

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

FORM 10-K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2024

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM TO

Commission File Number 001-39103

**CABALETTA BIO, INC.**

(Exact name of Registrant as specified in its Charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)  
2929 Arch Street, Suite 600  
Philadelphia, PA  
(Address of principal executive offices)

82-1685768  
(I.R.S. Employer  
Identification No.)

19104  
(Zip Code)

Registrant's telephone number, including area code: (267) 759-3100

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.00001 per share	CABA	The Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes  No

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes  No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller reporting company

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report.

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements.

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b).

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes  No

As of June 30, 2024 (the last business day of the registrant's most recently completed second fiscal quarter), the aggregate market value of the registrant's common stock held by non-affiliates was approximately \$350 million based on the last reported sale price of the registrant's common stock on the Nasdaq Global Select Market on June 30, 2024.

The number of shares of registrant's Common Stock outstanding as of March 27, 2025 was 50,743,101.

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## Summary of the Material and Other Risks Associated with Our Business

- We are a clinical-stage company with a limited operating history, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future.
- We are highly dependent on our relationships with University of Pennsylvania, or Penn, and/or WuXi Advanced Therapies, Inc., or WuXi, for our current manufacturing needs for our Phase 1/2 RESET™, or Restoring Self-Tolerance, clinical trials for resecabtagene autoleucel (rese-cel, formerly referred to as CABA-201) and our Phase 1 clinical trial of MuSK-CAART, or the MusCAARTes™ trial, and if Penn's or WuXi's manufacturing capacity is reduced or otherwise delayed or limited, including due to legislative action, or if we, Penn, WuXi or any third-party manufacturers encounter difficulties in manufacturing our product candidates, this could adversely impact the supply of product candidates for and enrollment in our trials.
- We are reliant on intellectual property licensed to us by Penn and Nanjing IASO Biotherapeutics Co., Ltd., or IASO, and termination of one of these license agreements would result in the loss of significant rights, which would have a material adverse effect on our business.
- If we are unable to obtain and maintain sufficient intellectual property protection for our current product candidates and technologies or any future product candidates, we may not be able to compete effectively in our markets.
- We will need to raise substantial additional funding before we can expect to complete development of any of our product candidates or generate any revenues from product sales.
- Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- If we are unable to successfully develop our current programs into a portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates.
- If we encounter difficulties enrolling patients in our RESET™ clinical trials for rese-cel or future clinical trials, these clinical development activities could be delayed or otherwise adversely affected.
- If we are unable to advance our product candidates through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.
- Results of earlier studies may not be predictive of future study or trial results, and we may fail to establish an adequate safety and efficacy profile to conduct clinical trials or obtain regulatory approval for our product candidates.
- If serious adverse events, undesirable side effects or unexpected characteristics are identified during the development of any of our product candidates, we may need to delay, abandon or limit our further clinical development of those product candidates.
- Manufacturing and administering our product candidates is complex and we may encounter difficulties in technology transfer to a contract manufacturing organization.
- We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.
- We may establish our own manufacturing facility and infrastructure in addition to or in lieu of relying on third parties for the manufacture of our product candidates, which will be costly and time-consuming, and which may not be successful.
- Our future success depends in part upon our ability to retain our key employees, consultants and advisors and to attract, retain and motivate other qualified personnel.
- We have identified conditions that raise substantial doubt about our ability to continue as a going concern. If we are unable to secure additional funding beyond our current cash position that enables our operations into the first half of 2026, we may be forced to delay, reduce or discontinue our product development programs efforts or other operations.

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, including the sections entitled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” contains express or implied forward-looking statements that are based on our management’s belief and assumptions and on information currently available to our management. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this Annual Report on Form 10-K include, but are not limited to, statements about:

- the success, cost and timing and conduct of our clinical trial program, including our Phase 1/2 RESET™ clinical trials for rese-cel, our MusCAARTes™ trial, and any other product candidates, including statements regarding the timing of initiation, enrollment and completion of the clinical trials and the period during which the results of the clinical trials will become available;
- the expected timing and significance around the announcement of safety, biologic activity and/or any additional clinical data from our RESET™ clinical trials for rese-cel or MusCAARTes™ trial;
- the timing of and our ability to obtain and maintain regulatory approval of our product candidates, including rese-cel, MuSK-CAART, and our other product candidates, in any of the indications for which we plan to develop them, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our expectations for the tolerability and clinical activity of rese-cel and ability to advance this product candidate through our license agreement with IASO;
- the potential benefits of our Orphan Drug, Rare Pediatric Disease and Fast Track designations;
- our expected use of proceeds from sales of our common stock in “at-the-market” offerings and other offerings, and the period over which such proceeds, together with existing cash, will be sufficient to extend our current cash runway beyond our current expectations into the first half of 2026 and meet our operating needs, including our ability to continue as a going concern;
- our plans to pursue research and development of other product candidates;
- the potential advantages of our proprietary Cabaletta Approach for B cell Ablation platform, called our CABA® platform, and our product candidates;
- the extent to which our scientific approach and CABA® platform may potentially address a broad range of diseases;
- the potential benefits and success of our arrangements with Penn, the Children’s Hospital of Philadelphia, or CHOP, and WuXi;
- our ability to successfully leverage our research and translational insights;
- our expectations regarding the results observed with the similarly-designed construct employed in recent academic publications, including the dosing regimen, and the implications on rese-cel;
- our ability to successfully commercialize our product candidates, including rese-cel, MuSK-CAART and any other product candidates;
- the potential receipt of revenue from future sales of rese-cel, MuSK-CAART and any other product candidates, if approved;
- the rate and degree of market acceptance and clinical utility of rese-cel, MuSK-CAART and any other product candidates;
- our estimates regarding the potential market opportunity for rese-cel, MuSK-CAART and any other product candidates, and our ability to serve those markets;
- our sales, marketing and distribution capabilities and strategy, whether alone or with potential future collaborators;
- our ability to establish and maintain arrangements or a facility for manufacture of rese-cel, MuSK-CAART and any other product candidates;

- our ability to obtain funding for our operations, including funding necessary to initiate and complete our RESET<sup>TM</sup> clinical trials of rese-cel, our MusCAARTes<sup>TM</sup> trial and any ongoing preclinical studies of other product candidates;
- our expectations for the efficiency of the trial design for our RESET<sup>TM</sup> clinical trials for rese-cel and the potential success and therapeutic benefits of rese-cel, including our belief that rese-cel may enable an “immune system reset” and provide deep and durable responses in patients across an increasing number of autoimmune diseases;
- the potential achievement of milestones and receipt of payments under our collaborations;
- our ability to enter into additional collaborations with existing collaborators or other third parties;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates and our ability to operate our business without infringing on the intellectual property rights of others;
- our expectations regarding international expansion and results of our efforts to do so;
- the success of competing therapies that are or become available, and our competitive position;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the impact of government laws and regulations in the United States and foreign countries; and
- our ability to attract and retain key scientific or management personnel.

These factors should not be construed as exhaustive and should be read in conjunction with the other cautionary statements that are included in this Annual Report on Form 10-K. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we undertake no obligations to publicly update or review any forward-looking statement, whether as a result of new information, future developments or otherwise. Therefore, you should not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Annual Report on Form 10-K.

## PART I

### Item 1. Business.

#### Overview

We are a clinical-stage biotechnology company focused on the discovery and development of innovative engineered T cell therapies that have the potential to provide deep and durable, perhaps curative, responses with one-time administration for patients with autoimmune diseases. Our proprietary CABA<sup>®</sup>, or Cabaletta Approach to B cell Ablation, platform encompasses two strategies. Our CARTA, or Chimeric Antigen Receptor T cells for Autoimmunity, approach is designed to potentially reset the immune system. Our legacy CAART, or Chimeric AutoAntibody Receptor T cells, approach is designed to engineer T cells to selectively engage and eliminate only disease-causing B cells. We believe our CABA<sup>®</sup> platform has the potential to safely enable complete and durable responses for a broad range of autoimmune diseases and that it has potential applicability across dozens of autoimmune diseases that we have identified, evaluated and prioritized.

Resecabtagene autoleucel (rese-cel, formerly referred to as CABA-201) is a 4-1BB co-stimulatory domain-containing fully human CD19-CAR T construct designed to treat patients with a broad range of autoimmune diseases and our lead product candidate within the CARTA strategy. Rese-cel is designed to achieve transient and deep depletion of all B cells following a single treatment by using T cells engineered to express an antibody fragment that recognizes a B cell receptor expressed on the surface of all B cells. The construct is designed to allow for the deep elimination of all B cells, including all B cells that contribute to disease, with subsequent repopulation by healthy naïve B cells. This approach has the potential to reset the immune system, providing meaningful durable and complete clinical responses to patients off immunosuppressive therapies.

In March 2023, the United States Food and Drug Administration, or the FDA, granted clearance of our rese-cel Investigational New Drug, or IND, application for treatment of SLE in patients with active lupus nephritis, or LN, or active SLE without renal involvement. SLE is a chronic, potentially severe, autoimmune disease, most commonly impacting young women between the ages of 15 and 40 with higher frequency and more severity in people of color, where the immune system attacks healthy tissue throughout the body. SLE affects an estimated up to 320,000 patients in the U.S., and 150,000 patients in Europe, with LN as the most common end-organ manifestation, affecting approximately 40% of SLE patients. In May 2023, we announced the FDA granted Fast Track Designation for rese-cel, designed to deplete CD19-positive B cells and improve disease activity in patients with SLE and LN. The RESET-SLE<sup>™</sup> Phase 1/2 clinical trial of rese-cel is designed to treat six SLE patients with active LN, and in a separate parallel cohort, six patients with active SLE without renal involvement, with a single weight-based dose of  $1.0 \times 10^6$  cells/kg. In March 2024, Health Canada issued a No Objection Letter in response to a Clinical Trial Application, or CTA, for the RESET-SLE<sup>™</sup> trial submitted by Cabaletta, enabling us to begin the process to activate clinical trial sites and pursue patient enrollment for the RESET-SLE<sup>™</sup> trial in Canada. In October 2024, the European Medicines Agency allowed a CTA submitted by Cabaletta for the RESET-SLE<sup>™</sup> trial to proceed, enabling us to begin the process of activating clinical trial sites and pursuing patient enrollment for the RESET-SLE<sup>™</sup> trial. The trial is open for enrollment across multiple sites in the United States and one site in the European Union.

In May 2023, the FDA granted clearance of our rese-cel IND application for treatment of idiopathic inflammatory myopathies, or IIM, or myositis. Myositis refers to a group of autoimmune diseases characterized by inflammation and muscle weakness. The three myositis subtypes being evaluated in the RESET-Myositis<sup>™</sup> Phase 1/2 clinical trial of rese-cel affect approximately 70,000 patients in the U.S. and approximately 85,000 patients in Europe and typically affect middle-aged individuals, particularly women. The RESET-Myositis<sup>™</sup> clinical trial, which is actively enrolling patients, is designed to treat six patients with dermatomyositis, six patients with anti-synthetase syndrome, and six patients with immune-mediated necrotizing myopathy, all in separate parallel cohorts. We announced the FDA granted Fast Track Designation for rese-cel for the treatment of patients with dermatomyositis to improve disease activity and Orphan Drug Designation for rese-cel for the treatment of myositis in January and February 2024, respectively. In March 2024, we announced the FDA granted Rare Pediatric Disease designation for rese-cel for juvenile dermatomyositis. The trial is open for enrollment across multiple sites in the United States and one site in the United Kingdom.

In October 2023, we announced that the FDA granted clearance of our rese-cel IND application for treatment of systemic sclerosis, or SSc. SSc is a rare and potentially fatal chronic autoimmune disease characterized by progressive skin and internal organ fibrosis that can be life-threatening, including interstitial lung disease, pulmonary hypertension, and scleroderma renal crisis. SSc affects approximately 90,000 patients in the U.S. and approximately 60,000 patients in Europe, typically middle-aged individuals, particularly women. The RESET-SSc<sup>™</sup> Phase 1/2 clinical trial of rese-cel is designed to treat six patients with severe skin manifestations and six patients with severe organ involvement associated with SSc. We announced the FDA granted Fast Track Designation for rese-cel for the treatment of patients with SSc to improve associated organ dysfunction and Orphan Drug Designation

for rese-cel for the treatment of systemic sclerosis in January and March 2024, respectively. The trial is open for enrollment across multiple sites in the United States.

In November 2023, the FDA granted clearance of our rese-cel IND application for treatment of generalized myasthenia gravis, or gMG, a subset of patients with myasthenia gravis, or MG. MG is a rare autoimmune disease characterized by autoantibodies that interfere with signaling at the neuromuscular junction, or NMJ, leading to potentially life-threatening muscle weakness. The majority of patients with MG have autoantibodies known to be pathogenic based on their interference with proteins in the NMJ, of which the majority target AChR. gMG affects approximately 55,000 patients in the U.S. and approximately 100,000 patients in Europe. Symptoms of gMG include profound muscle weakness throughout the body, disabling fatigue, and potential shortness of breath due to respiratory muscle weakness, with risk for episodes of respiratory failure. Standard of care therapies include cholinesterase inhibitors, steroids, immunomodulators, and biologics, which typically require chronic administration, increasing the risk of serious long-term side effects. The RESET-MG™ Phase 1/2 clinical trial of rese-cel is designed to treat six patients with AChR-positive gMG and six patients with AChR-negative gMG, each in separate parallel cohorts. The trial is open for enrollment across multiple sites in the United States.

In May 2024, we announced that we are working with active clinical sites to incorporate the RESET-PV™ trial as a sub-study within the Phase 1 DesCAARTes™ trial following the submission of a protocol amendment. The RESET-PV™ trial will evaluate rese-cel as a monotherapy without preconditioning in patients with mucosal pemphigus vulgaris, or mPV, and mucocutaneous pemphigus vulgaris, or mcPV. Approximately 15,000 patients in the U.S. and approximately 20,000 patients in Europe are affected by PV. The trial is open for enrollment across multiple sites in the United States.

In June 2024, we announced the initial clinical data from each of the first two patients dosed with rese-cel in the RESET-SLE™ and RESET-Myositis™ trials. In both of these patients, no cytokine release syndrome, or CRS, immune effector cell-associated neurotoxicity syndrome, or ICANS, infections or serious adverse events were observed through data cut-off of May 28, 2024. Rese-cel exhibited the anticipated profile of CAR T cell expansion and contraction with complete B cell depletion observed in both patients by day 15 post-infusion. Improvements in both patients' specific disease measures were observed, consistent with academic experience of a similar 4-1BB CD19-CAR T.

In late June 2024, a lupus nephritis patient with very active, refractory disease was dosed with rese-cel and experienced Grade 1 CRS and a protocol-defined dose-limiting toxicity of Grade 4 ICANS. The ICANS resolved rapidly following standard management. The study's Independent Data Monitoring Committee reviewed data from the patient, and recommended that the study proceed as designed, without delay, at the current dose. We recommended protocol modifications designed to improve patient safety, including enhanced monitoring for fever and neurologic symptoms along with seizure prophylaxis for all patients, in line with the practice at many academic sites including at Erlangen University, the site of the CD19-CAR T studies led by Dr. Georg Schett. In July 2024, we provided standard notice to the FDA and communicated details of the event and proposed protocol changes to all active clinical sites within the RESET™ clinical trial program, including the requirement that the investigator promptly discuss the appropriateness and timing of infusion with the trial's Medical Monitor.

In November 2024, we announced updated clinical data at the American College of Rheumatology Convergence 2024 conference in the first 8 patients dosed across the lupus, myositis and systemic sclerosis clinical trials. Clinical data from these patients in the RESET-SLE™, RESET-Myositis™ trial and RESET-SSC™ trial were presented in oral and poster presentations where compelling clinical responses were observed in lupus and myositis patients with up to six months of follow-up, and the first SSc patient demonstrated an emerging, drug-free clinical response.

In January 2025, we announced the FDA granted clearance of our rese-cel IND application for treatment of multiple sclerosis, or MS. In addition, we announced the FDA granted Fast Track Designation for rese-cel for the treatment of relapsing and progressive forms of MS. The RESET-MS™ trial is a Phase 1/2 open-label, dose escalation study of rese-cel in subjects with relapsing and progressive forms of MS, evaluated in separate cohorts. Approximately 750,000 patients in the U.S. and approximately 550,000 patients in Europe are affected by MS.

In February 2025, we announced updated clinical and translational data from the first 10 patients in the RESET™ clinical trial program, with a data cut off of January 8, 2025. Overall, clinical responses continued to deepen over three SLE patients in DORIS remission, the first LN patient achieving complete renal response, and the first dermatomyositis patient maintaining a major total improvement score, or TIS improvement. Each of these patients discontinued all immunosuppressants and were off or tapering steroids as of January 8, 2025. The safety profile continued to suggest a favorable risk-benefit in the first 10 patients dosed with 90% of patients having experienced either no CRS or Grade 1 CRS and 90% of patients having experienced no ICANS.

In March 2025, we learned of an important protocol deviation in the RESET-SSc™ trial. In this case, the patient reported a fever three days prior to infusion. The Cabaletta Medical Monitor was not notified by the investigator prior to the site infusing the patient. Nine days following rese-cel infusion, the patient experienced a dose-limiting toxicity of grade 3 immune effector

cell-associated neurotoxicity syndrome (ICANS) based on a transient period of confusion. There was no cerebral edema, seizures or motor dysfunction associated with the ICANS event, and the patient was arousable throughout. The ICANS resolved rapidly following treatment with dexamethasone, and the patient was discharged without further symptoms. After data review, the Independent Data Monitoring Committee recommended that the trial proceed at the current dose without delay and endorsed our proposal to require investigators to affirmatively confirm in writing to the company the absence of fevers or evidence of infection. As of March 14, 2025, 56 clinical sites across the U.S. and Europe were actively recruiting with 33 patients enrolled across the RESET™ clinical trial program.

Our manufacturing strategy is comprised of two stages. The initial stage is designed to leverage the extensive early-stage manufacturing expertise of our academic partners and contract development and manufacturing organizations, or CDMOs, for rapid early development and clinical supply, and in parallel partner with commercially compliant CDMOs capable of supporting late-stage clinical studies and commercial production. Our aim is to achieve full manufacturing readiness through expanded CDMO relationships, establishment of our own manufacturing facilities, and/or through strategic partnership(s). We are leveraging the expertise in cell and vector manufacturing of our partners at the Children’s Hospital of Philadelphia, or CHOP, and the University of Pennsylvania, or Penn. For supply of lentiviral vector for the late-stage clinical and commercial manufacturing of rese-cel, we are working with Oxford Biomedica (UK) Limited, or Oxford. We have also collaborated with WuXi Advanced Therapies, Inc., or WuXi, to serve as an additional and commercially compliant cell manufacturing partner for our MusCAARTes™ and RESET™ clinical trials to meet planned capacity and international supply requirements. In July 2024, we entered into a new technology transfer agreement with Lonza Houston Inc., or Lonza, another commercially compliant CDMO, to execute a technology transfer of our expected commercial manufacturing process for rese-cel. In December 2024, we entered into a Development and Manufacturing Services Agreement, or the Lonza Agreement, with Lonza to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and preparations for commercial readiness. This improved process is nearly fully closed, semi-automated, and more easily scaled than the current process. Transfer of this updated process will be performed from Cabaletta to Lonza in anticipation of being able to supply rese-cel drug product for any of Cabaletta’s ongoing and planned RESET™ clinical trials. We plan to secure commercial, scalable manufacturing capabilities through multiple potential strategies, including expanding existing or establishing new CDMO relationships, leasing, building, qualifying and operating our own manufacturing facility, and/or establishing a strategic partnership to rapidly and reliably scale manufacturing by leveraging the partner’s manufacturing expertise. We believe this later stage will enable control of product development and commercial supply for products arising from our CABA™ platform, enabling us to achieve continuous improvement of our product candidates. Multiple members of our team have previously built and led organizations that have constructed and commissioned cell therapy facilities, which we believe will enable us to build our own manufacturing organizations and facilities, if desirable.

As part of our innovative manufacturing strategy where we intend to increase scale, reduce cost of goods and improve patient experience, in November 2023, we partnered with Cellares Corp., or Cellares, to evaluate their automated manufacturing platform, the Cell Shuttle™, through the Cellares Technology Adoption Program, or TAP. As part of the collaboration, the companies have agreed on a proof-of-concept technology transfer process for the automated manufacture and release testing of rese-cel. In August 2024, we expanded our partnership with Cellares to facilitate the potential to incorporate the Cellares manufacturing platform to support the RESET™ clinical program. In March 2025, we and Cellares announced the successful conclusion of the Technology Adoption Program on Cellares’ automated cell therapy manufacturing Cell Shuttle™, facilitating the potential integration of the Cell Shuttle™ into our clinical and commercial, if approved, manufacturing strategy for rese-cel. We are also evaluating pathways to improve patient experience and scalability of collection through the removal of the requirement for apheresis. Data were published at the American Society of Cell and Gene Therapy conference in May 2024 illustrating the feasibility of this approach.



## Our History and Team

Our scientific co-founders, Aimee Payne, M.D., Ph.D., and Michael Milone, M.D., Ph.D., began partnering at Penn in 2013 to combine Dr. Payne's expertise in B cell-mediated autoimmune diseases with Dr. Milone's deep and experienced insights into the design and implementation of CAR T products. Dr. Payne is a worldwide leader in characterizing B cell-mediated autoantibody repertoires in PV and other autoimmune diseases. Dr. Milone is a renowned scientist in CAR T therapy and was a co-inventor of and a key driver in the preclinical discovery and development efforts that yielded Kymriah®, the first FDA-approved CAR T therapy for the treatment of B cell cancers. Their research attracted the attention of a colleague Steven Nichtberger, M.D., an adjunct professor at the Wharton School at the University of Pennsylvania, and in 2017, based on over a year of interaction and discussions regarding the optimal strategy to advance the scientific opportunity into a commercially developed product portfolio that could offer potentially curative treatment options to patients, Drs. Payne, Milone and Nichtberger decided to launch Cabaletta Bio. The longstanding and highly productive partnership between our co-founders has been complemented by additional management experience that brings a successful history of translating academic cellular therapy research from Penn and elsewhere into commercially sponsored clinical trials and the establishment of a GMP manufacturing facility and organization.

Gwendolyn Binder, Ph.D., our President, Science and Technology, was an early member of the Translational Research Program Operations team at Penn for over five years and participated in the submission and acceptance of multiple INDs for novel engineered T cell therapy products. As part of the cell therapy organization at Penn, Dr. Binder partnered with Dr. Milone and others to drive the IND-enabling translational studies that facilitated the initial CAR T clinical trial in B cell cancers at Penn. Dr. Binder also built and led a clinical stage biotechnology company's manufacturing operations and quality teams, including creation of a fully functioning commercial grade GMP facility. Dr. Binder also built the translational research program and ultimately led the company's research organization.

Our Chief Medical Officer, David Chang, M.D. was the late-stage clinical development leader of the only two drugs approved for SLE in the United States in over 60 years, belimumab, or Benlysta, and anifrolumab, or Saphnelo, through his roles at GlaxoSmithKline plc and AstraZeneca Pharmaceuticals LP prior to joining the team at Cabaletta Bio. Dr. Chang completed his fellowship in Rheumatology and was a faculty member in the Division of Rheumatology at the Perelman School of Medicine at the University of Pennsylvania prior to his transition to the biopharmaceutical industry.

Our legacy CAART platform has yielded two clinical-stage cell therapy programs in mPV and MuSK MG, which have required collaborations between specialists – dermatologists and neurologists – and oncologists who are responsible for administering the cell therapy at each site. Given our track record of timely clinical trial implementation with CAART cell therapy in autoimmune diseases over the past five years at over a dozen sites across the United States, we believe that our team's successful management of the complicated planning and logistics involved with implementation of these clinical trials has the potential to be an operating advantage. While advancing the CAART product candidates in our Phase 1 DesCAARTes™ and MusCAARTes™ trials and establishing our position in the field of autoimmunity, we were encouraged by results from an initial case report published in the *New England Journal of Medicine* in August 2021 and a follow-up academic clinical study published in *Nature Medicine* in September 2022, showing the potential for CD19-CAR T cell therapy to transform the course of SLE. In five patients with SLE, one-time treatment with a 4-1BB-containing CD19-CAR T cell therapy induced deep and durable clinical responses in all five patients within three months after treatment, with favorable tolerability. Healthy B cells repopulated in all patients within five months of treatment, and responses remained durable off SLE-associated medications for up to 2.5 years of follow-up, as of February 2024. These findings demonstrate the potential for CD19-CAR T cell therapy to “reset the immune system,” eliminating the cause of autoimmune disease with restoration of the healthy immune system.

Building on these results, we announced in October 2022 the development of rese-cel, a 4-1BB-containing CD19-CAR T investigational therapy, for the treatment of severe autoimmune diseases. Prompted by the initial case report in the *New England Journal of Medicine*, we conducted a global search to identify the optimally designed product candidate for patients with autoimmune diseases to be highly similar to the construct used by Dr. Schett in the *Nature Medicine* paper. We are employing a clinically-evaluated fully human CD19 binder that has high similarity, including identical epitopes and similar binding activity, to the construct employed in the academic reports. Our exclusive translational research partnership with Dr. Schett involves our robust translational research laboratory combined with confidential sharing of his unpublished clinical data to generate early and actionable insights from his trials that are informing our clinical development strategy and plans. As a result of the collaboration, in September 2023, Cabaletta scientists published “Cytokine and reactivity profiles in SLE patients following anti-CD19 CART therapy” in *Molecular Therapy: Methods and Clinical Development*, highlighting studies performed on serum samples from the first six SLE patients treated with CD19-CAR T cell therapy by Dr. Georg Schett's team. The publication reports that in the three months following infusion, cytokine markers of systemic inflammation resolved, autoantibody titers declined, and humoral immunity was maintained.

Since announcing rese-cel in October 2022, Cabaletta has had five IND applications cleared within the routine 30-day period for the RESET™ Phase 1/2 clinical trials in SLE, myositis, SSc, gMG and MS, in addition to the RESET-PV™ trial that is evaluating rese-cel without preconditioning. Accelerated by the emerging clinical data with rese-cel, our team's deep expertise in cell therapy in autoimmunity, demonstrated track record of strong clinical execution, positive regulatory interaction since 2018, and our successful cell therapy manufacturing, we are uniquely positioned to advance CD19-targeting cell therapy candidates to further our mission to develop therapies that deliver deep, durable and potentially curative responses for patients in a broad range of autoimmune diseases.

### **Our Strategy**

Our goal is to build upon our deep expertise in engineered T cell therapies for autoimmune diseases to accelerate the discovery, development and commercialization of our product candidates. We believe achieving this goal could result in potentially curative therapies for patients with unmet medical needs who suffer from a broad range of autoimmune diseases with B cell involvement. To achieve this goal, key elements of our strategy include:

***•Achieving compelling clinical responses and a favorable safety profile for our lead product candidate, rese-cel, across multiple autoimmune diseases driven by B cells.*** Academic clinical data published in *Nature Medicine* in September 2022 and in *NEJM* in February 2024, demonstrated that a CD19-CAR T cell therapy with a 4-1BB co-stimulatory domain following lymphodepletion with fludarabine and cyclophosphamide induced complete clinical responses in five out of five patients with moderate to severe, refractory SLE, with up to 2.5 years of follow up, as of February 2024. Our exclusive translational partnership with Dr. Schett, the lead investigator of the study, is focused on generation of additional translational data to gain a deeper understanding of the immunologic mechanisms of response and clinical insights from ongoing and continued clinical studies in multiple autoimmune disease indications. Due to the purposeful design of rese-cel to have a high level of functional and structural similarity to the construct used in these academic studies and engineered specifically for the use in autoimmune patients, we believe rese-cel may have the potential to transform the treatment of a broad range of autoimmune diseases. We have five IND applications cleared for the RESET™ Phase 1/2 clinical trials in systemic lupus erythematosus, myositis, systemic sclerosis, generalized myasthenia gravis and multiple sclerosis in addition to the RESET-PV™ trial, which is evaluating rese-cel without preconditioning. All of these clinical trials are currently recruiting patients. As the academic data published to date showed robust clinical improvement with multiple years of follow-up in autoimmune patients, and given the similarity of rese-cel to the academic construct, we expect our initial clinical and translational data to provide insights that may predict longer-term outcomes.

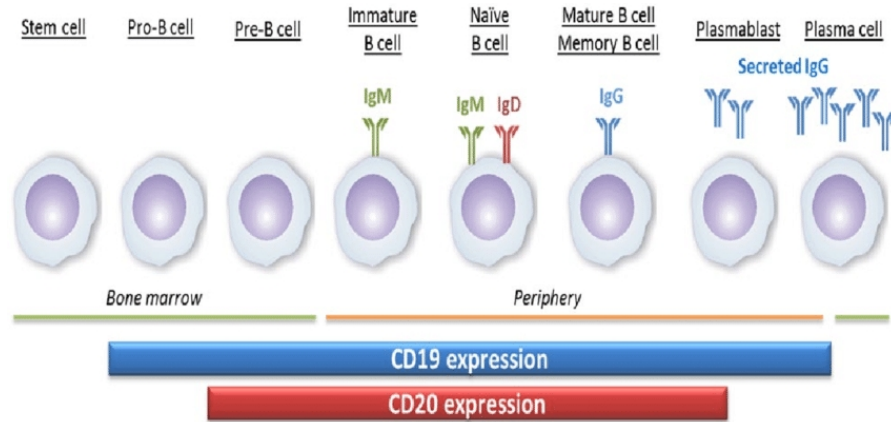
**•Utilizing our expertise in autoimmune cell therapy to enable a novel clinical strategy and efficient study design.** The first four RESET™ Phase 1/2 clinical trials – RESET-SLE, RESET-Myositis, RESET-SSc and RESET-MG – are comprised of independent cohorts with well-defined disease sub-types, each comprised of 6 patients, which can enroll and dose patients in parallel. These trials are initiating at a weight-based cell dose that is equivalent to the weight-based dose of the 4-1BB-containing CD19-CAR T cell therapy used as was done in the academic publications ( $1.0 \times 10^6$  cells/kg) with the same preconditioning regimen of fludarabine and cyclophosphamide. Each 6-patient cohort treated with an identical dose – without dose escalation requirement – is designed to inform discussions with the FDA on a registrational path for each indication, following the completion of any cohort. Our clinical strategy enables broad investigation of rese-cel across ten cohorts with well-defined patient populations in these four RESET™ Phase 1/2 clinical trials with the same dose and similar design. In addition, the RESET-PV™ trial is evaluating weight-based dosing of rese-cel without preconditioning. We have also received FDA Fast Track Designation for rese-cel in SLE, lupus nephritis, dermatomyositis, systemic sclerosis and multiple sclerosis.

**•Leveraging our cellular therapy knowledge and experience in addition to development efficiencies gained through our longstanding Penn collaboration to rapidly build fully integrated internal infrastructure.** We have differentiated expertise that we believe is uniquely suited to the continued buildout of our CABA™ platform, focused on autoimmune diseases. Our management team has expertise in conducting complex, interdisciplinary autoimmune-focused cell therapy clinical trials with a track record of positive regulatory interactions to support cell therapies in autoimmune diseases since 2018. We have had seven INDs for autoimmune cell therapies, each cleared within the routine 30-day period with six Fast Track Designations granted to date. In addition, we have a successful track record of manufacturing novel cell therapy product candidates with academic and industry partners. Not only does our team possess significant experience in the manufacturing of cell therapy product candidates, but our partnership with Penn allows us to utilize their existing infrastructure, which has accelerated our early development activities. In parallel, we continue to build out our internal capabilities while developing and continuing to implement a path to manufacturing independence, with established commercial CDMO partnerships to provide vector and cell processing capabilities at commercial grade and scale.

**•Advancing the RESET™ Phase 1/2 clinical trials to address significant unmet patient need while continuing to commit to optimize the patient experience.** We are focused on developing the first potentially curative targeted cellular therapies for patients with autoimmune diseases. We plan to expand development of rese-cel to a broad range of autoimmune diseases where the biologic opportunity for cure or treatment may be possible, addressing significant unmet patient need across multiple indications for rese-cel, with the goal of realizing the potential of rese-cel. While potentially serving a broad range of patients, we are committed to advancing to optimize the patient experience through minimizing the requirement for inpatient stay, optimizing the preconditioning regimen, reducing the burden of apheresis, and/or innovating to address scale in autoimmune disease.

## B Cells in Autoimmune Diseases: Overview and Current Treatment Paradigm

The body's immune system, which is designed to protect the body from infection and cancer, includes B cells and T cells. In addition to producing antibodies against antigens that the body perceives as foreign, B cells are responsible for producing inflammatory cytokines, co-stimulating other immune cells, and presenting antigen to T cells to enable cell-mediated immunity. Autoimmune disease occurs when the immune response becomes mistakenly targeted to healthy tissues and cells, and B cells can contribute to the incitement and/or maintenance of these processes through their varied immune mechanisms. In the case of many autoimmune diseases, B cells are responsible for driving disease through production of autoantibodies, or antibodies against the 'self,' that lead to disease, as well as through costimulation of T cells and through cytokine production.



Thomas G. Forsthuber, et al. "B cell-based therapies in CNS autoimmunity: differentiating CD19 and CD20 as therapeutic targets." *Therapeutic Advances in Neurological Disorders* (2018): Vol 11: 1-13

**Key markers of B cell lineage.** CD19 serves as a B cell marker from the pro B cell phase until differentiation to plasma cells, while CD20 is a surface marker expressed in a narrower range of the B cell maturation process. *Rese-cel* is directed to the CD19 B cell marker. CAAR T product candidates are designed to eliminate antigen specific B cells in each targeted disease, preventing their further development to antibody secreting plasma cells. IgM: immunoglobulin M; IgD: immunoglobulin D; IgA: immunoglobulin A; sIg: surface immunoglobulin, representing the autoantibody on the B cell surface.

There is currently no cure for autoimmune diseases. Current treatment options for autoimmune diseases involve generalized immune suppression, achieved through corticosteroids, immunosuppressive medications and biologics. Most commonly, corticosteroids are used on both a chronic and acute basis to control disease and act via a variety of mechanisms to control or downregulate multiple inflammatory pathways. In many cases, systemic immunosuppressive medications often used in chemotherapy such as mycophenolate, azathioprine and methotrexate, are added in an effort to minimize symptoms and manage the expected recurrences in patients. Biologic therapies have emerged as a new class of therapies and have a variety of targets including cytokines, B cells, and co-stimulation molecules. All of these current treatment options impair or destroy healthy B cells and/or other immune cells as well as pathogenic ones, weakening the patient's overall immune function, potentially putting them at risk for infection and impairing their response to vaccines. In general, these drugs require chronic administration and may have life-threatening side effects. We believe the ideal therapy in autoimmune diseases with B cell involvement would completely and specifically eliminate the pathogenic B cells with restoration of the normal immune system, enabling an "immune reset," restoring the body's immune system to its normal function of fighting foreign invaders, not healthy tissues.

### Our Pipeline and Product Candidates

We are a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies that have the potential to provide a deep and durable, perhaps curative, treatment for patients with autoimmune diseases. Our CABA™ platform encompasses two approaches – CARTA and CAART. Our current product candidate pipeline is illustrated below.

Program <sup>1</sup>	Trial	Preclinical	Phase 1/2	Pivotal
<b>Rese-cel</b> <sup>®</sup> <b>(CABA-201)</b> 4-1BB CD19-CAR T	<b>RESET-Myositis™</b>	Dermatomyositis		
		Antisynthetase syndrome		
		Immune-mediated necrotizing myopathy		
		Juvenile Myositis		
	<b>RESET-SLE™</b>	Lupus Nephritis		
		Non-Renal SLE		
	<b>RESET-SSc™</b>	Skin + Organ Cohort		
		Skin Cohort		
	<b>RESET-MG™</b>	AChR-Ab pos. gMG		
		AChR-Ab neg. gMG		
	<b>RESET-MS™</b>	Relapsing MS		
		Progressive MS		
	<b>RESET-PV™</b>	Mucocutaneous & mucosal pemphigus vulgaris		



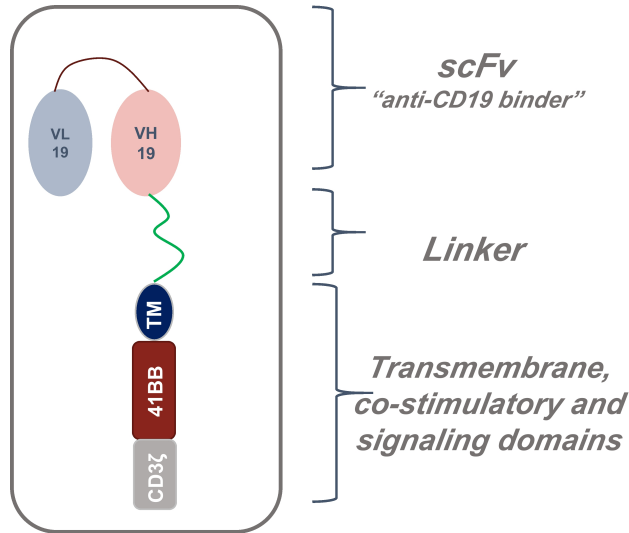
FTD – FDA Fast Track Designation received in dermatomyositis, SLE and lupus nephritis, systemic sclerosis, and multiple sclerosis.

1. Additional pipeline candidate includes MuSK-CAART for MuSK-Ab positive MG, currently being evaluated in a Phase 1 trial.

### ***Rese-cel for multiple autoimmune indications***

Rese-cel is designed to achieve transient but deep depletion of all B cells following a single infusion, allowing for the elimination disease-causing B cells with subsequent repopulation by translational naïve healthy B cells. This strategy may be able to reset the immune system, providing potentially meaningful clinical responses to patients off immunosuppressive therapies. Cabaletta is advancing six RESET™ Phase 1/2 clinical trials in SLE, myositis, SSc, gMG, PV and MS, with potential application in a broad range of other autoimmune diseases.

Rese-cel is comprised of a fully human anti-CD19 binder, which is the extracellular targeting domain. In addition, it contains a 4-1BB costimulatory domain and a CD3-zeta signaling domain, as shown in the figure below:



*Image showing the design of rese-cel, with a fully human anti-CD19 binder, the 4-1BB costimulatory domain and the CD3-Zeta signaling domain. The costimulatory and signaling domain are identical to the construct used in the academic clinical studies published in Nature Medicine and Lancet Rheumatology that were evaluated in SLE and myositis, respectively.*

Our exclusively licensed fully human CD19 binder has been clinically evaluated in a dual-CD19xCD22 CAR T candidate under development for B cell leukemia and lymphoma in an investigator-initiated trial in China in approximately 20 patients, where IASO has reported a tolerability profile that we believe is favorable for development in autoimmune diseases. Compared to FMC63-CART, rese-cel exhibits comparable biologic activity in vitro and in vivo (Peng, et al).

Given its structural and functional similarity to the CD19-CAR T construct employed in the academic clinical study published in *Nature Medicine*, including incorporation of a 4-1BB co-stimulatory domain, we believe rese-cel may have the potential to reset the immune system and transform treatment of a broad range of autoimmune diseases with high unmet need.

#### ***Disease Backgrounds***

Systemic lupus erythematosus is a chronic autoimmune disease, most common in young women between the ages of 15 and 40, with higher frequency and greater severity in people of color. In SLE, the immune system attacks healthy tissue throughout the body. It results in a range of clinical manifestations, including end organ damage and an increased risk of death. SLE affects an estimated up to 320,000 patients in the U.S., and 150,000 patients in Europe. Lupus nephritis is the most common end-organ manifestation of SLE, affecting approximately 40% of SLE patients. Among these patients, the risk of end-stage renal disease is approximately 17% and the risk of death is approximately 12%, each within 10 years of diagnosis.

Myositis refers to a group of autoimmune diseases characterized by severe inflammation and muscle weakness. It commonly impacts women of middle age, and in some cases, myositis may also affect other organs and systems in the body, such as the lungs, heart, or skin. Myositis is classified into several subtypes based on the underlying immune mechanisms and clinical characteristics. Although the pathogenesis of myositis is not well understood, there are several subtypes thought to be driven by B cells, including

dermatomyositis, or DM, anti-synthetase syndrome, or ASyS, and immune-mediated necrotizing myopathy, or IMNM. All three subtypes can lead to severe functional impairment and may be life-threatening. Including juvenile myositis, or JIIM, patients, approximately 70,000 patients are affected by these subtypes in the U.S. and approximately 85,000 patients in Europe. Current standard of care typically involves medications to suppress the immune system and/or chronic intensive therapies such as intravenous immunoglobulin, or IVIg. Despite these therapies, many myositis patients have disease that remains refractory to existing medications.

Systemic sclerosis is a rare and potentially fatal chronic autoimmune disease characterized by progressive fibrosis and scarring of the skin and internal organs that can lead to life-threatening conditions, including interstitial lung disease, pulmonary hypertension, and scleroderma renal crisis. Although the etiology of SSc is not well understood, the pathogenic role of autoantibodies and B cells in SSc provides a rationale for studying CAR T therapy in this population. Approximately 90,000 patients in the U.S. and 60,000 patients in Europe are affected by SSc, commonly middle-aged women. Current treatments, which have modest effects, include generalized immunosuppression or drugs targeted to specific symptomatic manifestations. In some cases, autologous hematopoietic stem cell transplant may provide some benefits in organ involvement, but is associated with significant risks, including mortality, infertility, and secondary autoimmune disease, limiting its potential to be applied broadly. The risk of mortality in SSc remains high due to the lack of adequate treatments, with an average survival of approximately 12 years following initial diagnosis.

Myasthenia gravis is a rare autoimmune disease characterized by autoantibodies that interfere with signaling at the neuromuscular junction, or NMJ, leading to potentially life-threatening muscle weakness. The majority of patients with MG have autoantibodies known to be pathogenic based on their interference with proteins in the NMJ, of which the majority target AChR. Generalized MG affects approximately 55,000 patients in the U.S. and 100,000 patients in Europe. Symptoms of gMG include profound muscle weakness throughout the body, disabling fatigue, shortness of breath due to respiratory muscle weakness and risk for episodes of respiratory failure. Standard of care therapies include cholinesterase inhibitors, steroids, immunomodulators, and biologics, which often require chronic administration, increasing the risk of serious long-term side effects.

Pemphigus vulgaris is an autoimmune disease that occurs when the immune system produces antibodies that attack a protein called desmoglein, or DSG. DSG normally enables skin cells and the cells lining the inside of your mouth, nose, throat, and eyelids to bind tightly together. Disruption by the antibodies directed to DSG can cause extensive, painful blistering and life-threatening wounds characteristic of PV. Approximately 15,000 patients in the U.S. and approximately 20,000 patients in Europe are affected by PV. Current therapies do not adequately control the underlying autoimmune process, and PV management often involves long-term immunosuppression, with high-dose steroids used in initial treatment and for relapses. The risk of mortality among patients with PV has been reported to be 2- to 3-fold higher than in the general population.

Multiple sclerosis is an autoimmune-mediated demyelinating disease of the central nervous system (CNS), affecting approximately 750,000 people in the U.S. and approximately 550,000 patients in Europe. The disease is more common in women than men, with onset of symptoms frequently between the ages of 20 and 40. The immune system attack on nerves within the brain and spinal cord impairs nerve electrical conduction, resulting in a wide range of symptoms including muscle weakness, sensory loss, visual impairment, incoordination, memory loss, fatigue, and walking difficulty. MS is classified into relapsing or progressive forms based on the pattern of clinical symptoms over time, with 85-90% experiencing a fluctuating (relapsing) course, and 10-15% steadily accumulating neurologic disability from disease onset (progressive). The pathogenesis of MS is multifactorial, with autoreactive B cells increasingly being recognized as a major driver of disease. Current standard of care therapies require chronic administration, which can be associated increased long-term risk of serious side effects.

#### *Clinical Development Plan*

Cabaletta is advancing RESET™ Phase 1/2 clinical trials in SLE, myositis, SSc, gMG, PV and MS, with potential application in a broad range of other autoimmune diseases. Our rese-cel Phase 1/2 RESET-SLE™, RESET-Myositis™, RESET-SSc™, RESET-MG™ and RESET-PV™ trials are currently enrolling patients. These five RESET™ Phase 1/2 clinical trials are comprised of independent cohorts with specific objectively defined disease sub-types, which can enroll and dose patients in parallel, and each cohort is expected to be comprised of 6 patients. The trials are initiating at a weight-based dose, which is equivalent to the dose of the 4-1BB CD19-CAR T used in the academic publications ( $1.0 \times 10^6$  cells/kg) with the same preconditioning regimen of fludarabine and cyclophosphamide, with the exception of the RESET-PV™ trial, which is evaluating rese-cel without preconditioning. Our clinical strategy enables broad investigation of rese-cel across cohorts with well-defined patient populations in our Phase 1/2 clinical trials with the same dose and similar design. We have also received FDA Fast Track Designation for rese-cel in SLE, lupus nephritis, dermatomyositis, systemic sclerosis and multiple sclerosis.

#### *DSG3-CAART for Mucosal PV*

DSG3-CAART is a CAAR T cell therapy expressing DSG3 as the extracellular domain of a chimeric immunoreceptor and is designed to enable specific cytotoxicity toward B cells autoreactive to DSG3. We believe this strategy has the potential to directly eliminate the disease-causing cells in mucosal pemphigus vulgaris, or mPV, an autoimmune disease that causes blisters on the skin and mucous membranes, which may lead to lasting clinical remission without damage to the healthy immune system. PV has two major subtypes: (1) mPV, which is caused by DSG3 autoantibodies and affects the mucous membranes; and (2) mcPV, which is caused by DSG3 and DSG1 autoantibodies, affecting both the mucous membranes and the skin. PV affects approximately 15,000 prevalent patients in the U.S., of which approximately 25% have mPV and 75% have mcPV. Like most autoimmune diseases, the current standard of care for PV relies on general immune suppression, which is often transiently effective but can lead to severe infection, potentially resulting in hospitalization and death.

#### *Clinical Development Plan*

The DesCAARTes™ trial is an open-label trial to assess the safety and tolerability of various dosing regimens of DSG3-CAART in the treatment of subjects with active mPV. The FDA granted DSG3-CAART Orphan Drug Designation for the treatment of PV in January 2020 and Fast Track Designation for improving healing of mucosal blisters in patients with mPV in May 2020. The DesCAARTes™ trial is no longer dosing patients for treatment with DSG3-CAART after evaluation of clinical and translational data from the combination cohort, where patients were pre-treated with IVIg, cyclophosphamide and fludarabine prior to DSG3-CAART infusion. In October 2024, we presented data in an oral presentation at the European Society of Gene and Cell Therapy, or ESGCT, 31st Annual Congress showing that the use of preconditioning with DSG3-CAART did not provide serologic or clinical improvement and did not deeply deplete B-cell levels in patients with mPV.

#### ***MuSK-CAART for MuSK Myasthenia Gravis***

MuSK-CAART is an investigational cell therapy aimed at treating patients with anti-MuSK antibody positive myasthenia gravis, or MuSK MG, who have active disease. We believe this strategy has the potential to directly eliminate the disease-causing cells in MuSK MG – induced by autoantibodies targeting the neuromuscular junction leading to potentially life-threatening muscle weakness to enable a complete and durable response for this disease. The current standard of care for patients with MuSK MG is typically corticosteroids in addition to one or more steroid-sparing immunosuppressive agents.

#### *Clinical Development Plan*

The MusCAARTes™ trial is an open-label trial to assess the safety and tolerability of various dosing regimens of MuSK-CAART in the treatment of subjects with active MuSK MG. In February 2022, MuSK-CAART received Fast Track Designation from the FDA to improve activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis. We are currently evaluating clinical and translational data from the A1 and A2 cohorts in the MusCAARTes™ trial, where patients were treated with MuSK-CAART without preconditioning.

#### **Manufacturing**

##### ***Manufacturing Strategy***

We are implementing a two-stage plan that we believe will ultimately enable us to achieve manufacturing independence. Part of our strategy relies on engaging non-profit and commercial suppliers early and in a staged manner. We believe partnering with proven and reputable manufacturing partners will allow us to efficiently deploy financial and personnel resources. The early phase of this plan is ongoing and utilizes the deep expertise in cell and vector manufacturing from our partners at Children’s Hospital of Philadelphia, or CHOP, and Penn, as well as commercial CDMO partnerships. We believe these facilities and partnerships will allow us to move efficiently into clinical trials with expanded commercial grade vector and cell product supply. We are aware that changes in any manufacturing process or facility introduces regulatory and scientific risk to a development program, if the changes result in a product that is not comparable. We plan to mitigate these risks across the two stages as follows:



•By securing contract development and manufacturing organizations, or CDMOs, as partners during the early phase of our manufacturing strategy early on for both vector and cell manufacturing. We plan to prioritize potential partners who are qualified to, and have an established track record of, the commercial production of vector and cell products. We believe this allows us to make one change in our supply partners during an early period of clinical development to facilitate in vitro comparability testing and clinical validation, prior to controlled clinical studies.

•As part of this strategy, in January 2021 and as amended in August 2022, we entered into an agreement with WuXi to serve as an additional cell processing manufacturing partner for our MusCAARTes™ trial and have since completed enabling engineering and patient production runs. In August 2023, as amended in August 2024, we entered into an agreement with WuXi to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel.

•In order to expand our clinical supply to address the increasing pace of enrollment in our clinical trials as well as to prepare for registrational trial(s) across the RESET clinical development program while expanding our manufacturing options for rese-cel, Cabaletta has expanded its CDMO agreements with Lonza to supply rese-cel clinical product under current Good Manufacturing Practices as soon as the second half of 2025.

•In December 2021, we entered into the LSA with Oxford to supply lentiviral vector for the clinical and commercial development of our DSG3-CAART candidate. In May 2023, we amended the LSA with Oxford to expand the license to include our rese-cel program. In August 2023, we entered into a vector supply agreement with Oxford, and a related second amendment to the LSA, for rese-cel. In February 2024, we and Oxford entered into a third amendment to the LSA to update the patent schedule. In June 2024, we and Oxford entered into a fourth amendment to the LSA eliminating royalties on net sales of products that incorporate the Oxford technology if Oxford manufactures the vector. We believe these efforts will provide us with sufficient clinical-grade vector to move forward with our anticipated clinical trials.

•In conjunction with our efforts to secure commercial supply for rese-cel, we plan to advance innovative manufacturing strategies in order to increase scale, reduce cost of goods and improve the patient experience. To this end, in November 2023, Cabaletta partnered with Cellares to evaluate Cellares' automated manufacturing platform, the Cell Shuttle™, through Cellares' Technology Adoption Program. As part of the collaboration, the companies have agreed on a proof-of-concept technology transfer process for the manufacture of rese-cel. In August 2024, we expanded our partnership with Cellares to facilitate the potential to incorporate the Cellares manufacturing platform to support the RESET™ clinical program. In March 2025, we and Cellares announced the successful conclusion of the Technology Adoption Program on Cellares' automated cell therapy manufacturing Cell Shuttle™, facilitating the potential integration of the Cell Shuttle™ into our clinical and commercial, if approved, manufacturing strategy for rese-cel.

### ***Cell Manufacturing***

We have entered into a collaboration with the Clinical Cell and Vaccine Production Facility, or CVPF, at Penn, to provide focused scientific, technical and regulatory support for CAAR T and CAR T cell manufacture. CVPF is accredited by the Foundation for the Accreditation of Cellular Therapy and is capable of and experienced at supporting manufacture for early-phase clinical trials of novel cell therapy products in first-in-man clinical trials. We rely upon CVPF as one of our manufacturing partners to provide initial Phase I clinical trial drug supply for rese-cel. Penn's manufacturing process for rese-cel is directly related to the process developed at Penn for early clinical trials of CD19-CAR T, which subsequently became known commercially as Kymriah. The process was later transferred to Novartis Pharmaceuticals Corporation and further modified for the Kymriah program.

As we scale our manufacturing for our product candidates to meet our expected needs for further clinical trials, and while we continue to utilize Penn for Phase 1/2 studies in the US, we have brought on board commercial CDMOs capable of global and fully GMP compliant manufacturing, as well as other third parties for the manufacturing and processing of our clinical trial materials. Any CDMO that we select will be subject to cGMP requirements. We believe the use of contract manufacturing for our pipeline programs will be cost-effective and allow us to rapidly prepare for clinical trials in accordance with our development plans. In preparation for this transition, we have engaged multiple third-party contractors to manufacture clinical grade viral vector used to deliver the applicable CAAR or CAR gene into the T cells. We have also initiated development work with certain contractors for cGMP and commercial vector production. We expect third-party manufacturers will be capable of providing and processing sufficient quantities of our product candidates to meet anticipated clinical trial demands and commercial need. In January 2021 and as amended in August 2022, we entered into an agreement with WuXi to serve as an additional cell processing manufacturing partner for our MusCAARTes™ trial and have since completed enabling engineering and patient production runs. In August 2023, as

amended in August 2024, we entered into an agreement with WuXi to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials for rese-cel. In order to expand our clinical supply to address the increasing pace of enrollment in our clinical trials as well as to prepare for registrational trial(s) across the RESET clinical development program while expanding our manufacturing options for rese-cel, Cabaletta has expanded its CDMO agreements with Lonza, a leading CDMO, to supply rese-cel clinical product under current Good Manufacturing Practices as soon as the second half of 2025.

### **Commercialization**

Our aim is to become a fully integrated cellular therapy company focused on improving the lives of patients with autoimmune diseases. The product candidates from our CABA™ platform are designed to address autoimmune indications where there is a compelling opportunity to improve clinical outcomes in comparison with the current standard of care.

Rese-cel is under development for autoimmune diseases with serious unmet medical need. Based on the differentiated expertise of Cabaletta's team members and our years of experience in conducting cell therapy clinical trials in autoimmunity, we are focused on being the first company to launch a cell therapy product for patients with autoimmune diseases, while continuing to innovate on next-generation approaches and differentiation strategies to deliver an optimal product candidate profile.

We aim to achieve full manufacturing independence through expanding our CDMO relationships, establishment of our own manufacturing facilities, and/or through a strategic partnership. Our development and commercialization efforts will focus initially on the United States, with expansion to the European Union and Asia-Pacific geographies, potentially with the support of strategic partners.

### **Competition**

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong focus on intellectual property. We face competition from many different players, including large and specialty pharmaceutical and biotechnology companies, academic research organizations and governmental agencies. Any therapeutic candidates we successfully develop and commercialize will compete with the existing standard of care as well as any novel therapies that may gain regulatory approval in the future.

There are multiple companies with marketed CAR T therapies for the treatment of hematologic cancers, including Novartis Pharmaceuticals Corporation, Gilead Sciences, Inc., Bristol Myers Squibb, Johnson and Johnson, Inc., Legend Biotech Corporation and Autolus. A subset of these companies along with other biopharmaceutical companies have announced CD19-targeting therapies and other methods of engineering T cells in development for the treatment of autoimmune diseases with B cell involvement, including SLE, myositis, SSc and MG, among others. There are also a number of companies with leading autoimmune franchises but without disclosed cell therapy platforms who may become competitors.

Within the CAR T field, we recognize that a subset of companies with an investment and expertise in CAR T cell development for oncology indications have announced they intend to leverage their technology in autoimmune disease-affected populations. We are aware of other pharmaceutical and biotechnology companies that are exploring CART-19 as well as other methods of engineering T cells, natural killer, or NK, cells or bispecific antibodies for the treatment of autoimmune conditions.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industry may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

### **Intellectual Property and Barriers to Entry**

We strive to protect the proprietary technologies that we believe are important to our business, including pursuing and maintaining patent protection intended to cover our product candidates and their use, as well as other inventions that are important to our business. In addition to patent protection, we also rely on know-how, confidentiality agreements, invention assignment agreements and trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for,

patent protection, to develop and maintain our proprietary position. The confidentiality agreements are designed to protect our proprietary information and the invention assignment agreements are designed to grant us ownership of technologies that are developed for us by our employees, consultants or certain other third parties. We seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in our agreements and security measures, either may be breached, and we may not have adequate remedies. In addition, our trade secrets may otherwise become known or independently discovered by competitors.

Our commercial success depends in part upon our ability to obtain and maintain patent and other proprietary protection for commercially important technologies, inventions and trade secrets related to our business, defend and enforce our intellectual property rights, particularly our patent rights, preserve the confidentiality of our trade secrets and operate without infringing valid and enforceable intellectual property rights of others.

The patent positions for biotechnology companies like us are generally uncertain and can involve complex legal, scientific and factual issues. In addition, the coverage claimed in a patent application can be significantly reduced before a patent is issued, and its scope can be reinterpreted and even challenged after issuance. As a result, we cannot guarantee that any of our product candidates will be protectable or remain protected by enforceable patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. Any patents that we hold may be challenged, circumvented or invalidated by third parties.

As of March 1, 2025, our in-licensed patent estate included seven granted U.S. patents, seven granted foreign patents, seven pending U.S. patent applications, and 50 pending foreign patent applications. See “Our Material Agreements - IASO Agreement” and “Our Material Agreements - Amended and Restated License Agreement with the Trustees of the University of Pennsylvania and the Children’s Hospital of Philadelphia.” As of March 1, 2025, our Cabaletta-owned patent estate included three U.S. provisional patent applications and two pending international patent applications.

With regard to our rese-cel product candidate, under the IASO Agreement, we have in-licensed one patent family which is directed to a CD19-specific chimeric antigen receptor and a CD19-specific antibody and contains one pending U.S. patent application and counterpart patent applications pending in Australia, Canada, China, Europe, and Hong Kong. These patent applications, if issued, would be expected to expire in 2040. The family also contains granted patents in China and Japan, which are scheduled to expire in 2040. This patent family is owned by IASO and is exclusively licensed to us in the field of the license. We also own two international patent applications drawn to methods of treating autoimmune diseases with a chimeric antigen receptor, including a CD19-specific chimeric antigen receptor. Any granted patents claiming priority to these applications would be expected to expire in 2044. We also own one U.S. provisional patent application drawn to methods of treating autoimmune diseases with a chimeric antigen receptor, including a CD19-specific chimeric antigen receptor; one U.S. provisional patent application drawn to CD19-binding antibodies and CD19-specific chimeric antigen receptors; and one U.S. provisional patent application drawn to methods of manufacturing cell-therapy compositions. Any granted patents claiming priority to these applications would be expected to expire in 2045.

With regard to our MuSK-CAAR T cell product candidate, under the Amended and Restated License Agreement with Penn, we have in-licensed one patent family containing one issued U.S. patent with claims directed to a CAAR containing an extracellular domain containing a MuSK autoantigen and nucleic acids encoding the CAAR. This patent is scheduled to expire in 2039, without taking potential patent term extensions into account. This family also contains a counterpart patent granted in Japan which is scheduled to expire in 2039, and counterpart patent applications pending in the U.S., Australia, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, Mexico and New Zealand, which if issued, would be expected to expire in 2039. This patent family is owned by Penn and exclusively licensed to us in the field of the license.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, the term of a patent covering an FDA-approved drug may be eligible for a patent term extension under the Hatch-Waxman Act as compensation for the loss of patent term during the FDA regulatory review process. The period of extension may be up to five years beyond the expiration of the patent but cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent among those eligible for an extension may be extended, and a given patent may only be extended once. Similar provisions are available in Europe and in certain other jurisdictions to extend the term of a patent that covers an approved drug. It is possible that issued U.S. patents covering each of our product candidates may be entitled to patent term extensions. If our product candidates receive FDA approval, we intend to apply for patent term extensions, if available, to extend the term of patents that cover the approved product candidates. We also intend to seek patent term extensions in any jurisdictions where they are available, however, there is no guarantee that the applicable authorities, including the FDA, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions.

In addition to patent protection, we also rely on know-how and trade secret protection for our proprietary information that is not amenable to, or that we do not consider appropriate for, patent protection, to develop and maintain our proprietary position.

However, trade secrets can be difficult to protect. Although we take steps to protect our proprietary information, including restricting access to our premises and our confidential information, as well as entering into agreements with our employees, consultants, advisors and potential collaborators, third parties may independently develop the same or similar proprietary information or may otherwise gain access to our proprietary information. As a result, we may be unable to meaningfully protect our know-how, trade secrets, and other proprietary information. In addition, we plan to rely on regulatory protection based on orphan drug exclusivities, data exclusivities, and market exclusivities. See “Government Regulation” for additional information.

## **Our Material Agreements**

### ***IASO Agreement***

On October 7, 2022, we entered into an Exclusive License Agreement, or the IASO Agreement, with IASO. Pursuant to the IASO Agreement, we received an exclusive, worldwide license under certain IASO intellectual property to use a novel clinical-stage anti-CD19 binder to develop, manufacture, commercialize and otherwise exploit T cell products directed to CD19 for the purpose of diagnosis, prevention or treatment of any autoimmune or alloimmune indications in humans. IASO has the right of first negotiation if we desire to grant a third party an exclusive license to develop, manufacture, commercialize or otherwise exploit the licensed products in the Greater China region. Pursuant to the IASO Agreement, we and IASO have agreed, subject to certain exceptions, to refrain from engaging in certain competitive activities with respect to certain programs. As partial consideration for the exclusive license, IASO received an upfront payment of \$2.5 million. IASO is also eligible to receive up to mid double digit millions in milestone payments based upon the achievement of specified pre-clinical, development and regulatory milestones, and up to an additional low triple digit millions in milestone payments based upon achievement of specified sales milestones, for a total consideration, inclusive of the upfront payment, of up to \$162 million, along with tiered mid-single digit royalties on future net sales for licensed products that may result from the IASO Agreement. We also may sublicense through multiple tiers the rights granted to it by IASO under the IASO Agreement at any time, however, we must pay IASO a low double-digit percentage of any revenue obtained from sublicenses or options to third parties, subject to certain customary exclusions. The IASO Agreement will continue on a country-by-country, licensed product-by-licensed product basis until the expiration of the royalty term as identified in the IASO Agreement, unless earlier terminated. We and IASO may terminate the IASO Agreement for a material, uncured breach or insolvency of the other party. We may also terminate the IASO Agreement at will upon advance written notice and in the event IASO rejects the IASO Agreement due to bankruptcy-related matters. IASO may also terminate the IASO Agreement if we fail to achieve certain specified diligence milestones in a timely manner and/or if we commence any patent challenges with respect to the patents and patent applications relating to the licensed sequence, in each case upon advance written notice. A milestone payment of \$1.5 million was paid to IASO in the first quarter of 2024 after the first patient in a rese-cel trial was dosed.

### ***Oxford Biomedica***

In December 2021, we entered into a Licence and Supply agreement, or LSA, with Oxford wherein the LSA grants us a non-exclusive license to Oxford's LentiVector® platform for its application in our DSG3-CAART program and puts in place a multi-year vector supply agreement. Under the terms of the agreement, we were required to pay Oxford an upfront fee, as well as costs associated with initial vector manufacturing activities. Oxford, is eligible to receive regulatory and sales milestones in the low tens of millions and royalties in the low single digits on net sales of products that incorporate the Oxford technology. We can terminate the agreement at will upon advance written notice and subject to certain manufacturing slot cancellation fees. In May 2023, we amended the LSA with Oxford to expand the license to include our rese-cel program for an upfront fee of \$0.5 million and in August 2023, we entered into a vector supply agreement with Oxford, and a related second amendment to the LSA, for rese-cel with a total cost of up to approximately \$5.0 million under the vector supply agreement. In February 2024, we and Oxford entered into a third amendment to the LSA to update the patent schedule. In June 2024, we and Oxford entered into a fourth amendment to the LSA eliminating royalties on net sales of products that incorporate the Oxford technology if Oxford manufactures the vector. In December 2024, the Company and Oxford entered into a work order for certain process characterization activities as part of commercial readiness activities. The Company can terminate the LSA or any work order under the LSA at will upon advance written notice and subject to certain cancellation fees.

### ***WuXi Manufacturing Agreement***

In January 2021, we entered into a Development and Manufacturing Services Agreement, or the WuXi Agreement, with WuXi to serve as an additional cell processing manufacturing partner for the MuSK-CAART Phase 1 clinical trial, or MusCAARTes™ trial. The WuXi Agreement is scheduled to expire upon completion of WuXi's services related to MuSK-CAART and rese-cel. In August 2023, as amended in August 2024, we entered into an agreement with WuXi to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel. Under the August 2023 work orders, WuXi converted our non-dedicated suite to a dedicated suite for GMP manufacturing for our rese-cel and MuSK-CAART programs, or the Dedicated Suite, for an initial term of 18 months with two 18 month extensions at our sole option on six months' notice prior to the end of the term. In August 2024, we notified WuXi that we would extend the initial term by 18 months through August 2026. In addition, we agreed to certain monthly minimum runs. In August 2024, the 2023 work order related to GMP manufacturing was amended to reduce the minimum monthly runs through the end of 2024. In lieu of the original \$1.5 million termination fee under the terms of the WuXi Agreement, we would

incur up to a \$1.08 million termination fee if we terminate both the rese-cel and MuSK-CAART work orders for any reason. We may terminate for convenience the WuXi Agreement or any work order with six months' prior written notice, however, we may not terminate the Dedicated Suite without terminating both the MuSK-CAART and rese-cel GMP run work orders. WuXi may terminate the WuXi Agreement or any work order for convenience on 18 months' prior written notice, but such notice may not be effective prior to February 2028.

***Amended and Restated License Agreement with the Trustees of the University of Pennsylvania and the Children's Hospital of Philadelphia***

In August 2018, we entered into a license agreement with Penn, which was amended and restated in July 2019 to include CHOP, collectively, the Institutions, and collectively with such amendment, as amended in May 2020 and October 2021, the License Agreement, pursuant to which we obtained (a) a non-exclusive, non-sublicensable, worldwide research license to make, have made and use products in two subfields of use, (b) effective as of October 2018, an exclusive, worldwide, royalty-bearing license, with the right to sublicense, under certain of the Institutions' intellectual property to make, use, sell, offer for sale and import products in the same two subfields of use, and (c) effective as of October 2018, a non-exclusive, worldwide, royalty-bearing license, with limited rights to sublicense, under certain of Penn's know-how to make, have made, use, sell, offer for sale, import and have imported products in the same two subfields of use. Our rights are subject to the rights of the U.S. government and certain rights retained by the Institutions.

Unless earlier terminated, the License Agreement expires on the expiration or abandonment or other termination of the last valid claim in Penn's intellectual property licensed by us. We may terminate the License Agreement at any time for convenience upon 60 days' written notice. In the event of an uncured, material breach, Penn may terminate the License Agreement upon 60 days' written notice.

***Master Translational Research Services Agreement***

In October 2018, we entered into a Master Translational Research Services Agreement with Penn, or the Services Agreement, pursuant to which Penn agreed to perform certain services related to the research and development of the technology licensed to us under the License Agreement, as well as certain clinical, regulatory and manufacturing services. The Services Agreement will expire on the later of (i) October 19, 2021 or (ii) completion of the services for which we have engaged Penn under the Services Agreement. Either party may terminate this agreement with or without cause upon a certain number of days' prior written notice. The services encompassed by the Services Agreement are performed by different organizations at Penn pursuant to certain addenda to the Services Agreement, including the Center for Advanced Retinal and Ocular Therapeutics, or CAROT, Addendum, as amended in May 2020, and the CVPF Addendum.

In February 2023, we entered into a second Master Translational Research Services Agreement with Penn, or the CARTA Services Agreement, pursuant to which Penn agreed to perform certain research, development and manufacturing activities. The CARTA Services Agreement will expire on the later of (i) February 9, 2026 or (ii) completion of the services for which we have engaged Penn under the CARTA Services Agreement. Either party may terminate this agreement with or without cause upon a certain number of days' prior written notice. The services encompassed by the CARTA Services Agreement are performed by different organizations at Penn pursuant to certain addenda to the CARTA Services Agreement.

**Government Regulation**

***U.S. Regulation***

As a biopharmaceutical company that operates in the United States, we are subject to extensive regulation. Our cell products will be regulated as biologics. With this classification, commercial production of our products will need to occur in registered facilities in compliance with cGMP for biologics. The FDA categorizes human cell- or tissue-based products as either minimally manipulated or more than minimally manipulated and has determined that more than minimally manipulated products require clinical trials to demonstrate product safety and efficacy and the submission of a BLA for marketing authorization. Our products are considered more than minimally manipulated and will require evaluation in clinical trials and the submission and approval of a BLA before we can market them.

Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of

biopharmaceutical products such as those we are developing. Our product candidates must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agency before they may be legally marketed in foreign countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way, but country-specific regulation remains essential in many respects. The process for obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

### ***U.S. Biological Product Development***

In the United States, the FDA regulates biologics under the Federal Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and their implementing regulations. Biologics are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may result in delays to the conduct of a study, regulatory review and approval or subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, license suspension or revocation, refusal to allow an applicant to proceed with clinical trials, imposition of a clinical hold, issuance of untitled or warning letters, product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations or penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Our drug product candidates must be approved by the FDA through the Biologics License Application, or BLA, process before they may be legally marketed in the United States. The process required by the FDA before a biologic may be marketed in the United States generally involves the following:

- completion of extensive nonclinical, sometimes referred to as preclinical, laboratory tests, animal studies and formulation studies in accordance with applicable regulations, including the FDA's Good Laboratory Practice, or GLP, regulations and standards;
- submission to the FDA of an IND which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, good clinical practices, or GCPs, and other clinical trial-related regulations to establish the safety and efficacy of the proposed drug product candidate for its proposed indication;
- submission to the FDA of a BLA, which includes not only the results of the clinical trials, but also, detailed information on the chemistry, manufacture and quality controls for the product candidate and proposed labeling;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the product is produced to assess compliance with the FDA's current good manufacturing practice, or cGMP, requirements to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality, purity and potency;
- potential FDA audit of the preclinical trial sites and/or clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval of the BLA prior to any commercial marketing or sale of the product in the United States.

The data required to support a BLA is generated in two distinct development stages: preclinical and clinical. The preclinical development stage generally involves laboratory evaluations of drug chemistry, formulation and stability, as well as studies to evaluate toxicity in animals, which support subsequent clinical testing. The conduct of the preclinical studies must comply with federal regulations, including GLPs. The sponsor must submit the results of the preclinical studies, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, as well as other information, to the FDA as part of the IND. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human trials. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the

proposed clinical trials and places the IND on clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a drug product candidate at any time before or during clinical trials due to safety concerns, non-compliance, or other issues affecting the integrity of the trial. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that could cause the trial to be suspended or terminated.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of institutional biosafety committees, or IBCs, as set forth in the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Under the NIH Guidelines, recombinant and synthetic nucleic acids are defined as: (i) molecules that are constructed by joining nucleic acid molecules and that can replicate in a living cell (i.e., recombinant nucleic acids); (ii) nucleic acid molecules that are chemically or by other means synthesized or amplified, including those that are chemically or otherwise modified but can base pair with naturally occurring nucleic acid molecules (i.e., synthetic nucleic acids); or (iii) molecules that result from the replication of those described in (i) or (ii). Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

The clinical stage of development involves the administration of the drug product candidate to healthy volunteers and patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed.

There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of certain clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

Clinical trials are generally conducted in three sequential phases, known as Phase 1, Phase 2 and Phase 3, and may overlap. Phase 1 clinical trials generally involve a small number of healthy volunteers who are initially exposed to a single dose and then multiple doses of the drug product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action tolerability, adverse effects, and safety of the drug product candidate and, if possible, to gain early evidence on effectiveness. Phase 2 clinical trials typically involve studies in disease-affected patients to determine the dose required to produce the desired benefits. At the same time, safety and further pharmacokinetic and pharmacodynamic information is collected, as well as identification of possible adverse effects and safety risks and preliminary evaluation of efficacy. Phase 3 clinical trials generally involve large numbers of patients at multiple sites, in multiple countries, and are designed to provide the data necessary to demonstrate the efficacy of the product for its intended use, its safety in use, and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product approval. Phase 3 clinical trials may include comparisons with placebo and/or other comparator treatments. The duration of treatment is often extended to mimic the actual use of a product during marketing. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of a BLA. In certain instances, FDA may condition approval of a BLA on the sponsor's agreement to conduct additional clinical trials to further assess the biologic's safety and effectiveness after BLA approval. Such post-approval trials are sometimes referred to as Phase 4 clinical trials. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and further document clinical benefit in the case of drugs approved under Accelerated Approval regulations. Failure to exhibit due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the biologic, findings from animal or



in vitro testing that suggest a significant risk for human subjects, and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA, the IRB, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated intervals based on access to certain data from the trial and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as interim data suggesting a lack of efficacy. We may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate. Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug product candidate and, among other things, must develop methods for testing the identity, strength, quality, potency and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug product candidate does not undergo unacceptable deterioration over its shelf life.

#### *BLA and FDA Review Process*

Following trial completion, trial data are analyzed to assess safety and efficacy. The results of preclinical studies and clinical trials are then submitted to the FDA as part of a BLA, along with proposed labeling for the product and information about the manufacturing process and facilities that will be used to ensure product quality, results of analytical testing conducted on the chemistry of the drug product candidate, and other relevant information. The BLA is a request for approval to market the biologic for one or more specified indications and must contain proof of safety, purity, potency and efficacy, which is demonstrated by extensive preclinical and clinical testing. The application may include both negative or ambiguous results of preclinical and clinical trials as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of a BLA must be obtained before a biologic may be marketed in the United States.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee, which is adjusted on an annual basis. PDUFA also imposes an annual prescription drug product program fee. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business.

Once a BLA has been accepted for filing, which occurs, if at all, sixty days after the BLA's submission, the FDA's goal is to review BLAs within 10 months of the filing date for standard review or six months of the filing date for priority review, if the application is for a product intended for a serious or life-threatening condition and the product, if approved, would provide a significant improvement in safety or effectiveness. The FDA has substantial discretion in the approval process and may refuse to accept any application or decide that the data is insufficient for approval, and may require additional preclinical, clinical or other studies before it accepts the filing. Additionally, the review process is often significantly extended by FDA requests for additional information or clarification.

After the BLA submission is accepted for filing, the FDA reviews the BLA to determine, among other things, whether the proposed drug product candidate is safe and effective for its intended use, and whether the drug product candidate is being manufactured in accordance with cGMP to assure and preserve the drug product candidate's identity, strength, quality, purity and potency. The FDA may refer applications for novel drug product candidates or drug product candidates which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. The FDA will likely re-analyze the clinical trial data, which could result in extensive discussions between the FDA and us during the review process. The review and evaluation of a BLA by the FDA is extensive and time consuming and may take longer than originally planned to complete, and we may not receive a timely approval, if at all.

Before approving a BLA, the FDA will conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether the facilities comply with cGMPs. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of

the product within required specifications. In addition, before approving a BLA, the FDA may also audit data from clinical trials to ensure compliance with GCP requirements. After the FDA evaluates the application, manufacturing process and manufacturing facilities, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the BLA identified by the FDA. The Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, withdraw the application or request a hearing. Even if such data and information is submitted, the FDA may ultimately decide that the BLA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive, and the FDA may interpret data differently than we interpret the same data.

There is no assurance that the FDA will ultimately approve a product for marketing in the United States, and we may encounter significant difficulties or costs during the review process. If a product receives marketing approval, the approval may be significantly limited to specific populations, severities of allergies, and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling or may condition the approval of the BLA on other changes to the proposed labeling, development of adequate controls and specifications, or a commitment to conduct post-market testing or clinical trials and surveillance to monitor the effects of approved products. For example, the FDA may require Phase 4 testing which involves clinical trials designed to further assess the product's safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. The FDA may also place other conditions on approvals including the requirement for a Risk Evaluation and Mitigation Strategy, or REMS, to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS. The FDA will not approve the BLA without an approved REMS, if required. a REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Product approvals may be withdrawn for non-compliance with regulatory standards or based on the results of post-market studies or surveillance programs. Additionally, post-approval, many types of changes to the approved product, such as adding new indications, changing manufacturing processes and adding labeling claims, are subject to further testing requirements and FDA review and approval. Such post-approval requirements can be costly and time-consuming and can affect the potential market and profitability of the product.

#### *Orphan Designation and Exclusivity*

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product.

Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug or biologic for the same indication for seven years from the date of such approval, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity on the basis of greater effectiveness or safety or providing a major contribution to patient care or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same indication or the same product for a different indication but that could be used off-label in the orphan indication. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval before we do for the same product, as defined by the FDA, for the same indication we are seeking approval, or if our product is determined to be contained within the scope of the competitor's product for the same indication or disease. If we pursue marketing approval for an indication broader than the orphan drug designation we have received, we may not be entitled to orphan drug exclusivity. Orphan drug status in the European Union has similar, but not identical, requirements and benefits.

#### *Expedited Development and Review Programs*

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track Designation if they are

intended to treat a serious or life-threatening condition and nonclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast Track Designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product concurrently with, or at any time after, submission of an IND, and the FDA must determine if the product qualifies for Fast Track Designation within 60 days of receipt of the sponsor's request. Under the Fast Track Designation, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review, or review within a six-month timeframe from the date a complete BLA is accepted for filing, if it has the potential to provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review.

Additionally, a product may be eligible for accelerated approval. An investigational drug may obtain accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials and, under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is now permitted to require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Under FDORA, the FDA has increased authority for expedited procedures to withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast Track Designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

#### *Breakthrough Designation*

A product can be designated as a breakthrough therapy if it is intended to treat a serious or life-threatening condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. A sponsor may request that a drug product candidate be designated as a breakthrough therapy concurrently with, or at any time after, the submission of an IND, and the FDA must determine if the drug product candidate qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor's request. If so designated, the FDA shall act to expedite the development and review of the product's marketing application, including by meeting with the sponsor throughout the product's development, providing timely advice to the sponsor to ensure that the development program to gather preclinical and clinical data is as efficient as practicable, involving senior managers and experienced review staff in a cross-disciplinary review, assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor, and taking steps to ensure that the design of the clinical trials is as efficient as practicable.

#### *Accelerated Approval for Regenerative Medicine Advanced Therapies*

FDA's regenerative medicine advanced therapy, or RMAT, program is intended to facilitate efficient development and expedite review of regenerative medicine advanced therapies, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A drug sponsor may request that FDA designate a drug as an RMAT concurrently with or at any time after submission of an IND. FDA has 60 calendar days to determine whether the drug meets the criteria, including whether there is preliminary clinical evidence indicating that the drug has the potential to address unmet medical needs for a serious or life-threatening disease or condition. A BLA for an RMAT may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. An RMAT that is granted accelerated approval and is subject to post approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post approval monitoring of all patients treated with such therapy prior to its approval.

### *Pediatric Trials*

Under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and efficacy of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDCA requires that a sponsor who is planning to submit a marketing application for a drug or biological product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within sixty days of an end-of-Phase 2 meeting or as may be agreed between the sponsor and FDA. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from nonclinical studies, early phase clinical trials, and/or other clinical development programs. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of data or full or partial waivers.

### *Post-Marketing Requirements*

Following approval of a new product, a manufacturer and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and recordkeeping activities, reporting to the applicable regulatory authorities of adverse experiences with the product, providing the regulatory authorities with updated safety and efficacy information, product sampling, distribution, and tracking and tracing requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available drugs and biologics for off-label uses, manufacturers may not market or promote such off-label uses.

Modifications or enhancements to the product or its labeling or changes of the site of manufacture are often subject to the approval of the FDA and other regulators, which may or may not be received or may result in a lengthy review process. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use.

In the United States, once a product is approved, its manufacture is subject to comprehensive and continuing regulation by the FDA. The FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMPs. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require, among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved products are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and quality assurance activities. BLA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms, and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by the FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such facilities or the ability to distribute products manufactured, processed or tested by them. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including, among other things, recall or withdrawal of the product from the market.

The FDA also may require post-approval testing, sometimes referred to as Phase 4 testing, REMS and post-marketing surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

#### *Other Regulatory Matters*

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in addition to the FDA, including, in the United States, the Centers for Medicare & Medicaid Services, or CMS, other divisions of the Department of Health and Human Services, or HHS (e.g., the Office of Inspector General, or OIG, and Office for Civil Rights), the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments. In the United States, sales, marketing and scientific/educational programs must also comply with federal and state fraud and abuse laws, data privacy and security laws, transparency laws, and pricing and reimbursement requirements in connection with governmental payor programs, among others. The handling of any controlled substances must comply with the U.S. Controlled Substances Act and Controlled Substances Import and Export Act. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities are also potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts. In addition, even if a firm complies with FDA and other requirements, new information regarding the safety or efficacy of a product could lead the FDA to modify or withdraw product approval. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

From time to time, legislation is drafted, introduced, passed in Congress and signed into law that could significantly change the statutory provisions governing the approval, manufacturing, and marketing of products regulated by the FDA. In addition to new legislation, FDA regulations, guidances, and policies are often revised or reinterpreted by the agency in ways that may significantly affect the manner in which pharmaceutical products are regulated and marketed. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

#### *U.S. Patent Term Restoration and Marketing Exclusivity*

Depending upon the timing, duration and specifics of the FDA approval of our drug product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

An abbreviated approval pathway for biological products shown to be biosimilar to, or interchangeable with, an FDA-licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, which was part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA. This amendment to the PHSA attempts to minimize duplicative testing. Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times, that the product and the reference product may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product. However, complexities associated with the larger, and often more complex,

structure of biological products as compared to small molecule drugs, as well as the processes by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted twelve years of exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after first licensure. "First licensure" typically means the initial date the particular product at issue was licensed in the United States. This does not include a supplement for the biological product or a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device, or strength, unless that change is a modification to the structure of the biological product and such modification changes its safety, purity, or potency. Whether a subsequent application, if approved, warrants exclusivity as the "first licensure" of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods. This six-month exclusivity, which attaches to the twelve-year exclusivity period for reference biologics, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial, provided that at the time pediatric exclusivity is granted there is not less than nine months of term remaining.

### ***Pricing and Reimbursement***

#### *United States*

Sales of our products will depend, in part, on the extent to which our products, once approved, will be covered and reimbursed by third-party payors, such as government health programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. The process for determining whether a third-party payor will provide coverage for a drug product, including a biologic, typically is separate from the process for setting the price of a drug product or for establishing the reimbursement rate that a payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the approved drugs for a particular indication.

In order to secure coverage and reimbursement for any drug product candidate that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the drug product candidate, in addition to the costs required to obtain FDA or other comparable regulatory approvals. Whether or not we conduct such studies, our drug product candidates may not be considered medically necessary or cost-effective. A third-party payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third party reimbursement may not be sufficient to enable us to maintain price levels high enough to realize an appropriate return on our investment in product development. In the United States, the principal decisions about reimbursement for new drug products are typically made by CMS, an agency within HHS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. However, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products can differ significantly from payor to payor. Additionally, one third-party payor's decision to cover a particular product or service does not ensure that other payors will also provide coverage for the product or service, and the level of coverage and reimbursement can differ significantly from payor to payor. As a result, the coverage determination process will often require us to provide scientific and clinical support for the use of our products to each payor separately and can be a time-consuming process, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Factors payors consider in determining reimbursement are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

The containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs, including biologics, have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for

substitution of generic products. In many countries, the prices of drug products are subject to varying price control mechanisms as part of national health systems. In general, the prices of drug products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for drug products but monitor and control company profits. Accordingly, in markets outside the United States, the reimbursement for drug products may be reduced compared with the United States. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for our drug product candidate or a decision by a third-party payor to not cover our drug product candidate could reduce physician usage of the drug product candidate and have a material adverse effect on our sales, results of operations and financial condition.

Outside of the United States, the pricing of pharmaceutical products is subject to governmental control in many countries. For example, in the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been approved. Some countries may require the completion of additional studies that compare the cost effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products but monitor and control product volumes and issue guidance to physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products will likely continue as countries attempt to manage healthcare expenditures. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower.

#### ***Other Healthcare Laws and Compliance Requirements***

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our business operations in the United States and our current and future arrangements with clinical investigators, healthcare providers, consultants, third-party payors and patients may expose us to broadly applicable federal and state fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any drugs for which we obtain marketing approval. In the United States, these laws include: the federal Anti-Kickback Statute, the False Claims Act, and HIPAA.

The Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by imprisonment, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it. Moreover, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution.

Although we would not submit claims directly to payors, drug manufacturers can be held liable under the federal civil False Claims Act, which imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) for, among other things, knowingly presenting, or causing to be presented to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties for each separate false claim, the potential for exclusion from participation in federal healthcare programs and, although the federal False Claims Act is a civil statute, conduct that results in a False Claims Act violation may also implicate various federal criminal statutes. The government may deem manufacturers to have “caused” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. Claims which include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. The federal False Claims Act also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the federal False Claims Act and to share in any monetary recovery. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, if approved, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our product candidates, are subject to scrutiny under this law.

The civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Additionally, we may be subject to data privacy and security regulations by both the federal government and states in which we conduct our business. For example, HIPAA created new federal criminal statutes that prohibit among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or the HITECH, and its implementing regulations, mandates, among other things, the adoption of uniform standards for the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information, which require the adoption of administrative, physical and technical safeguards to protect such information. Among other things, HITECH makes HIPAA's security standards directly applicable to business associates, defined as independent contractors or agents of covered entities, which include certain health care providers, health plans, and healthcare clearinghouses, that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities and business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, certain state laws govern the privacy and security of health information and other personal data in certain circumstances, some of which are more stringent or otherwise different than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and criminal penalties.

Further, the federal Physician Payments Sunshine Act, or the Sunshine Act, within the ACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to CMS information related to certain payments or other transfers of value made or distributed to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other licensed health care practitioners and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members. In addition, many states also govern the reporting of payments or other transfers of value, many of which differ from each other in significant ways, are often not pre-empted, and may have a more prohibitive effect than the Sunshine Act, thus further complicating compliance efforts.

We may become subject to federal government price reporting laws, which would require us to calculate and report complex pricing metrics in an accurate and timely manner to government programs, as well as federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Similar federal, state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services. Such laws are generally broad and are enforced by various state agencies and private actions. Also, many states have similar fraud and abuse statutes or regulations that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance, and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical and biotechnology companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales



and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, individual imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our financial results. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource- consuming and can divert a company's attention from the business.

#### *Current and Future Legislation*

In the United States and some foreign jurisdictions, there have been, and likely will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system directed at broadening the availability of healthcare, improving the quality of healthcare, and containing or lowering the cost of healthcare.

For example, in 2010, the ACA was enacted in the United States. The ACA includes measures that have significantly changed, and are expected to continue to significantly change, the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA of greatest importance to the pharmaceutical industry are that the ACA:

- made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on average manufacturer price, or AMP, on most branded prescription drugs and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP;
- imposed a requirement on manufacturers of branded drugs to provide a 70% point-of-sale discount as a condition for a manufacturer's outpatient drugs being covered under Medicare Part D;
- extended a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expanded the entities eligible for discounts under the 340B Drug Discount Program;
- imposed an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs, apportioned among these entities according to their market share in certain government healthcare programs, and
- established a Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products. The ACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted:

- The Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending reductions by Congress that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2031. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers were further reduced starting on January 1, 2025; however, legislation has been introduced in the U.S. Congress that would, if enacted, reverse these payment reductions. In addition to provider payment cuts under Medicare, the American Rescue Plan Act of 2021 also eliminated the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, effective January 1, 2024. These laws and regulations may result in additional reductions in Medicare and other healthcare funding available for healthcare providers and may otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.
- The American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.
- On May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides a federal framework for certain patients to request access to certain IND products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.
- On April 13, 2017, CMS published a final rule that gives states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.
- On May 23, 2019, CMS published a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

In addition, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient assistance programs, and reform government program reimbursement methodologies for drugs. At a federal level, President Trump reversed some of President Biden's executive orders including rescinding Executive Order 14087 entitled "Lowering Prescription Drug Costs for Americans." President Trump may issue new executive orders designed to impact drug pricing. A number of these and other proposed measures may require authorization through additional legislation to become effective. Congress and the Trump administration have indicated that they will continue to seek new legislative measures to control drug costs.

The Inflation Reduction Act of 2022, or IRA includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from \$7,050 to \$2,000 which became effective in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the HSS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The implementation of the IRA is currently subject to ongoing litigation challenging the constitutionality of the IRA's Medicare drug price negotiation program. The effects of the IRA on our business and the healthcare industry in general is not yet known.

Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain drug access and marketing cost disclosure and transparency measures, and designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other

restrictions could harm our business, financial condition, results of operations and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our drugs or put pressure on our drug pricing, which could negatively affect our business, financial condition, results of operations and prospects.

We cannot predict what healthcare reform initiatives may be adopted in the future. Further federal, state and foreign legislative and regulatory developments are likely, and we expect ongoing initiatives to increase pressure on drug pricing. Such reforms could have an adverse effect on anticipated revenues from product candidates and may affect our overall financial condition and ability to develop product candidates.

#### *The Foreign Corrupt Practices Act*

The FCPA prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

#### *Additional Regulation*

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

#### *Europe / Rest of World Government Regulation*

In addition to regulations in the United States, we may be subject to a variety of regulations in other jurisdictions that we may in the future select governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval of a product, we would need to obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the EU, for example, a clinical trial application must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the clinical trial application is approved in accordance with a country's requirements, clinical trial development may proceed. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under EU regulatory systems, we must submit an MAA. The application used to file the BLA in the United States is similar to that required in the EU, with the exception of, among other things, country-specific document requirements.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we or our potential collaborators fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

*European Union General Data Protection Regulation*

In addition to EU regulations related to the approval and commercialization of our products, we may be subject to the EU's General Data Protection Regulation, or GDPR. The GDPR imposes stringent requirements for controllers and processors of personal data of persons in the EU, including, for example, ensuring an appropriate legal basis or condition applies to the processing of personal data, more robust disclosures to individuals and a strengthened individual data rights regime, shortened timelines for data breach notifications, limitations on retention of information, increased requirements pertaining to special categories of data, such as health data, and additional obligations when we contract with third-party processors in connection with the processing of the personal data. The GDPR also imposes strict rules on the transfer of personal data out of the European Union to the United States and other third countries. In addition, the GDPR provides that EU member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

In addition, further to the UK's exit from the EU, the GDPR ceased to apply in the UK at the end of the transition period on December 31, 2020. However, as of January 1, 2021, the UK's European Union (Withdrawal) Act 2018 incorporated the GDPR (as it existed on December 31, 2020 but subject to certain UK specific amendments) into UK law, referred to as the UK GDPR. The UK GDPR and the UK Data Protection Act 2018 set out the UK's data protection regime, which is independent from but aligned to the EU's data protection regime.

The GDPR and UK GDPR apply extraterritorially, and we may be subject to the GDPR and UK GDPR because of our data processing activities that involve the personal data of individuals located in the European Union or United Kingdom, such as in connection with our EU and UK clinical trials. Failure to comply with the requirements of the GDPR or UK GDPR and the applicable national data protection laws of the EU member states and UK may result in fines of up to €20,000,000 (or £17.5 million for the UK) or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties. GDPR and UK GDPR regulations may impose additional responsibility and liability in relation to the personal data that we process and we may be required to put in place additional mechanisms to ensure compliance with the new data protection rules.

## Human Capital Resources

As of December 31, 2024, we had 164 employees, 161 of whom were full-time and 3 whom were part-time. Of those, 135 were engaged in research and development activities. Two full-time employees are located in Switzerland. We do not have any employees that are represented by a labor union or covered under a collective bargaining agreement. We consider our relationship with our employees to be good.

Our future success depends on our ability to attract, develop and retain key personnel, maintain our culture, and ensure diversity and inclusion in our board, management and broader workforce. Our human resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards. As these areas directly impact our ability to compete and innovate, they are key focus areas for our board of directors and senior executives. A testament to our strong culture is the strong results from our annual employee survey.

## Corporate History and Trademarks

We were incorporated under the laws of the State of Delaware in April 2017 under the name Tycho Therapeutics, Inc. In August 2018, our corporate name was changed to Cabaletta Bio, Inc. We have two subsidiaries, Cabaletta Bio GmbH, incorporated in Switzerland and Cabaletta Bio (Germany) GmbH, incorporated in Germany. Our principal executive offices are located at 2929 Arch Street, Suite 600, Philadelphia, PA 19104, and we have limited corporate and research operations in Germany and Switzerland. Our telephone number is (267) 759-3100, and our website address is [www.cabalettabio.com](http://www.cabalettabio.com). We do not incorporate the information on or accessible through our website into this Annual Report on Form 10-K, and you should not consider any information on, or that can be accessed through, our website to be part of this Annual Report on Form 10-K. We have included our website address in this Annual Report on Form 10-K solely as an inactive textual reference.

We view our operations and measure our business as one reportable segment. All of the Company's tangible assets are held in the United States. Refer to Note 8, Segment Information, to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K for additional information.

We own or have rights to various trademarks, service marks and trade names that we use in connection with the operation of our business. This Annual Report on Form 10-K may also contain trademarks, service marks and trade names of third parties, which are the property of their respective owners. Our use or display of third parties' trademarks, service marks, trade names or products in this Annual Report on Form 10-K is not intended to, and does not imply a relationship with, or endorsement or sponsorship by us. Solely for convenience, the trademarks, service marks and trade names referred to in this Annual Report on Form 10-K may appear without the ®, ™ or SM symbols, but the omission of such references is not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the right of the applicable owner of these trademarks, service marks and trade names.

## Available Information

Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, including exhibits, and any amendments to these reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge on our website located at [www.cabalettabio.com](http://www.cabalettabio.com) as soon as reasonably practicable after they are filed with or furnished to the SEC. These reports are also available at the SEC's Internet website at [www.sec.gov](http://www.sec.gov).

Investors and others should note that we announce material information to our investors using our investor relations website (<https://www.cabalettabio.com/investors>), SEC filings, press releases, public conference calls and webcasts. We use these channels as well as social media, including LinkedIn and X (formerly Twitter) (@CabalettaBio), to communicate with the public about our company, our business, our product candidates and other matters. It is possible that the information we post on social media could be deemed to be material information. Therefore, we encourage investors, the media, and others interested in our company to review the information we post on the social media channels listed on our investor relations website. Information that is contained in and can be accessed through our website or our social media posts are not incorporated into, and does not form a part of, this Annual Report on Form 10-K.

A copy of our Corporate Governance Guidelines, Code of Conduct and Business Ethics and the charters of the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee are posted on our website, [www.cabalettabio.com](http://www.cabalettabio.com), under the heading "Investors & Media."

## Item 1A. Risk Factors.

*Our business involves material and other risks, some of which are summarized and described below. You should carefully consider the risks and uncertainties described below, together with all of the other information contained in this Annual Report on Form 10-K, including "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the consolidated financial statements and the related notes. If any of the following risks actually occur, it could harm our business, prospects, operating results and financial condition and future prospects. In such event, the market price of our common stock could decline and you could lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this Annual Report.*

### Risks Related to Our Business, Technology and Industry

#### Risks Related to Clinical Development

***We are early in our development efforts. If we are unable to advance our product candidates through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.***

We are early in our development efforts and we have not yet completed any clinical trials. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of one or more of our product candidates. Even if we are able to develop and commercialize a marketable product, we may face challenges generating revenue from product sales. The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies resulting in data that is supportive of advancing to an investigational new drug, or IND, or clinical trial application, or CTA submission;
- successful submission and acceptance of INDs, CTAs or comparable applications;
- successful initiation of clinical trials;
- demonstration of adequate safety to progress to a therapeutic dose level;
- successful patient enrollment in and completion of clinical trials;
- receipt and related terms of regulatory and marketing approvals and licensures from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers for clinical supply and commercial manufacturing of our product candidates;
- making arrangements with various medical divisions across hospitals for administration of our product candidates, including with cancer treatment centers to conduct leukapheresis and with the relevant hospital divisions to perform infusion;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- establishing sales, marketing and distribution and patient administration capabilities and launching commercial sales of our products, if and when licensed, whether alone or in collaboration with others;
- acceptance of our products, if and when licensed, by patients, the medical community and third-party payors;
- effectively competing with established and emerging therapies targeting the same indications as our product candidates;
- obtaining and maintaining third-party coverage and adequate reimbursement; and
- maintaining a continued acceptable safety profile of our products following licensure.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or be unable to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

***Cellular therapies, including our engineered chimeric antigen receptor T cell, or CAR T, chimeric autoantibody receptor T cell, or CAAR T, product candidates, represent a novel approach to the treatment of autoimmune diseases, which creates***

*significant challenges for us. Negative perception or increased regulatory scrutiny of any product candidates that we develop could adversely affect our ability to conduct our business or obtain regulatory approvals for such product candidates.*

Cellular therapies are a novel approach to the treatment of autoimmune diseases, and negative perception or increased regulatory scrutiny of any product candidates that we develop could adversely affect our ability to conduct our business or obtain regulatory approvals for such product candidates. There are no cellular immunotherapies licensed to date in the United States or the European Union to treat autoimmune diseases or alloimmune responses. CAR T or CAAR T cell therapies for autoimmune or alloimmune diseases may not gain the acceptance of the public or the medical community. For example, CAR Ts and other cellular therapies have in some cases caused severe side effects, including death, and their broader use may therefore be limited. In the future, in the event such severe side effects are observed with other CAR T therapies (including those with a CD19 binder), it may increase negative perception of, and regulatory scrutiny on, our product candidates. For example, in November 2023, the FDA announced that it would be conducting an investigation into reports of T cell malignancies following treatment with BCMA-directed or CD19-directed autologous CAR T cell immunotherapies. The FDA also stated that patients and clinical trial participants receiving treatment with such approved products should be monitored life-long for new malignancies. In January 2024, the FDA determined that new safety information related to T cell malignancies should be included in the labeling with boxed warning language on these malignancies for all BCMA- and CD19-directed genetically modified autologous T cell immunotherapies. Public perception may be influenced by claims that gene therapy, including the insertion of a transgene, is unsafe, and products incorporating gene therapy may not gain the acceptance of the public or the medical community. The patient populations targeted by our product candidates are also typically not at risk of near-term death, even if they may suffer life-threatening symptoms, so patients will need to deem the benefits of cell therapy to be worth the risk of unknown potential adverse side effects. Our success will depend upon physicians who specialize in the treatment of autoimmune diseases targeted by our product candidates prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are more familiar and for which greater clinical data may be available. Adverse events in clinical trials of our product candidates, in clinical trials of others developing similar products or in the post-approval setting and the resulting publicity, as well as any other adverse events in the field of cellular therapies, could result in a decrease in demand for any product that we may develop.

We are developing a pipeline of CAR T and CAAR T product candidates that are intended for use in treating individuals with autoimmune diseases. Advancing these novel product candidates creates significant challenges for us, including:

- manufacturing our product candidates to our specifications and in a timely manner to support our clinical trials, and, if licensed, commercialization;
- sourcing clinical and, if licensed, commercial supplies for the materials used to manufacture our product candidates;
- understanding and addressing variability in the quality and quantity of a subject's T cells, which could ultimately affect our ability to manufacture clinical supply and, if licensed, commercial supply of our product candidates in a reliable and consistent manner;
- educating medical personnel regarding the potential side effect profile of our product candidates, if licensed, such as the potential adverse side effects related to worsening of systemic lupus erythematosus, or SLE, idiopathic inflammatory myopathy (IIM), or myositis, systemic sclerosis, or SSc, muscle-specific kinase myasthenia gravis, or MuSK MG, generalized myasthenia gravis, or gMG, pemphigus vulgaris, or PV, multiple sclerosis, or MS, adverse effects related to infusion of activated T cells or medication taper, including cytokine release syndrome, or CRS, immune effector cell-associated neurotoxicity syndrome, or ICANS, or other unexpected adverse effects of therapy with our product candidates or potential class-wide side effects, such as those related to CD19-directed autologous CAR T cell immunotherapies;
- facilitating patient access to the limited number of facilities able to administer our product candidates, if licensed;
- using medicines to manage adverse side effects of our product candidates that may not adequately control the side effects and/or may have a detrimental impact on the efficacy of the treatment;
- utilizing preconditioning agents in patients to enhance engraftment in advance of administering our product candidates, which may increase the risk of adverse side effects and potentially reduce the population eligible for therapy;
- obtaining and maintaining regulatory approval for our product candidates, as the FDA and other regulatory authorities have limited or no experience with development of engineered T cell therapies for the treatment of autoimmune diseases where B cells may play a role in initiating or maintaining disease;
- establishing sales and marketing capabilities upon obtaining any regulatory approval to gain market acceptance of a novel therapy; and
- managing costs of inputs and other supplies while scaling production.

In addition, preclinical murine and other animal models may not exist or be adequate for some or all of the autoimmune diseases where B cells may play a role in initiating or maintaining disease we choose to pursue in our programs, and because we are early in the clinical development process, we are unable to predict whether there may be short-term or long-term effects from treatment with any product candidates that we develop. In developing our product candidates, we have not exhaustively explored different options in the method for manufacturing CAR T or CAAR T cells. We may find our existing manufacturing process may be substantially improved with future design or process changes, necessitating further clinical testing, delaying commercial launch of our first products, and causing us to incur additional expenses. For example, while we have used a lentiviral vector in our manufacturing process, we may in the future find that another viral vector or non-viral vector-based process offers advantages. Switching from one lentiviral vector to another or switching from lentiviral to another delivery system would necessitate additional process development and clinical testing, and this may delay the development of existing product candidates.

In addition, we do not know the doses to be evaluated in pivotal trials or, if licensed, commercially. Finding a suitable dose may delay our anticipated clinical development timelines, and we may elect to pause clinical trials to find a suitable dose or make assessments ahead of continuing a trial. Our expectations with regard to our scalability and costs of manufacturing may vary significantly as we develop our product candidates and understand these critical factors. We may experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our product candidates on a timely or profitable basis, if at all.

Moreover, our product candidates may not perform successfully in clinical trials or may be associated with adverse events that distinguish them from the CAR T therapies that have previously been licensed. For instance, subjects in our CAAR T clinical trials will be infused with our proposed therapies, and may possess strongly activating soluble antibodies, which are not present in oncology patients, and when they interact with our infused product candidates, could result in potential adverse side effects, such as CRS or ICANS. Additionally, adverse side effects caused by even one of our CAR T or CAAR T product candidates could negatively affect our ability to develop future product candidates based on our CABA<sup>®</sup> platform. Unexpected side effects or clinical outcomes from any of our products candidates would significantly impact our business.

Further, the clinical study requirements of the FDA, the European Medicines Agency, or EMA, and other regulatory agencies and the criteria they use to determine the safety, potency and purity of a product candidate are determined according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours is less clear, and can be more complex and consequently have higher development risk, be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. Approvals by the FDA for existing cell therapies treating B cell-mediated diseases, such as Kymriah (Novartis Pharmaceuticals Corporation) and Yescarta<sup>®</sup> (Gilead Sciences, Inc.) in oncology indications, may not be indicative of what the FDA may require for approval of our therapies in autoimmune indications. Approvals by any regulatory agency may not be indicative of what any other regulatory agency may require for approval or what such regulatory agencies may require for approval in connection with new product candidates. As we advance our product candidates, we will be required to consult with these regulatory agencies and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. More restrictive statutory regimes, government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop.

In addition, responses by agencies at the federal and state level to negative public perception or ethical concerns may result in new legislation or regulations that could limit our ability to develop or commercialize any product candidates, obtain or maintain regulatory approval or otherwise achieve profitability. The FDA has expressed interest in further regulating biotechnology products, such as cellular therapies. Agencies at both the federal and state level in the United States, as well as the U.S. Congressional committees and other government entities or governing agencies have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates. Adverse developments in clinical trials of cellular therapy products conducted by others or in the post-approval setting may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. These regulatory review agencies and committees and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions.

***Patients receiving T cell-based immunotherapies, such as our product candidates, have in the past and may in the future experience serious adverse events, including ICANS, CRS and killing of cells other than the intended B cells that express the autoantibodies. If our product candidates are revealed to have high and unacceptable severity and/or prevalence of side effects or unexpected characteristics, their clinical development, regulatory approval, and commercial potential will be negatively impacted, which will significantly harm our business, financial condition and prospects.***

Our product candidates are CAR T or CAAR T cell-based immunotherapies. In our clinical trials and other similarly designed cellular immunotherapies to treat cancer, there have been life threatening events related to ICANS and CRS requiring intense medical



intervention, such as intubation or medications to support blood pressure, and in several cases, resulted in death. We have observed events of CRS and ICANS in our ongoing rese-cel REStoring SElf-Tolerance, or RESET™, trial. ICANS is a condition that is currently defined clinically by cerebral edema, confusion, drowsiness, speech impairment, tremors, seizures or other central nervous system side effects, when such side effects are serious enough to lead to intensive care. CRS is a condition that is currently defined clinically by certain symptoms related to the release of cytokines, which can include fever, chills and low blood pressure, when such side effects are serious enough to lead to intensive care with mechanical ventilation or significant medications to support blood pressure. There is a possibility that our product candidates could have similarly life threatening serious adverse side effects, such as ICANS and CRS.

Our product candidates may have serious and potentially fatal consequences due to the targeting of cells within the body due to unexpected protein interactions with the CAR or CAAR. Although we have completed multiple preclinical studies designed to screen for toxicity caused by unintended off-target recognition by the cell binding domain of the DSG3 CAAR, MuSK CAAR and rese-cel and intend to screen future CAR and CAAR candidates not yet tested in patients through preclinical studies, our product candidates may still recognize and react with one or more proteins unrelated to the intended surface immunoglobulin target protein to which it is designed to link. If unexpected binding occurs in normal tissue, our product candidates may target and kill the normal tissue in a patient, leading to serious and potentially fatal adverse events, undesirable side effects, toxicities or unexpected characteristics. Detection of any unexpected targeting may halt or delay any ongoing clinical trials for our product candidates and prevent or delay regulatory approval. While we have developed a preclinical screening process to identify cross-reactivity of our product candidates, we cannot be certain that this process will identify all potential tissue that our product candidates may target. For example, a membrane protein array with DSG3-CAART yielded one weak signal against a protein that is designed to bind to glycoproteins and which was detected in both the test and control conditions. Further analysis of this protein in confirmatory cell-based assays repeatedly demonstrated that DSG3-CAART does not recognize nor activate against this protein. We performed similar preclinical studies for the MuSK CAAR and rese-cel and did not observe any confirmed off target activity for MuSK-CAART or rese-cel. However, this further analysis may prove to be inaccurate. Any unexpected targeting that impacts patient safety could materially impact our ability to advance our product candidates into clinical trials or to proceed to marketing approval and commercialization. Furthermore, in the event subjects are re-treated, they may respond differently than other subjects given the same dose, and may not tolerate the dose or develop safety concerns.

Results of our studies could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory agencies. Our clinical trials of rese-cel represent the first evaluation of this product candidate in patients and rese-cel is directed against all B cells expressing CD19; therefore, there is a risk for prolonged B cell aplasia and/or hypogammaglobulinemia, which may predispose patients to infections. Given that the autoimmune and alloimmune diseases we are seeking to treat are, in some cases, less serious than the later stage cancers being treated with other immunotherapy products, we believe the FDA and other regulatory authorities likely will apply a different benefit-risk assessment thresholds such that even if our product candidate demonstrated a similar safety profile as current CAR T therapies, the FDA may ultimately determine that the harmful side effects outweigh the benefits and require us to cease clinical trials or deny approval of our product candidates. We believe tolerance for adverse events in the patient population being pursued with our CAAR T and CAR T cell therapies in autoimmune and alloimmune indications will be lower than it is in oncology, and the risks of negative impact from these toxicities may therefore be higher for us than for CAR T programs in oncology.

Furthermore, treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the studies or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from T cell-based immunotherapies are not normally encountered in routine medical care. Medical personnel may need additional training regarding T cell-based immunotherapy product candidates to understand their side effects. Inadequate training in recognizing or failure to effectively manage the potential side effects of T cell-based immunotherapy product candidates could result in patient deaths. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition to side effects caused by our product candidates, any preconditioning, administration process or related procedures, which we evaluate from time to time as part of our process improvement and optimization efforts, may also cause adverse side effects. For example, prolonged or persistent cytopenias and ICANS have been noted to be associated with the use of certain lymphodepleting regimens and CAR T therapies.

***Preconditioning regimens, as currently implemented in several of our clinical trials, may increase the risk of adverse side effects and impact our ability to accurately assess the efficacy of our product candidates.***

In oncology patients receiving CAR T cell therapy, a lymphodepleting preconditioning regimen is typically used to condition the patient prior to CAR T cell infusion in order to improve tumor immunogenicity and to promote the expansion of the infused CAR T cells. Together, these effects have been shown to enhance the clinical activity of CAR T cells in oncology patients. These regimens often include cyclophosphamide and fludarabine and are usually administered within the week prior to infusion of CAR T cells. We implemented a preconditioning regimen in the DesCAARTes™ trial where certain subjects were pre-treated with IVIg and cyclophosphamide, and other patients were pre-treated with IVIg, cyclophosphamide, and fludarabine prior to DSG3-CAART infusion, have included planned dosing cohorts in the MusCAARTes™ trial where subjects are pre-treated with fludarabine and

cyclophosphamide prior to MuSK-CAART infusion, and we have incorporated a lymphodepleting preconditioning regimen of fludarabine and cyclophosphamide in our RESET™ clinical trials. We are evaluating rese-cel without preconditioning in our RESET-PV™ trial in patients with mPV and mcPV. Serious adverse events have been observed in some patients following CAR T cell infusion, and these include infection, cytokine release syndrome and ICANS. The lymphodepleting and immunomodulatory preconditioning regimen may contribute to the occurrence and severity of these adverse events due to its role in inducing leukopenia, or low levels of white blood cells in the blood, including lymphopenia, or low levels of lymphocytes in the blood, and regulating the activation and effector functions of other immune cells and antibodies, and enhanced CAR T cell activity.

In addition, a lymphodepleting regimen may eliminate pathogenic B cells targeted by our CAAR T cell product candidates. As a result, any lymphodepleting regimen for preconditioning that we use may delay or otherwise adversely affect our ability to use DSG3 or MuSK autoantibody titers, a standard clinical assay, to assess the activity of DSG3-CAART and MuSK-CAART, respectively. An inability to use DSG3 or MuSK autoantibody levels to demonstrate the specific activity of our CAAR T cell product candidates may require us to rely on the subjective measurement of blister formation in patients in the DesCAARTes™ trial or muscle weakness in the MusCAARTes™ trial, which can be a less sensitive and accurate measurement of CAAR T cell activity. This therefore could delay a signal of potential biologic activity attributable to CAAR and therefore may slow clinical development. We will continue to evaluate emerging data from the MusCAARTes™ trial on an ongoing basis, as well as other relevant clinical trials in autoimmune disease, and may make additional modifications, as appropriate.

In addition to lymphodepleting preconditioning, other preconditioning regimens with immunomodulatory effects may be considered to prepare the body for CAR T or CAAR T infusion. For example, if autoantibody is found to reduce or inhibit function of CAAR T in the body, then pretreatment of patients with antibody reducing therapies, such as FcRN inhibitors, IVIg, plasmapheresis, or treatment of post rituximab patients may be considered. Some of these types of preconditioning are standard of care for this autoimmune population and therefore are already considered to have a beneficial risk profile in this patient population. These other preconditioning regimens may cause serious adverse events, including hypotension, thromboembolism, and opportunistic infections.

Subjects in our RESET™ trials, except for our RESET-PV™ trial, will be treated with a standard preconditioning regimen consisting of fludarabine and cyclophosphamide prior to rese-cel infusion. In addition, the lymphodepleting regimen may eliminate some of the pathogenic B cells targeted by rese-cel. As a result, the lymphodepleting regimen may contribute to the initial clinical response that may be observed after rese-cel, which may make interpretation of early efficacy difficult to assess and may also delay our ability to characterize the activity of rese-cel independent of the effects of fludarabine and cyclophosphamide. We intend to evaluate this potential impact of preconditioning in our CAAR and rese-cel studies, through the comparison to non-lymphodepletion arms.

Our clinical patients may experience increased or more severe adverse effects specifically related to the preconditioning regimens, such as severe allergic reactions, difficulty breathing, severe headaches, fevers and chills, serious infections, low blood counts, inflammation of the colon with bleeding, bladder irritation, blood clots, development of certain cancers, damage to the heart, lung or kidneys, and even death. These undesirable side effects, whether associated with the preconditioning regimen alone or in combination with our CAR T cell product candidates or CAAR T cell product candidates, could cause delays in patient enrollment in our clinical trials, could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a change to our clinical trial design, a more restrictive label or the delay or denial of regulatory approval by the FDA. Any of the foregoing may increase the duration and expense of the clinical development of our product candidates or limit market acceptance of such product candidates, if approved, any of which could have a material adverse effect on our business and financial condition.

***Our business is highly dependent on the success of our initial product candidates targeting autoimmune diseases where B cells may play a role in initiating or maintaining disease. All of our product candidates will require significant additional preclinical and/or clinical development before we can seek regulatory approval for and launch a product commercially.***

Our business and future success depend on our ability to obtain regulatory approval of, and then successfully launch and commercialize our initial product candidates targeting autoimmune diseases where B cells may play a role in initiating or maintaining disease. There is no guarantee that we will be able to advance our product candidates through clinical development or obtain marketing approval for any of our product candidates. The process for obtaining marketing approval for any product candidate is very long and risky and there will be significant challenges for us to address in order to obtain marketing approval as planned, if at all.

The initial clinical results we have observed may not be predictive of results of subsequent cohorts in this clinical trial, or of any future clinical trials. Because rese-cel, DSG3-CAART and MuSK-CAART are the first three product candidates that we are testing in the clinic, we may experience preliminary complications surrounding trial design, protocol establishment and execution, establishing trial protocols, patient recruitment and enrollment, quality and supply of clinical doses, or safety issues.

Additionally, a failure of our clinical trials of DSG3-CAART, MuSK-CAART or rese-cel RESET™ trials could influence physicians' and regulators' opinions with regard to the viability of our CABA® platform more broadly, particularly if treatment-related side effects are observed. The occurrence of any of these risks could significantly harm our development plans and business prospects. If treatment-related side effects are observed with the administration of DSG3-CAART, MuSK-CAART or rese-cel, or if they are viewed

as less safe, potent or pure than other therapies, our ability to develop other CAAR T or CAR T cell therapies may be significantly harmed.

***We have never successfully completed any clinical trials, and we may be unable to do so for any product candidates we develop.***

We have not yet demonstrated our ability to successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain regulatory approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Although our key employees have significant experience in leading clinical development programs, our experience conducting clinical trials with our product candidates is limited. We may not be able to file INDs or CTAs for any of our other product candidates on the timelines we expect, if at all. For example, we cannot be certain that the IND-enabling or CTA-enabling studies for our future product candidates will be completed in a timely manner or be successful or that the manufacturing process will be validated in a timely manner. Even if we submit an IND or CTA for a future product candidate, the FDA, the EMA, or other foreign regulatory authorities may not clear the IND or CTA and allow us to begin clinical trials in a timely manner or at all. The timing of submissions on future product candidates will be dependent on further preclinical and manufacturing success. Moreover, we cannot be sure that submission of an IND or a CTA will result in the FDA or other foreign regulatory authorities allowing further clinical trials to begin, or that, once begun, issues will not arise that require us to suspend or terminate clinical trials. Commencing each of these clinical trials is subject to finalizing the trial design based on discussions with the FDA and other foreign regulatory authorities. Any guidance we receive from the FDA or other foreign regulatory authorities is subject to change. These regulatory authorities could change their position, including, on the acceptability of our trial designs or the clinical endpoints selected, which may require us to complete additional clinical trials or impose stricter approval conditions than we currently expect.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- be subject to post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

***If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.***

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- inability to bring our product candidates to the market;
- decreased demand for our product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;

- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate; and
- a decline in our share price.

Since we have not yet commenced marketing of any products, we do not yet hold product liability insurance for commercialization of our product candidates. Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with corporate collaborators. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. Assuming we obtained clinical trial insurance for our clinical trials, we may have to pay amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

#### Risks Related to the Industry

***Our product candidates may cause undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.***

Undesirable or unacceptable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other foreign regulatory authorities. Further, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of subjects and limited duration of exposure, rare and severe side effects of our product candidates may only be uncovered with a significantly larger number of patients exposed to the drug. Undesirable side effects could also result in an expansion in the size of our clinical trials, increasing the expected costs and timeline of our clinical trials. Additionally, results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics.

Licensed CAR T cell therapies and those under development have shown frequent rates of CRS and ICANS, and adverse events have resulted in the death of patients. Similar adverse events have occurred and could occur during treatment with our current or future CAR T or CAAR T cell product candidates. For example, activation of CAAR T cells by patient autoantibodies or alloantibodies could stimulate CRS. When CAAR T cells are infused and the CAAR binds to soluble antibodies in the blood or tissues of treated patients, these soluble antibodies may cause the CAAR T cells to proliferate, resulting in an activation of the immune system that is too high, leading to CRS. Further, it is possible that patients will exhibit acute rejection of the CAAR T cells because of preexisting immunity to the antigen within the CAAR. This could render our product candidates ineffective.

If unacceptable toxicities or health risks, including risks inferred from other unrelated immunotherapy trials, arise in the development of our product candidates, we could suspend or terminate our trials or the FDA, the Safety Monitoring Boards for our trials (e.g. Data Safety Monitoring Board, or DSMB, or Independent Data Safety Monitoring Committee, or IDMC), or local regulatory authorities such as institutional review boards, or IRBs, or independent ethics committees, or IECs, as applicable, could recommend or order us to cease clinical trials. Regulatory authorities, such as the FDA, could also deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from T cell therapy are not normally encountered in the general patient population and by medical personnel. We expect to have to train medical personnel using CAR T or CAAR T cell product candidates to understand the side effect profile of our product candidates for both our preclinical studies and clinical trials and upon any commercialization of any of our product candidates, if licensed. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient deaths. Any of these occurrences may harm our business, financial condition and prospects significantly.

***Our preclinical studies and clinical trials may fail to demonstrate the safety, potency and purity of any of our product candidates, which would prevent or delay regulatory approval and commercialization.***

Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are safe, potent and pure for use in each target indication. Clinical trials are expensive and can take many years to complete, and their outcomes are inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, including in any post-approval studies of our product candidates. In addition, initial success in any clinical trials may not be indicative of results obtained when such trials are completed. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety, potency and purity profile despite having progressed through preclinical studies and initial clinical trials. Similarly, while we believe rese-cel has a similar overall design to the construct used for the patients in

the *Nature Medicine*, *Lancet*, *Annals of Rheumatic Diseases*, and *Rheumatology* publications, those studies involved a small number of patients, and a different product candidate, and the initial clinical results observed in those studies may not be predictive of clinical trial results with rese-cel or any of our other product candidates, additionally, because those studies are not our own, we may not have access to accurate follow-up information or peer-reviewed results.

A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of potency or efficacy, insufficient durability of potency or efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical and other nonclinical findings made while clinical trials were underway, or safety or efficacy observations made in preclinical studies and clinical trials, including previously unreported adverse events. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA or European Commission approval. Most product candidates that commence clinical trials are never approved as products.

Any preclinical studies or clinical trials that we may conduct may not demonstrate the safety, potency and purity necessary to obtain regulatory approval to market our product candidates. If the results of our ongoing or future preclinical studies and clinical trials are inconclusive with respect to evaluations of efficacy, the safety, potency and purity of our product candidates, if we do not meet the clinical endpoints with statistical and clinically meaningful significance, or if there are safety concerns associated with our product candidates, we may be prevented or delayed in obtaining marketing approval for such product candidates. In some instances, there can be significant variability in evaluations of efficacy, safety, potency or purity results between different preclinical studies and clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols and the rate of dropout among clinical trial participants. For example, because our CAAR T cell product candidates only target approximately 0.01% to 1% of the B cells in a patient, they may not engage enough of the target to achieve adequate engraftment necessary for elimination of all pathogenic B cells. Insufficient safety or potency in clinical trials may delay product development to enable time to modify the product candidate for next generation approaches or make manufacturing changes or may lead us to discontinue development of the product candidate.

Additionally, our ongoing clinical trials utilize, and our planned trials may utilize, an “open-label” trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an active drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with any of our product candidates for which we include an open-label clinical trial when studied in a controlled environment with a placebo or active control.

In addition, we cannot guarantee that the FDA or other foreign regulatory authorities will interpret the results of any of our ongoing or planned clinical trials as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or other foreign regulatory authorities to support a marketing application, approval of our product candidates may be significantly delayed, or we may be required to expend significant additional resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, an applicant will need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. For example, the process governing approval of medicinal products in the European Union generally follows the same lines as in the United States but can differ in significant ways as well. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application and granting of a marketing authorization by these authorities before the product can be marketed and sold in the European Union.

***Interim, topline or preliminary data from any preclinical studies or clinical trials that we conduct may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.***

Our DesCAARTes™ trial, MusCAARTes™ trial, and RESET™ trials in SLE, myositis, SSc, gMG, MS and PV are designed as open-label trials. From time to time, we may publicly disclose interim, preliminary or topline data from our preclinical studies and clinical trials, including safety data and evaluations of efficacy, which will be based on a preliminary analysis of then-available data,

and the results and related findings and conclusions are subject to change following our receipt of additional data or a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data.

As a result, the topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data are available. From time to time, we may also disclose interim data from planned interim analyses in our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects. Further, disclosure of interim data by us or our competitors, or by patients or caregivers who are aware that a patient is receiving investigational product, due to the open-label design of the trial, could result in volatility in the price of our common stock.

Regulatory agencies, including the FDA or other foreign regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general.

If the interim, topline or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

***The increasing use of social media platforms presents new risks and challenges.***

Social media is increasingly being used to communicate about our clinical development programs and the diseases our product candidates are being developed to treat. We intend to utilize appropriate social media in connection with communicating about our development programs. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to report an alleged adverse event during a clinical trial. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations, or we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our investigational products. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website, or a risk that a post on a social networking website by any of our employees may be construed as inappropriate promotion. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

***We may encounter substantial delays in our clinical trials or may not be able to conduct our trials on the timelines we expect or at all.***

Clinical testing is expensive, time consuming and subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Even if these trials begin as planned, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our ongoing and future clinical trials may not be successful. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology or other in vivo or in vitro data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- delays in developing suitable assays for screening patients for eligibility for clinical trials with respect to certain product candidates;
- delays in reaching a consensus with the FDA and other regulatory agencies on trial design;
- delays in reaching agreement on acceptable terms with prospective CMOs, CROs and clinical study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CMOs, CROs and clinical trial sites;
- delays in obtaining required institutional review board, or IRB, approval at each clinical trial site;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND submission or amendment, or equivalent application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; a negative finding from an inspection of our clinical study operations

or study sites; developments on trials conducted by competitors for related technology or by those that rely on a similar construct, design and/or third-party research that raise FDA or other foreign regulatory authorities concerns about risk to patients of the technology or construct broadly, and/or negative public perception of the same; or if FDA or other foreign regulatory authorities find that the investigational protocol or plan is clearly deficient to meet its stated objectives;

- delays in recruiting eligible patients to participate in our clinical trials, including pediatric patients who need parental consent;
- delays in treating one or more patients, once enrolled, due to a patient's inability to accommodate parts of the complex study procedures schedule;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties or us to adhere to clinical trial requirements and the potential termination of ongoing agreements with our CROs;
- limitations on our recourse in our CRO relationship with Penn as compared to a CRO that is not an academic institution;
- failure to perform in accordance with the FDA's Good Clinical Practice, or GCP, requirements or applicable regulatory guidelines in other countries;
- transfer of manufacturing processes to any new CMO or our own manufacturing facilities or any other development or commercialization partner for the manufacture of product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- patients dropping out of a trial;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- the cost of clinical trials of our product candidates being greater than we anticipate;
- clinical trials of our product candidates producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon product development programs;
- delays or failure to secure supply agreements with suitable raw material suppliers, or any failures by suppliers to meet our quantity or quality requirements for necessary raw materials; and
- delays in manufacturing or inability to manufacture sufficient clinical supply (for example, due to capacity constraints, supply interruption, or the need to engineer the process to meet higher dose requirements), testing, releasing, validating or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue. If we make manufacturing or formulation changes to our product candidates, we may be required to, or we may elect to, conduct additional trials to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our product candidates and products, if licensed, have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

We could also encounter delays if a clinical trial is suspended or terminated by us, the FDA or other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Delays in the initiation, conduct or completion of any clinical trial of our product candidates will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. In the event we identify any additional product candidates to pursue, we cannot be sure that submission of an IND or comparable foreign regulatory submission will result in the FDA or other foreign regulatory authorities allowing clinical trials to begin in a timely manner, if at all.

In addition, from time to time, we may publicly announce the expected timing of various scientific, clinical, regulatory, manufacturing and other product development milestones. These milestones may include the commencement, completion or development of data from our preclinical studies and clinical trials or the submission of regulatory filings, such as an IND or a CTA. All of these milestones are, and will be, based on a variety of assumptions. If any of the foregoing events impact our ability to meet the publicly announced timing of our milestones, we may experience adverse effects on our business, financial condition and prospects and the price of our common stock could decline.

***Monitoring safety of patients receiving our product candidates will be challenging, which could adversely affect our ability to obtain regulatory approval and commercialize our product candidates.***

For our RESET™, DSG3-CAART, MuSK-CAART and other planned clinical trials, we expect to continue to contract with academic medical centers and hospitals experienced in the assessment and management of toxicities arising during clinical trials. In the future, we may also contract with non-academic medical centers and hospitals with similar capabilities. Nonetheless, these centers and hospitals may have difficulty observing patients, including due to failure by patients to comply with post-clinical trial follow-up programs, and treating toxicities, which may be more challenging due to personnel changes, inexperience, inadequate institutional safety procedures, shift changes, house staff coverage or related issues. This could lead to more severe or prolonged toxicities or even patient deaths, which could result in us or the FDA delaying, suspending or terminating one or more of our clinical trials, and which could jeopardize regulatory approval. We also expect the centers using rese-cel, DSG3-CAART, MuSK-CAART and our other product candidates, if licensed, on a commercial basis could have similar difficulty in managing adverse events. Medicines used at centers to help manage adverse side effects of rese-cel, DSG3-CAART, MuSK-CAART and our other product candidates may not adequately control the side effects and/or may have a detrimental impact on the efficacy of the treatment.

***If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.***

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. The enrollment of patients depends on many factors, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- recruiting an adequate number of suitable patients to participate in a clinical trial;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining IRB and other required reviewing body approval at each clinical trial site;
- the proximity of patients to trial sites;
- the design of the trial and whether the FDA or other foreign regulatory authorities agree to the design and implementation of the trial;
- our ability to identify clinical trial sites and recruit clinical trial investigators with the appropriate capabilities, competencies and experience;
- clinicians', patients' and parents' (for juvenile patients) perceptions as to the potential advantages and risks of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating, or with CAR T cell therapies broadly following the FDA's investigation into reports of T cell malignancies for approved BCMA- and CD19-directed CAR T cell immunotherapies;



- the occurrence of dose-limiting toxicity in the clinical trial;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- our ability to obtain and maintain patient consents;
- the risk that patients enrolled in clinical trials will drop out of the trials before the infusion of our product candidates or trial completion; and
- the ability of patients to meet the complex follow-up requirements of the clinical trial.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, some of our clinical trial sites may also be used by some of our competitors, which may reduce the number of patients who are available for our clinical trials in that clinical trial site.

Moreover, because our product candidates represent a departure from more commonly used methods for autoimmune diseases where B cells may play a role in initiating or maintaining disease treatment, potential patients and their doctors may be inclined to use conventional therapies, such as corticosteroids or systemic immunosuppressive medications, rather than enroll patients in our clinical trial. Delays in patient enrollment may result in increased costs or may affect the timing or outcome of our ongoing and planned clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

Our DesCAARTes™ trial, our MusCAARTes™ trial, our RESET™ trials in SLE, myositis, SSc, gMG, PV and MS and any additional expected clinical trials for each of our product candidates will enroll a limited number of patients. The activity and toxicity data from these clinical trials of our product candidates may differ from future results of subsequent clinical trials that enroll a larger number of patients. Since the number of patients that we plan to dose in our MusCAARTes™ trial, our RESET™ trials in SLE, myositis, SSc, gMG, PV and MS is small, and the number of patients in clinical trials for any future product candidates may be small, the results from such clinical trials, once completed, may be less reliable than results achieved in larger clinical trials, which may hinder our efforts to obtain regulatory approval for our product candidates. In our RESET-PV™ trial, RESET-MS™ and our MusCAARTes™ trial, we plan to evaluate the toxicity profile of our product candidates and establish the recommended dose for the next clinical trial. The preliminary results of clinical trials with smaller sample sizes, such as our DesCAARTes™ trial, our MusCAARTes™ trial and our RESET™ trials, as well as any clinical trials for future product candidates, can be disproportionately influenced by various biases associated with the conduct of small clinical trials, such as the potential failure of the smaller sample size to accurately depict the features of the broader patient population, which limits the ability to generalize the results across a broader community, thus making the clinical trial results less reliable than clinical trials with a larger number of patients. As a result, there may be less certainty that such product candidates would achieve a statistically significant effect in any future clinical trials. If we conduct any future clinical trials of DSG3-CAART, MuSK-CAART, or rese-cel, we may not achieve a statistically significant result or the same level of statistical significance, if any, that we might have anticipated based on the results observed in our DesCAARTes™ trial, our MusCAARTes™ trial, and RESET™ trials, respectively.

#### Risks Related to Sales, Marketing and Competition

***The market opportunities for our product candidates may be limited to those patients who are ineligible for or have failed prior treatments and may be small.***

Our projections of both the number of people who have autoimmune diseases where B cells may play a role in initiating or maintaining disease we are targeting, as well as the subset of people with these diseases in a position to receive second or later lines of therapy and who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these autoimmune diseases where B cells may play a role in initiating or maintaining disease. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for our product candidates may be limited or may not be amenable to treatment with our product candidates.

***We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.***

The biopharmaceutical and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong focus on intellectual property. We face competition from many different players, including large and specialty pharmaceutical and biotechnology companies, academic research organizations and governmental agencies. Any therapeutic candidates we successfully develop and commercialize will compete with the existing standard of care as well as novel therapies that may gain regulatory approval in the future. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Although we are the first and only company developing CAAR T drug candidates, we are one of several developing CAR T drug candidates for the treatment of autoimmune diseases where B cells may play a role in initiating or maintaining disease. Additionally, despite the significant differences in discovery, development and target populations between oncology and autoimmune targets, we recognize that companies with an investment and expertise in CAR T cell development for oncology indications could also attempt to leverage their expertise into autoimmune diseases where B cells may play a role in initiating or maintaining disease affected populations. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products.

Specifically, while rituximab is the first drug to have received regulatory approval in the United States in over 60 years for the treatment of PV, the target indication for DSG3-CAART, and the indication we are evaluating in the RESET-PV™ study, we are aware that multiple biopharmaceutical companies have therapies in clinical development. We are also aware of other biopharmaceutical companies developing therapies for MuSK MG, SLE, myositis, SSc, gMG and MS. While we do not expect these product candidates to be directly competitive to our product candidates, even if we obtain regulatory approval of our product candidates, the availability and price of these other products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances.

***Even if we obtain regulatory approval of our product candidates, the products may not gain the market acceptance among physicians, patients, hospitals, treatment centers and others in the medical community necessary for commercial success.***

The use of engineered T cells as a potential treatment for B cell-mediated autoimmune diseases is a recent development and may not become broadly accepted by physicians, patients, hospitals, treatment centers and others in the medical community. We expect physicians to be particularly influential and we may not be able to convince them to use our product candidates for many reasons. Additional factors will influence whether our product candidates are accepted in the market, including:

- the clinical indications for which our product candidates are licensed;
- physicians, hospitals, treatment centers and patients considering our product candidates as a safe and effective treatment;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;
- the timing of market introduction of our product candidates as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and pricing by third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage and adequate reimbursement by third-party payors and government authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies; and
- the effectiveness of our sales and marketing efforts.

The product candidates we plan to develop and commercialize are premised on offering a potential cure for autoimmune diseases where B cells may play a role in initiating or maintaining disease, which may result in a high degree of uncertainty related to pricing

and long-term demand for our product. Our target patient populations are relatively small. Because of this pricing and demand for our product candidates, if licensed, may not be adequate to support an extended period of commercial viability, which could adversely affect our continued ability to successfully produce and market our product or any follow-on products.

In addition, if our product candidates are licensed but fail to achieve market acceptance among physicians, patients, hospitals, treatment centers or others in the medical community, we will not be able to generate significant revenue. Even if our products achieve market acceptance, we may not be able to maintain that market acceptance over time if new products or technologies are introduced that are more favorably received than our products, are more cost effective or render our products obsolete.

#### Risks Related to Business Development

***We may not be successful in our efforts to identify additional product candidates. Due to our limited resources and access to capital, we must prioritize development of certain product candidates, which may prove to be wrong and may adversely affect our business.***

Although we intend to explore other therapeutic opportunities, in addition to the product candidates that we are currently developing, we may fail to identify viable new product candidates for clinical development for a number of reasons. If we fail to identify additional potential product candidates, our business could be materially harmed.

Research programs to pursue the development of our existing and planned product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not they are ultimately successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may be identified but may not be able to be expressed on T cells in a manner that enables product activity;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective drugs; or
- it may take greater human and financial resources than we will possess to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, thereby limiting our ability to develop, diversify and expand our product portfolio.

Because we have limited financial and human resources, we intend to initially focus on research programs and product candidates for a limited set of indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, which could materially adversely affect our future growth and prospects. We may focus our efforts and resources on potential product candidates or other potential programs that ultimately prove to be unsuccessful.

***If we fail to develop additional product candidates, our commercial opportunity will be limited.***

One of our core strategies is to pursue clinical development of additional product candidates beyond rese-cel, DSG3-CAART and MuSK-CAART. Developing, obtaining regulatory approval and commercializing additional product candidates will require substantial additional funding and is prone to the risks of failure inherent in medical product development. We cannot provide you any assurance that we will be able to successfully advance any of these additional product candidates through the development process.

Even if we receive FDA or other foreign regulatory authority approval to market additional product candidates for the treatment of autoimmune diseases where B cells may play a role in initiating or maintaining disease, we cannot assure you that any such product candidates will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. If we are unable to successfully develop and commercialize additional product candidates, our commercial opportunity will be limited. Moreover, a failure in obtaining regulatory approval of additional product candidates may have a negative effect on the approval process of any other, or result in losing approval of any approved, product candidate.

***We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.***

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific, and medical personnel, including our Chief Executive Officer and President, our Scientific Advisory Board members, our President, Science and Technology, our Chief Medical Officer, and our Chief Financial Officer. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key person” insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

***We expect to grow the size of our organization, and we may experience difficulties in managing this growth.***

As of December 31, 2024, we had 160 full-time employees and two part-time employees. As our development and commercialization plans and strategies develop, and as we continue to broaden our operational capabilities, we expect to expand our employee base and continue to add managerial, operational, sales, research and development, marketing, financial and other personnel. Current and future growth imposes significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining and motivating additional employees in an increasingly competitive, inflationary market;
- managing our internal development efforts effectively, including the clinical and FDA or other regulatory authority review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize our product candidates will depend, in part, on our ability to effectively manage our growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including certain research and development as well as general and administrative support, pursuant to agreements which expire after a certain period of time. There can be no assurance that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, or if we are not able to raise sufficient funds in the future to support our hiring efforts beyond our research and development personnel, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

***Business disruptions, including due to natural disasters, global conflicts or political unrest, could seriously impact our operations, research and trials and harm our future revenue and financial condition.***

Our operations, Penn’s operations, WuXi’s operations and those of any CMOs, CROs and other contractors and consultants that we may engage could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for

which we are predominantly self-insured. Further, global conflicts or political unrest, such as the ongoing military conflict between Russia and Ukraine, the Israel-Hamas war, and the conflict in the Middle East may disrupt our global clinical trials and increase the likelihood of supply interruptions. Additionally, the effect of global financial and economic conditions and geopolitical events, including the presidential transition in the United States, events or changes related thereto or political unrest or otherwise, or similar events, may have an impact on our business. The occurrence of any of these business disruptions could seriously harm our research, clinical trials, operations and financial condition and increase our costs and expenses. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

There are also current geopolitical tensions with China. Recently, the Biden administration signed multiple executive orders regarding China. One particular executive order titled Advancing Biotechnology and Biomanufacturing Innovation for a Sustainable, Safe, and Secure American Bioeconomy signed on September 12, 2022 will likely impact the pharmaceutical industry to encourage U.S. domestic manufacturing of pharmaceutical products. Moreover, there have been Congressional legislative proposals, such as the bill titled the BIOSECURE Act, which would, among other things, prohibit U.S. federal funding in connection with biotechnology equipment or services produced or provided by certain named Chinese “biotechnology companies of concern” (which, under the version of legislation passed by the U.S. House of Representatives on September 9, 2024, are WuXi AppTec, WuXi Biologics, MGI, BGI, and Complete Genomics) and loans and grants to, and federal contracts with any entity that uses biotechnology equipment or services from one of these entities in performance of the government contract, grant, or loan. The legislation also gives the federal government the authority to name additional “biotechnology companies of concern” that are engaged in research activities with the Chinese government and that pose a risk of U.S. national security. Previously, on May 15, 2024, the House Committee on Oversight and Accountability approved an updated version of the BIOSECURE Act which would delay the application of the BIOSECURE Act’s provisions (1) until January 1, 2032, with respect to biotechnology equipment and services provided or produced by a named biotechnology company of concern under a contract or agreement entered before the effective date of the legislation and (2) for a period of 5 years after the identification of new biotechnology companies of concern, with respect to biotechnology equipment and services provided or produced by an entity that the government identifies in the future as a biotechnology company of concern. On December 23, 2024, WuXi AppTec, parent company of WuXi, announced that it entered into a definitive agreement to sell the US and UK based operations of WuXi to Altaris, LLC, a healthcare investment firm headquartered in New York, and expected the transaction to close in the first half of 2025. On February 28, 2024, President Biden signed Executive Order 14117 (“Preventing Access to Americans’ Bulk Sensitive Personal Data and United States Government-Related Data by Countries of Concern”) which implements a new framework to protect the privacy of personal data shared between the U.S. and Europe, which may, in effect, impact privacy laws with “countries of concern” such as China or Russia.

In addition, due to our adoption of a more flexible work model following the COVID-19 pandemic, our increased prevalence of personnel working from home may negatively impact productivity, or disrupt, delay, or otherwise adversely impact our business operations. Further, this could increase our cyber security risk, create data accessibility concerns, and make us more susceptible to communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, research or clinical trial sites and other important agencies and contractors.

#### **Risks Related to Our Financial Condition and Capital Requirements**

##### Risks Related to Past Financial Condition

***We have incurred net losses in every period since our inception and anticipate that we will incur substantial net losses over the next several years, and may never achieve or maintain profitability.***

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products licensed for commercial sale, and we will continue to incur significant research and development and other expenses related to our ongoing operations. Our net losses may fluctuate significantly from quarter to quarter and year to year. We have to date financed our operations primarily through private placements of our preferred stock, the sale of common stock in our initial and secondary public offerings and sales of our common stock from time to time in “at-the-market” offerings.

As a result, we are not profitable and have incurred net losses in each period since our inception. For the years ended December 31, 2024 and 2023, we recorded net losses of \$115.9 million and \$67.7 million, respectively. As of December 31, 2024, we had an accumulated deficit of \$349.1 million. We expect to incur significant losses for the foreseeable future, and we expect these losses to increase substantially if, and as, we:

- continue our research and development efforts and submit additional INDs and/or CTAs for our product candidates;
- conduct preclinical studies and clinical trials for our current and future product candidates;
- further develop our product candidate platform;

- continue to discover and develop additional product candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, scientific manufacturing and commercial personnel;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval, whether through a CMO or through a manufacturing facility that we establish;
- acquire or in-license other product candidates and technologies, including advanced manufacturing and translational capabilities that we will need for the further development and possible commercialization of our product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to support the sales and marketing of any product candidates for which we may obtain marketing approvals; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts, as well as to support our operations as a public company.

To become and remain profitable, we must succeed in developing, and eventually commercializing, a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, discovering additional product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling any products for which we may obtain regulatory approval. We are only in the preliminary stages of most of these activities and have not yet demonstrated our ability to successfully develop any product candidate, obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. We may never be able to develop, manufacture or commercialize a marketable product.

Even if we are able to succeed in these activities, we may never generate revenues that are significant enough to achieve profitability. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Our expenses will increase if, among other things:

- there are any delays in completing our clinical trials or the development of any of our product candidates;
- we are required by the FDA or other regulatory authorities to perform trials or studies in addition to, or different than, those expected; or
- there are any third-party challenges to our intellectual property or we need to defend against any intellectual property-related claim.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses we will incur or when, if ever, we will be able to achieve profitability. Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to develop, seek regulatory approval for and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

***We have a limited operating history, which may make it difficult to evaluate the success of our business to date and to assess our future viability, and we may face significant challenges and expense as we test our product candidates and build our capabilities.***

We were incorporated in 2017 and initially acquired rights to license certain patent rights from Penn in August 2018, and acquired rights to license certain patent rights from Nanjing IASO Biotherapeutics Co., Ltd., or IASO, in October 2022. All of our product candidates are still in the preclinical development or clinical stage. We have not yet demonstrated our ability to successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture clinical and commercial scale therapeutics, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Our ability to generate product revenue or profits, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We may never be able to develop or commercialize a marketable product.

Our limited operating history, particularly in light of the rapidly evolving cell therapy field, may make it difficult to evaluate our current business and predict our future performance. Our relatively short history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by clinical-stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer. Similarly, we expect that our financial condition and operating results will continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. As a result, our shareholders should not rely upon the results of any quarterly or annual period as an indicator of future operating performance.

We currently do not have in-house resources sufficient to enable the development of our product candidates, including our CAR T and CAAR T cell platforms. We are reliant on several manufacturing and support services from Penn through two Master Translational Research Service Agreements, or the CAART Services Agreement and CARTA Services Agreement, respectively, and collectively, the Services Agreements. We also rely on Penn for current manufacturing of rese-cel. Our ability to rely on services from Penn is limited to a specified period of time, to specific capabilities, and is subject to Penn's right to terminate these services with or without cause. We are reliant on WuXi manufacturing services for MuSK-CAART and rese-cel in multiple indications through a Development, Manufacturing and Testing Services Agreement, or the WuXi Agreement. Our ability to rely on services from WuXi is limited to a specified period of time, to specific capabilities, and is subject to WuXi's right to terminate these services with or without cause. If we are unable to establish necessary relationships with third party partners and/or build our own capabilities, our operating and financial results could differ materially from our expectations, and our business could suffer. As we build our own capabilities, and enter into agreements with third parties, we expect to encounter risks and uncertainties frequently experienced by growing companies in new and rapidly evolving fields, including the risks and uncertainties described herein.

All of our programs require additional preclinical research and development, clinical development, regulatory approval in multiple jurisdictions, obtaining manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. Other programs of ours require additional discovery research and then preclinical and clinical development. In addition, our product candidates must be licensed for marketing by the FDA or other foreign regulatory authorities before we may commercialize any product.

***We have not generated any revenue from our product candidates and our ability to generate revenue from product sales and become profitable depends significantly on our success in a number of areas.***

To become and remain profitable, we or any potential future collaborator must develop and eventually commercialize products with significant market potential at an adequate profit margin after cost of goods sold and other expenses. All of our product candidates are in the early stages of development and we will require additional preclinical studies, clinical development, regulatory review and approval, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. Our ability to generate revenue depends on a number of factors, including, but not limited to:

- timely completion of our preclinical studies and clinical trials, which may be significantly slower or cost more than we currently anticipate and will depend substantially upon the performance of third-party academic and commercial contractors;
- our ability to complete IND-enabling studies and successfully submit INDs or comparable applications;
- whether we are required by the FDA or other foreign regulatory authorities to conduct additional clinical trials or other studies beyond those planned to support the licensure and commercialization of our product candidates or any future product candidates;
- our ability to demonstrate to the satisfaction of the FDA or other foreign regulatory authorities, the safety, potency, purity and acceptable risk to benefit profile of our product candidates or any future product candidates;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates or future product candidates, if any;
- the cost of manufacturing and processing our product candidates being greater than we anticipate;
- the timely receipt of necessary marketing approvals from the FDA or other foreign regulatory authorities;
- the willingness of physicians, operators of clinics and patients to utilize or adopt any of our product candidates or future product candidates to treat autoimmune diseases where B cells may play a role in initiating or maintaining disease;
- our ability and the ability of third parties with whom we contract to manufacture adequate clinical and commercial supplies of our product candidates or any future product candidates, remain in good standing with regulatory authorities and develop, validate and maintain commercially viable manufacturing processes that are compliant with FDA's current Good Manufacturing Practices, or cGMP;

- our ability to successfully develop a commercial and competitive strategy and thereafter commercialize our product candidates or any future product candidates in the United States or other countries, if licensed for marketing, reimbursement, sale and distribution, whether alone or in collaboration with others;
- patient demand for our product candidates and any future product candidates, if licensed; and
- our ability to establish and enforce intellectual property rights in and to our product candidates or any future product candidates.

Many of the factors listed above are beyond our control and could cause us to experience significant delays or prevent us from obtaining regulatory approvals or commercialize our product candidates. Even if we are able to commercialize our product candidates, we may not achieve profitability soon after generating product sales, if ever. If we are unable to generate sufficient revenue through the sale of our product candidates or any future product candidates, we may be unable to continue operations without continued funding.

If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Additionally, even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to research, develop and market additional product candidates. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

#### Risks Related to Future Financial Condition

***We will require substantial additional financing to develop and commercialize our product candidates and implement our operating plans. We have also identified conditions that raise substantial doubt about our ability to continue as a going concern. If we fail to obtain additional financing or cannot obtain financing at the levels we require, we may be forced to delay, reduce or discontinue our plans and operations or unable to complete the development and commercialization of our product candidates.***

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the preclinical and clinical development of our product candidates, including our MusCAARTes™ trial and our RESET™ trials, and our research and development, preclinical studies and clinical trials for any future product candidates, to seek regulatory approvals for our product candidates, to enable commercial production of our products, if licensed, and to initiate and complete registration trials for multiple products. As of December 31, 2024, we had \$164.0 million of cash and cash equivalents. Since our initial public offering, we have generated cash from public offerings of our common stock and pre-funded warrants to purchase our common stock resulting in aggregate net proceeds of approximately \$280.0 million. While we currently expect our existing cash and cash equivalents to be sufficient to fund our operations into the first half of 2026 which includes initial clinical data on efficacy endpoints and tolerability from the initial rese-cel treated patients in the RESET™ clinical trials, we expect to require significant additional financing to complete these clinical trials and any future clinical trials of these and our other product candidates. Further, if marketing approval is received, we will require significant additional amounts of cash to launch and commercialize our product candidates. However, we have based this estimate on assumptions that may prove to be wrong. Additionally, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may require substantial additional capital for the further development and commercialization of our product candidates, including funding our internal manufacturing capabilities, and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate. Because the length of time and activities associated with development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near- and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates that we may develop or in-license;



- the terms of any collaboration agreements we may choose to conclude;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA or other foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the effect of competing technological and market developments;
- the costs of establishing and maintaining a supply chain for the development and manufacture of our product candidates;
- the cost and timing of establishing, expanding and scaling manufacturing capabilities;
- the cost of maintaining the amount patient data for which we would be responsible following commercialization of one or more of our product candidates; and
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own.

We cannot be certain that additional funding will be available on acceptable terms, or at all. As widely reported, global credit and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, inflation, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Until we are able to generate sufficient revenue to finance our cash requirements, we will need to finance our future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue our research and development initiatives and clinical development plans. We could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Based on our current operating plan, we believe there is substantial doubt about our ability to continue as a going concern for at least twelve months following the filing of this Annual Report on Form 10-K, and we will need to obtain additional funding. We may be forced to delay or reduce the scope of our product development programs, reduce our research and development costs and/or limit or cease our operations if we are unable to obtain additional funding to support our current operating plan. Our cash forecast contains estimates and assumptions based on success of our ongoing clinical trials, and management cannot predict the amount or timing of all expenditures with certainty. Nevertheless, our financial statements do not include any adjustments that might result from the outcome of this uncertainty. We will need to raise additional capital to fund our future operations and remain as a going concern. However, we cannot guarantee that we will be able to obtain any or sufficient additional funding or that such funding, if available, will be obtainable on terms satisfactory to us.

***Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.***

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

Pursuant to our equity incentive plans, our management is authorized to grant stock options to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under the 2019 Stock Option and Incentive Plan, or the 2019 Plan, automatically increased on January 1, 2025 and will automatically increase each January 1 thereafter through and including January 1, 2029, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall. In addition, on April 7, 2023, our board of directors adopted, and at our 2023 annual meeting our stockholders approved, an amendment to the 2019 Plan, or the Plan Amendment, to increase the aggregate number of shares authorized for issuance under the 2019 Plan by 3,000,000 shares, subject to adjustment. Our compensation committee determined the size of the increase to the reserved

pool under the Plan Amendment based on projected equity awards to anticipated new hires, projected annual equity awards to existing employees and an assessment of the magnitude of increase that our institutional investors and the firms that advise them would likely find acceptable. We anticipate that the increased share reserve under our 2019 Plan, as amended by the Plan Amendment, will be sufficient to provide equity incentives to attract, retain, and motivate employees for a period of two years following the effective date of the Plan Amendment.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

#### **Risks Related to Our Intellectual Property**

***We rely heavily on certain in-licensed patent and other intellectual property rights in connection with our development of our product candidates and, if we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business.***

Our ability to develop and commercialize our product candidates is heavily dependent on in-licenses to patent rights and other intellectual property granted to us by third parties. For example, we depend heavily on our License Agreement with Penn and CHOP, which was entered into in 2018, amended and restated in July 2019, and further amended in May 2020 and October 2021, pursuant to which we obtained (a) a non-exclusive, non-sublicensable, worldwide research license to intellectual property controlled by Penn and CHOP to make, have made and use products in two subfields of use, (b) effective as of October 2018, an exclusive, worldwide, royalty-bearing license, with the right to sublicense, under certain of such intellectual property to make, use, sell, offer for sale and import products in the same two subfields of use, and (c) effective as of October 2018, a non-exclusive, worldwide, royalty-bearing license, with limited rights to sublicense, under certain of Penn's know-how, which know-how satisfies certain criteria and is listed on a mutually agreed to schedule, to make, have made, use, sell, offer for sale, import and have imported products in the same two subfields of use. We also depend on our Exclusive License Agreement with IASO, which was entered into in October 2022, pursuant to which we obtained a worldwide, exclusive license under certain intellectual property to develop, manufacture, commercialize and otherwise exploit T cell products directed to CD19 for the purpose of diagnosis, prevention or treatment of an autoimmune or alloimmune indication in humans, or the IASO Agreement. We may enter into additional license agreements in the future. Our license agreements with Penn, CHOP and IASO impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under these licenses, our licensors, including Penn, CHOP and IASO may have the right to terminate these license agreements, in which event we might not be able to market our product candidates. Termination of any of our license agreements or reduction or elimination of our licensed rights may also result in our having to negotiate new or reinstated licenses with less favorable terms.

We may need to obtain additional licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

Furthermore, in many cases, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we in-license from third parties. For example, pursuant to our IASO Agreement, IASO controls such activities for the patent rights licensed to us under such agreement. Pursuant to our License Agreement with Penn and CHOP, Penn controls such activities for the patent rights licensed to us under such agreement. Therefore, although we provide input to IASO, Penn and CHOP on these activities, we cannot be certain that these patents will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our current or future licensors or collaboration partners fail to obtain, maintain or protect any patents or patent applications licensed to us, our rights to such patents and patent applications may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected.

Disputes may arise between us and our current and future licensors regarding intellectual property subject to a license agreement, including those related to:

- the scope of rights granted under the License Agreement or IASO Agreement and other interpretation-related issues;
- whether we have breached the License Agreement or IASO Agreement and whether any such breach is subject to a cure period;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;

- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

Furthermore, disputes may arise between us and our current or future licensors regarding the ownership of intellectual property developed by us, such that we may be required to assign or otherwise transfer such intellectual property to such licensor. In the event that the assigned or transferred intellectual property is covered by an existing license agreement with such licensor we may be required to make additional royalty or milestone payments, or both, to such licensor. If the assigned or transferred intellectual property is not covered by an existing license agreement, then we may be required to enter into an additional license agreement to advance our research or allow commercialization of our product candidates, which may not be available on commercially reasonable terms or at all.

If disputes over intellectual property that we have licensed, or license in the future, prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

***If our efforts to protect the proprietary nature of the intellectual property related to our current and any future product candidates are not adequate, we may not be able to compete effectively in our market.***

Our success depends in large part on our ability to obtain and maintain intellectual property protection in the United States and other countries with respect to our product candidates. If we do not adequately protect or enforce our intellectual property rights, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we have filed and in-licensed patent rights in the United States and abroad relating to the product candidates that are important to our business. The patent application and approval process is expensive, complex and time-consuming. We or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to biological and pharmaceutical products commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in the patents or pending patent applications, or that we and our licensors were the first to file for patent protection of such inventions.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, the patents or pending patent applications that we own or in-license may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in post-grant review procedures, derivation proceedings, reexaminations, or *inter partes* review in the United States, or oppositions and other comparable proceedings in foreign jurisdictions, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of the patents we in-license or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law is more restrictive than U.S. patent law in connection with the patentability of methods of treatment of the human body and Chinese bankruptcy law may not provide a licensee the same protections as U.S. bankruptcy law. This could impact our in-license under the IASO Agreement with IASO, a China-based company, if IASO declared bankruptcy, and could have a material adverse effect on the development of rese-cel.

A European Unified Patent Court, or the UPC, came into force during 2023. The UPC is a common patent court to hear patent infringement and revocation proceedings effective for member states of the European Union. This could enable third parties to seek revocation of our European patents in a single proceeding at the UPC rather than through multiple proceedings in each of the jurisdictions in which the European patent is validated. Any such revocation and loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and products. Moreover, the controlling laws and regulations of the UPC will develop over time, and may adversely affect our ability to enforce or defend the validity of our European patents. Although we have decided, and may continue to decide, to opt out certain of our European patents and patent applications from the UPC, if certain formalities and requirements are not met, then our European patents and patent applications could be challenged for non-compliance and brought under the jurisdiction of the UPC. Thus, we cannot be certain that our European patents and patent applications will avoid falling under the jurisdiction of the UPC.

We cannot predict whether the patent applications that we own or in-license will issue as patents, whether the claims of any patent that has or may issue will provide us with a competitive advantage or prevent competitors from designing around the claims to develop competing technologies in a non-infringing manner, or whether we or our licensors will be able to successfully pursue patent applications in the future relating to our current product candidates or future products and product candidates. Moreover, the patent application and approval process is expensive and time-consuming. We or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Furthermore, we, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to seek additional patent protection.

It is possible that defects of form in the preparation or filing of patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If there are material defects in the form, preparation, prosecution or enforcement of the patents or patent applications we in-license, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

Even if the patent applications we own or in-license issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patent rights by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our product candidates. Alternatively, our competitors may seek to market generic versions of any approved products by submitting abbreviated BLAs to the FDA during which process they may claim that our owned or in-licensed patents are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our intellectual property rights, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find the patents we own or in-license invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we own or in-license valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In the future, we likely will need to expand our patent portfolio to pursue patent coverage for new product candidates that we wish to develop. The patent prosecution process is competitive, and other companies, some which may have greater resources than we do in this area, may also be pursuing intellectual property rights that we may consider necessary or attractive in order to develop and commercialize future product candidates.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting, maintaining, defending and enforcing patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although our License Agreement and IASO Agreement grant us worldwide rights, there can be no assurance that we will obtain or maintain patent rights in or outside the United States under any future license agreements. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States even in jurisdictions where we and our licensors pursue patent protection. Consequently, we and our licensors may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we and our licensors pursue patent protection, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we and our licensors have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates and the patents we in-license or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of the patents we in-license or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights, even if obtained, in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put the patents we in-license at risk of being invalidated or interpreted narrowly and the patent applications we own or in-license at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

***We or our licensors may be subject to claims challenging the inventorship or ownership of the patents and other intellectual property that we own or license.***

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an ownership interest in the patents and intellectual property that we own or in-license or that we may own or in-license in the future. While it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own or such assignments may not be self-executing or may be breached. Our licensors may face similar obstacles. We or our licensors could be subject to ownership disputes arising, for example, from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against any claims challenging inventorship or ownership. If we or our licensors fail in defending any such claims, we may have to pay monetary damages and may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property, which could adversely impact our business, results of operations and financial condition.

***Some intellectual property which we have in-licensed was discovered through government funded programs and thus is subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers.***

Certain of the intellectual property rights we have licensed, including rights licensed to us by Penn relating to our DSG3-CAART and DSG3/1-CAART product candidates, was generated through the use of U.S. government funding and may therefore be subject to certain federal laws and regulations. As a result, the U.S. government has certain rights to intellectual property embodied in our DSG3-CAART and DSG3/1-CAART product candidates and may have rights in future product candidates pursuant to the Bayh-Dole Act of 1980. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations, also referred to as "march-in rights". The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, such as Penn, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for product candidates covered by such intellectual property.

***We may become involved in lawsuits to protect or enforce our patent rights or other intellectual property rights, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe, misappropriate or otherwise violate patents, trademarks, copyrights or other intellectual property that we own or in-license. To counter infringement, misappropriation or other unauthorized use, we may be required to file claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived violators could provoke these parties to assert counterclaims against us alleging that we infringe, misappropriate or otherwise violate their intellectual property, in addition to counterclaims asserting that the patents we in-license are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent we own or in-license is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue.

In the U.S., grounds for a validity challenge in a court proceeding could be an alleged failure to meet one or more statutory requirements for patentability, including, for example, lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Additionally, third parties are able to challenge the validity of issued patents through administrative proceedings in the patent offices of certain countries, including the USPTO and the European Patent Office.

Even if the validity of a patent is upheld during a court proceeding, there is a risk that the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that the patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving the patents we own or in-license could limit our ability to assert the patent against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition.

Even if we establish infringement, misappropriation or another violation of our intellectual property rights, the court may decide not to grant an injunction against the offender and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required to file and pursue such claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

***Changes in patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.***

Changes in either the patent laws or the interpretation of the patent laws in the United States or other jurisdictions could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. When implemented, the Leahy-Smith Act included several significant changes to U.S. patent law that impacted how patent rights could be prosecuted, enforced and defended. In particular, the Leahy-Smith Act also included provisions that switched the United States from a "first-to-invent" system to a "first-to-file" system, allowed third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO developed new regulations and procedures governing the administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, became effective on March 16, 2013. It remains unclear what impact, if any, the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of the patent applications we in-license and the enforcement or defense of the issued patents we in-license, all of which could have a material adverse effect on our business.

The patent positions of companies engaged in the development and commercialization of biologics are particularly uncertain. For example, the Supreme Court of the United States issued its decision in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, or *Myriad*, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent-eligible. Thereafter, the USPTO issued a guidance memorandum instructing USPTO examiners on the ramifications of the Prometheus and Myriad rulings and apply the Myriad ruling to natural products and principles including all naturally occurring nucleic acids. Certain claims of our owned and in-licensed patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties.

We cannot assure you that our efforts to seek patent protection for one or more of our product candidates will not be negatively impacted by this Supreme Court decision, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court's decisions in Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have a material adverse effect on our existing patent rights and our ability to protect and enforce our intellectual property in the future.

***If we are unable to protect the confidentiality of trade secrets, our business and competitive position would be harmed.***

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect certain proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce, and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors. Competitors and other third parties could infringe, misappropriate or otherwise violate our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

***Patent term may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.***

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, a patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of the product's approval by the FDA, only one patent applicable to an approved drug is eligible for the extension, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. In the future, if and when our product candidates receive FDA approval, we plan to apply for patent term extensions on patents covering those product candidates in any jurisdiction where these are available. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to the patents we own or in-license, or may grant more limited extensions than we request. Moreover, we may not receive an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

***We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.***

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel.

Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management. Our licensors may face similar risks, which could have an adverse impact on intellectual property that is licensed to us.

***We may become subject to claims that we are infringing certain third-party patents or other third-party intellectual property rights, any of which may prevent or delay our development and commercialization efforts and have a material adverse effect on our business.***

Our commercial success depends in part on avoiding infringing, misappropriating and otherwise violating the patents and other intellectual property and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, and administrative proceedings such as interferences, *inter partes* review and post grant review proceedings before the USPTO and opposition proceedings before foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned or controlled by third parties, including our competitors, exist in the fields in which we are pursuing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we or our licensors are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, methods of manufacture or methods for treatment relating to our product candidates and, because patent applications can take many years to issue, there may be currently pending third party patent applications which may later result in issued patents, in each case that our product candidates, their manufacture or use may infringe or be alleged to infringe. We may fail to identify potentially relevant patents or patent applications, incorrectly conclude that a patent is invalid or does not cover our activities, or incorrectly conclude that a patent application is unlikely to issue in a form of relevance to our activities.

Parties making patent infringement claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, including demonstrating non-infringement, invalidity or unenforceability of the respective patent rights in question, regardless of their merit, is time-consuming, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. For example, in order to successfully challenge the validity of any U.S. patent in federal court, we would need to overcome a presumption of validity. This is a high burden requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, and we can provide no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. We may not have sufficient resources to bring these actions to a successful conclusion. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our shares.

In the event that a holder of any such patents seeks to enforce its patent rights against us with respect to one or more of our product candidates, and our defenses against the infringement of such patent rights are unsuccessful, we may be precluded from commercializing our product candidates, even if approved, without first obtaining a license to some or all of these patents, which may not be available on commercially reasonable terms or at all. Moreover, we may be required to pay significant fees and royalties to secure a license to the applicable patents. Such a license may only be non-exclusive, in which case our ability to stop others from using or commercializing technology and products similar or identical to ours may be limited. Furthermore, we could be liable for damages to the holder of these patents, which may be significant and could include treble damages if we are found to have willfully infringed such patents. In the event that a challenge to these patents were to be unsuccessful or we were to become subject to litigation or unable to obtain a license on commercially reasonable terms with respect to these patents, it could harm our business, financial condition, results of operations and prospects.

We are aware of third-party U.S. patents relating to the lentiviral vectors which may be used in the manufacture or use of our product candidates. We are also aware of a patent family that includes granted U.S. and European patent rights directed to methods using, and compositions containing, genetically engineered T cells that may be relevant to our rese-cel product candidate. If these patent rights were enforced against us, we believe that we have defenses against any such action, including that these patents would not be infringed by our product candidates and/or that these patents are not valid. However, if these patents were enforced against us and defenses to such enforcement were unsuccessful, unless we obtain a license to these patents, which may not be available on commercially reasonable terms, or at all, we could be liable for damages and precluded from commercializing any product candidates that were ultimately held to infringe these patents, which could have a material adverse effect on our business, financial condition, results of operations and prospects. Furthermore, even if our arguments are successful, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results.

Even in the absence of a finding of infringement, we may need or may choose to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, or at all. In that event, we would be unable to further develop and commercialize our product candidates. Claims that



we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Any of the foregoing could materially adversely affect our business, results of operations and financial condition.

***Intellectual property rights do not necessarily address all potential threats.***

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to our product candidates or utilize similar cell therapy technology but that are not covered by the claims of our current or future patent portfolio;
- we, or our current or future licensors or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license now or that we may license or own in the future;
- we, or our current or future licensors or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our licensed intellectual property rights;
- it is possible that our current or future licensed patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors or other third parties;
- our competitors or other third parties might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business;
- we may choose not to file a patent application in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent application covering such intellectual property; and
- third-party patents may issue with claims covering our activities; we may have infringement liability exposure arising from such patents.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects.

**Risks Related to Our Reliance on Third Parties**

***We currently, and will likely continue to, rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or commercialize our product candidates.***

We depend and will continue to depend upon third parties, including independent investigators and collaborators, such as universities, medical institutions, CROs and strategic partners, to conduct our preclinical studies and clinical trials under agreements with us. Specifically, we depend on clinical trial sites to enroll patients and conduct the DesCAARTes™ trial, MusCAARTes™ trial and RESET™ trials in a timely and appropriate manner. If our clinical trial sites do not conduct the trials on the timeline we expect or otherwise fail to support the trials, our clinical trial results could be significantly delayed, thereby adversely impacting our leadership position in the autoimmune cell therapy space and our ability to progress additional product candidates. As we open additional clinical trial sites, we expect to have to negotiate budgets and contracts with CROs and study sites, which may result in delays to our development timelines and increased costs.

We will rely heavily on these third parties, including Penn and WuXi, to conduct our manufacturing, and as a result, will have limited control over pace at which these activities are carried out. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with FDA's GCPs which are regulations and guidelines enforced by the FDA for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to

perform additional clinical trials before approving our marketing applications. We cannot provide assurance that, upon inspection, such regulatory authorities will not determine that some or all of our clinical trials do not fully comply with the GCP requirements. For any violations of laws and regulations during the conduct of our clinical trials, we could be subject to untitled and warning letters or enforcement action that may include civil penalties up to and including criminal prosecution. In addition, our clinical trials must be conducted with biologic product produced under cGMPs and will require a large number of test patients. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

As widely reported, global credit and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. Regional or single-source dependencies may in some cases accentuate these risks. For example, the pharmaceutical industry in general, and in some instances we or our collaborators or other third parties on which we or they rely, depend on China-based suppliers or service providers for certain raw materials, products and services, or other activities. Our ability or the ability of our collaborators or such other third parties to continue to engage these China-based suppliers or service providers for certain preclinical research programs and clinical development programs could be restricted due to geopolitical developments between the United States and China, including as a result of the escalation of tariffs. In the event that one or more of our current or future service providers, manufacturers and other partners do not successfully carry out their contractual duties, meet expected deadlines, or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, due to the economic downturn, the enactment of legislative proposals or for any other reasons, then we may not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines. Our failure or the failure of these third parties to comply with applicable regulatory requirements or our stated protocols could also subject us to enforcement action. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

We currently rely on certain foreign or foreign-owned third-party vendors, including WuXi, to manufacture certain clinical materials or to provide services in connection with certain clinical trials. Such foreign and foreign-owned vendors may be subject to U.S. legislation or investigations, including legislation similar to the previously proposed BIOSECURE Act, sanctions, trade restrictions and other foreign regulatory requirements, which could increase the cost or reduce the supply of material available to us, delay the procurement or supply of such material, delay or impact clinical trials, have an adverse effect on our ability to secure significant commitments from governments to purchase our potential therapies and could adversely affect our financial condition and business prospects. In connection with WuXi, on December 23, 2024, WuXi AppTec, parent company of WuXi, announced that it entered into a definitive agreement to sell the US and UK based operations of WuXi to Altaris, LLC, a healthcare investment firm headquartered in New York, and expects that the transaction to close in the first half of 2025.

Any third parties conducting our clinical trials will not be our employees and, except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies or other drug development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

If any of our relationships with trial sites, or any CRO that we may use in the future, terminates, we may not be able to enter into arrangements with alternative trial sites or CROs or do so on commercially reasonable terms. Switching or adding third parties to conduct our clinical trials involves substantial cost and requires extensive management time and focus. In addition, there is often a natural transition period when a new third party commences work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any product candidates we may develop or commercialization of our medicines, producing additional losses and depriving us of potential product revenue.

***We intend to rely on third parties to manufacture our clinical product supplies, and we may have to rely on third parties to produce and process our product candidates, if licensed.***

Although we may eventually secure our own clinical manufacturing facility for any late phase clinical development that we undertake, we currently rely on third parties, including Penn and WuXi, to manufacture our product candidates, and we intend in the future to continue to rely on CMOs. In the case of any manufacturing performed for us by third parties, the services performed for us risk being delayed because of the competing priorities that such parties have for utilization of their manufacturing resources and any capacity issues that thereby arise.

We do not yet have sufficient information to reliably estimate the cost of the manufacturing and processing of our product candidates in clinical quantity or commercial quantity, and the actual cost to manufacture and process our product candidates could ultimately materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

In addition, our anticipated reliance on a limited number of third-party manufacturers exposes us to the following risks:

- We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA may have questions regarding any replacement contractor. This may require new testing and regulatory interactions. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA questions, if any.
- Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- Any contract manufacturers that we engage may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store and distribute our product candidates.
- Manufacturers are subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMP and other government regulations. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our product candidates.
- Our third-party manufacturers could breach or terminate their agreement with us.

Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks related to the production of such materials and products. As a result, failure to meet the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally. If the FDA does not approve these facilities for the manufacture of our product candidates or if any agency withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would negatively impact our ability to develop, obtain regulatory approval for or market our product candidates, if licensed.

Our contract manufacturers would also be subject to the same risks we face in developing our own manufacturing capabilities, as described above. Each of these risks could delay our clinical trials, the approval, if any of our product candidates by the FDA or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenue. In addition, we will rely on third parties to perform release tests on our product candidates prior to delivery to patients. If these tests are not appropriately done and test data are not reliable, patients could be put at risk of serious harm.

For more information, see "Risk Factors—Risks Related to Manufacturing and Supply".

***We may form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.***

We may form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our product candidates and any future product candidates that we may develop. Any of these relationships may require us to incur non-recurring and other charges, increase our near and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for

collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety, potency and purity. Any delays in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the results, revenue or specific net income that justifies such transaction.

***We may not realize the benefits of acquired assets or other strategic transactions, including any transactions whereby we acquire or license manufacturing and other advanced technologies.***

In August 2018, we entered into a License Agreement with Penn and CHOP which was amended and restated in July 2019, and further amended in May 2020 and October 2021, or the License Agreement, pursuant to which we were granted licenses to certain patent rights for the research and development of products, as well as an exclusive license under those same patent rights to make, use, sell and import such products, in the autoimmune disease and alloimmune response subfields, in each case, for the treatment of humans. In January 2021 and as amended in August 2022, we entered into an agreement with WuXi to serve as an additional cell processing manufacturing partner for our MusCAARTes™ trial, and have since completed enabling engineering and patient production runs. In August 2023, as amended in August 2024, we entered into an agreement with WuXi to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel. In October 2022, we entered into the IASO Agreement, pursuant to which we were granted worldwide license under certain intellectual property to develop, manufacture, commercialize and otherwise exploit T cell products directed to CD19 for the purpose of diagnosis, prevention or treatment of an autoimmune or alloimmune indication in humans.

We actively evaluate various strategic transactions on an ongoing basis. We may acquire other businesses, products or technologies as well as pursue joint ventures or investments in complementary businesses. The success of our strategic transactions, including the License Agreement, and any future strategic transactions depends on the risks and uncertainties involved including:

- unanticipated liabilities related to acquired companies or joint ventures;
- difficulties integrating acquired personnel, technologies and operations into our existing business;
- retention of key employees;
- diversion of management time and focus from operating our business to management of strategic alliances or joint ventures or acquisition integration challenges;
- increases in our expenses and reductions in our cash available for operations and other uses;
- disruption in our relationships with collaborators or suppliers as a result of such a transaction; and
- possible write-offs or impairment charges relating to acquired businesses or joint ventures.

If any of these risks or uncertainties occur, we may not realize the anticipated benefit of any acquisition or strategic transaction. Additionally, foreign acquisitions and joint ventures are subject to additional risks, including those related to integration of operations across different cultures and languages, currency risks, potentially adverse tax consequences of overseas operations and the particular economic, political, legal and regulatory risks associated with specific countries. For example, IASO is based in China and we may not receive the same protections under Chinese law, including with respect to applicable bankruptcy, insolvency, liquidation, arrangement, moratorium or similar laws relating to or affecting our rights.

Future acquisitions or dispositions could result in potentially dilutive issuances of our equity securities, the incurrence of debt, contingent liabilities or amortization expenses or write-offs of goodwill, any of which could harm our financial condition.

## Risks Related to Manufacturing and Supply

***We are reliant on Penn and WuXi for our current manufacturing activities and Penn and/or WuXi's failure to perform or termination would disrupt normal business operations, and we intend to continue to rely on other third parties for our future manufacturing needs prior to establishing our own manufacturing facility.***

We are reliant on Penn and WuXi for our current manufacturing activities for our preclinical and clinical research. If Penn and its affiliated entities were to fail to perform their obligations in accordance with the terms of the Services Agreements or terminate the Services Agreements, or if WuXi were to fail to perform their obligations in accordance with the terms of the WuXi Agreement or terminate the WuXi Agreement, we may have difficulty continuing our normal business operations and our business prospects, financial condition and results of operations could be harmed.

There are also current geopolitical tensions with China. These tensions and the related risks are described in the Risk Factor in this Report titled, "Risks Related to Business Development – Business disruptions, including due to natural disasters, global conflicts or political unrest, could seriously impact our operations, research and trials and harm our future revenue and financial condition."

In addition, the CAART Services Agreement is scheduled to expire on the later of October 19, 2021 or completion of all research and development projects, and unless the CAART Services Agreement is amended, Penn will not be obligated to provide any further services under the CAART Services Agreement after that time. We currently anticipate that research and development projects under the CAART Services Agreement will continue through at least 2025. In addition, Penn has the right to terminate the CAART Services Agreement in whole at any time with 90 days' notice and to terminate any research and development project being performed under the CAART Services Agreement if the Penn service provider appointed to lead such project is unavailable and Penn is unavailable to find a replacement within 60 days for such service provider. Penn also has the right to terminate certain manufacturing services being performed under the CAART Services Agreement with 180 days' written notice. From time to time, we may enter into further addenda to the CAART Services Agreement that provide Penn with the right to terminate such addenda with limited notice periods. If we do not have adequate personnel and capabilities at the time that we assume responsibilities for such services, we may not be successful in effectively or efficiently transitioning these services from Penn, which could disrupt our business and have a material adverse effect on our financial condition and results of operations. Even if we are able to successfully transition these services, they may be more expensive or less efficient than the services we are receiving from Penn during the transition period.

The CARTA Services Agreement is scheduled to expire on the later of February 9, 2026 or completion of all research and development projects, and unless the CARTA Services Agreement is amended, Penn will not be obligated to provide any further services under the CARTA Services Agreement after that time. In addition, Penn has the right to terminate the CARTA Services Agreement in whole at any time with 180 days' notice. From time to time, we may enter into further addenda to the CARTA Services Agreement that provide Penn with the right to terminate such addenda with limited notice periods. If we do not have adequate personnel and capabilities at the time that we assume responsibilities for such services, we may not be successful in effectively or efficiently transitioning these services from Penn, which could disrupt our business and have a material adverse effect on our financial condition and results of operations. Even if we are able to successfully transition these services, they may be more expensive or less efficient than the services we are receiving from Penn during the transition period.

The WuXi Agreement is scheduled to expire upon completion of WuXi's services related to MuSK-CAART and rese-cel. In August 2023, as amended in August 2024, we entered into new work orders under the WuXi Agreement for WuXi to serve as one of our cell processing manufacturing partners for the planned global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel. Under the August 2023 work orders, WuXi will convert our non-dedicated suite to a dedicated suite for GMP manufacturing for our rese-cel and MuSK-CAART programs, or the Dedicated Suite, for an initial term of 18 months with two 18 month extensions at our sole option on six months notice prior to the end of the term. In August 2024, we notified WuXi that we would extend the initial term by 18 months through August 2026. We may terminate for convenience with six months prior written notice, however, we may not terminate the Dedicated Suite without terminating both the MuSK-CAART and rese-cel GMP run work orders. In lieu of the existing 18 month termination right for convenience under the WuXi Agreement, WuXi may not terminate prior to February 2028. If WuXi were to fail to perform their obligations in accordance with the terms of the WuXi Agreement or terminate the WuXi Agreement, our clinical trials and commercial readiness may be adversely impacted which could in turn materially and adversely affect our business, results of operations and prospects.

Further, we may not be able to achieve clinical manufacturing and cell processing through our CMOs or on our own on a timely basis. While our current manufacturing process is similar to the well-established process developed at Penn for CD19 CAR-T, or CART19, which was later commercialized, we have limited experience as an organization in managing the CAR-T or CAAR T engineering process at commercial scale. Finally, because clinical manufacturing and cell processing is highly complex and patient donor material is inherently variable, we cannot yet be sure that our manufacturing process, will consistently result in product that meets specifications for release. Success in manufacturing in smaller early phase clinical trials may not predict the frequency of success at larger late phase clinical trials, or success at the commercial phase production until process qualification and validation is completed and submitted for BLA filing.

***Our product candidates are uniquely manufactured. If we or any of our third-party manufacturers encounter difficulties in manufacturing our product candidates, our ability to provide supply of our product candidates for clinical trials or, if licensed, for commercial sale, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.***

The manufacturing process used to produce our product candidates is complex and novel, and it has not yet been validated for commercial production. The manufacture of our product candidates includes harvesting white blood cells from each patient, stimulating certain T cells from the white blood cells and thereby causing them to activate and proliferate, combining patient T cells with lentiviral delivery vector through a process known as transduction, expanding the transduced T cells to obtain the desired dose, formulating and freezing the cell product, and ultimately infusing the modified T cells back into the patient's body. Because of the bespoke nature of this product for patients, the cost to manufacture our product candidates is higher than traditional small molecule chemical compounds and monoclonal antibodies. Furthermore, our manufacturing process development and scale-up is at an early stage, and evaluation of cost at large scale has not yet been finalized. The actual cost to manufacture and process our product candidates could be greater than we expect and could materially and adversely affect the commercial viability of our product candidates.

Our manufacturing process may be susceptible to technical and logistics delays or failures due to the fact that each patient is an independent manufacturing lot, and also due to unique supply chain requirements. These include the collection of white blood cells from patients' blood, variability in the quality of white blood cells collected from patients' blood, cryopreservation of the white blood cells collected, packaging and shipment of frozen white blood cells to the manufacturing site in order to enable multi-site studies, procurement of lentiviral vectors that meet potency and purity requirements and shipment to the product candidate manufacturing site, shipment of the final product to clinical centers, manufacturing issues associated with interruptions in the manufacturing process, scheduling constraints for cell manufacturing slots, process contamination, equipment or reagent failure or supply shortage(s)/interruption(s), improper installation or operation of equipment, vendor or operator error, and inconsistency in cell growth. Even minor deviations from normal manufacturing processes could result in reduced production yields, lot failures, product defects, product recalls, product liability claims and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, production at such manufacturing facilities may be interrupted for an extended period of time to investigate and remedy the contamination. Further, as product candidates are developed through preclinical studies to late-stage clinical trials toward approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize processes and results. Such changes may result in the need to enroll additional patients or to conduct additional clinical studies to evaluate the impact of changes on product safety and efficacy. Penn has informed us that it will be unable provide clinical supply for any late-phase or non-U.S. clinical trials of our product candidates that we may conduct. Therefore, we will need maintain and/or add new agreements with additional CMOs to produce clinical supply of our product candidates for late-phase clinical trials and at the necessary scale. We cannot guarantee that we will be able to enter into such agreements on commercially acceptable terms, if at all. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could cause our product candidates to perform differently and affect the results of ongoing and planned clinical trials or other future clinical trials.

Although we continue to optimize our manufacturing process for our product candidates, doing so is a difficult and uncertain task, and there are risks associated with scaling to the level required for advanced clinical trials or commercialization, including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, lot consistency and timely availability of reagents and/or raw materials. If we are unable to adequately scale-up the manufacturing process for our product candidates with WuXi, we may need to transfer to another manufacturer and/or our own facility, which can be lengthy. If we are able to adequately establish and scale-up the manufacturing process for our product candidates with an alternative manufacturer, we will still need to negotiate with such manufacturer an agreement for commercial supply and it is not certain we will be able to come to agreement on terms acceptable to us. This may impact our cost of goods and thus commercial viability and/or competitiveness.

In addition, many of the components which are required to support our cell manufacturing process, such as equipment, media, growth factors and disposables, are highly specialized and it is possible that the supply chain for these materials may be interrupted. If we are unable to promptly remedy such interruption, then there may be delays to our clinical development efforts.

***The manufacturing process for any products that we may develop is subject to the FDA approval process, and we will need to contract with manufacturers who can meet all applicable FDA requirements on an ongoing basis.***

The manufacturing process for any products that we may develop is subject to the FDA approval process, and we will need to contract with manufacturers who can meet all applicable FDA requirements on an ongoing basis. If we or our CMOs are unable to reliably produce products to specifications acceptable to the FDA, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CMOs will be able to manufacture the approved product in accordance with requirements from the FDA, to produce it in sufficient quantities to meet the requirements for the potential launch of the product, or to meet potential future demand. Any of these challenges could delay completion of clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, suspension of production or recalls of the product candidates or marketed biologics, operating restriction and criminal prosecutions, delay approval of our product candidates, impair commercialization efforts, increase our cost of goods, and have an adverse effect on our business, financial condition, results of operations and growth prospects. Our future success depends on our ability to manufacture our products, if licensed, on a timely basis with acceptable manufacturing costs, while at the same time maintaining good quality control and complying with applicable regulatory requirements, and an inability to do so could have a material adverse effect on our business, financial condition, and results of operations. In addition, we could incur higher manufacturing costs if manufacturing processes or standards change, and we could need to replace, modify, design, or build and install equipment, all of which would require additional capital expenditures. Specifically, because our product candidates may have a higher cost of goods than conventional therapies, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater.

***The manufacture of viral vectors is complex and variable, and there are a limited number of manufacturers able to supply us with viral vectors.***

Our MuSK-CAART and rese-cel product candidates utilize a lentiviral delivery vector and some or all of our other product candidates may require a lentiviral delivery vector, a key drug substance that delivers the CAR or CAAR to the target T cells. We do not have the capability to manufacture lentiviral vector and plan to obtain the vector we require from third parties. The manufacturing process for lentiviral vector is variable and still evolving. It is not uncommon for manufacturing runs to fail, whether due to contamination, supplier error, or equipment failure, or to be delayed. To the extent our product candidates use a lentiviral delivery vector, a lack of vector supply will cause us to be unable to manufacture our CAR T or CAAR T cells as well as a delay in patient enrollment, which may have a negative impact on our ability to successfully develop our product candidates.

Further, there are a limited number of manufacturers capable of producing lentiviral vectors. It can be challenging to secure a relationship with any of these manufacturers, and the manufacturing and release process can take a significant amount of time. We have secured a supply of lentiviral vector from CAROT sufficient for a portion of the patients we plan to enroll in our MusCAARTes™ trial and our RESET™ clinical trials. We have also reserved additional vector manufacturing capacity at Penn and CHOP and in December 2021, as amended in May 2023, February 2024 and May 2024, we secured a license and supply agreement with Oxford to establish a process and supply lentiviral vector for the clinical and commercial development of our rese-cel candidate. There is no assurance that we will be able to continue to secure adequate and timely supply of lentiviral vector. Moreover, we cannot be certain that our CAR T or CAAR T cell product candidates produced with lentiviral vector from different manufacturers will be comparable or that results of clinical trials will be consistent if conducted with lentiviral vector from different manufacturers.

Vector production also requires the production of high-quality DNA plasmids, for which there is also a limited number of suppliers. Although we have established relationships with suppliers for lentiviral vector and plasmids, we do not yet have our own clinical-scale manufacturing facility established, and are therefore highly dependent on the ability of these suppliers to manufacture necessary materials and to deliver these materials to us on a timely and reliable basis.

***If we are to operate our own manufacturing facility, significant resources will be required and we may fail to successfully operate our facility, which could adversely affect our clinical trials and the commercial viability of our product candidates.***

If we establish our own manufacturing facility, our operations will be subject to review and oversight by the FDA and the FDA could object to our use of our manufacturing facility. We must first receive approval from the FDA prior to licensure to manufacture our product candidates, which we may never obtain. Even if licensed, we would be subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMPs and other government regulations. Our license to manufacture product candidates will be subject to continued regulatory review. Our cost of goods development is at an early stage. The actual cost to manufacture and process our product candidates at a manufacturing facility of our own could be greater than we expect and could materially and adversely affect the commercial viability of our product candidates.

***The manufacture of biopharmaceutical products is complex and requires significant expertise, and can be impacted by resource constraints, labor disputes and workforce limitations.***

The manufacture of biopharmaceutical products is complex and requires significant expertise, including the development of advanced manufacturing techniques and process controls. Manufacturers of cell therapy products often encounter difficulties in production, particularly in scaling out and validating initial production and ensuring the absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our supply of product candidates or in the manufacturing facilities upon which we currently or will rely, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability or other issues relating to the manufacture of our product candidates, whether by Penn, WuXi, or other third-party CMOs, or at any manufacturing facility that we may establish, will not occur in the future.

Penn, WuXi or other third-party CMOs that we engage, or we may fail to manage the logistics of storing and shipping our product candidates. Storage failures and shipment delays and problems caused by us, our vendors or other factors not in our control, such as weather, could result in loss of usable product or prevent or delay the delivery of product candidates to patients.

Penn, WuXi, or other third-party CMOs that we engage, or we may also experience manufacturing difficulties due to resource constraints, labor disputes or workforce limitations arising from the expanding need for manufacturing in the cell therapy field and the limited number of training programs for technical staff. If we were to encounter any of these difficulties, our ability to provide our product candidates to patients would be jeopardized.

***We are dependent upon the availability of specialty raw materials and the production capabilities of small manufacturers to source the components of our product candidates.***

Our product candidates require many specialty raw materials, some of which are manufactured by small companies with limited resources and experience to support a commercial product, and the suppliers may not be able to deliver raw materials to our specifications. In addition, those suppliers generally do not have the capacity to support commercial products manufactured under cGMP by biopharmaceutical firms. The suppliers may be ill-equipped to support our needs, especially in non-routine circumstances like an FDA inspection or medical crisis, such as widespread contamination. We also do not have contracts with many of these suppliers, and we may not be able to contract with them on acceptable terms or at all. Accordingly, we may experience delays in receiving key raw materials to support clinical or commercial manufacturing.

In addition, some raw materials are currently available from a single supplier, or a small number of suppliers. We cannot be sure that these suppliers will remain in business or that they will not be purchased by one of our competitors or another company that is not interested in continuing to produce these materials for our intended purpose. In addition, the lead time needed to establish a relationship with a new supplier can be lengthy, and we may experience delays in meeting demand in the event we must switch to a new supplier. The time and effort to qualify a new supplier could result in additional costs, diversion of resources or reduced manufacturing yields, any of which would negatively impact our operating results. Further, we may be unable to enter into agreements with a new supplier on commercially reasonable terms, which could have a material adverse impact on our business. We are also unable to predict how changing global economic conditions or global health concerns will affect our third-party suppliers and manufacturers. Regional or single-source dependencies may in some cases accentuate these risks. For example, the pharmaceutical industry generally, and in some instances our Company or our collaborators or other third parties on which we rely, depend on China-based suppliers or service providers for certain raw materials, products and services, or other activities. Our ability or the ability of our collaborators or such other third parties to continue to engage these China-based suppliers or service providers for certain preclinical research programs and clinical development programs could be restricted due to geopolitical developments between the United States and China, including as a result of the escalation of tariffs or other trade restrictions or if the previously proposed federal legislation known as the BIOSECURE Act or a similar law were to be enacted. Any negative impact of such matters on our third-party suppliers and manufacturers may also have an adverse impact on our results of operations or financial condition.

***We may encounter difficulties in production, particularly with respect to process development or scaling up of our manufacturing capabilities. If we encounter such difficulties, our ability to provide supply of our CAR T or CAAR T cells for clinical trials or for commercial purposes could be delayed or stopped.***



Establishing clinical and commercial manufacturing and supply is a difficult and uncertain task, and there are risks associated with scaling to the level required for advanced clinical trials or commercialization, including, among others, increased costs, potential problems with process scale-out, process reproducibility, stability issues, lot consistency, and timely availability of reagents or raw materials. For example, we may find it difficult to establish a manufacturing process that is consistent. If this occurs, we may need to complete more than one manufacturing run for each treated patient, which would impact the availability of adequate coverage and reimbursement from third-party payors. Competitors that have developed CAR T cell therapies have had difficulty reliably producing engineered T cell therapies in the commercial setting. If we experience similar challenges manufacturing product candidates to approved specifications, this may limit our product candidates' utilization and our ability to receive payment for these product candidates once licensed. Alternatively, these challenges may require changes to our manufacturing processes, which could require us to perform additional clinical studies, incurring significant expense. We may ultimately be unable to reduce the expenses associated with our product candidates to levels that will allow us to achieve a profitable return on investment.

***If we or our third-party suppliers use hazardous, non-hazardous, biological or other materials in a manner that causes injury or violates applicable law, we may be liable for damages.***

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials. We and our suppliers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical and hazardous materials. Although we believe that we and our suppliers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we and our suppliers cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

***Changes in product candidate manufacturing or formulation may result in additional costs or delay, which could adversely affect our business, results of operations and financial condition.***

As product candidates are developed through preclinical studies to later-stage clinical trials towards approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods or formulation, are altered along the way in an effort to optimize processes and results. Any of these changes could cause our product candidates to perform differently and affect the results of ongoing and planned clinical trials or other future clinical trials conducted with the altered materials or with materials made with the altered methods. Such changes may also require additional testing, or notification to, or approval by the FDA or other regulatory authorities. This could delay completion of clinical trials, require the conduct of bridging clinical trials or studies, require the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and/or jeopardize our ability to commence product sales and generate revenue.

#### **Risks Related to Government Regulation**

***The regulatory approval process is lengthy and time-consuming, and we may experience significant delays in the clinical development and regulatory approval of our product candidates.***

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of drug products, including biologics, are subject to extensive regulation by the FDA and other regulatory authorities in the United States and comparable authorities in other jurisdictions, such as the EMA in Europe. We are not permitted to market any biological drug product in the United States until we receive approval of a Biologics License Application, or BLA, from the FDA. We have not previously submitted a BLA to the FDA, or similar licensure filings to comparable foreign authorities. A BLA must include extensive preclinical and clinical data and supporting information to establish the product candidate's safety, potency and purity for each desired indication. The BLA must also include significant information regarding the chemistry, manufacturing and controls for the product, including with respect to chain of identity and chain of custody of the product.

We expect the novel nature of our product candidates to create further challenges in obtaining regulatory approval. For example, to our knowledge, the FDA has not previously reviewed regulatory applications for marketing authorization of CAR T cells for treatment of autoimmune disease or CAAR T cells for treatment of pemphigus, and there is no cell therapy currently approved by the FDA for the treatment of mPV, MuSK myasthenia gravis, SLE, myositis, SSc, gMG or MS. Because of this, we have little guidance as to which endpoints will be accepted, how many clinical trials we may expect to conduct, and whether open-label clinical trials will be deemed acceptable, among other things. We may also request regulatory approval of future CAR T or CAAR T cell-based product candidates by target, regardless of disease type or origin, which the FDA may have difficulty accepting if our clinical trials only involved diseases of certain origins. The FDA may also require a panel of experts, referred to as an Advisory Committee, to deliberate on the adequacy of the safety, potency and purity data to support licensure. The opinion of the Advisory Committee, although not binding, may have a significant impact on our ability to obtain licensure of the product candidates based on the completed clinical trials, as the FDA often adheres to the Advisory Committee's recommendations. Further, given the rapidly evolving landscape of cell therapy, we could encounter a significant change in the regulatory environment for our product candidates once we have already begun one or more lengthy and expensive clinical trials for our product candidates. For example, the U.S. Supreme Court's July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which FDA's regulations, policies, and decisions may become subject to increasing legal challenges, delays, and/or changes. Moreover, the incoming U.S. administration may propose policy changes that create additional uncertainty for our business and may result in increased regulatory uncertainty, judicial interpretations overturning precedent and other impacts to the agency rulemaking process. Accordingly, the regulatory approval pathway for our product candidates may be uncertain, complex, expensive and lengthy, and approval may not be obtained.

We may also experience delays in completing ongoing and planned clinical trials for a variety of reasons, including delays related to:

- obtaining regulatory authorization to begin a trial, if applicable;
- the availability of financial resources to commence and complete the planned trials;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining approval at each clinical trial site by an independent IRB;
- recruiting suitable patients to participate in a trial;
- having patients complete a trial, including having patients enrolled in clinical trials dropping out of the trial before the product candidate is manufactured and returned to the site, or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol or dropping out of a trial;
- addressing any patient safety concerns that arise during a trial;
- adding new clinical trial sites; or
- manufacturing sufficient quantities of qualified materials under cGMPs and applying them on a patient by patient basis for use in clinical trials.

We could also encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. If we experience delays in the completion of, any future clinical trial of our product candidates, the commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may ultimately lead to the denial of regulatory approval of our product candidates.

***We expect the product candidates we develop will be regulated as biological products, or biologics, and therefore they may be subject to competition.***

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as “interchangeable” based on its similarity to a licensed biologic. Under the BPCIA, an application for a biosimilar product cannot be licensed by the FDA until 12 years after the reference product was licensed under a BLA. The law is complex and is still being interpreted and implemented by the FDA.

We believe that any of the product candidates we develop that is licensed in the United States as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

In the European Union, there is a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product. For such products, the results of appropriate preclinical or clinical trials must be provided in support of an application for market authorization. Guidelines from the EMA detail the type of quantity of supplementary data to be provided for different types of biological product.

***The regulatory landscape that will govern our product candidates is uncertain; regulations relating to more established cell therapies and other therapies for autoimmune diseases where B cells may play a role in initiating or maintaining disease are still developing, and changes in regulatory requirements could result in delays or discontinuation of development of our product candidates or unexpected costs in obtaining regulatory approval.***

Because we are developing novel CAR T and CAAR T cell product candidates that are unique biological entities, the regulatory requirements that we will be subject to are not entirely clear. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. For example, regulatory requirements governing gene therapy products and cell therapy products have changed frequently and may continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA established the Office of Tissues and Advanced Therapies, or OTAT, in 2016, within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. In September 2022, the FDA announced retitling of OTAT to the Office of Therapeutic Products, or OTP, and elevation of OTP to a “Super Office” to meet its growing cell and gene therapy workload. In addition, under guidelines issued by the National Institutes of Health, or NIH, gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. Before a clinical trial can begin at any institution, that institution’s institutional review board, or IRB, and its IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical study, even if the FDA has reviewed the study and approved its initiation. Conversely, the FDA can place an IND application on clinical hold even if such other entities have provided a favorable review. Furthermore, each clinical trial must be reviewed and approved by an independent IRB at or servicing each institution at which a clinical trial will be conducted. In addition, adverse developments in clinical trials of gene therapy products conducted by others or in the post-approval context may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates. For example, after the FDA’s November 2023 announcement of its investigation into reports of T cell malignancies for BCMA- and CD19-directed CAR T cell immunotherapies, the FDA informed us that, based on those reports, patients receiving rese-cel in our clinical trials will require life-long monitoring for new malignancies.

Complex regulatory environments exist in other jurisdictions in which we might consider seeking regulatory approvals for our product candidates, further complicating the regulatory landscape. For example, in the European Union, a special committee called the Committee for Advanced Therapies was established within the EMA in accordance with Regulation (EC) No 1394/2007 on advanced-therapy medicinal products, or ATMPs, to assess the quality, safety and efficacy of ATMPs, and to follow scientific developments in the field. ATMPs include gene therapy products as well as somatic cell therapy products and tissue engineered products. These various regulatory review committees and advisory groups and new or revised guidelines that they promulgate from time to time may lengthen

the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. Because the regulatory landscape for our CAR T and CAAR T cell product candidates is new, we may face even more cumbersome and complex regulations than those emerging for gene therapy products and cell therapy products. Furthermore, even if our product candidates obtain required regulatory approvals, such approvals may later be withdrawn because of changes in regulations or the interpretation of regulations by applicable regulatory agencies. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

***If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, we will not be able to commercialize, or will be delayed in commercializing, our product candidates, and our ability to generate revenue will be materially impaired.***

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States. Before we can commercialize any of our product candidates, we must obtain marketing approval. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction and it is possible that none of our product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval. We, as a company, have no experience in filing and supporting the applications necessary to gain regulatory approvals and expect to rely on third-party CROs and/or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the drug candidate's safety, potency and purity.

Securing regulatory approval also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining regulatory approvals is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted IND, BLA or comparable application types in other countries, may cause delays in the approval or rejection of an application. The FDA and foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. Our product candidates could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or other foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA that a drug candidate is safe, potent and pure for its proposed indication or a related companion diagnostic is suitable to identify appropriate patient populations;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or other foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or other foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an BLA, or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or other foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities that we may establish or of third-party manufacturers with which we may contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or other foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete the FDA approval process and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to

obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

We expect the novel nature of our product candidates to create further challenges in obtaining regulatory approval. As a result, our ability to develop product candidates and obtain regulatory approval may be significantly impacted. For example, the general approach for FDA approval of a new biologic or drug is for sponsors to seek licensure or approval based on dispositive data from well-controlled, Phase 3 clinical trials of the relevant product candidate in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete. We believe that we may be able to utilize the FDA's Regenerative Medicine Advanced Therapy designation for our product candidates given the limited alternatives for treatments for certain rare diseases and autoimmune diseases where B cells may play a role in initiating or maintaining disease, but the FDA may not agree with our plans.

Moreover, approval of genetic or biomarker diagnostic tests may be necessary to advance some of our product candidates to clinical trials or potential commercialization. In the future, regulatory agencies may require the development and approval of such tests. Accordingly, the regulatory approval pathway for such product candidates may be uncertain, complex, expensive and lengthy, and approval may not be obtained.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, if licensed, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

On November 28, 2023, the FDA issued a safety communication to provide information about reports of T-cell malignancy following BCMA-directed or CD19-directed autologous CAR T cell immunotherapies and subsequently initiated class safety labeling changes for the approved products in this class. B However, all currently approved CAR T-cell immunotherapies are in oncology indications, and the autoimmune diseases we are seeking to treat with rese-cel, a CD19-directed CAR T immunotherapy, are different indications from the approved oncology indications. Accordingly, the FDA and other regulatory authorities may apply a different benefit-risk assessment threshold such that even if our product candidate demonstrated a similar safety profile as current CAR T therapies, the FDA could ultimately determine that the harmful side effects outweigh the benefits and require us to cease clinical trials or deny approval of our product candidates. The FDA's investigation may impact the FDA's review of product candidates that we are developing, or that we may seek to develop in the future, which may, among other things, result in additional regulatory scrutiny of our product candidates, delay the timing for receiving any regulatory approvals or impose additional post-approval requirements on any of our product candidates that receive regulatory approval.

If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired.

***Even though we may apply for orphan drug designation for our product candidates, we may not be able to obtain orphan drug marketing exclusivity.***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 in the United States, or a patient population of 200,000 or more in the United States when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. In order to obtain orphan drug designation, the request must be made before submitting a BLA. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval of that particular product for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a BLA, to market the same biologic (meaning, a product with the same principal molecular structural features) for the same indication for seven years, except in limited circumstances such as a showing of clinical superiority to the product with orphan drug exclusivity or if FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. As a result, even if one of our product candidates receives orphan exclusivity, the FDA can still approve other biologics that do not have the same principal molecular structural features for use in treating the same indication or disease or the same biologic for a different indication or disease during the exclusivity period. Furthermore, the FDA can waive orphan exclusivity if we are unable to manufacture sufficient supply of our product or if a subsequent applicant demonstrates clinical superiority over our product.

We have obtained from the FDA orphan drug designation for DSG3-CAART for the treatment of pemphigus vulgaris, for MuSK-CAART for the treatment of MuSK MG and for rese-cel for the treatment of idiopathic inflammatory myopathies (IIM, or myositis) and systemic sclerosis. We may seek orphan drug designation for certain other of our product candidates, but may be unable to obtain orphan drug designation for some or all of our product candidates in specific orphan indications in which we believe there is a medically plausible basis for the use of these products. Even if we obtain orphan drug designation, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition, or if a subsequent applicant demonstrates clinical superiority over our products, if licensed. Although we may seek orphan drug designation for other product candidates, we may never receive such designations. In addition, the FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

***The FDA has granted rare pediatric disease designation to rese-cel for the treatment of juvenile dermatomyositis. However, a marketing application for rese-cel or any other product candidate, if approved, may not meet the eligibility criteria for a priority review voucher.***

The FDA has granted rare pediatric disease designation to rese-cel for the treatment of juvenile dermatomyositis. Designation of a drug as a drug for a rare pediatric disease does not guarantee that an NDA or BLA for such drug will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the FDCA, we will need to request a rare pediatric disease priority review voucher in our original BLA for rese-cel. The FDA may determine that a BLA for rese-cel, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- juvenile dermatomyositis no longer meets the definition of a rare pediatric disease;
- the BLA contains an active ingredient that has been previously approved by the FDA;
- the BLA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population (that is, if the BLA does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the BLA is approved for a different adult indication than the rare pediatric disease for which rese-cel is designated.

The FDA's rare pediatric disease priority voucher program began to sunset on December 20, 2024, on failure to pass a continuing resolution package that included its reauthorization. Under the amended statutory sunset provisions, after December 20, 2024, the FDA may award a priority review voucher for an approved rare pediatric disease product application only if the sponsor has rare pediatric disease designation for the drug and if that designation was granted by December 20, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. Congress may vote to reauthorize this program, but its future remains unknown at this time. Absent any such reauthorization, if a BLA for rese-cel is not approved prior to September 30, 2026 for any reason, regardless of whether it meets the criteria for a rare pediatric disease priority review voucher, it will not be eligible for a priority review voucher.

***A fast track designation by the FDA, even if granted, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our current product candidate and any future product candidates will receive marketing approval.***

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation for a particular indication. Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious or life-threatening conditions and address an unmet medical need. We have received fast track designation for DSG3-CAART for improving healing of mucosal blisters in patients with mPV, for MuSK-CAART for improving activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis and for rese-cel, designed to deplete CD19-positive B cells and improve disease activity in patients with SLE, LN and the myositis subtype of dermatomyositis, for the treatment of patients with systemic sclerosis to improve associated organ dysfunction and for the treatment of relapsing and progressive forms of MS. We may also apply for fast track designation for certain of our other product candidates, but there is no assurance that the FDA will grant this status to any of our other current or future product candidates. Marketing applications filed by sponsors of products in fast track development may qualify for priority review under the policies and procedures offered by the FDA, but the fast track designation does not assure any such qualification or ultimate marketing approval by the FDA. The FDA has broad discretion whether or not to grant fast track designation, so even if we believe a particular product candidate is eligible for this designation, there can be no assurance that the FDA would decide to grant it. Even though we have received fast track designation for certain of our product candidates, we may not experience a faster development process, regulatory

review or approval for these product candidates as compared to conventional FDA procedures, and receiving a fast track designation does not provide assurance of ultimate FDA approval. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. In addition, the FDA may withdraw any fast track designation at any time.

***Although we may pursue expedited regulatory approval pathways for a product candidate, it may not qualify for expedited development or, if it does qualify for expedited development, it may not actually lead to a faster development or regulatory review or approval process.***

Although we believe there may be an opportunity to accelerate the development of certain of our product candidates through one or more of the FDA's expedited programs, such as fast track, breakthrough therapy, Regenerative Medicine Advanced Therapy, accelerated approval or priority review, we cannot be assured that any of our product candidates will qualify for such programs.

For example, we may seek a Regenerative Medicine Advanced Therapy, or RMAT, designation for some of our product candidates. An RMAT is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells that lead to a durable modification of cells or tissues may meet the definition of a Regenerative Medicine Therapy. The RMAT program is intended to facilitate efficient development and expedite review of RMATs, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A new drug application or a BLA for an RMAT may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A Regenerative Medicine Therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval. Although RMAT designation or access to any other expedited program may expedite the development or approval process, it does not change the standards for approval. If we apply for RMAT designation or any other expedited program for our product candidates, the FDA may determine that our proposed target indication or other aspects of our clinical development plans do not qualify for such expedited program. Even if we are successful in obtaining a RMAT designation or access to any other expedited program, we may not experience faster development timelines or achieve faster review or approval compared to conventional FDA procedures. Access to an expedited program may also be withdrawn by the FDA if it believes that the designation is no longer supported by data from our clinical development program. Additionally, qualification for any expedited review procedure does not ensure that we will ultimately obtain regulatory approval for such product candidate.

***Disruptions at the FDA, the SEC and other government agencies, including from government shutdowns, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

Currently, federal agencies in the U.S. are operating under a continuing resolution that is set to expire on September 30, 2025. The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs or biologics to be reviewed, which would adversely affect our business. For example, over the past decade, the U.S. government has shut down several times, and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue to fund our operations.

***Significant political, trade, regulatory developments, and other circumstances beyond our control, could have a material adverse effect on our financial condition or results of operations.***

We operate beyond the United States and, if approved, we may sell our products in countries throughout the world. Significant political, trade, or regulatory developments in the jurisdictions in which we may sell our products, such as those stemming from the change in U.S. federal administration, are difficult to predict and may have a material adverse effect on us. Similarly, changes in U.S.

federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. For example, in 2025, the United States imposed tariffs on imports on its trading partners, including Canada, Mexico, the EU and China. Historically, tariffs have led to increased trade and political tensions. In response to tariffs, other countries have implemented retaliatory tariffs on U.S. goods. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our financial condition or results of operations.

#### Risks Related to Ongoing Regulatory Obligations

***Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.***

Any regulatory approvals that we receive for our product candidates will require surveillance to monitor the safety, potency and purity of the product candidate. We believe it is likely that the FDA will require a Risk Evaluation and Mitigation Strategy, or REMS, in order to approve our product candidates, which could entail requirements for a medication guide, physician communication plans or additional elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA approves our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any BLA, other marketing application and previous responses to inspectional observations. Additionally, manufacturers and manufacturers' facilities are required to comply with extensive FDA, and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations and applicable product tracking and tracing requirements. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control. In addition, the FDA could require us to conduct another study to obtain additional safety or biomarker information. Additionally, under the Food and Drug Omnibus Reform Act of 2022, or FDORA, sponsors of approved drugs and biologics must provide six months' notice to the FDA of any changes in marketing status, such as the withdrawal of a drug, and failure to do so could result in the FDA placing the product on a list of discontinued products, which would revoke the product's ability to be marketed.

Further, we will be required to comply with FDA promotion and advertising rules, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting products for uses or in patient populations that are not described in the product's approved uses (known as "off-label use"), limitations on industry-sponsored scientific and educational activities and requirements for promotional activities involving the internet and social media. Later discovery of previously unknown problems with our product candidates through follow-up programs with our clinical trial patients, including adverse events of unanticipated severity or frequency, or with our third-party suppliers or manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical studies to assess new safety risks, or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of our product candidates, withdrawal of the product from the market or voluntary or mandatory product recalls;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention, or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action. If these executive actions impose restrictions on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, and we may not achieve or sustain profitability.



***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our research and development activities involve the use of biological and hazardous materials and produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. Breach of certain environmental, health and safety laws and regulations could also in certain circumstances constitute a breach of our License Agreement with Penn. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Although we maintain workers' compensation insurance to cover us for costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological waste or hazardous waste insurance coverage, workers compensation or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological or hazardous waste exposure or contamination.

***Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to comply with the laws of the FDA, provide true, complete and accurate information to the FDA, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws, or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs.

#### Risks Related to Healthcare

***Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates, if licensed, profitably.***

Successful commercialization of our product candidates, if licensed, will depend in part on the extent to which reimbursement for those drug products will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drug products they will pay for and establish reimbursement levels. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford a drug product. Sales of drug products depend substantially, both domestically and abroad, on the extent to which the costs of drugs products are paid for by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. Any product candidate for which we seek regulatory approval and reimbursement will need to meet or surpass our target product profile, or TPP, to be deemed a viable alternative to currently approved therapies. In addition, because our product candidates represent new approaches to the treatment of autoimmune diseases where B cells may play a role in initiating or maintaining disease, we cannot accurately estimate the potential revenue from our product candidates. For more information, see the section titled "Business—Government regulation—Pricing and Reimbursement" included in this annual report on Form 10-K.

Obtaining coverage and reimbursement of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide the payor with supporting scientific, clinical and cost-effectiveness data for the use of our products, if licensed. In the United States, the principal decisions about reimbursement for new drug products are typically made by the Centers for Medicare and Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. However, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products can differ significantly from payor to payor. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Even if we obtain coverage for a given product, if the resulting reimbursement rates are insufficient, hospitals may not approve our product for use in their facility or third-party payors may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. Separate reimbursement for the product itself may or may not be available. Instead, the hospital or administering physician may be reimbursed only for providing the treatment or procedure in which our product is used. Further, from time to time, CMS revises the reimbursement systems used to reimburse health care providers, including the Medicare Physician Fee Schedule and Outpatient Prospective Payment System, which may result in reduced Medicare payments. In some cases, private third-party payors rely on all or portions of Medicare payment systems to determine payment rates. Changes to government healthcare programs that reduce payments under these programs may negatively impact payments from private third-party payors, and reduce the willingness of physicians to use our product candidates.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if government and other third-party payors fail to provide coverage and adequate reimbursement. We expect downward pressure on pharmaceutical pricing to continue. Further, coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

***Healthcare legislative measures aimed at reducing healthcare costs may have a material adverse effect on our business and results of operations.***

In the United States, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain regulatory approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and in additional downward pressure on the price that we, or any collaborators, may receive for any approved products. For more information, see the section titled "Business—Government regulation—Current and Future Legislation" included in this annual report on Form 10-K.

We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our product candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our products, if licensed;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

***Our relationships with customers, healthcare providers, physicians, and third-party payors will be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws, we could face substantial penalties.***

These laws may impact, among other things, our clinical research program, as well as our proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services is subject to extensive laws and regulations designed to prevent fraud, kickbacks, self-dealing and other abusive practices, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive and other business arrangements. We may also be subject to federal, state and foreign laws governing the privacy and security of individual identifiable health information and other personally identifiable information. For more information, see the section titled “Business—Government regulation—Other Healthcare Laws and Compliance Requirements” included in this annual report on Form 10-K.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company’s attention from the business.

The failure to comply with any of these laws or regulatory requirements subjects entities to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a pharmaceutical manufacturer to incur significant legal expenses and divert management’s attention from the operation of the business. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, or our arrangements with physicians, some of whom receive stock options as compensation, could be subject to challenge under one or more of such laws. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws, we may be subject to investigations, enforcement actions and/or significant penalties. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct or business noncompliance, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

#### **Risks Related to Data Privacy and Security**

***Data collection is governed by restrictive regulations governing the use, processing, and cross-border transfer of personal information.***

We are subject to stringent privacy and data protection requirements and these requirements may become more complex as we grow our business and begin to operate in other jurisdictions. For example, the collection, use, storage, disclosure, transfer, or other processing of personal data regarding individuals in the European Economic Area, or the EEA, including personal health data, is subject to the EU General Data Protection Regulation, or the EU GDPR, and similarly, processing of personal data regarding individuals in the UK is subject to the UK General Data Protection Regulation and the UK Data Protection Act 2018, or the UK GDPR, and together with the EU GDPR, or the GDPR. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process

personal data, including requirements relating to having a legal basis or condition for processing personal data, stricter requirements relating to the processing of sensitive data (such as health data), where required by the GDPR obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, requiring data protection impact assessments for high risk processing and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA/UK, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million (£17.5 million under UK GDPR) or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR includes restrictions on cross-border data transfers of personal data to countries outside the EEA/UK that are not considered by the European Commission and UK government as providing “adequate” protection to personal data, or third countries, including the United States. The GDPR may increase our responsibility and liability in relation to personal data that we process where such processing is subject to the GDPR, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, including as implemented by individual countries. Compliance with the GDPR is rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with our European activities.

To enable the transfer of personal data outside of the EEA or the UK, adequate safeguards (for example, the European Commission approved Standard Contractual Clauses, or SCCs) must be implemented in compliance with European and UK data protection laws. In addition, transfers made pursuant to the SCCs (and other similar appropriate transfer safeguards) need to be assessed on a case-by-case basis taking into account the legal regime applicable in the destination country, in particular regarding applicable surveillance laws and relevant rights of individuals with respect to the transferred personal data, to ensure an “essentially equivalent” level of protection to that guaranteed in the EEA in the jurisdiction where the data importer is based, or the Transfer Impact Assessment. On June 4, 2021, the EC issued new forms of standard contractual clauses for data transfers from controllers or processors in the EU/EEA (or otherwise subject to the GDPR) to controllers or processors established outside the EU/EEA. The UK is not subject to the EC’s new standard contractual clauses but has published its own transfer mechanism, the International Data Transfer Agreement and International Data Transfer Addendum, or the IDTA, which enable transfers from the UK, and has also implemented a similar Transfer Impact Assessment requirement. Further, the EU and United States have adopted its adequacy decision for the EU-U.S. Data Privacy Framework, or the Framework, which entered into force on July 11, 2023. This Framework provides that the protection of personal data transferred between the EU and the United States is comparable to that offered in the EU. This provides a further avenue to ensuring transfers to the United States are carried out in line with GDPR. There has been an extension to the Framework to cover UK transfers to the United States. The Framework could be challenged like its predecessor frameworks. We will be required to implement these safeguards and carry out Transfer Impact Assessments when conducting restricted data transfers under the GDPR and doing so will require significant effort and cost, and may result in us needing to make strategic considerations around where EEA or UK personal data is stored and transferred, and which service providers we can utilize for the processing of EEA/UK personal data.

Although the UK is regarded as a third country under the EU GDPR, the European Commission has issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or the Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. The UK government has confirmed that personal data transfers from the UK to the EEA remain free flowing. The UK Government had introduced a Data Protection and Digital Information Bill which failed in the UK legislative process. The UK Government introduced the Data Protection and Digital Information Bill which failed in the UK legislative process. A new Data (Use and Access) Bill (“UK Bill”) has now been introduced into parliament. The aim of the UK Bill is to reform the UK’s data protection regime. If passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regime. This may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

In the EEA, the NIS 2 Directive (“NIS 2”) is replacing the cybersecurity legal framework under the current NIS framework, aiming to ensure a high level of cybersecurity in the region. NIS 2 brings new medium and large organisations providing services in the EEA within scope of the legal framework. It extends to additional sectors and expands the list of in-scope healthcare organisations, including to certain providers engaged in research and development of medicinal products. The new regime imposes direct obligations on management in respect of an in-scope organization's compliance with NIS 2, requires covered organisations to put in place certain cyber risk management measures, strengthens incident reporting requirements and provides supervisory authorities with a greater oversight. The majority of obligations will come into force when national legislation implementing NIS 2 becomes effective in the relevant EU Member State. EU Member States had until 17 October 2024 to transpose NIS 2 into national legislation, although many countries have still not completed the transposition. As such, the cybersecurity regulatory landscape in the EU is currently fragmented and uncertain. Although we are not currently subject to NIS 2, we have evaluated its requirements and have already implemented various cybersecurity measures that align with NIS 2 obligations. To the extent that we become subject to NIS 2 in the future, we will continue working closely with our Data Protection Officer to ensure that our compliance programs are fully aligned with NIS 2 and national

implementing legislation where applicable. Under NIS 2 companies may be subject to administrative fines of up to the higher amount of €10 million or 2% of worldwide turnover.

In the United States, there has been a flurry of legislative activity related to privacy at the state level. In California, the California Consumer Privacy Act, or CCPA, went into effect on January 1, 2020, and became subject to enforcement by the California Attorney General's office on July 1, 2020. The CCPA broadly defines personal information, and creates comprehensive individual privacy rights and protections for California consumers (as defined in the law), places increased privacy and security obligations on entities handling personal data of consumers or households, and provides for civil penalties for violations and a private right of action for data breaches. The CCPA requires covered companies to provide certain disclosures to consumers about their data collection, use and sharing practices, and to provide affected California residents with ways to opt-out of certain sales or transfers of personal information.

Additionally, the California Privacy Rights Act, or CPRA, was passed in November 2020 and as of January 1, 2023 has imposed additional obligations on companies covered by the legislation. The CPRA significantly modified the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also created a new state agency that is vested with authority to implement and enforce the CCPA. While there is an exception for protected health information that is subject to HIPAA and clinical trial regulations, the effects of the CCPA, as amended by the CPRA are potentially significant and may require us to modify our data collection or processing practices and policies and to incur substantial costs and expenses in an effort to comply and decrease our potential exposure to regulatory enforcement and/or litigation.

Similar laws have been passed in numerous other states and proposed in others. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. The existence of comprehensive privacy laws in different states in the country would make our compliance obligations more complex and costly and may increase the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. There are also states that are specifically regulating health information. For example, Washington state recently passed a health privacy law that will regulate the collection and sharing of health information, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain specific types of information. For example, a small number of states have passed laws that regulate biometric data specifically. These various privacy and security laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. State laws are changing rapidly and there is discussion in the U.S. Congress of a new comprehensive federal data privacy law to which we may likely become subject, if enacted.

All of these evolving compliance and operational requirements impose significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. Further, various other jurisdictions around the world continue to propose new and/or amended laws that regulate the privacy and/or security of certain types of personal data. Complying with these laws, if enacted, would require significant resources and leave us vulnerable to possible fines and penalties if we are unable to comply. The regulatory framework governing the collection, processing, storage, use and sharing of certain information is rapidly evolving and is likely to continue to be subject to uncertainty and varying interpretations. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our existing data management practices or the features of our services and platform capabilities. Compliance with the above and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules, modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects. Any failure or perceived failure by us, or any third parties with which we do business, to comply with our posted privacy policies, evolving laws, rules and regulations, industry standards, or contractual obligations to which we or such third parties are or may become subject, may result in actions or other claims against us by governmental entities or private actors, the expenditure of substantial costs, time and other resources or the incurrence of significant fines, penalties or other liabilities. In addition, any such action, particularly to the extent we were found to be guilty of violations or otherwise liable for damages, would damage our reputation and adversely affect our business, financial condition and results of operations.

***If our security measures or those of our contractors, consultants or other service providers are breached or unauthorized access to confidential and/or proprietary information or other sensitive information, including individually identifiable health information or other personally identifiable information, is otherwise obtained, our reputation may be harmed, and we may incur significant liabilities.***

We and the third parties upon which we rely face a variety of evolving threats, which could cause cybersecurity incidents or data breaches, which, could result in unauthorized access to systems and data, loss of data, and compromise, misuse, or corruption of such data and information. The systems of any CMOs that we may engage now or in the future, and present and future CROs, contractors, consultants and other service providers also could experience breaches or cybersecurity incidents leading to the exposure of confidential

and sensitive information. Despite the implementation of security measures, our internal computer systems and information technology systems and infrastructure and those of our third party CROs, vendors, and other contractors and consultants upon which our business relies are vulnerable to breakdown or damage or interruption from, among other things, natural disasters, terrorism, war, telecommunication and electrical failures, and sophisticated cyber attacks, including the theft, fraud, and subsequent misuse of employee credentials, wrongful conduct by insider employees or vendors, denial-of-service attacks, ransomware attacks, business email compromises, computer malware, malicious codes, viruses, breakdown, wrongful intrusions, data breaches, and social engineering (including phishing attacks). Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources. Because the techniques used by computer programmers who may attempt to penetrate and sabotage our information technology systems and infrastructure, network security or our website change frequently and may not be recognized until launched against a target, we may be unable to anticipate these techniques or to adequately prevent or address them.

It is also possible that unauthorized access to our confidential and/or proprietary information or other sensitive information, including customer or employee information, may be obtained through inadequate use of security controls by customers, suppliers or other vendors. We rely on such third parties to implement effective security measures and identify and correct for any failures, deficiencies, compromises or breaches.

In the event of a cybersecurity incident or data breach, our company could be required to provide legal notifications and disclosures, and could suffer loss of business, severe reputational damage adversely affecting investor confidence, regulatory inquiries, investigations and orders, litigation, indemnity obligations, damages for contract breach, penalties and fines for violation of applicable laws or regulations, significant costs for remediation and other liabilities. For example, the loss of preclinical study or clinical trial data from completed or future preclinical studies or clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security compromise or breach were to result in a loss or misappropriation of, or damage to, our data, systems, or applications, or inappropriate disclosure of confidential or proprietary information or other sensitive information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

We have incurred and expect to incur significant expenses to prevent cybersecurity incidents and data breaches, including costs related to deploying additional personnel and protection technologies, training employees, and engaging third-party solution providers and consultants. Although we expend significant resources to create security protections that are designed to shield our confidential and/or proprietary information or other sensitive information, including customer data, against potential theft and security compromises or breaches, such measures cannot provide absolute security. Moreover, as we outsource more of our information systems to vendors and rely more on cloud-based information systems, the related security risks will increase, and we will need to expend additional resources to protect our technology and information systems.

Like other companies in our industry, we, and our third party vendors, have experienced and will continue to experience threats and cybersecurity incidents relating to our information technology systems and infrastructure. We remain at risk for future cybersecurity incidents and data breaches, including, without limitation, those that may occur as a result of third-party action, or wrongful conduct by insider employees or vendors or malfeasance and other causes. If, in the future, we experience a data breach or security incident, we could experience harm to our reputation, financial performance, and customer and vendor relationships. Further, we could experience possible legal notifications and disclosures, as well as litigation or regulatory investigations or actions by state and federal governmental authorities and non-U.S. authorities, including fines, penalties, and other legal and financial exposure and liabilities. Additionally, actual, potential or anticipated attacks or compromises may cause us to incur increasing costs, including costs to deploy additional personnel and protection technologies, train employees, conduct security incident investigation or remediation and engage third-party experts and consultants.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations. Further, although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or breach.

***Interruptions in the availability of server systems or communications with internet or cloud-based services, or failure to maintain the security, confidentiality, accessibility or integrity of data stored on such systems, could harm our business.***

We rely upon a variety of internet service providers, third-party web hosting facilities and cloud computing platform providers to support our business. Failure to maintain the security, confidentiality, accessibility or integrity of data stored on or processed by such systems could result in interruptions in our operations, damage our reputation in the market, increase our service costs, cause us to incur substantial costs, subject us to liability for damages and/or fines, and divert our resources from other tasks, any one of which could materially adversely affect our business, financial condition, results of operations and prospects. If our security measures or those of our third-party data center hosting facilities, cloud computing platform providers, or third-party service partners, are breached, and unauthorized access is obtained to or there is misuse of our data or our information technology systems, we may incur significant legal and financial exposure and liabilities.

We also do not have control over the operations of the facilities of our cloud service providers and our third-party web hosting providers, and they also may be vulnerable to damage, security compromise or interruption from natural disasters, cybersecurity attacks, terrorist attacks, power outages and similar events or acts of misconduct. In addition, any changes in these providers' service levels may adversely affect our ability to meet our requirements and operate our business.

## **Risks Related to Ownership of Our Common Stock**

### Risks Related to Ownership Generally

***Our principal stockholders and management own a significant percentage of our stock and could be able to exert significant control over matters subject to stockholder approval.***

As of December 31, 2024, our executive officers, directors, and 5% stockholders beneficially owned, in the aggregate, approximately 28% of our common stock. Such calculations assume all shares of non-voting common stock, if any outstanding, are converted into voting common stock in accordance with the terms of our Third Amended and Restated Certificate of Incorporation, or the amended and restated certificate of incorporation. Accordingly, these stockholders could have the ability to influence us through this ownership position and significantly affect the outcome of all matters requiring stockholder approval. For example, these stockholders may be able to significantly affect the outcome of elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

***If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.***

As a public company, we are required to maintain internal control over financial reporting and to report any material weaknesses in such internal controls. The Sarbanes-Oxley Act requires that we evaluate and determine the effectiveness of our internal control over financial reporting and, beginning with our second annual report following our initial public offering, provide a management report on internal control over financial reporting. However, while we remain a smaller reporting company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. In compliance with Section 404 of the Sarbanes-Oxley Act, we have begun and will continue to dedicate internal resources, potentially engage outside consultants, and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting. Additionally, we will enhance control processes as needed, validate their effectiveness through testing, and implement a continuous monitoring and improvement process.

Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes, and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our stock price and make it more difficult for us to effectively market and sell our service to new and existing customers.

***The dual class structure of our common stock may limit your ability to influence corporate matters and may limit your visibility with respect to certain transactions.***

The dual class structure of our common stock may limit your ability to influence corporate matters. Holders of our common stock are entitled to one vote per share, while holders of our non-voting common stock are not entitled to any votes. Nonetheless, each share of our non-voting common stock may be converted at any time into one share of our common stock at the option of its holder by providing written notice to us, subject to the limitations provided for in our amended and restated certificate of incorporation. Additionally, stockholders who hold, in the aggregate, more than 10% of our common stock and non-voting common stock, but 10% or less of our common stock, and are not otherwise a company insider, may not be required to report changes in their ownership due to transactions in our non-voting common stock pursuant to Section 16(a) of the Exchange Act, and may not be subject to the short-swing profit provisions of Section 16(b) of the Exchange Act. In May 2024, 1,444,295 shares of non-voting common stock were converted to voting common stock and no shares of non-voting common stock remain outstanding.

***Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Certain holders of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

On March 21, 2024, we filed a registration statement on Form S-3 (File No. 333-278126) with the SEC, as amended on March 31, 2025, which amendment is not yet declared effective, or the Shelf Registration Statement, in relation to the registration of common stock, preferred stock, debt securities, warrants and/or units of any combination thereof for the purposes of selling, from time to time, our common stock, debt securities or other equity securities in one or more offerings. We also simultaneously entered into a Sales Agreement, or the 2024 Sales Agreement, with Cowen and Company, LLC, or the Sales Agent, to provide for the offering, issuance and sale of up to an aggregate amount of \$200.0 million of our common stock from time to time in “at-the-market”, offerings under the Shelf Registration Statement and subject to the limitations thereof. The Shelf Registration Statement and 2024 Sales Agreement replaced our former registration statement and sales agreement, pursuant to which we had a \$100 million at-the-market offering program, all of which we sold. We will pay to the Sales Agent cash commissions of up to 3.0 percent of the aggregate gross proceeds of sales of common stock under the 2024 Sales Agreement. Sales of common stock, debt securities or other equity securities by us may represent a significant percentage of our common stock currently outstanding. If we sell, or the market perceives that we intend to sell, substantial amounts of our common stock under the Shelf Registration Statement or otherwise, the market price of our common stock could decline significantly. In December 2024, we sold 1,225,000 shares pursuant to the 2024 ATM Program for net proceeds of \$5.1 million, after deducting commissions of \$0.1 million.

We have also filed registration statements on Form S-8 to register shares issued or reserved for issuance under our equity compensation plans and will file additional registration statements on Form S-8 to register additional shares pursuant to the “evergreen” provisions under our equity compensation plans, the Plan Amendment and any subsequent amendments to our equity compensation plans. Shares registered under these registration statements on Form S-8 can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements described above. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

In addition, certain of our employees, executive officers, and directors may enter into Rule 10b5-1 trading plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 trading plan, a broker executes trades pursuant to parameters established by the employee, director, or officer when entering into the plan, without further direction from the employee, officer, or director. A Rule 10b5-1 trading plan may be amended or terminated in some circumstances. Our employees, executive officers, and directors also may buy or sell additional shares outside of a Rule 10b5-1 trading plan when they are not in possession of material, nonpublic information.

Risks Related to our Charter and Bylaws

***Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.***

Our amended and restated certificate of incorporation and amended and restated bylaws, as amended, or the amended and restated bylaws, contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;



- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of the holders of not less than 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors;
- a requirement of approval of not less than 75% of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our amended and restated certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

***Our amended and restated bylaws designate certain courts as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.***

Pursuant to our amended and restated bylaws, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for state law claims for: (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, or other employees to us or our stockholders; (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law or our amended and restated certificate of incorporation or amended and restated bylaws (including the interpretation, application or validity thereof); or (iv) any action asserting a claim governed by the internal affairs doctrine (the Delaware Forum Provision). The Delaware Forum Provision will not apply to any causes of action arising under the Securities Act of 1933, as amended (the Securities Act) or the Securities Exchange Act of 1934. Our amended and restated bylaws further provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America are the sole and exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, or the rules and regulations promulgated thereunder, or the Federal Forum Provision. In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing Delaware Forum Provision and Federal Forum Provision; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the U.S. federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision may impose additional litigation costs on stockholders in pursuing any such claims. Additionally, these forum selection clauses may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage the filing of lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. In addition, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. While the Delaware Supreme Court and other states have upheld the validity of federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court, there is uncertainty as to whether other courts will enforce our Federal Forum Provision. If the Federal Forum Provision is found to be unenforceable, we may incur additional costs with resolving such matters. The Federal Forum Provision may also impose additional litigation costs on us and/or our stockholders who assert that the provision is invalid or unenforceable. The Court of Chancery of the State of Delaware or the federal district courts of the United States of America may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

#### Risks Related to Tax

***Changes in tax laws could adversely affect our business and financial condition.***

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. Prospective investors in our common stock should consult with their

legal and tax advisors with respect to potential changes in tax laws and the tax consequences of investing in or holding our common stock.

In 2017, the U.S. Congress and the Trump administration made substantial changes to U.S. policies, which included comprehensive corporate and individual tax reform. In addition, the Trump administration called for significant changes to U.S. trade, healthcare, immigration and government regulatory policy. With the transition to the Biden administration in early 2021, changes to U.S. policy occurred and since the start of the Trump Administration in 2025, U.S. policy changes have been implemented at a rapid pace and additional changes are likely. Changes to U.S. policy implemented by the U.S. Congress, the Trump administration or any new administration have impacted and may in the future impact, among other things, the U.S. and global economy, international trade relations, unemployment, immigration, healthcare, taxation, the U.S. regulatory environment, inflation and other areas. Although we cannot predict the impact, if any, of these changes to our business, they could adversely affect our business. Until we know what policy changes are made, whether those policy changes are challenged and subsequently upheld by the court system and how those changes impact our business and the business of our competitors over the long term, we will not know if, overall, we will benefit from them or be negatively affected by them.

***Our ability to utilize our net operating losses and certain other tax attributes to offset future taxable income may be subject to certain limitations.***

As of December 31, 2024, we had U.S. federal, state and local net operating loss carryforwards of \$157.4 million, \$181.4 million and \$88.7 million, respectively. \$0.3 million of the federal amounts expire in 2037. The state net operating losses begin to expire in 2037 and the local net operating losses began to expire in 2024. Approximately \$157.2 million of the federal net operating losses can be carried forward indefinitely. Certain net operating loss carryforwards could expire unused and be unavailable to offset future taxable income. In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an “ownership change” is subject to limitations on its ability to utilize its pre-change net operating loss carryforwards or tax credits, or NOLs or credits, to offset future taxable income or taxes. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who owns at least 5% of a corporation’s stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a specified testing period. Our existing NOLs or credits may be subject to limitations arising from previous ownership changes, and if we undergo an ownership change, our ability to utilize NOLs or credits could be further limited by Sections 382 and 383 of the Code. In addition, future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the Code. Our NOLs or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or credits. Furthermore, our ability to utilize our NOLs or credits is conditioned upon our attaining profitability and generating U.S. federal and state taxable income. As described above under “—Risks Related to Our Financial Condition and Capital Requirements”, we have incurred significant net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future; and therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our NOLs or credits. Under current law, U.S. federal net operating loss carryforwards generated in taxable years beginning after December 31, 2017 will not be subject to expiration. However, any such net operating loss carryforwards may only offset 80% of our annual taxable income in taxable years beginning after December 31, 2020.

## General Risk Factors

***Adverse developments affecting the financial services industry could adversely affect our current and projected business operations and our financial condition and results of operations.***

Adverse developments that affect financial institutions, such as events involving liquidity that are rumored or actual, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation as receiver. Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that us, the financial institutions with which we have credit agreements or arrangements directly, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. While it is not possible at this time to predict the extent of the impact that high market volatility and instability of the financial services industry could have on economic activity and our business in particular, the failure of other banks and financial institutions and the measures taken by governments, businesses and other organizations in response to these events could adversely impact our business, financial condition and results of operations.

***Public health crises, such as a pandemic, epidemic or outbreak of other highly infectious or contagious diseases, could seriously harm our research, development and potential future commercialization efforts, increase our costs and expenses and have a material adverse effect on our business, financial condition and results of operations.***

Public health crises, such as a pandemic, epidemic or outbreak of other highly infectious or contagious diseases, could adversely impact our business, the business operations of third parties on whom we rely and our ongoing or planned research and development activities. Additionally, timely enrollment in our ongoing and planned clinical trials is dependent upon clinical trial sites which may be adversely affected by global health concerns. Public health crises could result in increased adverse events and deaths in our clinical trials. Some factors from public health crises that could delay or otherwise adversely affect enrollment in the clinical trials of our product candidates, as well as our business generally, include:

- the potential diversion of healthcare resources away from the conduct of clinical trials to focus on public health crises, including the attention of physicians serving as our clinical trial investigators, hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our prospective clinical trials and the need for drugs, such as tocilizumab, and other supplies that clinical trial sites must have on hand to conduct our clinical trials to be used to address such public health crises;
- limitations on travel that could interrupt key trial and business activities, such as clinical trial site initiations and monitoring, domestic and international travel by employees, contractors or patients to clinical trial sites, including any government-imposed travel restrictions or quarantines that will impact the ability or willingness of patients, employees or contractors to travel to our clinical trial sites or secure visas or entry permissions, a loss of face-to-face meetings and other interactions with potential partners, any of which could delay or adversely impact the conduct or progress of our prospective clinical trials;
- interruption in global shipping affecting the transport of clinical trial materials, such as patient samples, investigational drug product and conditioning drugs and other supplies used in our prospective clinical trials;
- interruptions in operations at our third-party manufacturers, which could result in delays or disruptions in the supply of our current product candidates and any future product candidates; and
- business disruptions caused by potential workplace, laboratory and office closures and an increased reliance on employees working from home, disruptions to or delays in ongoing laboratory experiments and operations, product manufacturing and supply, staffing shortages, travel limitations or mass transit disruptions, any of which could adversely impact our business operations or delay necessary interactions with local regulators, ethics committees and other important agencies and contractors.

Any of these factors, and other factors related to any such disruptions that are unforeseen, could have a material adverse effect on our business and our results of operations and financial condition. Further, uncertainty around these and related issues could lead to

adverse effects on the economy of the United States and other economies, which could impact our ability to raise the necessary capital needed to develop and commercialize our product candidates.

***The price of our stock may be volatile, and you could lose all or part of your investment.***

The trading price of our common stock has been, and is likely to be in the future, highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this “Risk Factors” section, these factors include:

- the commencement, enrollment or results of our planned preclinical studies or clinical trials of our product candidates or any preclinical studies or future clinical trials we may conduct, or changes in the development status of our product candidates;
- our decision to initiate a preclinical study or clinical trial, not to initiate a preclinical study or clinical trial or to terminate an existing preclinical study or clinical trial;
- adverse results or delays in preclinical studies or clinical trials of our product candidates;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such filings, including, without limitation, the FDA’s issuance of a “refusal to file” letter or a request for additional information;
- our failure to commercialize our product candidates;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- changes in laws or regulations applicable to our product candidates, including, but not limited to, clinical trial requirements for approvals;
- adverse developments concerning our manufacturers or suppliers;
- our inability to obtain adequate product supply for any licensed product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our product candidates;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth of our initial target markets;
- our ability to successfully treat additional types of autoimmune diseases where B cells may play a role in initiating or maintaining disease;
- actual or anticipated variations in annual or quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;

- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions, including inflation and tariffs;
- global health concerns; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and The Nasdaq Global Select Market and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations in recent years that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. Securities class action litigation has often been instituted against companies, particularly in the biopharmaceutical and life sciences industries, following periods of volatility in the market price of a company's securities. We have been subject to such a securities class action lawsuit filed in February 2022 and voluntarily dismissed by the plaintiff in October 2022, against certain of our officers and certain of our current and former directors, and may become subject to additional securities class action lawsuits in the future. This type of litigation could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

***Our business is affected by macroeconomic conditions, including rising inflation, interest rates and supply chain constraints.***

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates, the risk of economic slowdown or recession in the United States, instability in the banking system, and overall economic conditions and uncertainties such as those resulting from the current and future conditions in the global financial markets, including the presidential transition in the United States. Recent supply chain constraints have led to higher inflation, which if sustained could have a negative impact on our product development and operations. If inflation or other factors were to significantly increase our business costs, our ability to develop our current pipeline and new therapeutic products may be negatively affected. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the operation of our business and our ability to raise capital on favorable terms, or at all, in order to fund our operations. Similarly, these macroeconomic factors could affect the ability of our third-party suppliers and manufacturers to manufacture clinical trial materials for our product candidates.

***We do not intend to pay dividends on our common stock, so any returns will be limited to the value of our stock.***

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

***We are a "smaller reporting company," and we cannot be certain if the reduced reporting requirements applicable to smaller reporting companies will make our common stock less attractive to investors.***

We are considered a "smaller reporting company" under Rule 12b-2 of the Exchange Act. We are therefore entitled to rely on certain reduced disclosure requirements, such as an exemption from providing selected financial data and executive compensation information. These exemptions and reduced disclosures in our SEC filings due to our status as a smaller reporting company also mean our auditors are not required to review our internal control over financial reporting and may make it harder for investors to analyze our results of operations and financial prospects. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our common stock prices may be more volatile. We will remain a smaller reporting company for as long as (i) the market value of our common stock held by non-affiliates is less than \$250.0 million or (ii) our annual revenue is less than \$100.0 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is less than \$700.0 million as of the prior June 30.

***Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.***

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates or grant licenses on terms unfavorable to us.

***We could be subject to significant legal proceedings which may adversely affect our results of operations or financial condition.***

We are subject to the risk of litigation, derivative claims, securities class actions, regulatory and governmental investigations and other proceedings, including proceedings arising from investor dissatisfaction with us or our performance or claims brought by employees, government agencies or suppliers. In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years. In addition, if any individuals acting on our behalf fails to satisfy his or her relevant legal or contractual duties, we could have liability to third parties, including the government or investors. If any claims were brought against us and resulted in a finding of substantial legal liability, the finding could materially adversely affect our business, financial condition or results of operations or cause significant reputational harm to us, which could seriously adversely impact our business. Allegations of improper conduct by private litigants or regulators, regardless of veracity, also may harm our reputation and adversely impact our ability to grow our business. Even if the allegations against us in future legal matters are unfounded or we ultimately are not held liable, the costs to defend ourselves may be significant and the litigation may subject us to substantial settlements, fines, penalties or judgments against us and may consume management's bandwidth and attention, some or all of which may negatively impact our financial condition and results of operations. Litigation also may generate negative publicity, regardless of whether the allegations are valid, or we ultimately are liable, which could damage our reputation, and adversely impact our sales and our relationship with our employees, customers, and partners. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

***If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.***

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. In the event that one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

**Item 1B. Unresolved Staff Comments.**

None.

## **Item 1C. Cybersecurity.**

### **Cyber Risk Management and Strategy**

We, under the oversight of the audit committee of our board of directors, have implemented and maintain a cybersecurity risk management program that is designed to identify, assess, and mitigate risks from cybersecurity threats. Our risk management is supported by various tools and technologies, including third-party security solutions, monitoring, and alerting tools, designed to monitor, identify, and address risks from cybersecurity threats.

We engage with other third-party providers and consultants to support our cyber risk management efforts, including through periodic security testing and assessments. We have a process to assess certain major third-party vendors and service providers, including through a review of responses to compliance questionnaires and contractual requirements, as appropriate.

We, like other companies in our industry, face a number of risks from cybersecurity threats in connection with our business. Although such risks have not materially affected, and we do not believe they are reasonably likely to materially affect, us, including our business strategy, results of operations or financial condition, to date, we have, from time to time, experienced threats to and security incidents related to our data and systems. For more information about the risks from cybersecurity threats we face, please see Item 1A- Risk Factors.

### **Governance Related to Cybersecurity Risks**

Our board of directors, as a whole and through its committees, has responsibility for the periodic review and oversight of information technology risks, including risks from cybersecurity threats.

The audit committee is responsible for oversight of our cyber risk management program. The day-to-day management of the cyber program is directed by the Director of IT under the direction of the Chief Financial Officer. Currently, the Director of IT reports to the Chief Financial Officer and provides periodic updates to the Chief Financial Officer on cyber matters.

The Director of IT meets with the Chief Financial Officer periodically to discuss and review our cybersecurity risk management processes and to address matters related to potential cybersecurity and information technology risks, with input from the Company's third-party technology providers, as appropriate. The Chief Financial Officer provides periodic reports on cybersecurity and information technology matters to the audit committee, which is responsible for reviewing and overseeing the Company's risk management process, including risks from cybersecurity threats, as set forth in the audit committee charter. The audit committee periodically reports on cybersecurity risk management to the full board of directors.

## **Item 2. Properties.**

Our corporate headquarters are located in Philadelphia, PA, where we lease 7,672 square feet of office space subject to a lease agreement that is in effect through June 30, 2026. We also have a lease consisting of approximately 13,179 square feet of laboratory space in Philadelphia, PA subject to a lease agreement that is in effect through August 31, 2026. We believe our facilities are currently adequate for us to conduct our business.

## **Item 3. Legal Proceedings.**

From time to time, we may be involved in lawsuits, claims, investigations and proceedings, consisting of intellectual property, commercial, employment and other matters which arise in the ordinary course of business. While the outcome of any such proceedings cannot be predicted with certainty, as of December 31, 2024, we were not party to any legal proceedings that we would expect to have a material adverse impact on our financial position, results of operations or cash flow.

## **Item 4. Mine Safety Disclosures.**

Not applicable.



## PART II

### **Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.**

Our common stock trades on The Nasdaq Global Select Market under the symbol “CABA”. Trading of our common stock commenced on October 25, 2019, in connection with our initial public offering, or IPO. Prior to that time, there was no established public trading market for our common stock.

#### **Stockholders**

As of March 27, 2025, we had approximately 68 holders of record of our common stock. The actual number of holders of our common stock is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. The number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

#### **Dividend Policy**

We have never declared or paid any cash dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and growth of our business. We do not expect to pay any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors and will depend on various factors, including applicable laws, our results of operations, financial condition, future prospects, then applicable contractual restrictions and any other factors deemed relevant by our board of directors. Investors should not purchase our common stock with the expectation of receiving cash dividends.

#### **Equity Compensation Plan**

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report on Form 10-K.

#### **Issuer Purchases of Equity Securities**

We did not purchase any of our equity securities during the period covered by this Annual Report on Form 10-K.

#### **Recent Sales of Unregistered Securities**

None.

#### **Item 6. Reserved.**

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

*You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K, or this Annual Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the section entitled "Risk Factors," our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. You should carefully read the section entitled "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements." We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.*

### Overview

We are a clinical-stage biotechnology company focused on the discovery and development of innovative engineered T cell therapies that have the potential to provide deep and durable, perhaps curative, responses with one-time administration for patients with autoimmune diseases. Our proprietary CABA<sup>®</sup>, or Cabaletta Approach to B cell Ablation, platform encompasses two strategies. Our CARTA, or Chimeric Antigen Receptor T cells for Autoimmunity, approach is designed to potentially reset the immune system. Our legacy CAART, or Chimeric AutoAntibody Receptor T cells, approach is designed to engineer T cells to selectively engage and eliminate only disease-causing B cells. We believe our CABA<sup>®</sup> platform has the potential to safely enable complete and durable responses for a broad range of autoimmune diseases and that it has potential applicability across dozens of autoimmune diseases that we have identified, evaluated and prioritized.

Resecabtagene autoleucel (rese-cel, formerly referred to as CABA-201) is a 4-1BB co-stimulatory domain-containing fully human CD19-CAR T construct designed to treat patients with a broad range of autoimmune diseases and our lead product candidate within the CARTA strategy. Rese-cel is designed to achieve transient and deep depletion of all B cells following a single treatment by using T cells engineered to express an antibody fragment that recognizes a B cell receptor expressed on the surface of all B cells. The construct is designed to allow for the deep elimination of all B cells, including all B cells that contribute to disease, with subsequent repopulation by healthy naïve B cells. This approach has the potential to reset the immune system, providing meaningful durable and complete clinical responses to patients off immunosuppressive therapies.

In March 2023, the United States Food and Drug Administration, or the FDA, granted clearance of our rese-cel Investigational New Drug, or IND, application for treatment of SLE in patients with active lupus nephritis, or LN, or active SLE without renal involvement. SLE is a chronic, potentially severe, autoimmune disease, most commonly impacting young women between the ages of 15 and 40 with higher frequency and more severity in people of color, where the immune system attacks healthy tissue throughout the body. SLE affects an estimated up to 320,000 patients in the U.S., and 150,000 patients in Europe, with LN as the most common end-organ manifestation, affecting approximately 40% of SLE patients. In May 2023, we announced the FDA granted Fast Track Designation for rese-cel, designed to deplete CD19-positive B cells and improve disease activity in patients with SLE and LN. The RESET-SLE<sup>™</sup> Phase 1/2 clinical trial of rese-cel is designed to treat six SLE patients with active LN, and in a separate parallel cohort, six patients with active SLE without renal involvement, with a single weight-based dose of  $1.0 \times 10^6$  cells/kg. In March 2024, Health Canada issued a No Objection Letter in response to a Clinical Trial Application, or CTA, for the RESET-SLE<sup>™</sup> trial submitted by Cabaletta, enabling us to begin the process to activate clinical trial sites and pursue patient enrollment for the RESET-SLE<sup>™</sup> trial in Canada. In October 2024, the European Medicines Agency allowed a CTA submitted by Cabaletta for the RESET-SLE<sup>™</sup> trial to proceed, enabling us to begin the process of activating clinical trial sites and pursuing patient enrollment for the RESET-SLE<sup>™</sup> trial. The trial is open for enrollment across multiple sites in the United States and one site in the European Union.

In May 2023, the FDA granted clearance of our rese-cel IND application for treatment of idiopathic inflammatory myopathies, or IIM, or myositis. Myositis refers to a group of autoimmune diseases characterized by inflammation and muscle weakness. The three myositis subtypes being evaluated in the RESET-Myositis<sup>™</sup> Phase 1/2 clinical trial of rese-cel affect approximately 70,000 patients in the U.S. and approximately 85,000 patients in Europe and typically affect middle-aged individuals, particularly women. The RESET-Myositis<sup>™</sup> clinical trial, which is actively enrolling patients, is designed to treat six patients with dermatomyositis, six patients with anti-synthetase syndrome, and six patients with immune-mediated necrotizing myopathy, all in separate parallel cohorts. We announced the FDA granted Fast Track Designation for rese-cel for the treatment of patients with dermatomyositis to improve disease activity and Orphan Drug Designation for rese-cel for the treatment of myositis in January and February 2024,

respectively. In March 2024, we announced the FDA granted Rare Pediatric Disease designation for rese-cel for juvenile dermatomyositis. The trial is open for enrollment across multiple sites in the United States and one site in the United Kingdom.

In October 2023, we announced that the FDA granted clearance of our rese-cel IND application for treatment of systemic sclerosis, or SSc. SSc is a rare and potentially fatal chronic autoimmune disease characterized by progressive skin and internal organ fibrosis that can be life-threatening, including interstitial lung disease, pulmonary hypertension, and scleroderma renal crisis. SSc affects approximately 90,000 patients in the U.S. and approximately 60,000 patients in Europe, typically middle-aged individuals, particularly women. The RESET-SSc™ Phase 1/2 clinical trial of rese-cel is designed to treat six patients with severe skin manifestations and six patients with severe organ involvement associated with SSc. We announced the FDA granted Fast Track Designation for rese-cel for the treatment of patients with SSc to improve associated organ dysfunction and Orphan Drug Designation for rese-cel for the treatment of systemic sclerosis in January and March 2024, respectively. The trial is open for enrollment across multiple sites in the United States.

In November 2023, the FDA granted clearance of our rese-cel IND application for treatment of generalized myasthenia gravis, or gMG, a subset of patients with myasthenia gravis, or MG. MG is a rare autoimmune disease characterized by autoantibodies that interfere with signaling at the neuromuscular junction, or NMJ, leading to potentially life-threatening muscle weakness. The majority of patients with MG have autoantibodies known to be pathogenic based on their interference with proteins in the NMJ, of which the majority target AChR. gMG affects approximately 55,000 patients in the U.S. and approximately 100,000 patients in Europe. Symptoms of gMG include profound muscle weakness throughout the body, disabling fatigue, and potential shortness of breath due to respiratory muscle weakness, with risk for episodes of respiratory failure. Standard of care therapies include cholinesterase inhibitors, steroids, immunomodulators, and biologics, which typically require chronic administration, increasing the risk of serious long-term side effects. The RESET-MG™ Phase 1/2 clinical trial of rese-cel is designed to treat six patients with AChR-positive gMG and six patients with AChR-negative gMG, each in separate parallel cohorts. The trial is open for enrollment across multiple sites in the United States.

In May 2024, we announced that we are working with active clinical sites to incorporate the RESET-PV™ trial as a sub-study within the Phase 1 DesCAARTes™ trial following the submission of a protocol amendment. The RESET-PV™ trial will evaluate rese-cel as a monotherapy without preconditioning in patients with mucosal pemphigus vulgaris, or mPV, and mucocutaneous pemphigus vulgaris, or mcPV. Approximately 15,000 patients in the U.S. and approximately 20,000 patients in Europe are affected by PV. The trial is open for enrollment across multiple sites in the United States.

In June 2024, we announced the initial clinical data from each of the first two patients dosed with rese-cel in the RESET-SLE™ and RESET-Myositis™ trials. In both of these patients, no cytokine release syndrome, or CRS, immune effector cell-associated neurotoxicity syndrome, or ICANS, infections or serious adverse events were observed through data cut-off of May 28, 2024. Rese-cel exhibited the anticipated profile of CAR T cell expansion and contraction with complete B cell depletion observed in both patients by day 15 post-infusion. Improvements in both patients' specific disease measures were observed, consistent with academic experience of a similar 4-1BB CD19-CAR T.

In late June 2024, a lupus nephritis patient with very active, refractory disease was dosed with rese-cel and experienced Grade 1 CRS and a protocol-defined dose-limiting toxicity of Grade 4 ICANS. The ICANS resolved rapidly following standard management. The study's Independent Data Monitoring Committee reviewed data from the patient, and recommended that the study proceed as designed, without delay, at the current dose. We recommended protocol modifications designed to improve patient safety, including enhanced monitoring for fever and neurologic symptoms along with seizure prophylaxis for all patients, in line with the practice at many academic sites including at Erlangen University, the site of the CD19-CAR T studies led by Dr. Georg Schett. In July 2024, we provided standard notice to the FDA and communicated details of the event and proposed protocol changes to all active clinical sites within the RESET™ clinical trial program, including the requirement that the investigator promptly discuss the appropriateness and timing of infusion with the trial's Medical Monitor.

In November 2024, we announced updated clinical data at the American College of Rheumatology Convergence 2024 conference in the first 8 patients dosed across the lupus, myositis and systemic sclerosis clinical trials. Clinical data from these patients in the RESET-SLE™, RESET-Myositis™ trial and RESET-SSc™ trial were presented in oral and poster presentations where compelling clinical responses were observed in lupus and myositis patients with up to six months of follow-up, and the first SSc patient demonstrated an emerging, drug-free clinical response.

In January 2025, we announced the FDA granted clearance of our rese-cel IND application for treatment of multiple sclerosis, or MS. In addition, we announced the FDA granted Fast Track Designation for rese-cel for the treatment of relapsing and progressive forms of MS. The RESET-MS™ trial is a Phase 1/2 open-label, dose escalation study of rese-cel in subjects with relapsing and progressive forms of MS, evaluated in separate cohorts. Approximately 750,000 patients in the U.S. and approximately 550,000 patients in Europe are affected by MS.

In February 2025, we announced updated clinical and translational data from the first 10 patients in the RESET™ clinical trial program, with a data cut off of January 8, 2025. Overall, clinical responses continued to deepen over three SLE patients in DORIS remission, the first LN patient achieving complete renal response, and the first dermatomyositis patient maintaining a major total improvement score, or TIS improvement. Each of these patients discontinued all immunosuppressants and were off or tapering steroids as of January 8, 2025. The safety profile continued to suggest a favorable risk-benefit in the first 10 patients dosed with 90% of patients having experienced either no CRS or Grade 1 CRS and 90% of patients having experienced no ICANS.

In March 2025, we learned of an important protocol deviation in the RESET-SSc™ trial. In this case, the patient reported a fever three days prior to infusion. The Cabaletta Medical Monitor was not notified by the investigator prior to the site infusing the patient. Nine days following rese-cel infusion, the patient experienced a dose-limiting toxicity of grade 3 immune effector cell-associated neurotoxicity syndrome (ICANS) based on a transient period of confusion. There was no cerebral edema, seizures or motor dysfunction associated with the ICANS event, and the patient was arousable throughout. The ICANS resolved rapidly following treatment with dexamethasone, and the patient was discharged without further symptoms. After data review, the Independent Data Monitoring Committee recommended that the trial proceed at the current dose without delay and endorsed our proposal to require investigators to affirmatively confirm in writing to the company the absence of fevers or evidence of infection. As of March 14, 2025, 56 clinical sites across the U.S. and Europe were actively recruiting with 33 patients enrolled across the RESET™ clinical trial program.

Our manufacturing strategy is comprised of two stages. The initial stage is designed to leverage the extensive early-stage manufacturing expertise of our academic partners and contract development and manufacturing organizations, or CDMOs, for rapid early development and clinical supply, and in parallel partner with commercially compliant CDMOs capable of supporting late-stage clinical studies and commercial production. Our aim is to achieve full manufacturing readiness through expanded CDMO relationships, establishment of our own manufacturing facilities, and/or through strategic partnership(s). We are leveraging the expertise in cell and vector manufacturing of our partners at the Children’s Hospital of Philadelphia, or CHOP, and the University of Pennsylvania, or Penn. For supply of lentiviral vector for the late-stage clinical and commercial manufacturing of rese-cel, we are working with Oxford Biomedica (UK) Limited, or Oxford. We have also collaborated with WuXi Advanced Therapies, Inc., or WuXi, to serve as an additional and commercially compliant cell manufacturing partner for our MusCAARTes™ and RESET™ clinical trials to meet planned capacity and international supply requirements. In July 2024, we entered into a new technology transfer agreement with Lonza Houston Inc., or Lonza, another commercially compliant CDMO, to execute a technology transfer of our expected commercial manufacturing process for rese-cel. In December 2024, we entered into a Development and Manufacturing Services Agreement, or the Lonza Agreement, with Lonza to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and preparations for commercial readiness. This improved process is nearly fully closed, semi-automated, and more easily scaled than the current process. Transfer of this updated process will be performed from Cabaletta to Lonza in anticipation of being able to supply rese-cel drug product for any of Cabaletta’s ongoing and planned RESET™ clinical trials. We plan to secure commercial, scalable manufacturing capabilities through multiple potential strategies, including expanding existing or establishing new CDMO relationships, leasing, building, qualifying and operating our own manufacturing facility, and/or establishing a strategic partnership to rapidly and reliably scale manufacturing by leveraging the partner’s manufacturing expertise. We believe this later stage will enable control of product development and commercial supply for products arising from our CABA™ platform, enabling us to achieve continuous improvement of our product candidates. Multiple members of our team have previously built and led organizations that have constructed and commissioned cell therapy facilities, which we believe will enable us to build our own manufacturing organizations and facilities, if desirable.

As part of our innovative manufacturing strategy where we intend to increase scale, reduce cost of goods and improve patient experience, in November 2023, we partnered with Cellares Corp., or Cellares, to evaluate their automated manufacturing platform, the Cell Shuttle™, through the Cellares Technology Adoption Program, or TAP. As part of the collaboration, the companies have agreed on a proof-of-concept technology transfer process for the automated manufacture and release testing of rese-cel. In August 2024, we expanded our partnership with Cellares to facilitate the potential to incorporate the Cellares manufacturing platform to support the RESET™ clinical program. In March 2025, we and Cellares announced the successful conclusion of the Technology Adoption Program on Cellares’ automated cell therapy manufacturing Cell Shuttle™, facilitating the potential integration of the Cell Shuttle™ into our clinical and commercial, if approved, manufacturing strategy for rese-cel. We are also evaluating pathways to improve patient experience and scalability of collection through the removal of the requirement for apheresis. Data were published at the American Society of Cell and Gene Therapy conference in May 2024 illustrating the feasibility of this approach.

We were incorporated in April 2017 and started principal operations in August 2018. Our operations to date have been financed primarily by proceeds from the sale of convertible notes and convertible preferred stock prior to our initial public offering, or IPO, and proceeds from the sale of our common stock in public equity offerings, including our IPO, “at-the-market” offerings and

follow-on offerings of shares of our common stock and pre-funded warrants. As of December 31, 2024, we had \$164.0 million in cash and cash equivalents.

## **Material Agreements**

Refer to section titled “Business – Our Material Agreements” included in this annual report on Form 10-K for a discussion of our material agreements.

## **Components of Operating Results**

### ***Revenue***

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sales of products for several years, if at all. If our development efforts for our current or future product candidates are successful and result in marketing approval, we may generate revenue in the future from product sales. We cannot predict if, when or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates.

We may also in the future enter into license or collaboration agreements for our product candidates or intellectual property, and we may generate revenue in the future from payments as a result of such license or collaboration agreements.

### ***Operating Expenses***

#### ***Research and Development***

Our research and development expenses include:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with consultants and third-party contract organizations that conduct research and development activities on our behalf;
- costs related to sponsored research service agreements;
- costs related to production of preclinical and clinical materials, including fees paid to contract manufacturers;
- licensing fees for intellectual property and know-how;
- laboratory and vendor expenses related to the execution of preclinical studies and ongoing and planned clinical trials; and
- laboratory supplies and equipment used for internal research and development activities and related depreciation expense.

We have not reported program costs since inception because historically we have not tracked or recorded our research and development expenses on a pre-clinical program-by-program basis. We use our personnel and infrastructure resources across the breadth of our research and development activities, which are directed toward identifying and developing product candidates.

We expense all research and development costs in the periods in which they are incurred. Costs for certain research and development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and third-party service providers.

We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in manufacturing, as our programs advance and we conduct clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Because of the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration and completion costs of the current or future preclinical studies and clinical trials or if, when, or to what extent we will generate revenues from the commercialization and sale of our product candidates. We may never succeed in achieving regulatory approval for our product candidates. The duration, costs and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- successful completion of preclinical studies and IND and/or CTA-enabling studies;
- development of chemistry, manufacturing and controls, or CMC, processes and procedures for purposes of IND and/or CTA applications;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the impact of any business interruptions to our operations, including the timing and enrollment of patients in our ongoing and planned clinical trials, or to those of our clinical sites, manufacturers, suppliers, or other vendors resulting from public health crises;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety and efficacy profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

We may never succeed in achieving regulatory approval for any of our product candidates. We may obtain unexpected results from our preclinical studies and clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on others. A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA or another regulatory authority, were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in execution of or enrollment in any of our preclinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of preclinical and clinical development. We expect our research and development expenses to increase for the foreseeable future as we continue the development of product candidates.

#### ***General and Administrative Expenses***

Our general and administrative expenses consist primarily of personnel costs, costs related to maintenance and filing of intellectual property, depreciation expense and other expenses for outside professional services, including legal, human resources, information technology, audit and accounting services. Personnel costs consist of salaries, benefits and stock-based compensation expense. We expect our general and administrative expenses to increase over the next several years to support our continued research and development activities, manufacturing activities, increased costs of operating as a public company and the potential commercialization of our product candidates. We anticipate our general and administrative costs will increase with respect to the hiring of additional personnel, developing commercial infrastructure, fees to outside consultants, lawyers and accountants, and increased costs associated with being a public company such as expenses related to services associated with maintaining compliance with Nasdaq listing rules and SEC requirements, insurance and investor relations costs.

#### ***Other Income***

Other income consists of interest earned on our cash, cash equivalents and short-term investments and amortization of bond discount or premium.

### *Interest Expense*

Interest expense consists primarily of interest expense associated with finance lease arrangements.

### **Results of Operations for the years ended December 31, 2024 and 2023**

The following sets forth our results of operations:

	Year Ended December 31,		Change
	2024	2023	
	(in thousands)		
<b>Statements of Operations Data:</b>			
Operating expenses:			
Research and development	\$ 97,203	\$ 55,424	\$ 41,779
General and administrative	27,938	19,236	8,702
Total operating expenses	125,141	74,660	50,481
Loss from operations	(125,141)	(74,660)	(50,481)
Other income:			
Interest income	10,025	6,985	3,040
Interest expense	(748)	—	(748)
Net loss	<u>\$ (115,864)</u>	<u>\$ (67,675)</u>	<u>\$ (48,189)</u>

### *Research and Development*

Research and development expenses were \$97.2 million for the year ended December 31, 2024 as compared to \$55.4 million for the year ended December 31, 2023. The table below summarizes our research and development expenses:

	Year Ended December 31,		Change
	2024	2023	
	(in thousands)		
License of intellectual property	\$ 1,523	\$ 2,383	\$ (860)
Manufacturing of preclinical and clinical supplies	15,523	8,498	7,025
Clinical trials	22,286	8,302	13,984
Personnel	39,239	22,375	16,864
Development services	16,417	12,486	3,931
Other	2,215	1,380	835
	<u>\$ 97,203</u>	<u>\$ 55,424</u>	<u>\$ 41,779</u>

Specific changes in our research and development expenses year over year include a:

- \$16.9 million increase in personnel costs primarily driven by an increase in headcount to support overall growth related to our rese-cel program, including an increase of \$4.6 million in stock-based compensation expense;
- \$14.0 million increase in clinical trial costs primarily due to rese-cel clinical trial costs;
- \$7.0 million increase in manufacturing costs primarily due to expanded cell processing capabilities and related activities;
- \$3.9 million increase in development services due to expanded lab space and related costs to support increased headcount performing research and translational activities;
- \$0.8 million increase in other primarily due to increased travel, conference and training costs and other costs to support headcount growth; partially offset by a



- \$0.9 million decrease in license of intellectual property costs due to a \$1.2 million upfront fee to Autolus pursuant to the Autolus Option and License Agreement and a \$1.0 million milestone payment to IASO for IND clearance of rese-cel in the first quarter of 2023 compared to a \$1.5 million milestone payment to IASO in the first quarter of 2024 for the first patient dosed in the rese-cel trial.

#### *General and Administrative Expenses*

General and administrative expenses were \$27.9 million for the year ended December 31, 2024 as compared to \$19.2 million for the year ended December 31, 2023. The increase of \$8.7 million in our general and administrative expenses year over year includes:

- \$6.5 million of additional personnel costs, primarily driven by an increase in headcount to support overall company growth, including an increase of \$3.5 million in stock-based compensation expense; and
- \$2.2 million higher administrative costs, including legal, information technology, and travel as well as other administrative costs.

#### *Other Income*

Interest income has increased \$3.0 million for the year ended December 31, 2024 as compared to the year ended December 31, 2023, primarily as a result of increasing interest rates on a higher cash and cash equivalents balance due to a financing in May 2023 and proceeds from sales of common stock pursuant to the ATM Program in the fourth quarter of 2023, first quarter of 2024 and fourth quarter of 2024.

#### *Interest Expense*

Interest expense has increased \$0.8 million for the year ended December 31, 2024 as compared to the year ended December 31, 2023, primarily as a result of finance lease arrangements from embedded leases within our WuXi manufacturing agreement.

#### **Liquidity, Capital Resources and Going Concern**

From our inception in April 2017 to the time of our initial public offering, or IPO, our operations were financed by proceeds of \$86.4 million from the sale of convertible notes and our convertible preferred stock and proceeds of \$71.0 million from the sale of common stock in our IPO. Since our IPO, we have generated cash from public offerings of our common stock and pre-funded warrants to purchase our common stock resulting in aggregate net proceeds of approximately \$280.0 million. As of December 31, 2024, we had \$164.0 million in cash and cash equivalents which should enable us to fund our operations and capital expenditures into the first half of 2026. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

We have incurred losses since our inception and, as of December 31, 2024, we had an accumulated deficit of \$349.1 million. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding prepaid expenses and other current assets, accounts payable and accrued expenses.

Any product candidates we may develop may never achieve commercialization and we anticipate that we will continue to incur losses for the foreseeable future. We expect that our research and development expenses, general and administrative expenses, and capital expenditures will continue to increase. As a result, until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research, manufacturing and development services, costs relating to the build-out of our headquarters, laboratories and manufacturing facility, license payments or milestone obligations that may arise, laboratory and related supplies, clinical costs, manufacturing costs, legal and other regulatory expenses and general overhead costs.

We evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about our ability to continue as a going concern within one year after the date that the consolidated financial statements are issued. Based on our current operating plan, we believe there is substantial doubt about our ability to continue as a going concern for at least twelve months following the filing of this Annual Report on Form 10-K, and we will need to obtain additional funding. Our cash forecast

contains estimates and assumptions based on success of ongoing clinical trials, and we cannot predict the amount or timing of all expenditures with certainty. We will continue to seek funds through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise capital, we will need to delay, reduce or terminate certain planned activities to reduce costs.

#### ***At-The-Market Offering Sales Agreement***

On March 21, 2024, we filed an automatic shelf registration statement (File No. 333-278126), or 2024 S-3ASR, in relation to the registration of common stock, preferred stock, debt securities, warrants and/or units of any combination thereof for the purposes of selling, from time to time, our common stock, debt securities or other equity securities in one or more offerings. We also simultaneously entered into a Sales Agreement, or the 2024 Sales Agreement, with Cowen and Company, LLC, or the Sales Agent, to provide for the offering, issuance and sale of up to an aggregate amount of \$200.0 million of our common stock from time to time in "at-the-market" offerings, or the 2024 ATM Program, under the 2024 S-3ASR and subject to the limitations thereof. We intend to convert the S-3ASR to S-3 by post-effective amendments in connection with the filing of this Annual Report. In December 2024, we sold 1,225,000 shares pursuant to the 2024 ATM Program for net proceeds of \$5.1 million, after deducting commissions of \$0.1 million.

We previously had a Sales Agreement with Cowen to provide for the offering, issuance and sale of up to an aggregate amount of \$100.0 million of common stock from time to time in "at-the-market" offerings, or 2023 ATM Program, pursuant to our shelf registration statement on Form S-3 (File No. 333-270599), which was declared effective April 26, 2023. During the year ended December 31, 2023, we sold 4,760,899 shares pursuant to the 2023 ATM Program for net proceeds of \$91.7 million, after deducting commissions of \$2.4 million. In the first quarter of 2024, we sold 258,070 additional shares, completing the 2023 ATM Program for net proceeds of \$5.7 million, after deducting commissions of \$0.1 million.

#### ***December 2022 Financing***

In December 2022, we issued 126,815 shares of our common stock at a price of \$5.52 per share and to certain investors in lieu of common stock, pre-funded warrants to purchase 6,213,776 shares of our common stock at a price of \$5.51999 per pre-funded warrant. The purchase price per share of each pre-funded warrant represents the per share offering price for the common stock, minus the \$0.00001 per share exercise price of such pre-funded warrant. Aggregate net proceeds were \$32.6 million after deducting underwriting discounts and commissions and offering expenses of \$2.4 million. As of December 31, 2024, 6,213,775 pre-funded warrants had been exercised and no warrants remain outstanding.

#### ***May 2023 Financing***

In May 2023, we issued 8,337,500 shares of our common stock in an underwritten public offering, including the exercise in full by the underwriters of their option to purchase an additional 1,087,500 shares, at a public offering price of \$12.00 per share. Aggregate net proceeds were \$93.8 million after deducting underwriting discounts and commissions and offering expenses of \$6.3 million.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, progress, results and costs of researching, developing and manufacturing our lead product candidates or any future product candidates, and conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals or clearances for our lead product candidates or any future product candidates;
- the impact of any business interruptions to our operations or to those of our clinical sites, manufacturers, suppliers, or other vendors resulting from public health crises;

- the number and characteristics of any additional product candidates we develop or acquire;
- the timing of any cash milestone payments if we successfully achieve certain predetermined milestones;
- the cost of manufacturing our lead product candidate or any future product candidates and any products we successfully commercialize, including costs associated with building-out our manufacturing capabilities;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such agreements that we may enter into;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company; and
- the timing, receipt and amount of sales of any future approved or cleared products, if any.

Further, our operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

### ***Cash Flows***

The following table summarizes our cash flows for the periods indicated:

	<b>Year Ended December 31,</b>	
	<b>2024</b>	<b>2023</b>
	<b>(in thousands)</b>	
Net cash provided by (used in):		
Operating activities	\$ (88,222)	\$ (53,549)
Investing activities	47,289	(22,451)
Financing activities	11,677	187,631
Effect of exchange rate changes on cash and cash equivalents	(20)	—
Net (decrease) increase in cash and cash equivalents	<u>\$ (29,276)</u>	<u>\$ 111,631</u>

### ***Operating Activities***

During the year ended December 31, 2024, cash used in operating activities of \$88.2 million was attributable to our net loss of \$115.9 million, partially offset by non-cash charges of \$26.0 million for stock-based compensation charges, amortization of finance lease, non-cash operating lease expense, depreciation, amortization of premium on investments, accretion of operating lease liabilities and foreign currency exchange rates and a net change of \$1.7 million in our net operating assets and liabilities.

During the year ended December 31, 2023, cash used in operating activities of \$53.5 million was attributable to our net loss of \$67.7 million, partially offset by non-cash charges of \$14.1 million for stock-based compensation charges, amortization of premium on investments, non-cash lease expense, accretion of lease liabilities and depreciation and a net change of \$0.1 million in our net operating assets and liabilities.

### ***Investing Activities***

During the year ended December 31, 2024, cash provided by investing activities of \$47.3 million was attributable to \$49.5 million from the maturity of short-term investments, partially offset by purchases of \$2.2 million of property and equipment.

During the year ended December 31, 2023, cash used in investing activities of \$22.5 million was attributable to \$84.3 million of purchases of investments and \$0.7 million of purchases of property and equipment, partially offset by \$62.5 million of proceeds from maturities of investments.

### ***Financing Activities***

During the year ended December 31, 2024, cash provided by financing activities of \$11.7 million was from \$10.9 million in sales of common stock, net of issuance costs paid, \$1.7 million from the exercise of employee stock and purchases of shares under our 2019 Employee Stock Purchase Plan, or 2019 ESPP, offset by \$0.9 million in principal payments on finance leases.

During the year ended December 31, 2023, cash provided by financing activities of \$187.6 million was from \$185.2 million in sales of common stock, net of issuance costs paid and \$2.4 million from the exercise of employee stock options and purchases of shares under our 2019 ESPP.

### **Contractual Obligations and Commitments**

We lease our headquarters office space and lab space under non-cancelable operating lease agreements. The lease term of our office space commenced in May 2019 and was amended in October 2024 for an additional 12 months through June 30, 2026. The lease term of our lab space was amended in September 2024 for 23 months through August 2026. See Note 9 of the consolidated financial statements for additional detail on our leases. We also have an embedded lease with WuXi, our cell processing manufacturing partner through August 2026. Total undiscounted aggregate future finance and operating lease obligations under all of our leases as of December 31, 2024 are \$17.1 million.

We have no material contractual obligations not fully recorded on our balance sheets or fully disclosed in the notes to the consolidated financial statements. Our commitments include:

- *IASO Exclusive License Agreement.* As partial consideration for the exclusive license, IASO received an upfront payment of \$2.5 million. IASO is also eligible to receive up to mid double digit millions in milestone payments based upon the achievement of specified pre-clinical, development and regulatory milestones, and up to an additional low triple digit millions in milestone payments based upon achievement of specified sales milestones, for a total consideration, inclusive of the upfront payment, of up to \$162 million, along with tiered mid-single digit royalties on future net sales for licensed products that may result from the IASO Agreement. We also may sublicense through multiple tiers the rights granted to it by IASO under the IASO Agreement at any time, however, we must pay IASO a low double-digit percentage of any revenue obtained from sublicenses or options to third parties, subject to certain customary exclusions. The IASO Agreement will continue on a country-by-country, licensed product-by-licensed product basis until the expiration of the royalty term as identified in the IASO Agreement, unless earlier terminated. A milestone payment of \$1.5 million was paid to IASO in the first quarter of 2024 after the first patient in a rese-cel trial was dosed.

- *Oxford Licence and Supply Agreement.* Under the terms of the agreement, we were required to pay Oxford an upfront fee, as well as costs associated with initial vector manufacturing activities for our DSG3-CAART program. In May 2023, we amended the LSA with Oxford to expand the license to include our rese-cel program for an upfront fee of \$0.5 million and in August 2023, we entered into a vector supply agreement with Oxford, and a related second amendment to the LSA, for rese-cel with a total cost of up to approximately \$5.0 million under the vector supply agreement. In February 2024, the Company and Oxford entered into a third amendment to the LSA to update the patent schedule. We can terminate the agreement at will upon advance written notice and subject to certain manufacturing slot cancellation fees.

- *Autolus Option and License Agreement.* In January 2023, we entered into an Option and License Agreement, or the Autolus Agreement, with Autolus Holdings (UK) Limited (Autolus), wherein the Autolus Agreement grants us a non-exclusive license to access Autolus' RQR8 technology in our CD19-CAR T cell therapy program, and subject to additional nominal option exercise fees, up to four additional targets. In January 2025, the Company and Autolus agreed to novate the Autolus Agreement with both Autolus and Autolus Limited jointly assuming the rights, obligations and liabilities of Autolus under the Autolus Agreement. Under the terms of the Autolus Agreement, we were required to pay Autolus an upfront license fee of \$1.2 million, of which \$1.1 million was paid in 2023 and \$0.1 million was paid in January 2024. Autolus is also eligible to receive regulatory milestones of up to \$12 million for each licensed target, sales milestones of up to a total of \$15 million and royalties in the low single digits on net sales of all products that incorporate the RQR8 technology. The Autolus Agreement will continue on a country-by-country, licensed product-by-licensed product basis until the expiration of the royalty term as identified in the Autolus Agreement, unless earlier terminated. We can terminate the Autolus Agreement at will upon advance written notice. Each of Autolus and us may terminate the Agreement for a material, uncured breach or insolvency of the other party.

•*The Penn License Agreement.* Under the License Agreement, we are required to make milestone payments upon successful completion of certain development, regulatory and sales milestones on a product-by-product and geographical basis. The payment obligations under the License Agreement are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones and we will be required to make development milestone payments and royalty payments in connection with the sale of products developed under the License Agreement. As of December 31, 2024, we are unable to estimate the timing or likelihood of achieving the milestones or making future product sales.

Under the License Agreement, we must use commercially reasonable efforts to develop and commercialize a product in each subfield. During the term of the License Agreement until the first commercial sale of the first product, we are obligated to pay Penn a non-refundable, non-creditable annual license maintenance fee of \$10,000. We are required to pay certain milestone payments upon the achievement of specified clinical and commercial milestones. Milestone payments are reduced by a certain percentage for the second product that achieves a milestone, by an additional percentage for the third product that achieves a milestone, and so on, for each subsequent product that achieves a milestone. In the event that we are able to successfully develop and launch multiple products under the License Agreement, total milestone payments could be approximately \$21.0 million. Penn is also eligible to receive tiered royalties at percentage rates in the low single-digits, subject to an annual minimum royalty, on annual worldwide net sales of any products that are commercialized by us or our sublicensees that contain or incorporate, or are covered by, the intellectual property licensed by us. To the extent we sublicense our license rights under the License Agreement, Penn would be eligible to receive tiered sublicense income at percentage rates in the mid-single to low double-digits. We have also entered into a subscription and technology transfer agreement with Penn, pursuant to which we owed Penn an upfront subscription fee, which was paid in 2019, and a nominal non-refundable royalty on net sales of products, a portion of which will be credited toward milestone payments and royalties under this License Agreement. Technology transfer activities would be at our cost and subject to agreement as to the technology to be transferred.

•*Master Translational Research Services Agreement with Penn.* Under the Services Agreement, we have contracted for additional research and development services from various laboratories within Penn. The Services Agreement will expire upon completion of the services for which we have engaged Penn under the Services Agreement. In May 2020, the Company amended its Addendum with CAROT to expand access to vector manufacturing. We may incur additional expenses up to \$0.6 million through the remaining term of the CAROT Amended Addendum. In February 2023, we entered into a second Master Translational Research Services Agreement with Penn, or the CARTA Services Agreement, pursuant to which Penn agreed to perform certain research, development and manufacturing activities. The services encompassed by the CARTA Services Agreement are performed by different organizations at Penn pursuant to certain addenda to the CARTA Services Agreement. The CARTA Services Agreement will expire on the later of (i) February 9, 2026 or (ii) completion of the services for which we have engaged Penn under the CARTA Services Agreement. Either party may terminate this agreement with or without cause upon a certain number of days' prior written notice. We are committed to pay up to \$3.0 million for cell processing manufacturing under the CARTA Services Agreement through December 31, 2025.

•*WuXi Manufacturing agreement.* In January 2021, we entered into the WuXi Agreement to serve as an additional cell processing manufacturing partner for the MusCAARTes<sup>TM</sup> trial. In August 2023, we entered into new work orders under the WuXi Agreement for WuXi to serve as one of our cell processing manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel. Under the August 2023 work orders, WuXi will convert our non-dedicated suite to a dedicated suite for GMP manufacturing for our rese-cel and MuSK-CAART programs for an initial term of 18 months with two 18 month extensions at our sole option on six months' notice prior to the end of the term. In addition, we agreed to certain monthly minimum runs. We would incur up to a \$1.08 million termination fee if we terminate both the rese-cel and MuSK-CAART work orders for any reason. We may terminate for convenience the WuXi Agreement or any work order with six months' prior written notice, however, we may not terminate the Dedicated Suite without terminating both the MuSK-CAART and rese-cel GMP run work orders.

•*Lonza Manufacturing agreement.* In December 2024, we entered into the Lonza Agreement with Lonza to serve as one of our manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and preparations for commercial readiness. Under the initial work order, Lonza will perform cell therapy manufacturing activities for our CAR-T cell therapy product rese-cel for a term of 12 months and total cost of approximately \$16.0 million with the ability to extend the manufacturing period subject to the terms of the Lonza Agreement.

•*Other Purchase Commitments.* In the normal course of business, we enter into various purchase commitments with third-party contract manufacturers for the manufacture and processing of our product candidates and related raw materials, contracts with contract research organizations for clinical trials and agreements with vendors for other services and products for operating purposes. These agreements generally provide for termination or cancellation other than for costs already incurred.

#### **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles, or GAAP. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing elsewhere in this Annual Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

#### ***Research and Development Costs***

We estimate costs of research and development activities conducted by service providers, which include the conduct of preclinical studies, contract manufacturing activities and clinical trial activities. We record the estimated costs of research and development activities based upon the estimated amount of services provided and include these costs in the accrued and other current liabilities on the balance sheets and within research and development expense on the statements of operations. Non-refundable advance payments made for goods or services that will be used or rendered for future research and development activities are deferred and capitalized and recognized as expense as the goods are received or the related services are rendered.

We estimate these costs based on factors such as estimates of the work completed and budget provided and in accordance with agreements established with our collaboration partners and third-party service providers. We make significant judgments and estimates in determining the accrued liabilities balance in each reporting period. As actual costs become known, we adjust our accrued liabilities. We have not experienced any material differences between accrued costs and actual costs incurred since our inception.

### **Smaller Reporting Company Status**

We are a “smaller reporting company,” as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to be a smaller reporting company if we have a public float in excess of \$250 million, or have annual revenues in excess of \$100 million and a public float in excess of \$700 million, determined on an annual basis. This status allows us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

### **Recently Adopted Accounting Pronouncements**

For a discussion of recently adopted accounting pronouncements please read Note 2, Summary of Significant Accounting Policies, to our consolidated financial statements included in this report.

### **Recently Issued Accounting Pronouncements**

For a discussion of recently issued accounting pronouncements please read Note 2, Summary of Significant Accounting Policies, to our consolidated financial statements included in this report.

**Item 7A. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities. We held cash and cash equivalents of \$164.0 million as of December 31, 2024. We generally hold our cash in interest-bearing money market treasury fund accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents. Declines in interest rates, however, would reduce future investment income.

We do not have any foreign currency or derivative financial instruments. Inflation generally affects us by increasing our cost of labor and program costs. Although we do not believe that inflation has had a material impact on our financial position or results of operations to date, we may experience some effect in the near future (especially if inflation rates continue to rise) due to an impact on the costs to conduct clinical trials, labor costs we incur to attract and retain qualified personnel, and other operational costs. Inflationary costs could adversely affect our business, financial condition and results of operations.

**Item 8. Consolidated Financial Statements and Supplementary Data.**

The consolidated financial statements required to be filed pursuant to this Item 8 are appended to this Annual Report. An index of those consolidated financial statements is found in Item 15.

**Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.**

There has been no change of accountants nor any disagreements with accountants on any matter of accounting principles or practices of financial disclosure required to be reported under this Item.



## **Item 9A. Controls and Procedures.**

### **Evaluation of Disclosure Controls and Procedures**

The Company has established disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) designed to ensure that information required to be disclosed in the reports that the Company files or submits under the Securities Exchange Act of 1934, as amended, or Exchange Act, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and is accumulated and communicated to management, including the principal executive officer (our Chief Executive Officer) and principal financial officer (our Chief Financial Officer), to allow timely decisions regarding required disclosure. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of December 31, 2024, the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures have been designed to provide reasonable assurance of achieving their objectives. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) were effective at the reasonable assurance level as of December 31, 2024.

### **Management's Annual Report on Internal Control Over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is a process designed to provide reasonable assurance of the reliability of financial reporting and of the preparation of consolidated financial statements for external reporting purposes, in accordance with U.S. generally accepted accounting principles.

Internal control over financial reporting includes policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect transactions and disposition of assets; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of consolidated financial statements in accordance with U.S. generally accepted accounting principles, and that receipts and expenditures are being made only in accordance with the authorization of its management and directors; and (3) provide reasonable assurance regarding the prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on its consolidated financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of the effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies and procedures included in such controls may deteriorate.

Our management has assessed the effectiveness of our internal control over financial reporting as of December 31, 2024. In making this assessment, management used the criteria established by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control - Integrated Framework (2013). These criteria are in the areas of control environment, risk assessment, control activities, information and communication, and monitoring. Management's assessment included extensive documentation, evaluating and testing the design and operating effectiveness of its internal controls over financial reporting.

Based on management's processes and assessment, as described above, management has concluded that, as of December 31, 2024, our internal control over financial reporting was effective.

### **Attestation Report of the Registered Public Accounting Firm**

As a non-accelerated filer, we are not required to provide an attestation report of our independent registered public accounting firm on the effectiveness of our internal control over financial reporting.

### Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) that occurred during the fiscal quarter ended December 31, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

### Item 9B. Other Information.

#### Rule 10b5-1 Trading Plans

During the fiscal quarter ended on December 31, 2024, none of our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as those terms are defined in Item 408(a) of Regulation S-K, except as described in the table below:

Name and Title	Action	Action Date	Duration of Trading Arrangements <sup>(1)</sup>	Rule 10b5-1 Trading Arrangement? (Y/N)*	Aggregate Number of Securities Subject to Trading Arrangement
Steven Nichtberger Chief Executive Officer	Terminate <sup>(2)</sup>	October 18, 2024	July 8, 2024 - July 31, 2025	Y	Up to 350,000 shares to be sold
Steven Nichtberger Chief Executive Officer	Adopt <sup>(2)</sup>	October 18, 2024	February 16, 2025 – July 27, 2026	Y	Up to 350,000 shares to be sold
Gwendolyn Binder President of Science and Technology	Terminate	November 21, 2024	February 20, 2024 – February 21, 2025	Y	Up to 100,756 shares to be sold
Gwendolyn Binder President of Science and Technology	Adopt	December 18, 2024	March 27, 2025 to December 12, 2025	Y	Up to 100,756 shares to be sold

\* Denotes whether the trading plan is intended, when adopted, to satisfy the affirmative defense of Rule 10b5-1(c).

(1) Except as indicated by footnote, each trading arrangement permitted or permits transactions through and including the earlier to occur of (a) the completion of all purchases or sales or (b) the date listed in the table.

(2) Represents the modification, as described in Rule 10b5-1(c)(1)(iv) under the Exchange Act, of a written trading arrangement adopted on November 9, 2023 that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.

### Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

## PART III

### Item 10. Directors, Executive Officers and Corporate Governance.

The following table sets forth information about our directors and executive officers as of March 27, 2025.

Name	Positions and Offices Held with Cabaletta	Officer/Director Since	Class and Year in Which Term Will Expire	Age
<b>Executive Officers</b>				
Steven Nichtberger, M.D.	Chief Executive Officer, President, Chairman of the Board of Directors and Director	2017	Class III—2025	63
Gwendolyn K. Binder, Ph.D.	President, Science and Technology	2019	—	50
Anup Marda, MBA	Chief Financial Officer	2019	—	47
David J. Chang, M.D., M.P.H.	Chief Medical Officer	2019	—	62
Michael Gerard, J.D.	General Counsel and Secretary	2021	—	45
Arun Das, M.D.	Chief Business Officer	2022	—	35
<b>Non-Employee Directors</b>				
Mark Simon, MBA (1)(2)(3)	Director	2018	Class III—2025	63
Scott Brun, M.D. (1)(3)	Director	2021	Class I—2026	57
Shawn Tomasello, MBA (2)	Director	2023	Class I—2026	66
Catherine Bollard, MBChB, M.D. (3)	Director	2019	Class II—2027	57
Richard Henriques, MBA (1)(2)	Director	2019	Class II—2027	69

(1) Member of the Audit Committee.

(2) Member of the Compensation Committee.

(3) Member of the Nominating and Corporate Governance Committee.

#### Executive Officers

**Steven Nichtberger, M.D.** Dr. Nichtberger has served as our Chief Executive Officer, President and Chairman of our board of directors since our founding in 2017. He is also a Professor of Professional Practice in the Vagelos LSM program and adjunct professor at The Wharton School of the University of Pennsylvania in addition to being a member of the board of directors of the BioAdvance Greenhouse Fund. Previously, Dr. Nichtberger served as president, chief executive officer, and a member of the board of directors of Tengion, Inc. from 2004 to 2011. In 2008, his leadership was recognized nationally with the National Ernst & Young Entrepreneur of the Year Award for emerging companies. Earlier in his career, Dr. Nichtberger served as the head of global marketing for Merck and in various commercial operating roles at the company from 1995 to 2003. He has previously served as a member of the boards of directors of the Alliance for Regenerative Medicine (as a founding member), Pennsylvania Bio (including as chairman), and Biotechnology Industry Organization. From 2008 to 2019, he was a member of the board of overseers for the School of Arts & Sciences at the University of Pennsylvania. Dr. Nichtberger received his M.D. from the State University of New York at Buffalo, his B.S. from The Wharton School at the University of Pennsylvania and his B.A. from the University of Pennsylvania. He completed internship, residency and fellowship in the Department of Medicine and the Division of Cardiology of the Mount Sinai Medical Center in New York. We believe Dr. Nichtberger is qualified to serve as a member and Chairman of our board of directors because of his scientific and medical background as well as his corporate leadership experience.

**Gwendolyn K. Binder, Ph.D.** Dr. Binder joined our company in February 2019 as our Executive Vice President, Science and Technology and was appointed President, Science and Technology in January 2022. Dr. Binder is an R&D executive with specialized expertise in the discovery and early clinical development and optimization of engineered T cell therapies. Prior to joining Cabaletta, Dr. Binder was the Chief Technology Officer of Adaptimmune Therapeutics Plc from March 2016 to January 2019, where she ultimately led the research team focusing on understanding mechanisms of tumor response and resistance and translating this understanding to next generation products and clinical study designs. In prior roles, from March 2011 to February 2016, she established and led the translational research group, in addition to the manufacturing and quality teams including oversight of the buildout of an in-house manufacturing facility. Earlier in her career, Dr. Binder served as Director of Operations for the Translational Research Program at the University of Pennsylvania, where she progressed multiple investigational new drug submissions for novel engineered T-cell therapies in HIV and oncology, including the CD19 CAR IND acquired by Novartis (now Kymriah), the first human gene editing IND, and three TCR engineered T cell therapy studies in oncology and HIV. Prior to that, she served as the

Director of Scientific Affairs at VIRxSYS Corporation in Gaithersburg Maryland where she supported the development of the first clinical lentiviral vector used in humans, for the application of engineered T cell therapy for HIV. In addition, Dr. Binder has served on the Board of Directors of Instil Bio (Nasdaq: TIL), a clinical-stage biopharmaceutical company focused on developing a pipeline of novel therapies including a lead PD-L1xVEGF bispecific antibody for solid tumors, since July 2020, and as a member of the Scientific Advisory Board for Immatics, which is a publicly traded company developing engineered T cell immunotherapy for cancer, since July 2020. Dr. Binder studied viral immunology and translational research at the Johns Hopkins University in Baltimore, Maryland where she earned a Ph.D. in Cellular and Molecular Medicine in 2002. She has authored over 30 publications in the field, including top international journals such as “Science”, and “Nature Medicine”. She is a recognized leader in the biotechnology sector for the translational and clinical advancement of novel T cell therapies for patients with serious diseases.

**Anup Marda, MBA.** Mr. Marda joined our company in January 2019 as our Chief Financial Officer. From April 2001 to January 2019, Mr. Marda held positions at Bristol-Myers Squibb, including, most recently, as Vice President, Head of Global Corporate Financial Planning & Analysis, and previously as Head of Finance, R&D Operations and Head of Finance, R&D Portfolio Management and Global Medical from 2014 to 2018. Prior to this, Mr. Marda held the role of Executive Director, U.S. Pharmaceuticals from 2012 to 2014 and Assistant Treasurer, Capital Markets from 2008 to 2012. Mr. Marda received his MBA from the Krannert School of Business of Purdue University and his B.Tech in Chemical Engineering from the Indian Institute of Technology Bombay.

**David J. Chang, M.D., M.P.H.** Dr. Chang joined our company in June 2019 as our Chief Medical Officer. From June 2015 to May 2019, he was Senior Vice President and Head, Inflammation, Autoimmunity & Neuroscience, or IA&NS, Global Medicines Development at AstraZeneca Pharmaceuticals LP, where he oversaw development projects of both biologics and small molecules in a variety of indications and led the late-stage clinical development organization for IA&NS. From October 2007 to June 2015, Dr. Chang served in various positions at GlaxoSmithKline plc, including VP and Head, Immuno-Inflammation, Clinical Development. While at AstraZeneca and GlaxoSmithKline, he provided leadership in driving the clinical development and registration of the only two products approved for systemic lupus erythematosus in the US in over 60 years. He received his M.D. from New York University School of Medicine, his M.P.H. from Emory University and his B.S. from Yale University. Dr. Chang completed his internship and residency in internal medicine at the New York Hospital—Cornell University Medical Center and fellowship in rheumatology at the Hospital for Special Surgery—Cornell University Medical Center.

**Michael Gerard, J.D.** Mr. Gerard joined our company in September 2021 as our General Counsel and Secretary. Prior to joining Cabaletta, Mr. Gerard served as Associate General Counsel of Spark Therapeutics, Inc., or Spark, from September 2020 to August 2021. Prior to Spark, Mr. Gerard served in various legal roles at Sandoz, Inc., a division of the Novartis Group, beginning in 2013, with the most recent role being Executive Director, Associate General Counsel, Business Development and Licensing, Strategy and Portfolio. Earlier in his career, Mr. Gerard served as Assistant General Counsel at Aramark and as a litigator at the law firms of Morrison & Foerster, LLP and K&L Gates, LLP in New York. Mr. Gerard received a B.A. in Political Science from the University of Michigan, Ann Arbor and a J.D. from Cornell Law School.

**Arun Das, M.D.** Dr. Das joined our company in July 2019 after serving as a consultant to the company since 2017, and served in several roles, most recently Executive Director of New Product Planning and Business Development, prior to being appointed Chief Business Officer in January 2022. He is also a Lecturer in the Health Care Management Department at the Wharton School of the University of Pennsylvania. Prior to joining Cabaletta, Dr. Das was a resident physician in General Pediatrics at the Children’s Hospital of Philadelphia from June 2018 to June 2019. Previously, he was an investment banking analyst in the healthcare group at Goldman Sachs from 2010 to 2012. In addition, Dr. Das has spent multiple years consulting in business development and operations for several start-ups in the biotechnology space. Dr. Das received his M.D. from Johns Hopkins University School of Medicine and a B.A. and B.S. dual degree from the University of Pennsylvania.

#### **Non-Employee Directors**

**Mark Simon, MBA.** Mr. Simon joined our board of directors in October 2018. He is a Strategic Advisor since 2020 to the Founder and CEO of Sun Pharmaceutical Industries Ltd., which is the largest pharmaceutical company in India, and a board observer of Sun Pharma Advanced Research Company Ltd., both public companies in India, where Mr. Simon is focused on accelerating their innovation initiatives. He is also a consultant to Stifel Nicolaus & Company (NYSE: SF) and co-founder of Torrey Partners, LLC, a global investment banking firm serving companies in the life sciences industry that Stifel acquired in 2023. Prior to co-founding Torrey, Mr. Simon was a managing director and head of life sciences investment banking at Citigroup from 2002 to 2005 where he covered global pharmaceutical companies with a focus on large-capitalization players in the U.S., Europe and Japan. Prior to Citigroup, Mr. Simon served as a Managing Director at Robertson Stephens in San Francisco and New York where he was responsible for covering the biotechnology investment banking sector. During his tenure overseeing the group, he was involved in raising 45% of all biotechnology dollars raised in the U.S. from 1999-2001. Prior to joining the investment banking group, Mr.

Simon was a senior biotechnology research analyst at Robertson Stephens, and was named a Wall Street All-Star Analyst for three years in a row. He began his career at Kidder Peabody as a biotechnology research associate in 1984 and participated in the IPO of Genzyme and the early financings of companies such as Amgen. Mr. Simon currently serves on the boards of directors of several disease advocacy and philanthropic foundations in the oncology, CNS/neuroscience and metabolic areas. He previously served on the board of directors of Portage Biotech Inc. (Nasdaq: PRTG) until April 2024. Mr. Simon received his MBA from Harvard Business School and his B.A. from Columbia College. We believe that Mr. Simon is qualified to serve on our board of directors because of his extensive experience of over 35 years in the life sciences industry and biotechnology and specialty pharmaceutical transactions.

**Scott Brun, M.D.** Dr. Brun joined our board of directors in June 2021. Dr. Brun is currently President at Gold Mast Consulting, LLC, an advisory firm he founded in 2019 to provide technical advice and strategic guidance related to biopharmaceutical research and development, pipeline portfolio management, commercialization of new therapeutics and strategic communications related to R&D activities. Dr. Brun serves as a Venture Partner at Abingworth LLP and a Senior Medical Advisor to Launch Therapeutics. Prior to his current roles, Dr. Brun had two decades of experience in various leadership roles at AbbVie, Inc., including 15 years at the predecessor company, Abbott Laboratories. The majority of his career has been focused on leading teams and clinical development organizations across a broad variety of therapeutic areas including autoimmune, neurologic, and renal among others. He was most recently Corporate Vice President of Scientific Affairs and Head of AbbVie Ventures, a corporate venture fund responsible for investment opportunities within AbbVie's R&D therapeutic areas as well as technology platforms of interest from 2016 to 2019. Previously, Dr. Brun served as Corporate Vice President and Head of Pharmaceutical Development at AbbVie from 2013 to 2016. During his tenure at AbbVie, Dr. Brun oversaw a global organization with responsibilities for AbbVie's entire portfolio of early and late-stage clinical pre-registration pipeline compounds as well as marketed compounds within oncology, neurology, immunology, renal, infectious disease, and women's and men's health therapeutic areas. Prior to joining AbbVie, Dr. Brun spent over 15 years at Abbott Laboratories, where he held positions of increasing leadership responsibility in drug development within the R&D organization. Dr. Brun is a member of the boards of directors of Axial Biotherapeutics, Inc. and Trishula Therapeutics, Inc., both private, clinical-stage biopharmaceutical companies, Jasper Therapeutics, Inc. (Nasdaq: JSRP) a biotechnology company focused on targeting mast cells and stem cells to treat chronic diseases and Forte Biosciences, Inc. (Nasdaq: FBRX) a preclinical-stage company focused on autoimmune diseases. Previously, Dr. Brun served as a Senior Advisor to the business development team at Horizon Therapeutics plc (Nasdaq: HZNP) from 2020 to 2023. Dr. Brun received his B.S. in Biochemistry from the University of Illinois at Urbana-Champaign and earned his M.D. from the Johns Hopkins University School of Medicine. He completed his residency in ophthalmology at the Massachusetts Eye and Ear Infirmary, Harvard Medical School. We believe Dr. Brun is qualified to serve as a member of our board of directors because of his scientific background and significant experience in clinical and research efforts.

**Shawn Tomasello, MBA.** Ms. Tomasello joined our Board of Directors in July 2023. She is currently Chief Executive Officer at CoRegen, Inc., a private company. Prior to her current role, Ms. Tomasello served as the Chief Commercial Officer of Kite Pharma, where she oversaw the global commercialization of Yescarta, from 2015 to 2018 including through its acquisition by Gilead for \$11.9 billion in October 2017. She was previously Chief Commercial Officer at Pharmacyclics, where she led commercial and medical affairs activities for Imbruvica®, a first-in-class treatment for hematologic malignancies, from August 2014 until its acquisition by AbbVie for \$21.0 billion in August 2015. Prior to Pharmacyclics, Ms. Tomasello served in leading commercial roles with multiple major pharmaceutical companies, including Celgene as President of the Americas Hematology and Oncology, where she led the company through five successful product launches encompassing 11 indications and played a critical role in acquisitions. At Genentech for 14 years prior, Ms. Tomasello was the National Director of Hematology for Rituxan. She currently serves on the board of directors of AlloVir, Inc. (Nasdaq: ALVR) since March 2022, 4D Molecular Therapeutics Inc. (Nasdaq: FDMT) since November 2020 and Centrexion Therapeutics Corp., a private, clinical-stage biopharmaceutical company, since 2017. She previously served as a board member of TCR2 Therapeutics Inc. (Nasdaq: TCRR) until June 2023, Urogen Pharma Ltd (Nasdaq: URGN) until 2022, Mesoblast, Ltd. (Nasdaq: MESO) until August 2022, Clementia Pharmaceuticals, Diplomat Pharmacy, Inc. until 2020, Abeona Therapeutics (Nasdaq: ABEO) until 2020, Principia Biopharma until 2020, and Gamida Cell Ltd. (Nasdaq: GMDA) until 2024, as well as several private boards. On such boards, she at various times has served and serves on the Compensation, Nominating and Corporate Governance, Compliance, and Transaction committees. Ms. Tomasello received her B.S. in Marketing from the University of Cincinnati and her M.B.A. from Murray State University in Kentucky. We believe Ms. Tomasello is qualified to serve as a member of our Board of Directors because of her experience serving in various commercial roles in the life sciences industry.

**Catherine Bollard, MBChB, M.D.** Dr. Bollard joined our board of directors in April 2019. She currently serves as Senior Vice President and Chief Research Officer and Director of the Center for Cancer and Immunology Research at the Children's National Research Institute (CNRI). She is also a faculty member of the Division of the Blood and Marrow Transplantation at CNH. Since 2013, Dr. Bollard has served as a tenured Professor of Pediatrics and Microbiology, Immunology and Tropical Medicine at the George Washington University and is the Associate Center Director (ACD) for Translational Research and Innovation within the George Washington Cancer Center. Prior to her move in 2013, Dr. Bollard was Professor of Pediatrics, Medicine and

Immunology at Baylor College of Medicine. She is a past president of the International Society for Cell Therapy (ISCT), and she served on the Cellular, Tissues and Gene Therapy Advisory Committee of the FDA until 2019. Dr. Bollard chaired the Non-Hodgkin's Lymphoma Committee of the Children's Oncology Group from 2012 to 2020 and in 2021 became the President of the Foundation for the Accreditation of Cellular Therapy (FACT). Dr. Bollard was the scientific co-founder of Catamaran Bio, a company developing novel, off-the-shelf chimeric antigen receptor (CAR)-NK cell therapies designed to treat a broad range of cancers and served on its Scientific Advisory Board until 2024. Dr. Bollard was previously on the board of directors and subsequently the Scientific Advisory Board of Mana Therapeutics. She currently serves on the SAB of Minovia Tx Ltd and serves as Chair of the DSMB for SOBI. Bollard also serves on the board of directors for Alliance for Regenerative Medicine and a nonprofit organization she co-founded called Access4Kids. Dr. Bollard received her medical degree (MBChB) and her doctor of medicine (MD) from the University of Otago Medical School in New Zealand. We believe Dr. Bollard is qualified to serve as a member of our board of directors because of her scientific background and significant experience in clinical and translational research efforts.

**Richard Henriques, MBA.** Mr. Henriques joined our board of directors in February 2019. Since 2015, he has been a senior fellow at the Center for High Impact Philanthropy at the University of Pennsylvania, with a focus on impact investing. From 2010 to 2014, Mr. Henriques was the Chief Financial Officer of the Bill & Melinda Gates Foundation, where he was responsible for finance and accounting, financial planning and analysis, strategic planning, measurement and evaluation, program related investments and information technology. From 1981 to 2008, Mr. Henriques held positions at Merck & Co., Inc., including as Senior Vice President of Finance for Global Human Health, Vice President and Corporate Controller, and Principal Accounting Officer. Mr. Henriques serves on the boards of directors or trustees of the Bucks County Playhouse, International Vaccine Institute, Heritage Conservancy, Delaware Canal 21, Fisherman's Mark and The Franklin Institute. Mr. Henriques served on the board of directors of Arbutus Biopharma Corporation (Nasdaq: ABUS) from December 2014 to February 2025. Mr. Henriques received his MBA from the Wharton School of the University of Pennsylvania and a BA from the University of Pennsylvania. We believe that Mr. Henriques is qualified to serve as a member of our board of directors because of his extensive corporate leadership experience and expertise in finance and investment management.

#### **Family Relationships and Legal Proceedings**

There are no family relationships between or among any of our directors or executive officers. Our executive officers are appointed by, and serve at the discretion of, our board of directors. The principal occupation and employment during the past five years of each of our directors was carried on, in each case except as specifically identified above, with a corporation or organization that is not a parent, subsidiary or other affiliate of us. There is no arrangement or understanding between any of our directors and any other person or persons pursuant to which he or she is to be selected as a director.

There are no material legal proceedings to which any of our directors, officers or affiliate, any owner of record or beneficially of more than five percent of any class of our voting securities, or any associate of any such director, officer, affiliate, or security holder is a party adverse to us or any of our subsidiaries or in which any such person has a material interest adverse to us or our subsidiary.

#### **Board Committees**

Our board of directors has established an audit committee, a compensation committee, and a nominating and corporate governance committee. Each of the audit committee, compensation committee, and nominating and corporate governance committee operates under a charter that satisfies the applicable standards of the SEC and Nasdaq. Our board of directors has also established a science and technology committee, an advisory committee of the board of directors which operates under a charter duly adopted by the board of directors. Each such committee reviews its respective charter at least annually. A current copy of the charter for each of the audit committee, compensation committee, nominating and corporate governance committee and science and technology committee is posted on the corporate governance section of our website, [www.cabalettabio.com/investors/corporate-governance/governance-documents](http://www.cabalettabio.com/investors/corporate-governance/governance-documents). From time to time, our board of directors may establish certain committees for scientific, business or other matters.

##### ***Audit Committee***

Scott Brun, M.D., Richard Henriques, MBA and Mark Simon, MBA serve on the audit committee, which is chaired by Richard Henriques, MBA. Our board of directors has determined that each member of the audit committee is "independent" for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and each has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated Mr. Henriques as an "audit

committee financial expert,” as defined under the applicable rules of the SEC. During the fiscal year ended December 31, 2024, the audit committee met seven times. The audit committee’s responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee’s review and discussions with management and our independent registered public accounting firm whether our audited financial statements and our related disclosures under the “Management’s Discussion and Analysis” section shall be included in our 2024 Annual Report;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions;
- reviewing quarterly earnings releases; and
- discussing the Company’s risk assessment and management guidelines, including the Company’s major financial risk exposures, cybersecurity risks and the steps that the Company’s management has taken to monitor and control such exposures and risks.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

#### ***Compensation Committee***

Richard Henriques, MBA, Mark Simon, MBA and Shawn Tomasello, MBA serve on the compensation committee, which is chaired by Mark Simon, MBA. Our board of directors has determined that each member of the compensation committee is “independent” as defined in the applicable Nasdaq rules. During the fiscal year ended December 31, 2024, the compensation committee met four times. The compensation committee’s responsibilities include:

- annually reviewing and recommending to the board of directors corporate goals and objectives relevant to the compensation of our chief executive officer;
- evaluating the performance of our chief executive officer in light of such corporate goals and objectives and based on such evaluation (i) recommending to the board of directors the cash compensation of our chief executive officer and (ii) reviewing and approving grants and awards to our chief executive officer under equity-based plans;
- reviewing and approving the compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy, and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- reviewing and making recommendations to our board of directors about our policies and procedures for the grant of equity-based awards;

- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement;
- reviewing and discussing annually with management our “Compensation Discussion and Analysis,” if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention or termination of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

#### *Nominating and Corporate Governance Committee*

Catherine Bollard, MBChB, M.D., Scott Brun, M.D., and Mark Simon, MBA, serve on the nominating and corporate governance committee, which is chaired by Scott Brun, M.D. Our board of directors has determined that each member of the nominating and corporate governance committee is “independent” as defined in the applicable Nasdaq rules. During the fiscal year ended December 31, 2024, the nominating and corporate governance committee met two times. The nominating and corporate governance committee’s responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board’s committees;
- reviewing and discussing with the board of directors corporate succession plans for our chief executive officer and other key officers;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines; and
- overseeing the evaluation of our board of directors.

The nominating and corporate governance committee considers candidates for board of directors membership suggested by its members and the chief executive officer. Additionally, in selecting nominees for directors, the nominating and corporate governance committee will review candidates recommended by stockholders in the same manner and using the same general criteria as candidates recruited by the committee and/or recommended by our board of directors. Any stockholder who wishes to recommend a candidate for consideration by the committee as a nominee for director should follow the procedures described in our proxy statement under the heading “Stockholder Proposals.” The nominating and corporate governance committee will also consider whether to nominate any person proposed by a stockholder in accordance with the provisions of our bylaws relating to stockholder nominations as described in our proxy statement under the heading “Stockholder Proposals.”

*Identifying and Evaluating Director Nominees.* Our board of directors is responsible for filling vacancies on our board of directors and for nominating candidates for election by our stockholders each year in the class of directors whose term expires at the relevant annual meeting. The board of directors delegates the selection and nomination process to the nominating and corporate governance committee, with the expectation that other members of the board of directors, and of management, will be requested to take part in the process as appropriate.

Generally, the nominating and corporate governance committee identifies candidates for director nominees in consultation with management, through the recommendations submitted by stockholders or through such other methods as the nominating and corporate governance committee deems to be helpful to identify candidates. Once candidates have been identified, the nominating and corporate governance committee confirms that the candidates meet all of the minimum qualifications for director nominees established by the nominating and corporate governance committee. The nominating and corporate governance committee may gather information about the candidates through interviews, detailed questionnaires, comprehensive background checks or any other means that the nominating and corporate governance committee deems to be appropriate in the evaluation process. The nominating and corporate governance committee then meets as a group to discuss and evaluate the qualities and skills of each candidate, both



on an individual basis and taking into account the overall composition and needs of our board of directors. Based on the results of the evaluation process, the nominating and corporate governance committee recommends candidates for the board of directors' approval to fill a vacancy or as director nominees for election to the board of directors by our stockholders each year in the class of directors whose term expires at the relevant annual meeting.

#### ***Science and Technology Committee***

Catherine Bollard, MBChB, M.D., Scott Brun, M.D., and Shawn Tomasello, MBA, serve on the science and technology committee, which is chaired by Catherine Bollard, MBChB, M.D. The science and technology committee supports Board oversight of the Company's research and development, manufacturing and supply activities, by providing a forum for review of strategic considerations and issues in such areas, as well as to evaluate relevant emerging technology and advances in the field. The science and technology committee was formed in July 2023 and held four meetings in during the fiscal year ended December 31, 2024.

#### **Code of Business Conduct and Ethics**

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on the corporate governance section of our website, which is located at [www.cabalettabio.com/investors/corporate-governance/governance-documents](http://www.cabalettabio.com/investors/corporate-governance/governance-documents). If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

#### **Insider Trading Policies and Procedures**

We have adopted an insider trading policy that governs the purchase, sale, and/or other transactions of our securities by our directors, officers, employees and designated consultants that we believe is reasonably designed to promote compliance with applicable insider trading laws, rules and regulations, and listing standards applicable to us. A copy of our insider trading policy is filed as Exhibit 19.1 to this Annual Report on Form 10-K for the fiscal year ended December 31, 2024. In addition, with regard to trading in our own securities, it is our policy to comply with the federal securities laws and the applicable exchange listing requirements.

Our insider trading policy prohibits our directors, officers, employees and certain designated consultants, and their affiliated persons, from trading in company securities while in possession of material nonpublic information about our company. The policy also prohibits tipping (i.e., disclosing material nonpublic information about our company to others who may trade on the basis of that information). Under our insider trading policy, designated insiders may only trade in company securities at a time when they do not possess material nonpublic information about our company. We also require our directors, officers and certain other employees to receive approval before trading in company securities. Our insider trading policy also expressly prohibits short sales; purchases or sales of puts, calls, or other derivative securities or hedging transactions; using company securities as collateral in a margin account; or pledging company securities as collateral for a loan. Any waiver of the provisions of this policy requires the approval of our Audit Committee. To date, no such requests have been made or approved. We have also adopted an additional policy that governs adoption, modification and termination of written securities trading plans, known as Rule 10b5-1 plans, by our directors, executive officers and certain other persons. Our policy provides that all Rule 10b5-1 plans must comply with SEC rules applicable to the Rule 10b5-1 safe harbor and imposes additional requirements and limitations.

## Item 11. Executive Compensation.

Our named executive officers for the year ended December 31, 2024 are:

- Steven Nichtberger, M.D., our President and Chief Executive Officer;
- Gwendolyn Binder, Ph.D., our President, Science and Technology; and
- David J. Chang, M.D., M.P.H., our Chief Medical Officer.

### 2024 Summary Compensation Table

The following table presents the compensation awarded to, earned by or paid to each of our named executive officers for the years indicated. Dr. Nichtberger also serves as a member of our board of directors but does not receive any additional compensation for his service as a director.

	Year	Salary (\$)	Option Awards (\$)(1)	Non-Equity Incentive Plan Compensation (\$)(2)	All Other Compensation (\$)(3)	Total (\$)
Steven Nichtberger, M.D. <i>President and Chief Executive Officer</i>	2024	646,328	7,008,417	428,157	14,045	8,096,947
	2023	597,417	3,784,529	412,288	14,045	4,808,279
Gwendolyn K. Binder, Ph.D. <i>President, Science and Technology</i>	2024	529,687	2,581,003	246,869	—	3,357,559
	2023	477,417	1,618,263	245,203	—	2,340,883
David J. Chang, M.D., M.P.H. <i>Chief Medical Officer</i>	2024	496,833	2,581,003	268,290	—	3,346,126
	2023	476,910	1,379,202	244,940	—	2,101,052

(1) Amounts reflect the grant date fair value of option awards granted in 2024 and 2023 in accordance with the Financial Accounting Standards Board's Accounting Standards Codification Topic 718, or ASC 718. Such grant date fair value does not take into account any estimated forfeitures related to service-vesting conditions. For information regarding assumptions underlying the valuation of equity awards, see Note 10 to our financial statements for the year ended December 31, 2024. These amounts do not correspond to the actual value that may be recognized by the named executive officers upon vesting of applicable awards.

(2) The amounts represent bonuses earned in 2024 and 2023 and paid in 2025 and 2024, respectively, pursuant to the Company's cash incentive plan based on the factors described under "Non-Equity Incentive Plan Compensation" below.

(3) Amount includes reimbursements for life insurance premiums.

### Narrative to 2024 Summary Compensation Table

Our board of directors and compensation committee review compensation annually for our chief executive officer and his direct reports. In setting executive base salaries and bonuses, and granting equity incentive awards, our board of directors considers compensation for comparable positions in the market, the historical compensation levels of our executives, individual performance as compared to our expectations and objectives, our desire to motivate our employees to achieve short-and long-term results that are in the best interests of our stockholders, and a long-term commitment to our company. We target a general competitive position, based on independent third-party benchmark analytics to inform the mix of compensation of base salary, bonus or long-term incentives.

Our board of directors has historically determined our executives' compensation. Our compensation committee typically reviews and discusses management's proposed compensation with the chief executive officer for all of his direct reports. Based on those discussions and its discretion, taking into account the factors noted above, the compensation committee determines the compensation for each executive officer other than the chief executive officer. Our board of directors discusses the compensation committee's recommendations and ultimately approves the compensation of our chief executive officer without the chief executive officer and chief financial officer present. In 2024, the compensation committee continued to retain the services of Radford, an AON Hewitt company, as its external compensation consultant and the board of directors and the compensation committee considered Radford's input on certain compensation matters as they deemed appropriate.

### Annual base salary

Each named executive officer's base salary is a fixed component of annual compensation for performing specific duties and functions, and has been established by our board of directors taking into account each individual's role, responsibilities, skills, and

experience. Base salaries for our named executive officers are reviewed annually by our compensation committee, typically in connection with our annual performance review process, and adjusted from time to time, based on the recommendation of the compensation committee, to realign salaries with market levels after taking into account individual responsibilities, performance, and experience. The base salary of each named executive officer in 2024 is noted below:

Name	2024 Base Salary through February 29, 2024	Base Salary Effective March 1, 2024
Steven Nichtberger, M.D.	602,200	638,300
Gwendolyn K. Binder, Ph.D.	480,500	500,200
David J. Chang, M.D., M.P.H.	480,000	500,200

#### *Non-Equity Incentive Plan Compensation*

Annual cash awards are determined based on company performance, which is assessed based on predetermined annual objectives, and on individual performance. When determining the individual component of our annual incentive awards, the compensation committee considers each executive's contributions to the company's strategic achievements and financial and operational performance. In addition, the compensation committee considers how each executive demonstrates Cabaletta behaviors.

Each of our named executive officers was eligible to receive a bonus with respect to 2024 as a percentage of base salary listed below, modified by company and individual performance.

Name	2024 Target Bonus Rate (% of base salary)
Steven Nichtberger, M.D.	55 %
Gwendolyn K. Binder, Ph.D.	40 %
David J. Chang, M.D., M.P.H.	40 %

Company performance was assessed as achievement of 2024 company objectives, including certain clinical development, business development and financial and operational goals and development of a strong team culture. As a result, the compensation committee recommended and our board of directors approved 2024 company achievement at 108%.

The compensation committee recommended (and/or approved) and the board of directors approved (as applicable, in the instance of the chief executive officer), a further adjustment of bonuses based on the individual achievement of each of the named executive officers, as noted below:

Name	Individual Achievement (%)
Steven Nichtberger, M.D.	114 %
Gwendolyn K. Binder, Ph.D.	115 %
David J. Chang, M.D., M.P.H.	125 %

#### *Long-term equity incentives*

Our equity grant program is intended to align the interests of our named executive officers with those of our stockholders and to motivate them to make important contributions to our performance. In 2024, we granted stock options to each of our named executive officers, as reflected in the "Outstanding Equity Awards at 2024 Fiscal Year End Table" below.

#### *Employment arrangements with our named executive officers*

In October 2019, we entered into amended and restated employment agreements with each of our named executive officers. Each of our named executive officers is employed at will.

#### ***Steven Nichtberger, M.D.***

Under his amended and restated employment agreement, Dr. Nichtberger is entitled to an annual base salary, which is subject to review and redetermination by our board of directors or our compensation committee, and he is eligible to receive an annual bonus determined by our board of directors or the compensation committee (subject to the terms of any applicable incentive compensation plan that may be in effect from time to time), with a target amount calculated as a percentage of his annual base salary. Dr.

Nichtberger is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans, and is reimbursed for his existing, personal life insurance policy premiums.

Dr. Nichtberger's employment agreement provides that, in the event that his employment is terminated by us without "cause" or by him for "good reason," then subject to the execution and effectiveness of a separation agreement and release and compliance with his continuing obligations, he will be entitled to receive (i) an amount equal to (x) 12 months of base salary payable on our normal payroll cycle if such termination occurs outside of the period commencing 60 days before and ending 12 months after the occurrence of the first event constituting a "change in control", or the Change in Control Period, or (y) 1.5 times the sum of his base salary plus his target bonus for the then-current year payable on our or our successor's normal payroll cycle over 18 months if such termination occurs within Change in Control Period; (ii) payment of the monthly employer COBRA premium for the same level of group health coverage as in effect for Dr. Nichtberger on the date of termination for up to (x) 12 months if such termination occurs outside of the Change in Control Period, and (y) 18 months if such termination occurs within the Change in Control Period; and (iii) full acceleration of all stock options and other stock-based awards held by Dr. Nichtberger if such termination occurs within the Change in Control Period. In addition to standard good reason triggers, Dr. Nichtberger may elect to resign for good reason if within six months following the appointment of a chairperson of the board of directors, Dr. Nichtberger provides written notice of his reasonable dissatisfaction with such chairperson and the board fails to designate a new chairperson within 30 days of such notice. In the event of such a resignation for good reason outside of the Change in Control Period, subject to the execution and effectiveness of a separation agreement and release and compliance with his continuing obligations, in addition to the severance benefits described above, all stock options and other stock-based awards held by Dr. Nichtberger will accelerate and become fully exercisable or nonforfeitable.

The payments and benefits provided to Dr. Nichtberger under his employment agreement in connection with a sale event may not be eligible for a federal income tax deduction for the company pursuant to Section 280G of the Code. These payments and benefits also may be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Dr. Nichtberger in connection with a sale event would be subject to the excise tax on golden parachutes imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after tax benefit to Dr. Nichtberger.

In addition, Dr. Nichtberger has entered into a non-competition, non-solicitation, confidentiality and assignment agreement with us that contains, among other things, non-competition and non-solicitation provisions that apply during the term of Dr. Nichtberger's employment and for 12 months thereafter.

***Gwendolyn K. Binder, Ph.D.***

Under her amended and restated employment agreement, Dr. Binder is entitled to an annual base salary, which is subject to review and redetermination by our board of directors or our compensation committee, and she is eligible to earn an annual bonus with a target amount calculated as a percentage of her base salary. Dr. Binder is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Dr. Binder's employment agreement provides that, in the event that her employment is terminated by us without "cause" or by her for "good reason," then subject to the execution and effectiveness of a separation agreement and release and compliance with her continuing obligations, she will be entitled to receive (i) an amount equal to (x) nine months of base salary payable on our normal payroll cycle if such termination occurs outside of the Change in Control Period or (y) a lump sum in cash in the amount of 1.0 times the sum of her base salary plus her target bonus for the then-current year payable on our or our successor's normal payroll cycle over 12 months if such termination is occurs within the Change in Control Period; (ii) payment of the monthly employer COBRA premium for the same level of group health coverage as in effect for Dr. Binder on the date of termination up to (x) nine months if such termination occurs outside of the Change in Control Period, and (y) 12 months if such termination occurs within the Change in Control Period; and (iii) full acceleration of all stock options and other stock-based awards subject to time-based vesting held by Dr. Binder if such termination occurs within the Change in Control Period.

The payments and benefits provided to Dr. Binder under her employment agreement in connection with a sale event may not be eligible for a federal income tax deduction for the company pursuant to Section 280G of the Code. These payments and benefits also may be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Dr. Binder in connection with a sale event would be subject to the excise tax on golden parachutes imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to such officer.

In addition, Dr. Binder has entered into a non-competition, non-solicitation, confidentiality and assignment agreement with us that contains, among other things, non-competition and non-solicitation provisions that apply during the term of Dr. Binder's employment and for 12 months thereafter.

***David J. Chang, M.D., M.P.H.***

Under his amended and restated employment agreement, Dr. Chang is entitled to an annual base salary, which is subject to review and redetermination by our board of directors or our compensation committee, and he is eligible to earn an annual bonus with a target amount calculated as a percentage of his base salary. Dr. Chang is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Dr. Chang's employment agreement provides that, in the event that his employment is terminated by us without "cause" or by him for "good reason," then subject to the execution and effectiveness of a separation agreement and release and compliance with his continuing obligations, he will be entitled to receive (i) an amount equal to (x) nine months of base salary payable on our normal payroll cycle if such termination occurs outside of the Change in Control Period or (y) a lump sum in cash in the amount of 1.0 times the sum of his base salary plus his target bonus for the then-current year payable on our or our successor's normal payroll cycle over 12 months if such termination occurs within the Change in Control Period; (ii) payment of the monthly employer COBRA premium for the same level of group health coverage as in effect for Dr. Chang on the date of termination up to (x) nine months if such termination occurs outside of the Change in Control Period, and (y) 12 months if such termination occurs outside of the Change in Control Period; and (iii) full acceleration of all stock options and other stock-based awards subject to time-based vesting held by Dr. Chang if such termination occurs within the Change in Control Period.

The payments and benefits provided to Dr. Chang under his employment agreement in connection with a sale event may not be eligible for a federal income tax deduction for the company pursuant to Section 280G of the Code. These payments and benefits also may be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Dr. Chang in connection with a sale event would be subject to the excise tax on golden parachutes imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to such officer.

In addition, Dr. Chang has entered into a non-competition, non-solicitation, confidentiality and assignment agreement with us that contains, among other things, non-competition and non-solicitation provisions that apply during the term of Dr. Chang's employment and for 12 months thereafter.

**Outstanding Equity Awards at 2024 Fiscal Year End Table**

The following table presents information regarding all outstanding stock awards and stock options held by each of our named executive officers as of December 31, 2024.

Name	Number of Securities Underlying Unexercised Options (#) Exercisable	Option awards			Stock awards	
		Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price (\$)	Option Expiration Date	Number of Shares or Units of Stock that Have Not Vested (#)	Market Value of Shares or Units of Stock that Have Not Vested (\$)
Steven Nichtberger, M.D.	186,451	—	1.01	10/28/2028	—	—
	229,677	—(1)	1.01	10/28/2028	—	—
	99,714	—(2)	11.00	10/23/2029	—	—
	300,000	—(3)	14.94	2/28/2030	—	—
	312,656	20,844(7)	11.47	2/28/2031	—	—
	261,250	118,750(8)	3.21	1/17/2032	—	—
	180,075	231,525(9)	11.09	1/17/2033	—	—
Gwendolyn K. Binder, Ph.D.	—	353,000(10)	23.97	2/28/2034	—	—
	100,756	—(4)	1.01	10/28/2028	—	—
	66,667	—(5)	9.54	8/21/2029	—	—
	29,908	—(2)	11.00	10/23/2029	—	—
	117,188	7,812(7)	11.47	2/28/2031	—	—
	110,000	50,000(8)	3.21	1/17/2032	—	—
	77,000	99,000(9)	11.09	1/17/2033	—	—
David J. Chang, M.D., M.P.H.	—	130,000(10)	23.97	2/28/2034	—	—
	185,709	—(6)	6.30	6/23/2029	—	—
	48,836	—(2)	11.00	10/23/2029	—	—
	38,238	—(3)	14.94	2/28/2030	—	—
	106,406	7,094(7)	11.47	2/28/2031	—	—
	92,813	42,187(8)	3.21	1/17/2032	—	—
	65,625	84,375(9)	11.09	1/17/2033	—	—

(1) These options vest over four years, with 25% vesting on October 11, 2019, and the remaining shares vesting in 36 equal monthly installments thereafter, subject to a continued service relationship with us.

(2) These options vest over four years, with 25% vesting on October 24, 2020, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(3) These options vest over four years, with 25% vesting on March 1, 2021, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(4) These options vest over four years, with 25% vesting on October 11, 2019 and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(5) These options vest over four years, with 25% vesting on August 22, 2020, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(6) These options vest over four years, with 25% vesting on June 17, 2020, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(7) These options vest over four years, with 25% vesting on March 1, 2022, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(8) These options vest over four years, with 25% vesting on January 18, 2023, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(9) These options vest over four years, with 25% vesting on January 18, 2024, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

(10) These options vest over four years, with 25% vesting on March 1, 2025, and the remaining shares vesting in 12 equal quarterly installments thereafter, subject to a continued service relationship with us.

### ***Additional Narrative Disclosure***

*401(k) Savings Plan.* We participate in a retirement savings plan, or 401(k) plan, that is intended to qualify for favorable tax treatment under Section 401(a) of the Code, and contains a cash or deferred feature that is intended to meet the requirements of Section 401(k) of the Code. U.S. employees who are at least 21 years of age are generally eligible to participate in the 401(k) plan, subject to certain criteria. Participants may make pre-tax and certain after-tax (Roth) salary deferral contributions to the plan from their eligible earnings up to the statutorily prescribed annual limit under the Code. Participants who are 50 years of age or older may contribute additional amounts based on the statutory limits for catch-up contributions. Participant contributions are held in trust as required by law. An employee's interest in his or her salary deferral contributions is 100% vested when contributed. We have the ability to make discretionary contributions under the plan and all eligible employees will receive a Safe Harbor matching contribution up to 4% of eligible pay for the 2024 plan year.

*Health and Welfare Benefits.* All of our full-time employees, including our executive officers are eligible to participate in certain medical, disability and life insurance benefit programs offered by us. We pay the premiums for term life insurance and long-term disability for all of our employees, including our executive officers. We also provide all employees, including executive officers, with a flexible spending account plan and paid time off benefits including, vacation, sick time and holidays. We do not sponsor any qualified or non-qualified defined benefit plans for any of our employees or executives.

### **Compensation Risk Assessment**

We believe that although a portion of the compensation provided to our executive officers and other employees is performance-based, our executive compensation program does not encourage excessive or unnecessary risk taking. Our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals, in particular in connection with our pay-for-performance compensation philosophy. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

### **Policy on Trading, Pledging and Hedging of Company Stock**

Certain transactions in our securities (such as purchases and sales of publicly traded put and call options, and short sales) create a heightened compliance risk or could create the appearance of misalignment between management and stockholders. In addition, securities held in a margin account or pledged as collateral may be sold without consent if the owner fails to meet a margin call or defaults on the loan, thus creating the risk that a sale may occur at a time when an officer or director is aware of material, non-public information or otherwise is not permitted to trade in Company securities. Our insider trading policy expressly prohibits short sales and derivative transactions of our stock by our executive officers, directors, employees and certain designated consultants and contractors, including short sales of our securities. Our insider trading policy expressly prohibits, without the advance approval of our audit committee, purchases or sales of puts, calls, or other derivative securities of the company or any derivative securities that provide the economic equivalent of ownership.

### **Compensation Recovery Policy (Clawback Policy)**

On September 12, 2023, we adopted a Compensation Recovery Policy, or the Clawback Policy, in compliance with the requirements of the Dodd-Frank Act, final SEC rules and applicable Nasdaq listing standards (the "final clawback rules"), which covers our current and former executive officers, including all of our named executive officers. Under the Clawback Policy, in the event that we are required to prepare a restatement of our previously issued financial statements due to our material noncompliance with any financial reporting requirement under securities laws, we are required to recover (subject to certain limited exceptions described in the Clawback Policy and permitted under the final clawback rules) any cash or equity incentive-based compensation received by any current or former executive officer after the effective date of the Clawback Policy and in the three years prior to the date we are required to restate our financial statements that is in excess of the amount that would have been received based on the restated financial statements.

### **Compensation Committee Interlocks and Insider Participation**

Each of Richard Henriques, Mark Simon and Shawn Tomasello, served on the compensation committee during fiscal year 2024. No member of the compensation committee has ever been an officer or employee of the Company. None of our executive officers serve, or have served during the last fiscal year, as a member of the board of directors, compensation committee, or other

board committee performing equivalent functions of any other entity that has one or more executive officers serving as one of our directors or on our compensation committee.

#### Policy on the Timing of Awards of Options and Other Option-Like Instruments

Our compensation committee has generally granted annual equity awards, including stock option grants to our named executive officers, in the first quarter of each fiscal year, specifically early March. In addition, new hires receive stock option grants at the time of their hiring. Eligible employees, including our named executive officers, may voluntarily enroll in our Employee Stock Purchase Plan and receive an option to purchase shares at a discount using payroll deductions accumulated during the prior six months, with purchase dates occurring in May and November. During 2024, our compensation committee did not take into account any material nonpublic information when determining the timing and terms of equity incentive awards, and we did not time the disclosure of material nonpublic information for the purpose of affecting the value of executive compensation. During 2024, we did not grant stock options to our named executive officers during any period beginning four business days before and ending one business day after the filing or furnishing of a Form 10-Q, 10-K or 8-K that discloses material nonpublic information.

Please see Item 10 for details regarding corporate governance, including the composition of our compensation committee, as required by Regulation S-K 407(c)(4) and (e)(5).

#### Director Compensation

The following table presents the total compensation for each person who served as a non-employee member of our board of directors and received compensation for such service during the fiscal year ended December 31, 2024. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2024. We reimburse members of our board of directors for reasonable travel expenses. Dr. Nichtberger, our Chief Executive Officer and President, did not receive any compensation for his service as a member and Chairman of our board of directors in 2024. Dr. Nichtberger's compensation for service as an employee for fiscal years 2024 and 2023 is presented in "Executive Compensation—2024 Summary Compensation Table."

Name	Fees Earned or Paid In Cash (\$)	Option Awards (1) (2)	All Other Compensation	Total (\$)
Catherine Bollard, MBChB, M.D.	60,000	181,810	—	241,810
Scott Brun, M.D.	65,000	181,810	—	246,810
Richard Henriques, MBA	60,500	181,810	—	242,310
Mark Simon, MBA	63,500	181,810	—	245,310
Shawn Tomasello, MBA	53,000	181,810	—	234,810

(1) Amount shown reflects the grant date fair value of an option award granted during 2024. The grant date fair value was computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718, Compensation—Stock Compensation, or FASB ASC Topic 718, disregarding the effect of estimated forfeitures related to service-based vesting. See Note 10 to the financial statements in our 2024 Annual Report on Form 10-K regarding assumptions we made in determining the fair value of option awards.

(2) As of December 31, 2024, the aggregate number of shares subject to option awards held by our non-employee directors were: Dr. Bollard: 134,428; Dr. Brun: 110,000; Mr. Henriques: 134,428; Mr. Simon: 134,428 and Ms. Tomasello 66,000. As of December 31, 2024, the aggregate number of unvested shares outstanding held by our non-employee directors were: Dr. Bollard: 22,000; Dr. Brun: 22,000; Mr. Henriques: 22,000; Mr. Simon: 22,000 and Ms. Tomasello 47,667.

#### Amended and Restated Non-Employee Director Compensation Policy

Under our director compensation policy, we pay our non-employee directors a cash retainer for service on the board of directors and for service on each committee on which the director is a member. The chairman of each committee receives a higher retainer for such service. These fees are payable in arrears in four equal quarterly installments on the last day of each quarter, *provided that* the amount of such payment is prorated for any portion of such quarter that the director is not serving on our board of directors.

The fees paid to non-employee directors for service on the board of directors and for service on each committee of the board of directors on which the director is a member for 2024 are as follows:



	Member Annual Retainer (\$)	Chairperson Annual Retainer (\$)
Board of Directors	40,000	—
Audit Committee	7,500	15,000
Compensation Committee	5,500	11,000
Nominating and Corporate Governance Committee	5,000	10,000
Science and Technology Committee	7,500	15,000

In February 2025, we amended our director compensation policy. Accordingly, the fees paid to non-employee directors for service on the board of directors and for service on each committee of the board of directors on which the director is a member for 2025 are as follows:

	Member Annual Retainer (\$)	Chairperson Annual Retainer (\$)
Board of Directors	40,000	—
Audit Committee	10,000	20,000
Compensation Committee	6,250	12,500
Nominating and Corporate Governance Committee	5,000	10,000
Science and Technology Committee	7,500	15,000

We also reimburse our non-employee directors for reasonable out-of-pocket expenses incurred in connection with attending our board of director and committee meetings.

In addition, each new non-employee director elected to our board of directors will be granted an initial, one-time award of a stock option to purchase 44,000 shares, which shall vest in equal quarterly installments over a three-year period from the date of vesting commencement, subject to continued service as a director through each such vesting date. In addition, on the date of each annual meeting of stockholders of our company, each non-employee director will receive an annual stock option award of 22,000 shares, which shall vest in full upon the earlier to occur of the first anniversary of the date of grant or the date of the next annual meeting *provided, however*, that all vesting shall cease if the director resigns from the board of directors or otherwise ceases to serve as a director, unless the board of directors determines that the circumstances warrant continuation of vesting.

This program is intended to provide a total compensation package that enables us to attract and retain qualified and experienced individuals to serve as directors and to align our directors' interests with those of our stockholders.

## Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

### Equity Compensation Plan Information

The following table provides information as of December 31, 2024 regarding shares of common stock that may be issued under our equity compensation plans, consisting of the 2018 Plan, the 2019 Plan and the 2019 Employee Stock Purchase Plan, or the ESPP. We have no equity compensation plans that were not approved by security holders.

Plan Category	Number of securities to be issued upon exercise of outstanding options, units and rights (#)	Weighted-average exercise price of outstanding options, units and rights (\$)	Number of securities remaining available for future issuance under equity compensation plan (excluding securities referenced in first column)
Equity compensation plans approved by security holders(1)	11,231,148(2)	\$ 10.63	1,693,564(3)(4)
Equity compensation plans not approved by security holders	—	—	—
<b>Total</b>	<b>11,231,148</b>	<b>\$ 10.63</b>	<b>1,693,564</b>

(1) Consists of our 2019 Stock Option and Incentive Plan, 2018 Stock Option and Grant Plan, and 2019 Employee Stock Purchase Plan. Following our initial public offering, we have not and will not grant any awards under our 2018 Stock Option and Grant Plan, but all outstanding awards under the 2018 Stock Option and Grant Plan will continue to be governed by their existing terms. The shares of common stock underlying any awards granted under the 2018 Stock Option and Grant Plan or 2019 Stock Option and Incentive Plan that are forfeited, canceled, reacquired by us prior to vesting, satisfied without the issuance of stock, or otherwise terminated (other than by exercise) and the shares of common stock that are withheld upon exercise of a stock option or settlement of such award to cover the exercise price or tax withholding will be added to the shares of common stock available for issuance under the 2019 Stock Option and Incentive Plan.

(2) Includes 11,231,148 shares of common stock issuable upon the exercise of outstanding options, of which 1,235,458 are subject to our 2018 Stock Option and Grant Plan and 9,995,690 are subject to our 2019 Stock Option and Incentive Plan.

(3) As of December 31, 2024, there were 1,379,305 shares available for grant under the 2019 Stock Option and Incentive Plan and 314,259 shares available for grants under the 2019 Employee Stock Purchase Plan.

(4) The 2019 Stock Option and Incentive Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2020, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31. The 2019 Employee Stock Purchase Plan provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2020, through January 1, 2029, by the least of 234,229 shares of our common stock, 1% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee.

### Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information, to the extent known by us or ascertainable from public filings, with respect to the beneficial ownership of our common stock as of March 7, 2025 by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own greater-than-5.0% of our common stock.

The column entitled “Shares Beneficially Owned” is based on a total of 50,743,101 shares of our common stock outstanding as of March 7, 2025.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options or warrants that are currently exercisable or exercisable within 60 days of March 7, 2025 are considered outstanding and beneficially owned by the person holding the options or warrants for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property

laws, where applicable. Except as otherwise indicated in the table below, addresses of named beneficial owners are in care of Cabaletta Bio, Inc., 2929 Arch Street, Suite 600, Philadelphia, Pennsylvania 19104.

Name and address of beneficial owner(1)	Shares beneficially owned	
	Number	Percentage
<b>&gt; 5% Stockholders:</b>		
BlackRock, Inc.(2)	3,397,260	6.7%
Entities Affiliated with Adage Capital Partners, L.P.(3)	3,634,475	7.2%
T. Rowe Price Investment Management, Inc.(4)	3,673,724	7.2%
Entities affiliated with Prudential Financial, Inc.(5)	2,798,863	5.5%
Bain Capital Life Sciences Opportunities III, LP(6)	2,760,888	5.4%
The Vanguard Group(7)	2,553,710	5.0%
<b>Named Executive Officers and Directors:</b>		
Steven Nichtberger, M.D.(8)	3,127,350	5.95%
Gwendolyn K. Binder, Ph.D.(9)	603,831	1.18%
David Chang, M.D., M.P.H.(10)	612,846	1.19%
Catherine Bollard, MBChB, M.D.(11)	113,428	0.22%
Scott Brun, M.D.(12)	88,000	0.17%
Richard Henriques, MBA(13)	112,428	0.22%
Mark Simon, MBA(14)	255,572	0.50%
Shawn Tomasello, MBA(15)	25,667	0.05%
All executive officers and directors as a group (11 persons)(15)	6,056,069	10.96%

\* Represents beneficial ownership of less than one percent.

(1) Unless otherwise indicated, the address for each beneficial owner is c/o Cabaletta Bio, Inc., 2929 Arch Street, Suite 600, Philadelphia, Pennsylvania 19104.

(2) The information reported is based on information in a Schedule 13G/A, as filed by BlackRock, Inc. with the SEC on February 5, 2025. In that filing, BlackRock, Inc. reports sole voting power with respect to 3,329,097 shares of common stock and sole dispositive power with respect to 3,397,260 shares, and lists its address as 50 Hudson Yards, New York, New York 10001.

(3) The information is based on a Schedule 13G/A filed with the SEC on November 12, 2024. Consists of 3,036,645 shares of common stock and 597,830 shares of common stock issuable upon the exercise of pre-funded warrants held by Adage Capital Partners, L.P., or ACP. Adage Capital Partners GP, L.L.C., or ACPGP, is the general partner of ACP. Adage Capital Management, L.P., or ACM, is the investment manager of ACP. Adage Capital Advisors, L.L.C., or ACA, is managing member of ACPGP. Adage Capital Partners LLC, or ACPLLC, is the general partner of ACM, with respect to the shares of voting common stock directly held by ACP. Robert Atchinson and Phillip Gross are managing members of ACA and ACPGP and are general partners of ACP. Each of ACP, ACPGP, ACA, ACM, Robert Atchinson, and Phillip Gross have shared voting and dispositive power over all of the shares. The ability of the Adage entities to exercise their pre-funded warrants is subject to a beneficial ownership limitation, which prevents the Adage entities from exercising any portion of the warrants to the extent such exercise would result in the Adage entities exceeding a certain percentage of the total number of issued and outstanding shares of our common stock following such exercise. The address for Adage Capital Partners, L.P. is 200 Clarendon Street, 52nd Floor, Boston, Massachusetts 02116.

(4) The information reported is based on information in a Schedule 13G/A, as filed by T. Rowe Price Investment Management, Inc. (Price Investment Management) with the SEC on November 14, 2024. In that filing, T. Rowe Price Investment Management, Inc. (Price Investment Management) reports sole voting power with respect to 3,666,024 shares of common stock and sole dispositive power with respect to 3,673,724 shares, and lists its address as 101 E. Pratt Street, Baltimore, MD 21201.

(5) The information reported is based on information in a Schedule 13G/A, as filed by Prudential Financial, Inc. with the SEC on December 5, 2024. In that filing, Prudential Financial, Inc. reports shared voting and dispositive power over (i) 2,796,053 shares of our common stock, being 2,512,053 shares held through its subsidiary Jennison Associates LLC and 284,000 shares held through its subsidiary PGIM, Inc., and (ii) sole voting and dispositive power over 2,810 shares of our common stock held through its subsidiary PGIM Quantitative Solutions LLC. The address provided for Prudential Financial, Inc. is 751 Broad Street, New Jersey 07102.

(6) The information reported is based on information in a Schedule 13G, as filed by Bain Capital Life Sciences Opportunities III, LP with the SEC on August 22, 2024. In that filing, Bain Capital Life Sciences Opportunities III, LP reports shared voting power and shared dispositive power with respect to 2,760,888 shares of common stock. Bain Capital Life Sciences Investors, LLC, or BCLSI, is the manager of Bain Capital Life Sciences III General Partner, LLC, which is the general partner of Bain Capital Life Sciences Fund III, L.P., which is the sole member of Bain Capital Life Sciences Opportunities III GP, LLC, which is the general partner of Bain Capital Life Sciences Opportunities III, LP. As a result, BCLSI may be deemed to share voting and dispositive power with respect to the securities held by Bain Capital Life Sciences Opportunities III, LP. The address of BCLSI is 200 Clarendon Street, Boston, MA 02116.

(7) The information reported is based on information in a Schedule 13G/A, as filed by The Vanguard Group with the SEC on November 12, 2024. In that filing, The Vanguard Group reports shared voting power with respect to 31,135 shares of common stock, sole dispositive power with respect to 2,500,994 shares and shared dispositive power with respect to 52,716 shares, and lists its address as 100 Vanguard Blvd., Malvern, PA 19355.

(8) Consists of (i) 986,483 shares of common stock held by Dr. Nichtberger, (ii) 363,000 shares of common stock held by the 2017 Nichtberger Family Trust, or the Trust Shares, and (iii) 1,177,867 shares of common stock underlying options exercisable within 60 days of March 7, 2025. Dr. Nichtberger may be deemed to beneficially own the Trust Shares. Dr. Nichtberger disclaims beneficial ownership of the Trust Shares and this shall not be deemed an admission that he is the beneficial owner of the Trust Shares.

(9) Consists of (i) 20,000 shares of common stock held and (ii) 583,831 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(10) Consists of 612,846 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(11) Consists of (i) 1,000 shares of common stock held and (ii) 112,428 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(12) Consists of 88,000 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(13) Consists of 112,428 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(14) Consists of (i) 143,144 shares of common stock held and (ii) 112,428 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(15) Consists of 25,667 shares of common stock underlying options exercisable within 60 days of March 7, 2025.

(16) See notes (7) through (14); also includes an aggregate of (i) 50,000 shares of common stock and (ii) 1,066,947 shares of common stock subject to options exercisable within 60 days of March 7, 2025 held by Anup Marda, our Chief Financial Officer, Michael Gerard, our General Counsel and Secretary, and Arun Das, M.D., our Chief Business Officer.

### Item 13. Certain Relationships and Related Transactions, and Director Independence.

#### Certain Relationships and Transactions

Other than the compensation agreements and other arrangements described under “Executive compensation” and “Director compensation” in this Annual Report on Form 10-K for the fiscal year ended December 31, 2024 and the transactions described below, since January 1, 2023, there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, \$120,000 (or, if less, 1% of the average of our total assets amounts at December 31, 2023 and 2024) and in which any director, executive officer, holder of five percent or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

#### May 2023 Offering

In May 2023, we issued 8,337,500 shares of our common stock at a price of \$12.00 per share in connection with an underwritten public offering. Certain existing holders of five percent or more of a class of our capital stock participated in the transaction, as evidenced by the table below.

Greater than Five Percent Holder	Aggregate Common Stock Purchased		Aggregate Purchase Price
Adage Capital Partners LP	725,000	\$	8,700,000
Fred Alger Management Inc	400,000	\$	4,800,000
Cormorant Global Healthcare Master Fund LP	400,000	\$	4,800,000
Commodore Capital LP	400,000	\$	4,800,000

#### Limitation of Liability and Indemnification of Officers and Directors

Our certificate of incorporation contains provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by Delaware law. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for the following:

- any breach of their duty of loyalty to our company or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which they derived an improper personal benefit.

Any amendment to, or repeal of, these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to that amendment or repeal. If the Delaware General Corporation Law is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the Delaware General Corporation Law.

In addition, we adopted bylaws which provide that we will indemnify, to the fullest extent permitted by law, any person who is or was a party or is threatened to be made a party to any action, suit or proceeding by reason of the fact that he or she is or was one of our directors or officers or is or was serving at our request as a director or officer of another corporation, partnership, joint venture, trust, or other enterprise. Our bylaws provide that we may indemnify to the fullest extent permitted by law any person who is or was a party or is threatened to be made a party to any action, suit, or proceeding by reason of the fact that he or she is or was one of our employees or agents or is or was serving at our request as an employee or agent of another corporation, partnership, joint venture, trust or other enterprise. Our bylaws also provide that we must advance expenses incurred by or on behalf of a director or officer in advance of the final disposition of any action or proceeding, subject to very limited exceptions.

We have entered into and in the future plan to enter into agreements to indemnify our directors and executive officers. These agreements, among other things, require us to indemnify these individuals for certain expenses (including attorneys’ fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person’s status as a member of our board of directors to the maximum extent allowed under Delaware law.

### **Related person transaction policy**

Our board of directors adopted a written related person transaction policy providing that transactions with our directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related person, must be approved by our audit committee. This policy became effective on October 29, 2019, the date our registration statement for our initial public offering became effective. Pursuant to this policy, the audit committee has the primary responsibility for reviewing and approving or disapproving “related person transactions,” which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed \$120,000 and in which a related person has or will have a direct or indirect material interest. For purposes of this policy, a related person is defined as a director, executive officer, nominee for director, or greater than 5% beneficial owner of our common stock, in each case since the beginning of the most recently completed year, and their immediate family members.

As appropriate for the circumstances, the audit committee will review and consider:

- the related person’s interest in the related person transaction;
- the approximate dollar amount involved in the related person transaction;
- the approximate dollar amount of the related person’s interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the related-party transaction; and
- any other information regarding the related-party transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

### **Director Independence**

Applicable Nasdaq rules require a majority of a listed company’s board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq rules require that, subject to specified exceptions, each member of a listed company’s audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act and that compensation committee members satisfy independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable Nasdaq rules, a director will only qualify as an “independent director” if, in the opinion of the listed company’s board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In addition, in affirmatively determining the independence of any director who will serve on a company’s compensation committee, Rule 10C-1 under the Exchange Act requires that a company’s board of directors must consider all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director’s ability to be independent from management in connection with the duties of a compensation committee member, including: the source of compensation to the director, including any consulting, advisory or other compensatory fee paid by such company to the director, and whether the director is affiliated with the company or any of its subsidiaries or affiliates.

Our board of directors has determined that all members of the board of directors, except Dr. Nichtberger are independent directors, including for purposes of the rules of Nasdaq and the SEC. In making such independence determination, our board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances that our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director. In considering the independence of the directors listed above, our board of directors considered the association of our directors with the holders of more than 5% of our common stock. There are no family relationships among any of our directors or executive officers. Dr. Nichtberger is not an independent director under these rules because he is an executive officer of the Company.

**Item 14. Principal Accountant Fees and Services.**

Our independent public accounting firm is Ernst & Young, Philadelphia, PA, PCAOB Auditor ID 42.

Cabaletta incurred the following fees from Ernst & Young for the audit of the financial statements and for other services provided during the years ended December 31, 2024 and 2023.

	2024		2023	
Audit fees(1)	\$	646,500	\$	830,000
Audit-Related fees(2)		—		—
Tax fees(3)		66,950		65,000
All other fees(4)		—		—
Total fees	\$	713,450	\$	895,000

(1) Audit fees consist of fees for the audit of our annual financial statements, the review of our interim financial statements included in our quarterly reports on Form 10-Q, comfort letters and consents.

(2) Audit-related fees consist of services that are reasonably related to the performance of the audit or review of our financial statements. There were no such fees incurred in 2024 or 2023.

(3) Tax Fees consist of fees for tax compliance, advice and tax services.

(4) There were no other fees incurred in 2024 or 2023.

**Audit Committee Pre-approval Policy and Procedures**

Our audit committee has adopted policies and procedures relating to the approval of all audit and non-audit services that are to be performed by our independent registered public accounting firm. This policy provides that we will not engage our independent registered public accounting firm to render audit or non-audit services unless the service is specifically approved in advance by our audit committee or the engagement is entered into pursuant to the pre-approval procedure described below.

From time to time, our audit committee may pre-approve specified types of services that are expected to be provided to us by our independent registered public accounting firm during the next 12 months. Any such pre-approval details the particular service or type of services to be provided and is also generally subject to a maximum dollar amount.

During our 2024 and 2023 fiscal years, no services were provided to us by Ernst & Young other than in accordance with the pre-approval policies and procedures described above.

## PART IV

### Item 15. Exhibits, Consolidated Financial Statement Schedules.

(a) The following documents are filed as part of this Annual Report on Form 10-K:

(1) Consolidated Financial Statements:

	<b>Page</b>
<a href="#">Report of Independent Registered Public Accounting Firm</a>	F-2
<a href="#">Consolidated Balance Sheets as of December 31, 2024 and 2023</a>	F-4
<a href="#">Consolidated Statements of Operations and Comprehensive Loss for the Years Ended December 31, 2024 and 2023</a>	F-5
<a href="#">Consolidated Statements of Stockholders' Equity for the Years Ended December 31, 2024 and 2023</a>	F-6
<a href="#">Consolidated Statements of Cash Flows for the Years Ended December 31, 2024 and 2023</a>	F-7
<a href="#">Notes to Consolidated Financial Statements</a>	F-8

(2) Consolidated Financial Statement Schedules:

All consolidated financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the consolidated financial statements or the notes thereto.

(3) Exhibits. The exhibits required by Item 601 of Regulation S-K and Item 15(b) of this Annual Report on Form 10-K are listed in the Exhibit Index immediately preceding the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index are incorporated by reference herein.

### Item 16. Form 10-K Summary

None.



## INDEX TO FINANCIAL STATEMENTS

	<b>Page</b>
<a href="#"><u>Report of Independent Registered Public Accounting Firm</u></a>	F-2
<a href="#"><u>Consolidated Balance Sheets as of December 31, 2024 and 2023</u></a>	F-4
<a href="#"><u>Consolidated Statements of Operations and Comprehensive Loss for the Years Ended December 31, 2024 and 2023</u></a>	F-5
<a href="#"><u>Consolidated Statements of Stockholders' Equity for the Years Ended December 31, 2024 and 2023</u></a>	F-6
<a href="#"><u>Consolidated Statements of Cash Flows for the Years Ended December 31, 2024 and 2023</u></a>	F-7
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## **Report of Independent Registered Public Accounting Firm**

To the Stockholders and the Board of Directors of Cabaletta Bio, Inc.

### **Opinion on the Financial Statements**

We have audited the accompanying consolidated balance sheets of Cabaletta Bio, Inc. (the Company) as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles.

### **The Company's Ability to Continue as a Going Concern**

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

### **Basis for Opinion**

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

**Critical Audit Matter**

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the account or disclosures to which it relates.

***Accounting for Accrued Clinical Trial Expenses******Description of the Matter***

As disclosed in Note 2 to the consolidated financial statements, the Company expenses research and development costs as incurred, which include costs relating to clinical trial activities. The Company's accrued clinical trial expenses are based on factors such as estimates of the work completed in accordance with agreements established with a contract research organization. As disclosed in Note 5 to the consolidated financial statements, the Company's total accrued expenses and other current liabilities related to research and development costs were \$3.5 million at December 31, 2024, which included the estimated clinical trial expenses incurred as of December 31, 2024 but not paid as of that date.

Auditing the Company's accrued clinical trial expenses required subjective auditor judgement due to the estimation required by management in determining the services provided but not yet invoiced. Specifically, the amount of accrued clinical trial expenses recognized is sensitive to the availability of information to make the estimate, including the estimate of the period over which services will be performed, the associated cost of such services, and the level of services performed and progress in the period for which the Company has not yet received an invoice.

***How We Addressed the Matter in Our Audit***

To test the accrued clinical trial expenses our audit procedures included, among others, reviewing a sample of agreements with the service providers to corroborate key financial and contractual terms, and testing the accuracy and completeness of the underlying data used in the accrual expense computations. We also evaluated management's estimates of the progress of a sample of clinical trial activities by making inquiries of the Company's operations personnel that oversee the clinical trials and confirming information directly with the contract research organization. To evaluate the completeness of the accruals, we also examined subsequent invoices from the service providers and cash disbursements to the service providers, to the extent such invoices were received, or payments were made prior to the date that the consolidated financial statements were issued.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2018.

Philadelphia, Pennsylvania

March 31, 2025

**CABALETTA BIO, INC.**  
**Consolidated Balance Sheets**  
(in thousands, except share and per share amounts)

	December 31,	
	2024	2023
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 163,962	\$ 193,238
Short-term investments	—	48,011
Prepaid expenses and other current assets	2,713	3,241
Total current assets	166,675	244,490
Property and equipment, net	2,743	2,541
Finance lease right-of-use assets	7,020	—
Operating lease right-of-use assets	6,315	4,910
Other assets	2,293	1,709
<b>Total Assets</b>	<u>\$ 185,046</u>	<u>\$ 253,650</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 4,923	\$ 4,547
Accrued and other current liabilities	12,188	7,887
Finance lease liabilities, current portion	5,989	—
Operating lease liabilities, current portion	3,986	3,560
Total current liabilities	27,086	15,994
Finance lease liabilities, net of current portion	3,241	—
Operating lease liabilities, net of current portion	2,384	1,458
<b>Total Liabilities</b>	32,711	17,452
Commitments and contingencies (see Notes 6 and 7)		
Stockholders' equity:		
Preferred stock, \$0.00001 par value: 10,000,000 shares authorized as of December 31, 2024 and December 31, 2023; no shares issued or outstanding at December 31, 2024 and December 31, 2023	—	—
Voting and non-voting common stock, \$0.00001 par value: 150,000,000 (143,590,481 voting and 6,409,519 non-voting) shares authorized as of December 31, 2024 and December 31, 2023; 50,743,101 voting shares issued and outstanding as of December 31, 2024 and 47,823,232 (46,378,937 voting and 1,444,295 non-voting) shares issued and outstanding as of December 31, 2023	1	—
Additional paid-in capital	501,435	469,396
Accumulated other comprehensive income	—	39
Accumulated deficit	(349,101)	(233,237)
<b>Total stockholders' equity</b>	152,335	236,198
<b>Total liabilities and stockholders' equity</b>	<u>\$ 185,046</u>	<u>\$ 253,650</u>

The accompanying notes are an integral part of these consolidated financial statements.

**CABALETTA BIO, INC.**  
**Consolidated Statements of Operations and Comprehensive Loss**  
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2024	2023
Operating expenses:		
Research and development	\$ 97,203	\$ 55,424
General and administrative	27,938	19,236
Total operating expenses	125,141	74,660
Loss from operations	(125,141)	(74,660)
Other income:		
Interest income	10,025	6,985
Interest expense	(748)	—
Net loss	\$ (115,864)	\$ (67,675)
Other comprehensive income:		
Net unrealized (loss) gain on available-for-sale investments, net of tax	(39)	86
Net comprehensive loss	<u>\$ (115,903)</u>	<u>\$ (67,589)</u>
Net loss per share of voting and non-voting common stock, basic and diluted	<u>\$ (2.34)</u>	<u>\$ (1.65)</u>

The accompanying notes are an integral part of these consolidated financial statements.

**CABALETTA BIO, INC.**  
**Consolidated Statements of Stockholders' Equity**  
(in thousands, except share and per share amounts)  
**Common Stock**

	Shares	Amount	Additional Paid-in Capital	Accumulated Other Comprehen sive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance—December 31, 2022	29,445,134	\$ —	\$ 270,129	\$ (47)	\$ (165,562)	\$ 104,520
Stock-based compensation	—	—	11,343	—	—	11,343
Issuance of common stock, net of issuance costs of \$8,647	13,098,399	—	185,495	—	—	185,495
Issuance of common stock in connection with exercise of stock options	489,672	—	2,321	—	—	2,321
Issuance of common stock under employee stock purchase plan	16,053	—	108	—	—	108
Issuance of common stock upon exercise of pre-funded warrants	4,773,974	—	—	—	—	—
Net unrealized losses on available-for-sale securities	—	—	—	86	—	86
Net loss	—	—	—	—	(67,675)	(67,675)
Balance—December 31, 2023	47,823,232	\$ —	\$ 469,396	\$ 39	\$ (233,237)	\$ 236,198
Stock-based compensation	—	—	19,465	—	—	19,465
Issuance of common stock, net of issuance costs of \$252	1,483,070	1	10,875	—	—	10,876
Issuance of common stock in connection with exercise of stock options	210,695	—	1,430	—	—	1,430
Issuance of common stock under employee stock purchase plan	58,056	—	269	—	—	269
Issuance of common stock upon exercise of pre-funded warrants	1,168,048	—	—	—	—	—
Net unrealized losses on available-for-sale securities	—	—	—	(39)	—	(39)
Net loss	—	—	—	—	(115,864)	(115,864)
Balance—December 31, 2024	50,743,101	\$ 1	\$ 501,435	\$ —	\$ (349,101)	\$ 152,335

The accompanying notes are an integral part of these consolidated financial statements.

**CABALETTA BIO, INC.**  
**Consolidated Statements of Cash Flows**  
(in thousands)

	Year Ended December 31,	
	2024	2023
<b>Cash flows from operating activities:</b>		
Net loss	\$ (115,864)	\$ (67,675)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	19,465	11,343
Depreciation	1,711	1,426
Amortization of finance lease	3,102	—
Non-cash operating lease expense	2,838	2,070
Accretion of operating lease liabilities	405	408
Amortization of discount on investments	(1,528)	(1,222)
Loss on foreign currency exchange rates	38	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	528	(954)
Other assets	(584)	(1,144)
Accounts payable	1,011	1,675
Accrued and other current liabilities	3,947	2,980
Lease liabilities	(3,291)	(2,456)
Net cash used in operating activities	(88,222)	(53,549)
<b>Cash flows from investing activities:</b>		
Purchases of property and equipment	(2,211)	(687)
Purchases of investments	—	(84,264)
Proceeds from maturities of investments	49,500	62,500
Net cash provided by (used in) investing activities	47,289	(22,451)
<b>Cash flows from financing activities:</b>		
Proceeds from the issuance of common stock, net of issuance costs	10,875	185,202
Proceeds from issuance of common stock in connection with the exercise of stock options	1,430	2,321
Proceeds from issuance of common stock under employee stock purchase plan	269	108
Principal payments on finance leases	(897)	—
Net cash provided by financing activities	11,677	187,631
Effect of exchange rate changes on cash and cash equivalents	(20)	—
Net (decrease) increase in cash and cash equivalents	(29,276)	111,631
Cash and cash equivalents—beginning of period	193,238	81,607
Cash and cash equivalents—end of period	<u>\$ 163,962</u>	<u>\$ 193,238</u>
<b>Supplemental disclosures</b>		
Cash paid for interest	\$ 748	\$ —
Property and equipment purchases included in accounts payable and accrued expenses	\$ 404	\$ 702
Right-of-use assets obtained in exchange for finance lease obligations	\$ 10,963	\$ 2,048
Right-of-use assets obtained in exchange for operating lease obligations	\$ 4,242	\$ —

The accompanying notes are an integral part of these consolidated financial statements.

## CABALETTA BIO, INC.

### Notes to the Consolidated Financial Statements (in thousands, except share and per share amounts)

#### 1. Basis of Presentation

Cabaletta Bio, Inc. (the Company or Cabaletta) was incorporated in April 2017 in the State of Delaware as Tycho Therapeutics, Inc. and, in August 2018, changed its name to Cabaletta Bio, Inc. The consolidated financial statements include the accounts of Cabaletta Bio and its wholly owned subsidiaries: Cabaletta Bio GmbH and Cabaletta Bio (Germany) GmbH. All intercompany balances and transactions have been eliminated in consolidation.

The Company is headquartered in Philadelphia, Pennsylvania. Cabaletta is a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for autoimmune diseases. Principal operations commenced in April 2018.

#### *Risks and Uncertainties*

The Company does not expect to generate revenue from sales of engineered T cell therapies for autoimmune diseases or any other revenue unless and until the Company completes preclinical and clinical development and obtains regulatory approval for one or more product candidates. If the Company seeks to obtain regulatory approval for any of its product candidates, the Company expects to incur significant commercialization expenses.

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. As a result, the Company is unable to predict the timing or amount of increased expenses or when or if the Company will be able to achieve or maintain profitability. Further, the Company is dependent on third parties for certain research and development activities, including manufacturing services (Note 6 and Note 7). Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. Even if the Company is able to generate revenues from the sale of its product candidates, if approved, it may not become profitable. If the Company fails to become profitable or is unable to sustain profitability on a continuing basis, then it may be unable to continue its operations at planned levels and be forced to reduce its operations.

#### *Liquidity and Going Concern*

In accordance with Accounting Standards Codification ("ASC") 205-40, Going Concern, ("ASC 205-40") the Company evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern. The Company has sustained annual operating losses since inception and expects to continue to generate operating losses for the foreseeable future. As of December 31, 2024, the Company has incurred an accumulated deficit of \$349,101 and has a cash and cash equivalents balance of \$163,962. The Company's ultimate success depends on the outcome of its research and development activities. Management expects to incur additional losses in the future as it continues its research and development and will need to raise additional capital to fully implement its business plan and to fund its operations. The Company intends to raise such additional capital through a combination of equity offerings, debt financings, government funding arrangements, strategic alliances or other sources. However, if such financing is not available at adequate levels and on a timely basis, or such agreements are not available on favorable terms, or at all, as and when needed, the Company will need to reevaluate its operating plan and may be required to delay or discontinue the development of one or more of its product candidates or operational initiatives. The Company expects that its current cash and cash equivalents may not be sufficient to fund operations for at least the next twelve months from the date of issuance of these consolidated financial statements. The Company's cash forecast contains estimates and assumptions based on success of ongoing clinical trials, and management cannot predict the amount or timing of all expenditures with certainty. Accordingly, the Company has concluded that substantial doubt exists about the Company's ability to continue as a going concern. The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business.



## 2. Summary of Significant Accounting Policies

### *Principles of Consolidation*

The accompanying consolidated financial statements included herein have been prepared in conformity with accounting principles generally accepted in the United States ("GAAP") and under the rules and regulations of the U.S. Securities and Exchange Commission ("SEC"). All intercompany balances and transactions have been eliminated in consolidation.

### *Use of Estimates*

The preparation of consolidated financial statements in conformity with accounting principles generally accepted in the United States of America (GAAP) requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities as of the date of the consolidated financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions made in the accompanying consolidated financial statements include but are not limited to advance payments and accruals related to the Company's research and development expenses. The Company evaluates its estimates and assumptions on an ongoing basis using historical experience and other factors and adjusts those estimates and assumptions when facts and circumstances dictate. Actual results could differ from those estimates.

### *Off-Balance Sheet Risk and Concentrations of Credit Risk*

Financial instruments, which potentially subject the Company to significant concentrations of credit risk, consist of cash and cash equivalents, which are invested in U.S. treasury-based money market funds. As of December 31, 2023, the Company held available-for-sale securities, which were invested in U.S. treasury securities. These investments matured in 2024. A portion of the Company's cash is maintained at two federally insured financial institutions, and account balances may at times exceed federally insured limits. The Company has not experienced any losses in such accounts and management believes that the Company is not exposed to significant credit risk. The Company has no off-balance sheet risk, such as foreign exchange contracts, option contracts, or other foreign hedging arrangements.

The Company considers all highly liquid investments purchased with original maturities of three months or less from the purchase date to be cash equivalents. Cash equivalents consist primarily of amounts invested in money market accounts.

### *Investments*

Investments are available-for-sale and carried at estimated fair value. The Company's valuations of available-for-sale debt securities are generally derived from independent pricing services based upon quoted prices in active markets for similar securities, with prices adjusted for yield and number of days to maturity, or based on industry models using data inputs, such as interest rates and prices that can be directly observed or corroborated in active markets. Management determines the appropriate classification of its investments in debt securities at the time of purchase and at the end of each reporting period. Investments with original maturities beyond three months at the date of purchase and which mature at, or less than, twelve months from the balance sheet date are classified as current.

Unrealized gains and losses are excluded from earnings and are reported as a component of comprehensive income. The Company periodically evaluates whether declines in fair values of its available-for-sale securities below their book value are other-than-temporary. This evaluation consists of several qualitative and quantitative factors including the severity and duration of the unrealized loss as well as the Company's ability and intent to hold the available-for-sale security until a forecasted recovery occurs. Additionally, the Company assesses whether it has plans to sell the security or if more likely than not it will be required to sell any available-for-sale securities before recovery of its amortized cost basis. Realized gains and losses and declines in fair value judged to be other-than-temporary, if any, on available-for-sale securities are included in interest and other income, net. The cost of investments sold is based on the specific-identification method. Interest income on investments as well as amortization of discount or premium is included in interest income.

### ***Property, Plant and Equipment***

Property, plant and equipment are recorded at cost less accumulated depreciation. Cost includes the acquisition costs and all costs necessary to bring the asset to the location and working condition necessary for its intended use. Depreciation expense is recognized using the straight-line method over the estimated useful life of each asset. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the accompanying statements of operations. Expenditures for normal, recurring or periodic repairs and maintenance related to property and equipment are charged to expense as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if it will result in future economic benefits.

Estimated useful lives for property and equipment are as follows:

<b>Property and equipment</b>	<b>Estimated useful life</b>
Laboratory equipment	Three years
Furniture and fixtures	Three years
Computer equipment	Three years
Leasehold improvements	Lesser of estimated useful life or remaining lease term

### ***Fair Value Measurement***

Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Observable inputs such as unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2—Inputs (other than quoted prices included in Level 1) that are either directly or indirectly observable for the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active.

Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

### ***Leases***

The Company has leases related to its facilities used for offices and laboratory space, which are classified as operating leases as well as leases related to its manufacturing arrangements, which are classified as finance leases.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease and its classification based on the unique facts and circumstances present in the arrangement. Leases with a term greater than one year are recognized on the balance sheet as right-of-use assets and current and non-current lease liabilities, as applicable.

Lease liabilities and their corresponding right-of-use assets are initially recorded based on the present value of lease payments over the expected remaining lease term. Certain adjustments to the right-of-use asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate to discount lease payments. Subsequent to the initial measurement, the Company will continue to account for:

- the lease liability at the net present value using the incremental borrowing rate that was in effect as of the lease commencement, lease modification, or transition date, and;

- the finance lease and operating lease expense on a straight line basis.

Leases with terms of 12 months or less are considered short-term leases and right-of-use assets and lease obligations are not recognized. Payments associated with short-term leases are expensed on a straight-line basis over the lease term.

In accordance with ASC 842, components of a lease should be split into three categories: lease components, non-lease components and non-components. The fixed and in-substance fixed contract consideration (including any consideration related to non-components) must be allocated based on the respective relative fair values to the lease components and non-lease components. Entities may elect not to separate lease and non-lease components. Rather, entities would account for each lease component and related non-lease component together as a single lease component. The Company has elected to aggregate all lease and non-lease components for each class of underlying assets into a single lease component for real-estate leases and embedded manufacturing arrangements.

ASC 842 allows for the use of judgment in determining whether the assumed lease term is for a major part of the remaining economic life of the underlying asset and whether the present value of lease payments represents substantially all of the fair value of the underlying asset. The Company applies the guidance in ASC 842-10-55-2 to assist in determining lease classification across its leases.

#### ***Research and Development Expenses***

Research and development costs include costs incurred for internal and external research and development activities and are expensed as incurred in the accompanying statements of operations. Research and development costs consist of salaries and benefits, including associated stock-based compensation, and laboratory supplies and facility costs, as well as fees paid to entities that conduct certain research and development and clinical trial activities on the Company's behalf.

The Company records accrued liabilities for estimated costs of research and development activities conducted by service providers, which include preclinical studies, contract manufacturing activities and clinical trial activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided and in accordance with agreements established with service providers and includes these costs in accrued and other current liabilities in the accompanying balance sheets and within research and development expense in the accompanying statements of operations. Non-refundable advance payments made for goods or services that will be used or rendered for future research and development activities are deferred and capitalized and recognized as expense as the goods are received or the related services are rendered. The Company makes significant judgments and estimates in determining the accrued liabilities and prepaid expenses in each reporting period. As actual costs become known, the Company adjusts its accrued liabilities and prepaid expenses. The Company has not experienced any material differences between accrued costs and actual costs incurred since its inception.

#### ***Stock-based Compensation***

The Company measures its stock-based awards granted to employees and non-employees based on the estimated fair values of the awards on the respective grant dates. The Company uses the Black-Scholes option-pricing model (Black-Scholes) to estimate the fair value of its stock-based awards. The Company recognizes compensation expense for time-based awards on a straight-line basis over the requisite service period, which is generally the vesting period of the award. The Company accounts for forfeitures of stock option awards as they occur.

#### ***Income Taxes***

The Company uses the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to temporary differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax base, as well as for net operating loss carryforwards and research and development credits, and are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

Valuation allowances are established when necessary to reduce deferred tax assets where, based upon the available evidence, the Company concludes that it is more-likely-than-not that the deferred tax assets will not be realized. In evaluating its ability to recover deferred tax assets, the Company considers all available positive and negative evidence, including its operating results, ongoing tax planning and forecasts of future taxable income on a jurisdiction-by-jurisdiction basis. Because of the uncertainty of the realization of deferred tax assets, the Company has recorded a full valuation allowance against its deferred tax assets.

Reserves are provided for tax benefits for which realization is uncertain. Such benefits are only recognized when the underlying tax position is considered more-likely-than-not to be sustained on examination by a taxing authority, assuming they possess full knowledge of the position and facts. Interest and penalties related to uncertain tax positions are recognized in the provision of income taxes; however, the Company currently has no interest or penalties related to uncertain income tax benefits.

#### ***Net Loss Per Share***

The Company calculates basic and diluted net loss per share in conformity with the two-class method required for participating securities because the Company had both voting and non-voting common stock outstanding in 2023 and for a portion of 2024. The rights, including the liquidation and dividend rights, of the holders of the voting and non-voting common stock are identical, except with respect to voting. Each share of non-voting common stock may be converted at any time into one share of voting common stock at the option of its holder by providing written notice to the Company, subject to the limitations provided for in the amended and restated certificate of incorporation. As of December 31, 2024, no shares of non-voting common stock remain outstanding.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss by the weighted average number of shares of common stock, including any pre-funded warrants to purchase shares of common stock that may be outstanding. The undistributed loss for each year is allocated to common stockholders based on the contractual participation rights of the voting and non-voting common stock as if the losses for the year had been distributed. As the liquidation and dividend rights are identical, the undistributed losses are allocated on a proportionate basis. Diluted net loss per share attributable to common stockholders is computed under the if-converted method and assumes that all non-voting common stock has been converted to common stock. Since the Company was in a loss position for all periods presented, the effects of the other potentially dilutive securities are antidilutive.

#### ***Segments***

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Maker (CODM) in deciding how to allocate resources to an individual segment and in assessing performance. The Company's CODM is its Chief Executive Officer (CEO). The Company has determined it operates in a single operating segment and has one reportable segment. Refer to Note 8 – Segment Reporting for further information related to our segment.

#### ***Deferred Offering Costs***

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated.

After consummation of the equity financing, these costs are recorded as a reduction to the carrying value of stockholders' equity as a reduction of additional paid-in capital or equity generated as a result of such offering. Should an in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the statements of operations and comprehensive loss.

#### ***Emerging Growth Company Status***

Prior to December 31, 2024, the Company was an "emerging growth company" (EGC) as defined in the Jumpstart Our Business Startups Act, (JOBS Act), and elected to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies until the Company no longer qualifies as an EGC, including using the extended transition period for complying with new or revised accounting standards. As of December 31, 2024, the Company ceased to qualify as an EGC.

#### ***Recently Adopted Accounting Pronouncements***

In November 2023, the FASB issued Accounting Standards Update, or ASU, 2023-07, *Segment Reporting (Topic 280) – Improvements to Reportable Segment Disclosures*. This ASU requires that a public entity provide additional segment disclosures on an interim and annual basis. The amendments in this ASU should be applied retrospectively to all prior periods presented in the consolidated financial statements, unless impracticable. Upon transition, the segment expense categories and amounts disclosed in the prior periods should be based on the significant segment expense categories identified and disclosed in the period of adoption. The Company adopted this ASU for the annual period ended December 31, 2024 and the amendments have been applied retrospectively to all prior periods presented in the financial statements. Refer to Note 8 – Segment Reporting for further information related to our segment.

#### ***Recently Issued Accounting Pronouncements***

In December 2023, the FASB issued ASU 2023-09, *Improvements to Income Tax Disclosures*. ASU 2023-09 enhances the transparency about income tax information through improvements to income tax disclosures primarily related to the rate reconciliation and income taxes paid information. The guidance is effective for public business entities for annual periods beginning after December 15, 2024. Early adoption is permitted. This update is effective beginning with the Company's 2025 fiscal year annual reporting period. The Company is currently planning to adopt this guidance when effective and is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes.

In November 2024, the FASB issued ASU 2024-03, *Income Statement (Subtopic 220-40): Reporting Comprehensive Income-Expense Disaggregation Disclosures*. ASU 2024-03 requires enhanced disclosures about components of expense captions on the face of the income statement. This standard will be effective for fiscal years beginning after December 15, 2026. Early adoption is permitted. ASU 2024-03 applies on a prospective basis for periods beginning after the effective date. However, retrospective application to any or all prior periods presented is permitted. The Company is currently planning to adopt this guidance when effective and is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes.

There have been no other new accounting pronouncements or changes to accounting pronouncements that, if adopted, would have or may have a material impact on the Company's consolidated statements or disclosures.

### 3. Fair Value Measurements

As of December 31, 2024 and 2023, the Company's financial instruments included cash and cash equivalents, available-for-sale debt securities, accounts payable and accrued expenses. The carrying amounts for cash and cash equivalents, accounts payable and accrued expenses reported in the Company's consolidated financial statements for these instruments approximate their respective fair values because of the short-term nature of these instruments.

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

	December 31, 2024			
	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<b>Financial assets</b>				
Cash equivalents:				
Money market funds	\$ 162,634	\$ 162,634	\$ —	\$ —
Short-term investments:				
U.S. Treasury securities	—	—	—	—
Total	<u>\$ 162,634</u>	<u>\$ 162,634</u>	<u>\$ —</u>	<u>\$ —</u>

	December 31, 2023			
	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<b>Financial assets</b>				
Cash equivalents:				
Money market funds	\$ 180,124	\$ 180,124	\$ —	\$ —
U.S. Treasury securities - original maturity less than three months	12,371	—	12,371	—
Short-term investments:				
U.S. Treasury securities	48,011	—	48,011	—
Total	<u>\$ 240,506</u>	<u>\$ 180,124</u>	<u>\$ 60,382</u>	<u>\$ —</u>

Money market funds are measured at fair value on a recurring basis using quoted prices and are classified as Level 1. Investments are measured at fair value based on inputs other than quoted prices that are derived from observable market data and are classified as Level 2 inputs.

For debt securities classified as available-for-sale investments, the Company records unrealized gains or losses resulting from changes in fair value between measurement dates as a component of other comprehensive income. The Company did not hold any available-for-sale securities as of December 31, 2024:

	December 31, 2023			Fair value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
<b>Financial assets</b>				
Cash	\$ 743	\$ —	\$ —	\$ 743
Money market funds	180,124	—	—	180,124
U.S. Treasury securities - original maturity less than three months	12,367	4	—	12,371
<i>Included in cash and cash equivalents</i>	193,234	4	—	193,238
U.S. Treasury securities - due in one year or less	47,976	39	(4)	48,011
<i>Included in short-term investments</i>	47,976	39	(4)	48,011
Total	<u>\$ 241,210</u>	<u>\$ 43</u>	<u>\$ (4)</u>	<u>\$ 241,249</u>

#### 4. Property, Plant and Equipment

Property, plant and equipment consists of the following:

	December 31,	
	2024	2023
Laboratory equipment	\$ 7,663	\$ 5,893
Furniture and fixtures	277	277
Computer equipment	196	53
Leasehold improvements	113	113
Total property, plant and equipment	8,249	6,336
Less: accumulated depreciation	(5,506)	(3,795)
Property, plant and equipment, net	<u>\$ 2,743</u>	<u>\$ 2,541</u>

Depreciation expense was \$1,711 and \$1,426 for the years ended December 31, 2024 and 2023, respectively.

#### 5. Accrued and Other Current Liabilities

Accrued and other current liabilities consist of the following:

	December 31,	
	2024	2023
Research and development services	\$ 3,491	\$ 2,459
General and administrative services	342	188
Compensation expense	8,315	5,200
Other	40	40
	<u>\$ 12,188</u>	<u>\$ 7,887</u>

#### 6. Collaborations, Licensing Agreements and other Agreements

##### *Amended and Restated License Agreement with the Trustees of the University of Pennsylvania and Children's Hospital of Philadelphia*

In August 2018, the Company entered into a license agreement with Penn, as amended and restated in July 2019 to include the Children's Hospital of Philadelphia (CHOP) as a party, and as amended in May 2020 and October 2021 (the License Agreement) pursuant to which the Company obtained (a) a non-exclusive, non-sublicensable worldwide license to certain of Penn's intellectual property to conduct research, product development, clinical trials, cell manufacturing and other activities, and (b) an exclusive, worldwide, royalty-bearing right and license, with a right to sublicense, on a target-by-target basis, under certain of Penn's intellectual property to make, use, sell, offer for sale, import, and otherwise commercialize products for the treatment of autoimmune and alloimmune diseases. Unless earlier terminated, the License Agreement expires on the expiration or abandonment or other termination of the last valid claim in Penn's intellectual property licensed by the Company. The Company may terminate the License Agreement at any time for convenience upon 60 days' written notice. In the event of an uncured, material breach, Penn may terminate the License Agreement upon 60 days' written notice. Under the terms of the License Agreement, the Company was obligated to pay \$2,000 annually for three years beginning August 2018 for funding to the laboratories of each of Drs. Milone and Payne. This was satisfied through completed sponsored research agreements with a total cost of \$12,560. During the term of the License Agreement until the first commercial sale of the first product, the Company is obligated to pay Penn a non-refundable, non-creditable annual license maintenance fee of \$10. In May 2020, the Company paid Penn an additional, non-refundable, non-creditable license fee of \$33 under the amended License Agreement.



The Company is required to pay certain milestone payments upon the achievement of specified clinical and commercial milestones. Milestone payments are reduced by a certain percentage for the second product that achieves a milestone, by an additional percentage for the third product that achieves a milestone, and so on, for each subsequent product that achieves a milestone. In the event that the Company is able to successfully develop and launch multiple products under the License Agreement, total milestone payments could be approximately \$21,000. Penn is also eligible to receive tiered royalties at percentage rates in the low single-digits, subject to an annual minimum royalty, on annual worldwide net sales of any products that are commercialized by the Company or its sublicensees that contain or incorporate, or are covered by, the intellectual property licensed by the Company. To the extent the Company sublicenses its license rights under the License Agreement, Penn would be eligible to receive tiered sublicense income at percentage rates in the mid-single to low double-digits. There were no amounts due under the License Agreement as of December 31, 2024.

#### ***Master Translational Research Services Agreement***

In October 2018 and February 2023, the Company entered into services agreements (the CAART and CARTA Services Agreements) with Penn for research, development and manufacturing services from various laboratories within Penn. The activities are detailed in separately executed Penn organization-specific addenda. In May 2020, the Company amended its Addendum with the Center for Advanced Retinal and Ocular Therapeutics (CAROT) to expand access to vector manufacturing.

Research and development expense related to executed addenda under the master translational research service agreements with Penn recognized in the accompanying statements of operations was \$3,250 and \$3,786 for the years ended December 31, 2024 and 2023, respectively. The Company is committed to pay up to \$3,000 under the CARTA Services Agreement for cell processing manufacturing through December 31, 2025. The Company may incur additional expenses up to \$643 through the remaining term of the CAROT Amended Addendum.

#### ***Exclusive License Agreement with IASO Biotherapeutics***

On October 7, 2022, the Company entered into an Exclusive License Agreement (the IASO Agreement) with Nanjing IASO Biotherapeutics Co., Ltd. (IASO). Pursuant to the IASO Agreement, the Company received an exclusive, worldwide license under certain IASO intellectual property to use a novel clinical-stage anti-CD19 binder to develop, manufacture, commercialize and otherwise exploit T cell products directed to CD19 for the purpose of diagnosis, prevention or treatment of any autoimmune or alloimmune indications in humans. As partial consideration for the exclusive license, IASO received an upfront payment of \$2,500. IASO is also eligible to receive up to mid double digit millions in milestone payments based upon the achievement of specified pre-clinical, development and regulatory milestones, and up to an additional low triple digit millions in milestone payments based upon achievement of specified sales milestones, for a total consideration, inclusive of the upfront payment, of up to \$162,000, along with tiered mid-single digit royalties on future net sales for licensed products that may result from the IASO Agreement. Upon the U.S. Food and Drug Association's clearance of the rese-cel Investigational New Drug application for the treatment of systemic lupus erythematosus in March 2023, a milestone payment of \$1,000 was recognized in the accompanying statements of operations. A milestone payment of \$1,500 was paid to IASO in the first quarter of 2024 after the first patient in a rese-cel trial was dosed.

IASO has the right of first negotiation if the Company desires to grant a third party an exclusive license to develop, manufacture, commercialize or otherwise exploit the licensed products in the Greater China region. Pursuant to the IASO Agreement, each of IASO and the Company have agreed, subject to certain exceptions, to refrain from engaging in certain competitive activities with respect to certain programs. The Company also may sublicense through multiple tiers the rights granted to it by IASO under the IASO Agreement at any time, however, it must pay IASO a low double-digit percentage of any revenue obtained from sublicenses or options to third parties, subject to certain customary exclusions. The IASO Agreement will continue on a country-by-country, licensed product-by-licensed product basis until the expiration of the royalty term as identified in the IASO Agreement, unless earlier terminated. Each of the Company and IASO may terminate the Agreement for a material, uncured breach or insolvency of the other party. The Company may also terminate the Agreement at will upon advance written notice and in the event IASO rejects the Agreement due to bankruptcy-related matters. IASO may also terminate the Agreement if the Company fails to achieve certain specified diligence milestones in a timely manner and/or if the Company commences any patent challenges with respect to the patents and patent applications relating to the licensed sequence, in each case upon advance written notice.

### ***Licence and Supply Agreement with Oxford Biomedica***

In December 2021, the Company entered into a Licence and Supply agreement (LSA) with Oxford Biomedica (UK) Limited (Oxford), wherein the LSA grants the Company a non-exclusive license to Oxford's LentiVector® platform for its application in the Company's DSG3-CAART program and put in place a multi-year vector supply agreement. Under the terms of the agreement, the Company was required to pay Oxford an upfront fee, as well as costs associated with initial vector manufacturing activities for a total cost incurred of \$1,100. Oxford is eligible to receive regulatory and sales milestones in the low tens of millions and royalties in the low single digits on net sales of products that incorporate the Oxford technology. In May 2023, the Company amended the LSA with Oxford to expand the license to include the Company's rese-cel program for an upfront fee of \$500, and in August 2023, the Company and Oxford entered into a vector supply agreement for rese-cel, and a related second amendment to the LSA, with a total cost under the vector supply agreement of up to approximately \$5,000, of which approximately \$3,500 and \$1,500 were recognized as research and development expense for the years ended December 31, 2024 and 2023, respectively. In February 2024, the Company and Oxford entered into a third amendment to the LSA to update the patent schedule. In June 2024, the Company and Oxford entered into a fourth amendment to the LSA eliminating royalties on net sales of products that incorporate the Oxford technology if Oxford manufactures the vector. In December 2024, the Company and Oxford entered into a work order with a cost of approximately \$9,000 for certain process characterization activities as part of commercial readiness activities. The Company can terminate the LSA or any work order under the LSA at will upon advance written notice and subject to certain cancellation fees.

### ***Option and License Agreement with Autolus***

In January 2023, the Company entered into an Option and License Agreement (Autolus Agreement) with Autolus Holdings (UK) Limited (Autolus), wherein the Autolus Agreement granted the Company a non-exclusive license to access Autolus' RQR8 technology in its CD19-CAR T cell therapy program, and subject to additional nominal option exercise fees, up to four additional targets. In January 2025, the Company and Autolus agreed to novate the Autolus Agreement with both Autolus and Autolus Limited jointly assuming the rights, obligations and liabilities of Autolus, under the Autolus Agreement. Under the terms of the Autolus Agreement, the Company was required to pay Autolus an upfront license fee of \$1,200 that was recognized as expense in the first quarter of 2023 in the accompanying statements of operations, of which \$1,100 was paid in 2023 and \$100 was paid in January 2024. Autolus is also eligible to receive regulatory milestones of up to \$12,000 for each licensed target, sales milestones of up to a total of \$15,000 and royalties in the low single digits on net sales of all products that incorporate the RQR8 technology. The Autolus Agreement will continue on a country-by-country, licensed product-by-licensed product basis until the expiration of the royalty term as identified in the Autolus Agreement, unless earlier terminated. The Company can terminate the Autolus Agreement at will upon advance written notice. Each of the Company and Autolus may terminate the Agreement for a material, uncured breach or insolvency of the other party.

## **7. Commitments and Contingencies**

### ***Manufacturing Agreements***

In January 2021, the Company entered into a Development and Manufacturing Services Agreement (WuXi Agreement) with WuXi Advanced Therapies, Inc. (WuXi) to serve as an additional cell processing manufacturing partner for the MuSK-CAART Phase 1 clinical trial, or MuSK-CAARTes™ trial. The WuXi Agreement is scheduled to expire upon completion of WuXi's services related to MuSK-CAART and rese-cel. In August 2023, and as extended in August 2024, the Company entered into an agreement with WuXi to serve as one of the Company's manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and commercial readiness activities for rese-cel. Under the August 2023 work orders, WuXi converted the Company's non-dedicated suite to a dedicated suite for GMP manufacturing for the Company's rese-cel and MuSK-CAART programs, or the Dedicated Suite, for an initial term of 18 months with two 18-month extensions at the Company's sole option on six months' notice prior to the end of the term. In August 2024, the Company sent a notice to WuXi to extend the initial term of the WuXi Agreement by 18 months through August 2026. In addition, the Company agreed to certain monthly minimum runs. In August 2024, the 2023 work order related to GMP manufacturing was amended to reduce the minimum monthly runs through the end of 2024. In lieu of the original \$1,500 termination fee under the terms of the WuXi Agreement, the Company would incur up to a \$1,080 termination fee if it terminates both the rese-cel and MuSK-CAART work orders for any reason. The Company may terminate for

convenience the WuXi Agreement or any work order with six months' prior written notice. However, the Company may not terminate the Dedicated Suite without terminating both the MuSK-CAART and rese-cel GMP run work orders. WuXi may terminate for convenience the WuXi Agreement or any work order on 18 months' prior written notice, but such notice may not be effective prior to February 2028.

In December 2024, the Company entered into a Development and Manufacturing Services Agreement (the Lonza Agreement) with Lonza Houston Inc. (Lonza) to serve as one of the Company's manufacturing partners for the global clinical development of rese-cel in multiple indications, including potential late-stage clinical trials and preparations for commercial readiness. Under the initial work order, Lonza will perform cell therapy manufacturing activities for Cabaletta's CAR-T cell therapy product rese-cel for a term of 12 months and total cost of approximately \$16,000 with the ability to extend the manufacturing period subject to the terms of the Lonza Agreement.

#### ***Other Purchase Commitments***

In the normal course of business, the Company enters into various purchase commitments with third-party contract manufacturers for the manufacture and processing of its product candidates and related raw materials, contracts with contract research organizations for clinical trials and agreements with vendors for other services and products for operating purposes. These agreements generally provide for termination or cancellation, other than for costs already incurred.

#### ***Indemnification***

The Company enters into certain types of contracts that contingently require the Company to indemnify various parties against claims from third parties. These contracts primarily relate to (i) the Company's Amended and Restated Bylaws, as amended, (bylaws) under which the Company must indemnify directors and executive officers, and may indemnify other officers and employees, for liabilities arising out of their relationship, (ii) contracts under which the Company must indemnify directors and certain officers and consultants for liabilities arising out of their relationship, (iii) contracts under which the Company may be required to indemnify partners against certain claims, including claims from third parties asserting, among other things, infringement of their intellectual property rights, and (iv) procurement, consulting, or license agreements under which the Company may be required to indemnify vendors, consultants or licensors for certain claims, including claims that may be brought against them arising from the Company's acts or omissions with respect to the supplied products, technology or services. From time to time, the Company may receive indemnification claims under these contracts in the normal course of business. In addition, under these contracts, the Company may have to modify the accused infringing intellectual property and/or refund amounts received.

In the event that one or more of these matters were to result in a claim against the Company, an adverse outcome, including a judgment or settlement, may have a material adverse effect on the Company's future business, operating results or financial condition. It is not possible to determine the maximum potential amount under these contracts due to the limited history of prior indemnification claims and the unique facts and circumstances involved in each particular agreement.

#### ***Litigation***

From time to time, the Company may become involved in litigation or legal proceedings. While the outcome of any such proceedings cannot be predicted with certainty, as of December 31, 2024, the Company is not involved in any material litigation or legal proceedings that it would expect to have a material adverse impact on its financial position, results of operations, or cash flows.

### **8. Segment Information**

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the CODM in deciding how to allocate resources to an individual segment and in assessing performance. The Company's CODM is the CEO. The Company has determined that it has one operating and reportable segment. The segment consists of the development of clinical and preclinical product candidates for the discovery and development of innovative engineered T cell therapies and related administrative activities. The

CODM assesses performance and allocates resources based on the Company's consolidated statement of operations, which is reported on the income statement as consolidated net loss and considers forecast-to-actuals variances on a quarterly basis for expenses that are deemed significant. Further, the CODM reviews the segment's assets based on the consolidated balance sheet to assess liquidity and funding capacity. The majority of the Company's assets are located in the United States.

The Company's CODM views specific categories within expenses as significant given the direct correlation between cash burn and profitability as a pre-revenue company. Significant segment expenses, as provided to the CODM, are presented below. For additional information on the year over year change of segment expenses refer to Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Research and development expenses:		
Personnel	\$ 39,239	\$ 22,375
Clinical trials	22,286	8,302
Development services	16,417	12,486
Manufacturing of preclinical and clinical supplies	15,523	8,498
License of intellectual property	1,523	2,383
Other research and development costs <sup>(1)</sup>	2,215	1,380
General and administration expenses	27,938	19,236
Interest income	(10,025)	(6,985)
Interest expense	748	—
Net loss	<u>\$ 115,864</u>	<u>\$ 67,675</u>

<sup>(1)</sup> Other research and development costs includes costs associated with information technology, travel, medical and scientific symposiums and conferences.

## 9. Leases

The Company leases office and laboratory space in addition to a manufacturing agreement which has been determined to contain an embedded lease.

### *Operating lease commitments*

The Company leases office and laboratory space which include rent escalations and are subject to additional variable charges, including common area maintenance, property taxes and property insurance. Given the variable nature of such costs, they are recognized as expense as incurred. Additionally, some of the Company's leases are subject to certain fixed fees which the Company has determined to be non-lease components. The Company has elected the practical expedient to account for lease and non-lease components as a single-lease component and has included fixed payments related to non-lease components in calculating the operating lease liability. In 2024, the Company extended the terms of its lab and office operating leases and leased additional lab space resulting in an increase in the right-of-use asset and lease liability of \$4,242.

### *Finance lease commitments - Embedded leases*

As described further in Note 7, in August 2023, the Company entered into new work orders under the WuXi Agreement for WuXi to serve as one of the Company's cell processing manufacturing partners for the global clinical development of rese-cel. WuXi converted the Company's non-dedicated suite to a Dedicated Suite for GMP manufacturing for the Company's rese-cel and MuSK-CAART programs, for an initial term of 18 months. The terms of the August 2023 work orders included both fixed costs and contingent variable costs. The lease commenced October 1, 2023. In 2024, the Company remeasured the lease due to resolution of the contingency related to variable costs and an amendment to the 2023 work order to extend the term of the agreement for an additional 18 months through August

2026 resulting in an increase in the right-of-use asset and lease liability of \$10,963. The Company may terminate the Dedicated Suite lease for convenience with six months' prior written notice and up to a \$1,080 termination fee if both the rese-cel and MuSK-CAART work orders are terminated.

As described further in Note 7, in December 2024, the Company entered into the Lonza Agreement with Lonza to serve as one of our manufacturing partners. Under the initial work order, Lonza will perform cell therapy manufacturing activities for our CAR-T cell therapy product rese-cel for a term of 12 months and total cost of approximately \$16,000 with the ability to extend the manufacturing period subject to the terms of the Lonza Agreement. The Lonza Agreement is currently being evaluated for lease accounting under ASC 842. No right-of-use asset or liability was recognized as of December 31, 2024, as lease commencement begins in fiscal year 2025.

*Summary of lease costs recognized under ASC 842*

The following table contains a summary of the lease costs recognized under ASC 842 and other information pertaining to the Company's operating and finance leases for the years ended December 31, 2024 and 2023.

	<b>For the year ended December 31,</b>	
	<b>2024</b>	<b>2023</b>
<b>Finance leases</b>		
Interest expense	\$ 748	\$ —
Amortization expense	3,102	—
Total fixed finance lease cost	\$ 3,850	\$ —
Variable lease cost	930	—
Total finance lease cost	<u>\$ 4,780</u>	<u>\$ —</u>
<b>Operating leases</b>		
Fixed lease cost	\$ 3,247	\$ 2,477
Total fixed operating lease cost	\$ 3,247	\$ 2,477
Variable lease cost	296	65
Short-term lease cost	750	717
Total operating lease cost	<u>\$ 4,293</u>	<u>\$ 3,259</u>
<b>Cash paid in the measurement of lease liabilities</b>		
Operating cash flows used for operating leases	\$ 3,291	\$ 2,456
Operating cash flows used for finance leases	748	—
Financing cash flows for finance leases	897	—
<b>Other information</b>		
Weighted average remaining lease term - finance leases (in years)	1.7	—
Weighted average discount rate - finance leases	11.5 %	—
Weighted average remaining lease term - operating leases (in years)	1.7	1.5
Weighted average discount rate - operating leases	11.0 %	9.5 %

For finance leases embedded in CDMO arrangements, interest expense is recognized using the effective interest method, applying the Company's incremental borrowing rate as required by ASC 842, and amortization expense is recognized on a straight-line basis over the shorter of the life of the asset or the term of the lease.

Future lease payments under the non-cancelable operating and finance leases as of December 31, 2024 are as follows:

	Finance Leases	Operating Leases
2025	\$ 6,328	\$ 4,227
2026	3,792	2,769
Total undiscounted lease payments	10,120	6,996
Less imputed interest	(890)	(626)
Total lease liability	<u>\$ 9,230</u>	<u>\$ 6,370</u>

## 10. Common Stock

### *Common Stock*

Pursuant to the Company's Third Amended and Restated Certificate of Incorporation filed in October 2019, the Company is authorized to issue 143,590,481 shares of voting common stock and 6,409,519 shares of non-voting common stock. Holders of voting common stock shall have the exclusive right to vote for the election of directors of the Company and on all other matters requiring stockholder action. Each share of the Company's non-voting common stock may be converted at any time into one share of common stock at the option of its holder by providing 61 days written notice to the Company, subject to certain limitations, as described in the amended and restated certificate of incorporation. In May 2024, 1,444,295 shares of non-voting common stock was converted to voting common stock and no shares of non-voting common stock remain outstanding.

### *May 2023 Financing*

In May 2023, the Company issued 8,337,500 shares of its common stock in an underwritten public offering, including the exercise in full by the underwriters of their option to purchase an additional 1,087,500 shares, at a public offering price of \$12.00 per share. Aggregate net proceeds were \$93,755 after deducting underwriting discounts and commissions and offering expenses of \$6,295.

### *December 2022 Financing*

In December 2022, the Company issued 126,815 shares of its common stock at a price of \$5.52 per share and to certain investors in lieu of common stock, pre-funded warrants to purchase 6,213,776 shares of common stock at a price of \$5.51999 per pre-funded warrant. The purchase price per share of each pre-funded warrant represents the per share offering price for the common stock, minus the \$0.00001 per share exercise price of such pre-funded warrant. Aggregate net proceeds were \$32,562 after deducting underwriting discounts and commissions and offering expenses. As of December 31, 2024, 6,213,775 pre-funded warrants had been exercised and no warrants remain outstanding.

The pre-funded warrants were classified as a component of permanent stockholders' equity within additional paid-in capital and were recorded at the issuance date using a relative fair value allocation method. The pre-funded warrants are equity classified because they (i) are freestanding financial instruments that are legally detachable and separately exercisable from the equity instruments, (ii) are immediately exercisable, (iii) do not embody an obligation for the Company to repurchase its shares, (iv) permit the holders to receive a fixed number of shares of common stock upon exercise, (v) are indexed to the Company's common stock and (vi) meet the equity classification criteria. In addition, such pre-funded warrants do not provide any guarantee of value or return.

### *At-the-Market Offering*

On March 21, 2024, the Company filed an automatic shelf registration statement (S-3ASR) in relation to the registration of common stock, preferred stock, debt securities, warrants and/or units of any combination thereof for the purposes of selling, from time to time, the Company's common stock, debt securities or other equity securities in one or more offerings. This S-3ASR became effective immediately. We intend to convert the S-3ASR to S-3 by post-effective amendments in connection with the filing of this Annual Report.

The Company has a Sales Agreement with Cowen and Company, LLC (Cowen) to provide for the offering, issuance and sale of up to an aggregate amount of \$200.0 million of common stock from time to time in "at-the-market" offerings (2024 ATM Program) pursuant to its S-3ASR, and subject to the limitations thereof. In December 2024, the Company sold 1,225,000 shares pursuant to the 2024 ATM Program for net proceeds of \$5,129, after deducting commissions of \$105.

The Company previously had a Sales Agreement with Cowen to provide for the offering, issuance and sale of up to an aggregate amount of \$100.0 million of common stock from time to time in "at-the-market" offerings (2023 ATM Program) pursuant to its shelf registration statement on Form S-3 (File No. 333-270599), which was declared effective April 26, 2023. During the year ended December 31, 2023, the Company sold 4,760,899 shares pursuant to the 2023 ATM Program for net proceeds of \$91,740, after deducting commissions of \$2,352. In the first quarter of 2024, the Company sold 258,070 additional shares, completing the 2023 ATM Program for net proceeds of \$5,746, after deducting commissions of \$147.

#### ***2018 Stock Option and Grant Plan***

In September 2018, the Company adopted the 2018 stock option and grant plan (the 2018 Plan), which provided for the Company to sell or issue common stock, or other stock-based awards, to employees, members of the board of directors and consultants of the Company. The Company generally granted stock-based awards with service conditions only (service-based awards), although there was one grant with performance conditions. As of December 31, 2020, there are no unvested options with performance conditions. Stock options granted under the 2018 Plan generally vest over three to four years. There were 1,959,411 options granted under the 2018 Plan prior to the Company's IPO in October 2019. No further grants may be made under the 2018 Plan subsequent to the IPO.

#### ***2019 Stock Option and Incentive Plan***

The 2019 Stock Option and Incentive Plan (2019 Plan) was approved by the Company's board of directors on October 14, 2019, and became effective on October 23, 2019. The 2019 Plan provides for the grant of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock units, restricted stock awards, unrestricted stock awards, cash-based awards and dividend equivalent rights to the Company's officers, employees, directors and consultants. The number of shares initially reserved for issuance under the 2019 Plan was 2,342,288, which will be increased each January 1 thereafter by 4% of the number of shares of the Company's common stock outstanding on the immediately preceding December 31 or such lesser number of shares determined by the Company's board of directors or compensation committee of the board of directors. On June 1, 2023, at the 2023 Annual Meeting of Stockholders of the Company, the stockholders of the Company approved Amendment No. 1 to the 2019 Plan, increasing the number of shares of common stock reserved for issuance under the 2019 Plan by 3,000,000 shares. On January 1, 2025, the total number of shares under the 2019 Plan was increased by 2,029,724 shares pursuant to the 2019 Plan Evergreen Provision. As of December 31, 2024, there were 1,379,305 shares remaining available for issuance under the 2019 Plan.

A summary of the stock option activity is presented below:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value
Outstanding as of January 1, 2024	8,141,035	\$ 8.28	7.6	\$ 117,384
Granted	3,476,700	16.13		
Exercised	(210,695)	6.79		2,889
Forfeited/Cancelled	(175,892)	15.04		
Outstanding as of December 31, 2024	<u>11,231,148</u>	\$ 10.63	7.4	\$ 989
Options Exercisable at December 31, 2024	<u>5,642,705</u>	\$ 7.92	6.1	\$ 966

The aggregate intrinsic value of options granted is calculated as the difference between the exercise price of the options and the estimated fair value of the Company's common stock. The weighted average grant-date fair value of stock options granted during the year ended December 31, 2024 and 2023 was \$13.44 and \$9.51, respectively.

The fair value of each award is estimated using Black-Scholes based on the following assumptions:

	Year Ended December 31,	
	2024	2023
Risk-free interest rate	3.43%—4.60%	3.38%—4.68%
Expected term	5.5 years—6.2 years	5.5 years—6.1 years
Expected volatility	106%—111%	105%—107%
Expected dividend yield	0%	0%

Black-Scholes requires the use of subjective assumptions which determine the fair value of stock-based awards. These assumptions include:

*Expected term*—The expected term represents the period that stock-based awards are expected to be outstanding. The expected term for option grants is determined using the simplified method, which is the midpoint between the vesting period and the contractual term of the option.

*Expected volatility*—As a privately held company prior to the Company's IPO in October 2019, the Company has limited trading history for its common stock and, as such, the expected volatility is estimated based on a weighted average volatility for the Company's stock price and comparable publicly traded biotechnology companies over a period equal to the expected term of the stock-based awards. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

*Risk-free interest rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of a stock-based award.

*Expected dividend*—The Company has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

#### ***Stock-based Compensation***

The Company has recorded stock-based compensation in the accompanying statements of operations as follows:

	Year Ended December 31,	
	2024	2023
Research and development	\$ 10,163	\$ 5,520
General and administrative	9,302	5,823
Total	<u>\$ 19,465</u>	<u>\$ 11,343</u>



As of December 31, 2024, there was \$51,239 of unrecognized compensation cost related to unvested option awards, which is expected to be recognized over a weighted-average period of 2.8 years.

### 2019 Employee Stock Purchase Plan

The 2019 Employee Stock Purchase Plan (2019 ESPP) was approved by the Company's board of directors on October 14, 2019, and became effective on October 23, 2019. A total of 234,229 shares of common stock were initially reserved for issuance under the 2019 ESPP, and such number of shares will be increased each January 1 thereafter through January 1, 2029 by the least of (i) 234,229 shares of common stock, (ii) 1% of the number of shares of the Company's common stock outstanding on the immediately preceding December 31 or (iii) such lesser number of shares determined by the 2019 ESPP's administrator. There was no increase to the total number of shares available under the 2019 ESPP on January 1, 2024, 2023 or 2022, respectively. As of December 31, 2024, there were 314,259 shares remaining available for issuance under the 2019 ESPP.

Employee contributions are made through payroll deductions of up to 15% of eligible compensation over the offering period. A participant may not accrue rights to purchase more than \$25 worth of the Company's common stock for each calendar year in which such right is outstanding. At the end of each offering period, shares of the Company's common stock may be purchased at 85% of the lesser of the Company's common stock on (i) the first trading day of the relevant offering period and (ii) the last trading day of the relevant offering period. Each offering period will be six months in duration and will commence on each December 1 and June 1.

## 11. Income Taxes

The reconciliation of federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,	
	2024	2023
Expected income tax benefit at the federal statutory rate	21.0 %	21.0 %
State and local taxes, net of federal benefit	7.7	7.6
Research and development credit, net	5.3	6.4
Non-deductible items and other	(1.1)	(0.5)
Change in state and local tax rate	—	(0.7)
Change in valuation allowance	(32.9)	(33.8)
Total	0.0 %	0.0 %

Deferred income taxes reflect the net effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The principal components of the Company's deferred tax assets and liabilities consisted of the following:

	December 31,	
	2024	2023
Deferred tax assets:		
Federal, state and local net operating loss carryforwards	\$ 44,369	\$ 34,534
Capitalized research and development costs	36,646	19,830
Research and development tax credits	16,296	10,192
Stock-based compensation deductions	9,919	6,066
License fee deductions	221	240
Operating lease liabilities	4,607	1,482
Accrued expenses	2,603	1,665
Gross deferred tax assets	114,661	74,009
Less: valuation allowance	(110,723)	(72,560)
Total deferred tax assets	3,938	1,449
Deferred tax liabilities:		
Operating lease right-of-use assets	(3,938)	(1,449)
Net deferred tax assets	<u>\$ —</u>	<u>\$ —</u>

The Company increased its valuation allowance by \$38,163 for the year ended December 31, 2024 in order to maintain a full valuation allowance against its deferred tax assets. Based on the Company's history of losses, the Company recorded a full valuation allowance against its deferred tax assets as of December 31, 2024. The Company intends to maintain a valuation allowance until sufficient positive evidence exists to support a reversal of the allowance.

As of December 31, 2024, the Company had federal, state and local net operating loss carryforwards of \$157,448, \$181,362 and \$88,701, respectively; \$157,198 of the federal net operating losses do not expire, and the remaining \$250 expire in 2037. The state net operating losses begin to expire in 2037. The local net operating losses begin to expire in 2042. As of December 31, 2024, the Company had federal and state research and development tax credit carryforwards of \$15,684 and \$612, respectively, which begin to expire in 2038 and 2036, respectively. Under the provisions of Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the IRC), these net operating losses, credit carryforwards and other tax attributes may be subject to limitation based on previous significant changes in ownership and upon future significant changes in ownership of the Company, as defined by the IRC.

The Company files income tax returns in the U.S. federal jurisdiction as well as in Pennsylvania and Philadelphia. The tax years 2023, 2022 and 2021 remain open to examination by the jurisdictions where the Company is subject to tax.

The Company evaluates tax positions for recognition using a more-likely-than-not recognition threshold, and those tax positions eligible for recognition are measured as the largest amount of tax benefit that is greater than 50% likely of being realized upon the effective settlement with a taxing authority that has full knowledge of all relevant information. As of December 31, 2024, the Company had no unrecognized income tax benefits that would affect the Company's effective tax rate if recognized.

## 12. Net Loss Per Share

The Company calculates basic and diluted net loss per share in conformity with the two-class method required for participating securities. For the year ended December 31, 2023, the Company had voting and non-voting common stock outstanding. In May 2024, 1,444,295 shares of non-voting common stock was converted to voting common stock and as of December 31, 2024, no shares of non-voting common stock remain outstanding. Since the rights of the voting and non-voting common stock are identical, except with respect to voting, the undistributed losses of the Company have been allocated on a proportionate basis to the two classes. Basic net loss per share of common stock is computed by dividing the net loss per share of common stock by the weighted average number of shares of common stock outstanding for the period.

The weighted average shares of common stock outstanding as of December 31, 2023 included outstanding pre-funded warrants to purchase up to an aggregate of 1,168,054 shares of common stock. No pre-funded warrants remain outstanding as of December 31, 2024.

Diluted net loss per share is calculated using the if-converted method, which assumes conversion of all non-voting common stock to voting common stock.

	Year ended December 31, 2024	
	Voting common stock	Non-voting common stock
<b>Basic net loss per share:</b>		
Numerator		
Allocation of undistributed losses	\$ (114,728)	\$ (1,136)
Denominator		
Weighted average number of shares used in basic per share computation	49,038,726	485,378
Net loss per share, basic	<u>\$ (2.34)</u>	<u>\$ (2.34)</u>
<b>Diluted net loss per share:</b>		
Numerator		
Allocation of undistributed losses for basic computation	\$ (114,728)	\$ (1,136)
Reallocation of undistributed losses as a result of conversion of non-voting to voting common shares	(1,136)	—
Allocation of undistributed losses	\$ (115,864)	\$ (1,136)
Denominator		
Weighted average number of shares used in basic per share computation	49,038,726	485,378
Add: conversion of non-voting to voting common shares outstanding	485,378	—
Weighted average number of shares used in diluted per share computation	49,524,104	485,378
Net loss per share, diluted	<u>\$ (2.34)</u>	<u>\$ (2.34)</u>

	<b>Year ended December 31, 2023</b>	
	<b>Voting common stock</b>	<b>Non-voting common stock</b>
<b>Basic net loss per share:</b>		
Numerator		
Allocation of undistributed losses	\$ (64,776 )	\$ (2,899 )
Denominator		
Weighted average number of shares used in basic per share computation	39,192,445	1,753,505
Net loss per share, basic	<u>\$ (1.65)</u>	<u>\$ (1.65)</u>
<b>Diluted net loss per share:</b>		
Numerator		
Allocation of undistributed losses for basic computation	\$ (64,776 )	\$ (2,899 )
Reallocation of undistributed losses as a result of conversion of non-voting to voting common shares	(2,899 )	—
Allocation of undistributed losses	\$ (67,675 )	\$ (2,899 )
Denominator		
Weighted average number of shares used in basic per share computation	39,192,445	1,753,505
Add: conversion of non-voting to voting common shares outstanding	1,753,505	—
Weighted average number of shares used in diluted per share computation	40,945,950	1,753,505
Net loss per share, diluted	<u>\$ (1.65)</u>	<u>\$ (1.65)</u>

The following outstanding potentially dilutive shares have been excluded from the calculation of diluted net loss per share, as their effect is anti-dilutive:

	<b>As of December 31,</b>	
	<b>2024</b>	<b>2023</b>
Stock options to purchase common stock	11,231,148	8,141,035
	<u>11,231,148</u>	<u>8,141,035</u>

### **13. 401(k) Savings Plan**

The Company maintains a defined-contribution savings plan under Section 401(k) of the IRC, or the 401(k) Plan. The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. Effective January 1, 2020, the Plan provided for matching contributions on a portion of participant contributions pursuant to the 401(k) Savings Plan's matching formula, up to 4% of eligible compensation. All matching contributions and participant contributions vest immediately. Contributions totaled \$894 and \$521 for the years ended December 31, 2024 and 2023, respectively, and have been recorded in the statements of operations.

## EXHIBIT INDEX

Exhibit Number	Description
3.1	<a href="#"><u>Third Amended and Restated Certificate of Incorporation of the Registrant, as currently in effect (incorporated by reference to Exhibit 3.1 of the Registrant's Current Report on Form 8-K (File No. 001-39103) filed on October 30, 2019)</u></a>
3.2	<a href="#"><u>Amended and Restated Bylaws of the Registrant, as currently in effect (incorporated by reference to Exhibit 3.2 of the Registrant's Current Report on Form 8-K (File No. 001-39103) filed on October 30, 2019)</u></a>
3.3	<a href="#"><u>Amendment No. 1 to the Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.3 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-39103) filed with the SEC on May 12, 2022)</u></a>
4.1	<a href="#"><u>Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 of the Registrant's Registration Statement on Form S-1 (File No. 333-234017) filed on October 16, 2019)</u></a>
4.2	<a href="#"><u>Description of Securities (incorporated by reference to Exhibit 4.3 to the Registrant's Annual Report on Form 10-K (File No. 001-39103) filed on March 21, 2024)</u></a>
4.3	<a href="#"><u>Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 of the Registrant's Current Report on Form 8-K (File No. 001-39103) filed with the SEC on December 12, 2022)</u></a>
10.1#	<a href="#"><u>2018 Stock Option and Grant Plan, as amended, and form of award agreements thereunder (incorporated by reference to Exhibit 10.1 of the Registrant's Registration Statement on Form S-1 (File No. 333-234017) filed on September 30, 2019)</u></a>
10.2#	<a href="#"><u>2019 Stock Option and Incentive Plan, and form of award agreements thereunder. (incorporated by reference to Exhibit 10.2 of the Registrant's Registration Statement on Form S-1/A (File No. 333-234017) filed on October 16, 2019)</u></a>
10.3#	<a href="#"><u>Amendment No. 1 to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on June 1, 2023)</u></a>
10.4#	<a href="#"><u>2019 Employee Stock Purchase Plan. (incorporated by reference to Exhibit 10.3 of the Registrant's Registration Statement on Form S-1/A (File No. 333-234017) filed on October 16, 2019)</u></a>
10.5#	<a href="#"><u>Senior Executive Cash Incentive Bonus Plan (incorporated by reference to Exhibit 10.4 of the Registrant's Quarterly Report on Form 10-Q filed on December 5, 2019)</u></a>
10.6#	<a href="#"><u>Form of Indemnification Agreement between the Registrant and each of its directors (incorporated by reference to Exhibit 10.5 of the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019)</u></a>
10.7#	<a href="#"><u>Form of Indemnification Agreement between the Registrant and each of its executive officers (incorporated by reference to Exhibit 10.4 of the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019)</u></a>
10.8+	<a href="#"><u>Amended and Restated License Agreement, dated as of July 23, 2019, among the Registrant, the Trustees of the University of Pennsylvania and the Children's Hospital of Philadelphia (incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019)</u></a>

- 10.9+ [Master Translational Research Services Agreement, dated as of October 2018, between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.10+ [CAROT Master Services Addendum to Master Translational Research Services Agreement, dated as of February 4, 2019, between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.11+ [CVPF Master Services Addendum to Master Translational Research Services Agreement, dated as of October 22, 2018, between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.11 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.12 [Lease, dated as of February 11, 2019, between the Registrant and Brandywine Cira, L.P. \(incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.13# [Employment Agreement between the Registrant and Steven Nichtberger \(incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.14# [Employment Agreement between the Registrant and Anup Marda \(incorporated by reference to Exhibit 10.17 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.15# [Employment Agreement between the Registrant and Gwendolyn Binder \(incorporated by reference to Exhibit 10.18 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.16# [Employment Agreement between the Registrant and David Chang \(incorporated by reference to Exhibit 10.19 to the Registrant's Registration Statement on Form S-1, filed with the SEC on September 30, 2019\)](#)
- 10.17\* [Fourth Amended and Restated Non-Employee Director Compensation Policy](#)
- 10.18+ [First Amendment, dated May 27, 2020, to the Amended and Restated License Agreement, dated July 23, 2019, among the Registrant, the Trustees of the University of Pennsylvania and the Children's Hospital of Philadelphia \(incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K \(File No. 001-39103\) filed on May 28, 2020\)](#)
- 10.19+ [Amendment to CAROT Master Services Addendum to Master Translational Research Services Agreement, dated as of May 18, 2020 between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on August 6, 2020\)](#)
- 10.20+ [Second Amendment, dated October 19, 2021, to the First Amended and Restated License Agreement, dated May 27, 2020, among the Registrant, the Trustees of the University of Pennsylvania and the Children's Hospital of Philadelphia \(incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 1, 2021\)](#)
- 10.21 [First Amendment, dated February 15, 2022, to the Lease, dated as of February 11, 2019, between the Registrant and Brandywine Cira, L.P. \(incorporated by reference to Exhibit 10.26 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 17, 2022\)](#)
- 10.22# [Form of Employment Agreement \(incorporated by reference to Exhibit 10.28 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 17, 2022\)](#)

- 10.23+ [Option Agreement, dated December 23, 2021, by and between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.30 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 17, 2022\)](#)
- 10.24+ [Licence and Supply Agreement, dated December 30, 2021, by and between the Registrant and Oxford Biomedica \(UK\) Limited \(incorporated by reference to Exhibit 10.31 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 17, 2022\)](#)
- 10.25+ [First Amendment to the Licence and Supply Agreement, dated as of May 2, 2023, between the Registrant and Oxford Biomedica \(UK\) Limited \(incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on August 10, 2023\)](#)
- 10.26+ [Second Amendment to the Licence and Supply Agreement, dated as of August 18, 2023, between the Registrant and Oxford Biomedica \(UK\) Limited \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 9, 2023\)](#)
- 10.27+ [Exclusive License Agreement, dated October 7, 2022, by and between the Registrant and Nanjing IASO Biotherapeutics Co., LTD.\(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 10, 2022\)](#)
- 10.28+ [Development, Manufacturing, and Testing Services Agreement, dated January 11, 2021, between the Registrant and WuXi Advanced Therapies Inc. \(incorporated by reference to Exhibit 10.34 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 16, 2023\)](#)
- 10.29+ [Master Translational Research Services Agreement, dated as of February 9, 2023, between the Registrant and the Trustees of the University of Pennsylvania \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on May 11, 2023\)](#)
- 10.30+ [Third Amendment to the Licence and Supply Agreement, dated as of February 21, 2024, between the Registrant and Oxford Biomedica \(UK\) Limited \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on May 15, 2024\)](#)
- 10.31+ [Fourth Amendment to the Licence and Supply Agreement, dated as of June 24, 2024, between the Registrant and Oxford Biomedica \(UK\) Limited \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on August 8, 2024\)](#)
- 10.32+ [Services Agreement, dated as of February 1, 2019, between the Registrant and CIC Innovation Communities, LLC \(incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 14, 2024\)](#)
- 10.33+ [Amendment to the Services Agreement, dated as of December 1, 2021, between the Registrant and CIC Innovation Communities, LLC \(incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 14, 2024\)](#)
- 10.34+ [Amendment to the Services Agreement, dated as of September 30, 2024, between the Registrant and CIC Innovation Communities, LLC \(incorporated by reference to Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 14, 2024\)](#)
- 10.35+ [Second Amendment, dated September 24, 2024, to the Lease, dated as of February 11, 2019, between the Registrant and Brandywine Cira, L.P., as amended. \(incorporated by reference to Exhibit 10.4 to the Registrant's Quarterly Report on Form 10-Q \(File No. 001-39103\) filed on November 14, 2024\)](#)
- 19.1\* [Insider Trading Policy](#)
- 21.1\* [List of Subsidiaries of the Registrant](#)



- 23.1\* [Consent of Ernst & Young, independent registered public accounting firm](#)
- 31.1\* [Certification of Principal Executive Officer Pursuant to Rules 13a-14\(a\) and 15d-14\(a\) under the Securities Exchange Act of 1934, as amended](#)
- 31.2\* [Certification of Principal Financial Officer Pursuant to Rules 13a-14\(a\) and 15d-14\(a\) under the Securities Exchange Act of 1934, as amended](#)
- 32.1\*\* [Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to 18 U.S.C. Section 1350, as adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002](#)
- 32.2\*\* [Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to 18 U.S.C. Section 1350, as adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002](#)
- 99.7 [Cabaletta Bio, Inc.'s Compensation Recovery Policy \(incorporated by reference to Exhibit 99.7 to the Registrant's Annual Report on Form 10-K \(File No. 001-39103\) filed on March 21, 2024\)](#)
- 101.INS\* Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
- 101.SCH\* Inline XBRL Taxonomy Extension Schema Document
- 101.CAL\* Inline XBRL Taxonomy Extension Calculation Linkbase Document
- 101.DEF\* Inline XBRL Taxonomy Extension Definition Linkbase Document
- 101.LAB\* Inline XBRL Taxonomy Extension Label Linkbase Document
- 101.PRE\* Inline XBRL Taxonomy Extension Presentation Linkbase Document
- 104\* Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101\*)

# Management Contract or compensatory plan or arrangement.

+ Portions of this exhibit (indicated by asterisks) have been omitted pursuant to Item 601(b)(10) of Regulation S-K.

\* Filed herewith.

\*\* The certifications furnished in Exhibit 32.1 and 32.2 hereto are deemed to accompany this Annual Report on Form 10-K and will not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended. Such certifications will not be deemed to be incorporated by reference into any filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Cabaletta Bio, Inc.

Date: March 31, 2025

By: */s/ Steven Nichtberger*  
**Steven Nichtberger, M.D.**  
*Chief Executive Officer and President*

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Annual Report on Form 10-K has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
<i>/s/ Steven Nichtberger</i> <b>Steven Nichtberger, M.D.</b>	<i>Director, Chief Executive Officer and President</i> <i>(Principal Executive Officer)</i>	March 31, 2025
<i>/s/ Anup Marda</i> <b>Anup Marda</b>	<i>Chief Financial Officer</i> <i>(Principal Financial and Accounting Officer)</i>	March 31, 2025
<i>/s/ Catherine Bollard</i> <b>Catherine Bollard, M.D.</b>	<i>Director</i>	March 31, 2025
<i>/s/ Scott Brun</i> <b>Scott Brun, M.D.</b>	<i>Director</i>	March 31, 2025
<i>/s/ Richard Henriques</i> <b>Richard Henriques</b>	<i>Director</i>	March 31, 2025
<i>/s/ Mark Simon</i> <b>Mark Simon</b>	<i>Director</i>	March 31, 2025
<i>/s/ Shawn Tomasello</i> <b>Shawn Tomasello</b>	<i>Director</i>	March 31, 2025



**CABALETTA BIO, INC.**  
**FOURTH AMENDED AND RESTATED NON-  
EMPLOYEE DIRECTOR COMPENSATION POLICY**

The purpose of this Third Amended and Restated Non-Employee Director Compensation Policy of Cabaletta Bio, Inc. (the “Company”), is to provide a total compensation package that enables the Company to attract and retain, on a long-term basis, high-caliber directors who are not employees or officers of the Company or its subsidiaries. In furtherance of the purpose stated above, all non-employee directors shall be paid compensation for services provided to the Company as set forth below:

**Cash Retainers**

Annual Retainer for Board Membership: \$40,000 for general availability and participation in meetings and conference calls of our Board of Directors, to be paid quarterly in arrears, prorated based on the number of actual days served by the director during such calendar quarter.

Additional Retainers for Committee Membership:

Audit Committee Chair: \$20,000

Audit Committee member: \$10,000

Compensation Committee Chair: \$12,500

Compensation Committee member: \$6,250

Nominating and Corporate Governance Committee Chair: \$10,000

Nominating and Corporate Governance Committee member: \$5,000

Science and Technology Committee Chair: \$15,000

Science and Technology Committee member: \$7,500

Note: Chair and committee member retainers are in addition to retainers for members of the Board of Directors.

**Equity Retainers**

Initial Award: An initial, one-time stock option award (the “Initial Award”) of 44,000 shares will be granted to each new non-employee director upon his or her election to the Board of Directors, which shall vest in equal quarterly installments over three years from the date of vesting commencement, provided, however, that all vesting shall cease if the director resigns from the Board of Directors or otherwise ceases to serve as a director of the Company. The Initial Award shall expire ten years from the date of grant, and shall have a per share exercise

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price equal to the Fair Market Value (as defined in the Company's 2019 Stock Option and Incentive Plan) of the Company's common stock on the date of grant. This Initial Award applies only to non-employee directors who are first elected to the Board of Directors subsequent to the Company's initial public offering.

Annual Award: Commencing in 2021, on each date of the Company's Annual Meeting of Stockholders following the completion of the Company's initial public offering (the "Annual Meeting"), each continuing non-employee member of the Board of Directors, other than a director receiving an Initial Award, will receive an annual stock option award (the "Annual Award") of 22,000 shares, which shall vest in full upon the earlier to occur of the first anniversary of the date of grant or the date of the next Annual Meeting; provided, however, that all vesting shall cease if the director resigns from the Board of Directors or otherwise ceases to serve as a director, unless the Board of Directors determines that the circumstances warrant continuation of vesting. Such Annual Award shall expire ten years from the date of grant, and shall have a per share exercise price equal to the Fair Market Value (as defined in the Company's 2019 Stock Option and Incentive Plan) of the Company's common stock on the date of grant.

Each Initial Award and Annual Award will become immediately vested and exercisable upon a Sale Event (as defined in the Company's 2019 Stock Option and Incentive Plan).

#### **Expenses**

The Company will reimburse all reasonable out-of-pocket expenses incurred by non-employee directors in attending meetings of the Board or any Committee.

Effective January 1, 2025

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**CABALETTA BIO, INC.**  
**STATEMENT OF COMPANY POLICY ON**  
**INSIDER TRADING AND DISCLOSURE**

This memorandum sets forth the policy of Cabaletta Bio, Inc. and its subsidiaries (collectively, the “Company”) regarding trading in the Company’s securities as described below and the disclosure of information concerning the Company. This Insider Trading Policy (the “Insider Trading Policy”) is designed to prevent insider trading or the appearance of impropriety, to satisfy the Company’s obligation to reasonably supervise the activities of Company personnel, and to help Company personnel avoid the severe consequences associated with violations of insider trading laws. **It is your obligation to understand and comply with this Insider Trading Policy.** If you have a question regarding the Insider Trading Policy, you are encouraged to consult with the Company’s General Counsel (the “General Counsel”).

**PART I. OVERVIEW**

***A. To Whom does this Insider Trading Policy Apply?***

This Insider Trading Policy is applicable to the Company’s directors, officers, employees, and designated consultants (collectively, and solely for the purposes of this Insider Trading Policy, these persons are referred to as “Insiders”), and applies to any and all transactions (e.g. purchase, sale, exercise or other transfer) by such persons and their Affiliated Persons (as defined below) in the Company’s securities, including its common stock, options to purchase common stock, any other type of securities that the Company may issue (such as preferred stock, convertible debentures, warrants, exchange-traded options or other derivative securities), and any derivative securities that provide the economic equivalent of ownership of any of the Company’s securities or an opportunity, direct or indirect, to profit from any change in the value of the Company’s securities.

Part II of this Insider Trading Policy (the “Trading Procedures”) requires pre-clearance of all transactions in the Company’s securities by Insiders and their Affiliated Persons and describes trading windows outside of which the persons covered by the Trading Procedures will be restricted from trading in the Company’s securities.

“Affiliated Persons” means:

- your spouse, child, parent, significant other or other family member, in each case, living in the same household;
  - all trusts, family partnerships and other types of entities formed for your benefit of the Insider or for the benefit of a member of your family over which you have the ability to influence or direct investment decisions concerning securities;
  - all persons who execute trades on your behalf; and
  - all investment funds, trusts, retirement plans, partnerships, corporations and other types of entities over which you have the ability to influence or direct investment decisions concerning securities.
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You are responsible for ensuring compliance with this Insider Trading Policy, including the Trading Procedures contained herein, by all of your Affiliated Persons.

In the event that you leave the Company for any reason, this Insider Trading Policy, including, if applicable, the Trading Procedures contained herein, will continue to apply to you and your Affiliated Persons until the later of: (1) the first trading day following the public release of earnings for the fiscal quarter in which you leave our Company or (2) the second trading day after any material nonpublic information known to you has become public or is no longer material.

### ***B. What is Prohibited by this Insider Trading Policy?***

It is generally illegal for you to trade in the securities of the Company, whether for your account or for the account of another, while in the possession of material, nonpublic information about the Company. It is also generally illegal for you to disclose material, nonpublic information about the Company to others who may trade on the basis of that information. These illegal activities are commonly referred to as “*insider trading*.”

#### **Prohibited Activities**

When you know or are in possession of material, nonpublic information about the Company, whether positive or negative, you are prohibited from the following activities:

- trading (whether for your account or for the account of another) in the Company’s securities, which includes common stock, options to purchase common stock, any other type of securities that the Company may issue (such as preferred stock, convertible debentures, warrants, exchange-traded options or other derivative securities), and any derivative securities that provide the economic equivalent of ownership of any of the Company’s securities or an opportunity, direct or indirect, to profit from any change in the value of the Company’s securities, except for trades made in compliance with the affirmative defense of Rule 10b5-1 under the Exchange Act, such as when trades are made pursuant to a written plan that was adopted, or trading instructions that were given, before you knew or had possession of such material, nonpublic information and certain other conditions are satisfied;
- giving trading advice of any kind about the Company; and
- disclosing such material, nonpublic information about the Company, whether positive or negative, to anyone else (commonly known as “*tipping*”).

**This Insider Trading Policy does not apply to (1) an exercise of an employee stock option when payment of the exercise price is made in cash or (2) the withholding by the Company of shares of stock upon vesting of restricted stock or upon settlement of restricted stock units to satisfy applicable tax withholding requirements if (a) such withholding is required by the applicable plan or award agreement or (b) the election to exercise such tax withholding right was made by the Insider in compliance with the Trading Procedures. The policy does apply, however, to the use of outstanding Company securities to pay part or all of the exercise price of an option, any sale of stock as part of**

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**a broker-assisted cashless exercise of an option, or any other market sale for the purpose of generating the cash needed to pay the exercise price of an option.**

These prohibitions continue whenever and for as long as you know or are in possession of material, nonpublic information. Remember, anyone scrutinizing your transactions will be doing so after the fact, with the benefit of hindsight. As a practical matter, before engaging in any transaction, you should carefully consider how enforcement authorities and others might view the transaction in hindsight.

**Definition of Material, Nonpublic Information**

This Insider Trading Policy prohibits you from trading in the Company's securities if you are in possession of information about the Company that is both "*material*" and "*nonpublic*." If you have a question whether certain information you are aware of is material or has been made public, you are encouraged to consult with the General Counsel.

***What is "Material" Information?***

Information about the Company is "material" if it could reasonably be expected to affect the investment or voting decisions of a stockholder or investor, or if the disclosure of the information could reasonably be expected to significantly alter the total mix of information in the marketplace about the Company. In simple terms, material information is any type of information that could reasonably be expected to affect the market price of the Company's securities. Both positive and negative information may be material. While it is not possible to identify all information that would be deemed "material," the following items are types of information that should be considered carefully to determine whether they are material:

- developments regarding any programs in clinical development or subject to regulatory approval, including recent regulatory interaction and/or data that have been recently generated from ongoing or recently completed clinical trials;
  - developments regarding the intellectual property and/or freedom to operate for any of the current programs or product candidates under development;
  - developments regarding a change in the Company's publicly announced cash runway;
  - potential restatements of the Company's financial statements, changes in auditors or auditor notification that the Company may no longer rely on an auditor's audit report;
  - pending or proposed corporate mergers, acquisitions, tender offers, joint ventures or dispositions of significant assets;
  - changes in management or the Board of Directors;
  - significant actual or threatened litigation or governmental investigations or major developments in such matters;
  - a cybersecurity incident;
  - significant developments regarding products, customers, suppliers, orders, contracts or financing sources (e.g., the acquisition or loss of a contract);
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- changes in dividend policy, declarations of stock splits, or public or private sales of additional securities;
- potential defaults under the Company's credit agreements or indentures, or the existence of material liquidity deficiencies; and
- bankruptcies or receiverships.

By including the list above, the Company does not mean to imply that each of these items above is per se material. The information and events on this list still require determinations as to their materiality (although some determinations will be reached more easily than others). For example, some new products or contracts may clearly be material to an issuer; yet that does not mean that all product developments or contracts will be material. This demonstrates, in our view, why no "bright-line" standard or list of items can adequately address the range of situations that may arise. Furthermore, the Company cannot create an exclusive list of events and information that have a higher probability of being considered material. The Securities and Exchange Commission (the "SEC") has stated that there is no fixed quantitative threshold amount for determining materiality, and that even very small quantitative changes can be qualitatively material if they would result in a movement in the price of the Company's securities.

#### ***What is "Nonpublic" Information?***

Material information is "nonpublic" if it has not been disseminated in a manner making it available to investors generally. To show that information is public, it is necessary to point to some fact that establishes that the information has become publicly available, such as the filing of a report with the SEC, the distribution of a press release through a widely disseminated news or wire service, or by other means that are reasonably designed to provide broad public access. Before a person who possesses material, nonpublic information can trade, there also must be adequate time for the market as a whole to absorb the information that has been disclosed. For the purposes of this Insider Trading Policy, information will be considered public after the close of trading on the second full trading day following the Company's public release of the information. As a general rule, you should consider information to be "non-public" until at least one full trading day has elapsed after the information has been broadly disseminated to the public in a press release, a public filing with the SEC, a pre-announced public webcast or another broad, non-exclusionary form of public communication. The term "trading day" means a day on which national stock exchanges are open for trading. A "full" trading day has elapsed when, after the public disclosure, trading in the relevant security has opened and then closed.

For example, if the Company announces material nonpublic information of which you are aware before trading begins on a Tuesday, the first time you can buy or sell Company securities is the opening of the market on Wednesday. However, if the Company announces this material information after trading begins on that Tuesday, the first time that you can buy or sell Company securities is the opening of the market on Thursday.

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#### ***C. What are the Penalties for Insider Trading and Noncompliance with this Insider Trading Policy?***

Both the SEC and the national securities exchanges, through the Financial Industry

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Regulatory Authority (“FINRA”), investigate and are very effective at detecting insider trading. The SEC, together with the U.S. Attorneys, pursue insider trading violations vigorously. For instance, cases have been successfully prosecuted against trading by employees in foreign accounts, trading by family members and friends, and trading involving only a small number of shares.

The penalties for violating insider trading or tipping rules can be severe and include:

- disgorgement of the profit gained or loss avoided by the trading;
- payment of the loss suffered by the persons who, contemporaneously with the purchase or sale of securities that are subject of such violation, have purchased or sold, as applicable, securities of the same class;
- payment of criminal penalties of up to \$5,000,000;
- payment of civil penalties of up to three times the profit made or loss avoided; and
- imprisonment for up to 20 years.

The Company and/or the supervisors of the person engaged in insider trading may also be required to pay civil penalties of up to the greater of \$1,525,000 or three times the profit made or loss avoided, as well as criminal penalties of up to \$25,000,000, and could under certain circumstances be subject to private lawsuits.

Violation of this Insider Trading Policy or any federal or state insider trading laws may subject the person violating such policy or laws to disciplinary action by the Company up to and including termination. The Company reserves the right to determine, in its own discretion and on the basis of the information available to it, whether this Insider Trading Policy has been violated. The Company may determine that specific conduct violates this Insider Trading Policy, whether or not the conduct also violates the law. It is not necessary for the Company to await the filing or conclusion of a civil or criminal action against the alleged violator before taking disciplinary action.

***D. How Do You Report a Violation of this Insider Trading Policy?***

If you have a question about this Insider Trading Policy, including whether certain information you are aware of is material or has been made public, you are encouraged to consult with the General Counsel. In addition, if you violate this Insider Trading Policy or any federal or state laws governing insider trading, or know of any such violation by any director, officer or employee of the Company, you must report the violation immediately to the General Counsel.

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## PART II. TRADING PROCEDURES

### *A. Special Trading Restrictions Applicable to Insiders*

In addition to the restrictions on trading in Company securities set forth above, Insiders and their Affiliated Persons are subject to the following special trading restrictions:

#### **1. Special Closed Trading Periods**

There are times when the Company or certain members of its board of directors or senior management or support staff may be aware of a material, nonpublic development. Although an Insider may not know the specifics of such development, if an Insider engages in a trade before such development is disclosed to the public or resolved, such Insider and the Company might be exposed to a charge of insider trading that could be costly and difficult to refute. In addition, a trade by an Insider during such a period could result in adverse publicity for the Company. Therefore, Insiders may not trade in Company securities if they are notified by the General Counsel that the trading window is closed because of the existence of a material, nonpublic development or closed with respect to the securities of other companies, including specified peers or competitors of the Company. The General Counsel will subsequently notify the Insiders once the material, nonpublic development is disclosed to the public or resolved and that, as a result, the trading window is again open. While the General Counsel will undertake reasonable efforts to notify the Insiders that material, nonpublic events have developed, or are soon likely to develop, it is each Insider's individual duty to ensure that they do not make any trade in Company securities when material, nonpublic information exists, regardless of whether such Insider is aware of such development. The imposition of such a closed trading window announced to the Company generally should not be communicated to any other person, and may itself be considered under this Insider Trading Policy to be material nonpublic information

#### **2. Prohibited Transactions**

• **No Short Sales.** No Insider may at any time sell any securities of the Company that are not owned by such Insider at the time of the sale (a "short sale").

• **No Purchases or Sales of Derivative Securities or Hedging Transactions.** No Insider may buy or sell puts, calls, other derivative securities of the Company or any derivative securities that provide the economic equivalent of ownership of any of the Company's securities or an opportunity, direct or indirect, to profit from any change in the value of the Company's securities or engage in any other hedging transaction with respect to the Company's securities, at any time.

• **No Company Securities Subject to Margin Calls.** No Insider may use the Company's securities as collateral in a margin account.

• **No Pledges.** No Insider may pledge Company securities as collateral for a loan (or modify an existing pledge).

#### **3. Distributions, Gifts and Other Transfers for No Consideration are Gifts Subject to Same Restrictions as All Other Securities Trades.**

No Insider may give or make any other transfer of Company securities without consideration (e.g., a partnership distribution gift) during a period when the Insider is

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not permitted to trade.

### ***B.Pre-Clearance Procedures***

No Insider may trade (e.g. purchase, sale, exercise or other transfer) in Company securities unless the trade has been approved by the General Counsel in accordance with the procedures set forth below. The General Counsel will review and either approve or prohibit all proposed trades by Insiders in accordance with the procedures set forth below. The General Counsel may consult with the Company's other officers and/or outside legal counsel and will receive approval for their own trades from the Chief Financial Officer.

***Procedures.*** No Insider may trade in Company securities until:

- The Insider has provided the General Counsel with either a pre-clearance request in the Morgan Stanley Shareworks system ("Shareworks") for trades to be conducted through Shareworks and/or a Stock Transaction Request Form (form attached hereto) for trades to be conducted outside of Shareworks; which shall each provide the following information:

- o the amount and nature of the proposed trade(s); and
- o Certifying to the General Counsel in writing prior to the proposed trade(s) that (i) the Insider is not in possession of material, nonpublic information concerning the Company; (ii) to the Insider's best knowledge, (a) the Insider has (or is deemed to have) engaged in any opposite way transactions within the previous six months that were not exempt from Section 16(b) of the Exchange Act and (b) if the transaction involves a sale by an "affiliate" of the Company or of "restricted securities" (as such terms are defined under Rule 144 under the Securities Act of 1933, as amended ("Rule 144")), whether the transaction meets all of the applicable conditions of Rule 144.

- The General Counsel or their designee has approved the trade(s) in writing on the Stock Transaction Request Form or in Shareworks.

If practicable, an Insider should provide the General Counsel with the Stock Transaction Request Form and/or a pre-clearance request in Shareworks at least (2) business days prior to the intended trade date. The General Counsel does not assume the responsibility for, and approval from the General Counsel does not protect the Insider from, the consequences of prohibited insider trading.

***Additional Information.*** Insiders shall provide to the General Counsel any documentation reasonably requested by them in furtherance of the foregoing procedures. Any failure to provide such requested information will be grounds for denial of approval by the General Counsel.

***No Obligation to Approve Trades.*** The existence of the foregoing approval procedures does not in any way obligate the General Counsel to approve any trade requested by an Insider. The General Counsel may reject any trading request at their sole discretion. From time to time,

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an event may occur that is material to the Company and is known by only a few directors or executives. Insiders may not trade in Company securities if they are notified by the General Counsel that a proposed trade has not been cleared because of the existence of a material, nonpublic development in accordance with a restricted period or otherwise. Even if that particular Insider is not aware of the material, nonpublic development involving the Company, if any Insider engages in a trade before a material, nonpublic development is disclosed to the public or resolved, the Insider and the Company might be exposed to a charge of insider trading that could be costly and difficult to refute even if the Insider was unaware of the development. So long as the event remains material and nonpublic, the General Counsel may determine not to approve any transactions in the Company's securities. The General Counsel will subsequently notify the Insider once the material, nonpublic development is disclosed to the public or resolved. If an Insider requests clearance to trade in the Company's securities during the pendency of such an event, the General Counsel may reject the trading request without disclosing the reason.

***Completion of Trades.*** After receiving written clearance to engage in a trade signed by the General Counsel, an Insider must complete the proposed trade within two (2) business days or make a new trading request.

***Post-Trade Reporting.*** Any transactions in the Company's securities (including transactions under a Rule 10b5-1 Plan) by an Insider who is an executive officer or director required to make Section 16 filings must be reported to the General Counsel by completing the "Confirmation of Transaction" section of the Stock Transaction Request form attached to this Insider Trading Policy on the same day in which such a transaction occurs. Each report an Insider makes to the General Counsel should include the date of the transaction, quantity of shares, price and broker-dealer through which the transaction was effected. This reporting requirement may be satisfied by sending (or having such Insider's broker send) duplicate confirmations of trades to the General Counsel if such information

is received by the General Counsel on or before the required date. Compliance by directors and executive officers with this provision is imperative given the requirement of Section 16 of the Exchange Act that these persons generally must report changes in ownership of Company securities within two (2) business days. The sanctions for noncompliance with this reporting deadline include mandatory disclosure in the Company's proxy statement for the next annual meeting of stockholders, as well as possible civil or criminal sanctions for chronic or egregious violators.

### ***C.Exemptions***

**1.Pre-Approved Rule 10b5-1 Plans.** Transactions effected pursuant to a Rule 10b5-1 Plan (as defined below) will not be subject to the Company's restricted period or pre-clearance procedures, and Insiders are not required to complete a Stock Transaction Request form for such transactions. Rule 10b5-1 of the Exchange Act provides an affirmative defense from insider trading liability under the federal securities laws for trading plans, arrangements or instructions that meet certain requirements. A trading plan, arrangement or instruction that meets the requirements of Rule 10b5-1 (a "Rule 10b5-1 Plan") enables Insiders to establish arrangements to trade in Company securities outside of the Company's trading windows, even when in possession of material, nonpublic information. The Company has adopted a separate Rule 10b5-1 Plan with respect to Company securities. If an Insider intends to trade pursuant to a

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Rule 10b5-1 Plan, such plan, arrangement or instruction must:

- satisfy the requirements of Rule 10b5-1;
- be documented in writing;
- be established during a trading window when such Insider does not possess material, nonpublic information; and
- be pre-approved by the General Counsel.

Any deviation from, or alteration to, the specifications of an approved Rule 10b5-1 Plan (including, without limitation, the amount, price or timing of a purchase or sale) must be reported immediately to the General Counsel. Any transaction pursuant to a Rule 10b5-1 Plan must be timely reported following the transaction in accordance with the procedures set forth above.

The General Counsel may refuse to approve a Rule 10b5-1 Plan as they deem appropriate including, without limitation, if they determine that such plan does not satisfy the requirements of Rule 10b5-1.

Any modification or termination of an Insider's prior Rule 10b5-1 Plan requires pre-approval by the General Counsel. A modification or termination must occur during a trading window and while such Insider is not aware of material, nonpublic information.

**2.Exercise of Stock Options.** The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to the exercise of an option to purchase securities of the Company when payment of the exercise price is made in cash. However, the exercise of an option to purchase securities of the Company is subject to the current reporting requirements of Section 16 of the Exchange Act and, therefore, Insiders must comply with the post-trade reporting requirement described in Section B above for any such transaction. In addition, the securities acquired upon the exercise of an option to purchase Company securities are subject to all of the requirements of this Insider Trading Policy, including the Trading Procedures contained herein. Moreover, the Trading Procedures apply to the use of outstanding Company securities to pay part or all of the exercise price of an option, any net option exercise, any exercise of a stock appreciation right, share withholding, any sale of stock as part of a broker-assisted cashless exercise of an option, or any other market sale for the purpose of generating the cash needed to pay the exercise price of an option.

**3.Tax Withholding on Restricted Stock/Units.** The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to the withholding by the Company of shares of stock upon vesting of restricted stock or upon settlement of restricted stock units to satisfy applicable tax withholding requirements if (a) such withholding is required by the applicable plan or award agreement or (b) the election to exercise such tax withholding right was made by the Insider in compliance with the Trading Procedures.

**4.Employee Stock Purchase Plan.** The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to periodic wage withholding contributions by the Company or employees of the Company which are used to purchase the Company's securities pursuant to the employees' advance instructions under the Company's 2019 Employee Stock Purchase Plan. However, no Insider may: (a) elect to participate in the plan or alter their instructions regarding the level of withholding or purchase by the Insider of Company securities under such plan; or (b) make cash contributions to such plan (other than through periodic wage

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withholding) without complying with the Trading Procedures including Part II.B (Pre-Clearance Procedures). Any sale of securities acquired under such plan is subject to the prohibitions and restrictions of the Trading Procedures including Part II.B (Pre-Clearance Procedures).

***D. WAIVERS***

A waiver of any provision of this Insider Trading Policy, or the Trading Procedures contained herein, in a specific instance may be authorized in writing by the General Counsel, their designee or the Audit Committee of the Board of Directors, and any such waiver shall be reported to the Company's Board of Directors.

***E. ACKNOWLEDGMENT***

This Insider Trading Policy will be delivered to all current Insiders and to all directors, officers, employees and designated consultants at the start of their employment or relationship with the Company. Upon first receiving a copy of this Insider Trading Policy, each individual must acknowledge that they have received a copy and agrees to comply with the terms of this Insider Trading Policy, and, if applicable, the Trading Procedures contained herein. The acknowledgment attached hereto must be returned within ten (10) days of receipt to the Company's General Counsel.

This acknowledgment will constitute consent for the Company to impose sanctions for violation of the Insider Trading Policy, including the Trading Procedures, and to issue any necessary stop-transfer orders to the Company's transfer agent to ensure compliance.

All directors, officers, employees and designated consultants will be required upon the Company's request to re-acknowledge and agree to comply with the Insider Trading Policy (including any amendments or modifications). For such purpose, an individual will be deemed to have acknowledged and agreed to comply with the Insider Trading Policy, as amended from time to time, when copies of such items have been delivered by regular or electronic mail (or other delivery option used by the Company) by the General Counsel or their designee.

Questions regarding this Insider Trading Policy are encouraged and may be directed to the General Counsel.

Amended & restated effective February 13, 2024.

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## STOCK TRANSACTION REQUEST

Pursuant to Cabaletta Bio, Inc.'s Special Trading Procedures for Insiders (the "Trading Procedures"), I hereby notify Cabaletta Bio, Inc. (the "Company") of my intent to trade the securities of the Company as indicated below:

### REQUESTER INFORMATION

Insider's Name:

### INTENT TO PURCHASE

Number of shares: Intended trade date:

Means of acquiring shares:

Acquisition through employee benefit plan (please specify):

Purchase through a broker on the open market

Other (please specify):

Date

Insider's Signature

### CERTIFICATION

I hereby certify that (1) I am not in possession of any material, nonpublic information concerning the Company, as defined in the Company's Statement of Company Policy on Insider Trading and Disclosure, (2) to the best of my knowledge, the proposed trade(s) listed above does not violate the trading restrictions of Section 16 of the Securities Exchange Act of 1934, as amended, or Rule 144 under the Securities Act of 1933, as amended, and (3)

I am not purchasing any securities of the Company on margin in contravention of the Company's Trading Procedures. I understand that, if I trade while possessing such information or in violation of such trading restrictions, I may be subject to severe civil and/or criminal penalties, and may be subject to discipline by the Company including termination.

Other (please specify):

Sale through a broker on the open market

Sale through employee benefit plan (please specify):

### INTENT TO SELL

Number of shares:

Intended trade date: Means of selling shares:

### AUTHORIZED APPROVAL

Signature of General Counsel (or designee)

Date

### CONFIRMATION OF TRANSACTION

I hereby confirm that the transaction(s) requested above was (were) executed as follows:

Purchase of shares:

\*Number of shares:

Price per share: Date and approximate time of purchase:

Sale of shares:

\*Number of shares:

Price per share:

Date and approximate time of sale:

Insider's Signature

Insider's Name:

Date

*\*NOTE: Multiple lots must be listed on separate forms or broken out herein.*

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**Cabaletta Bio, Inc.**

The following is a list of subsidiaries of Cabaletta Bio, Inc. as of December 31, 2024.

<b>SUBSIDIARY</b>	<b>STATE OR OTHER JURISDICTION OF INCORPORATION OR ORGANIZATION</b>
Cabaletta Bio, Inc.	Delaware
Cabaletta Bio GmbH	Switzerland
Cabaletta Bio (Germany) GmbH	Germany

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Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

- (1) Registration Statement (Form S-8 No. 333-234367) pertaining to the Cabaletta Bio, Inc. 2018 Stock Option and Incentive Plan, the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan, and the Cabaletta Bio, Inc. 2019 Employee Stock Purchase Plan,
- (2) Registration Statement (Form S-8 No. 333-237484) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan and the Cabaletta Bio, Inc. 2019 Employee Stock Purchase Plan,
- (3) Registration Statement (Form S-8 No. 333-254342) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan and the Cabaletta Bio, Inc. 2019 Employee Stock Purchase Plan,
- (4) Registration Statement (Form S-8 No. 333-263637) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan,
- (5) Registration Statement (Form S-8 No. 333-270595) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan,
- (6) Registration Statement (Form S-8 No. 333-273863) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan;
- (7) Registration Statement (Form S-3 No. 333-278126) of Cabaletta Bio, Inc., and
- (8) Registration Statement (Form S-8 No. 333-278124) pertaining to the Cabaletta Bio, Inc. 2019 Stock Option and Incentive Plan;

of our report dated March 31, 2025, with respect to the consolidated financial statements of Cabaletta Bio, Inc. included in this Annual Report (Form 10-K) of Cabaletta Bio, Inc. for the year ended December 31, 2024.

/s/ Ernst & Young LLP

Philadelphia, Pennsylvania  
March 31, 2025

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**CERTIFICATION PURSUANT TO  
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,  
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Steven Nichtberger, certify that:

1. I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2024 of Cabaletta Bio, Inc. (the "registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

- (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
- (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
- (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
- (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

- (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
- (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2025

By  
:

/s/ Steven Nichtberger  
**Steven Nichtberger, M.D.**  
**Chief Executive Officer and President**  
**(Principal Executive Officer)**





**CERTIFICATION PURSUANT TO  
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,  
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Anup Marda, certify that:

1. I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2024 of Cabaletta Bio, Inc. (the "registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

- (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
- (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
- (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
- (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

- (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
- (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2025

By  
:

/s/ Anup Marda  
**Anup Marda**  
**Chief Financial Officer**  
**(Principal Financial and Accounting Officer)**



**CERTIFICATION PURSUANT TO  
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report of Cabaletta Bio, Inc. (the "Company") on Form 10-K for the fiscal year ended December 31, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 31, 2025

By  
:

/s/ Steven Nichtberger  
**Steven Nichtberger, M.D.**  
**Chief Executive Officer**  
**(Principal Executive Officer)**

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**CERTIFICATION PURSUANT TO  
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report of Cabaletta Bio, Inc. (the "Company") on Form 10-K for the fiscal year ended December 31, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 31, 2025

By  
:

/s/ Anup Marda  
**Anup Marda**  
**Chief Financial Officer**  
**(Principal Accounting and Financial Officer)**

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