2025 Annual Report

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended December 31, 2025

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 001-38210

Krystal Biotech, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**82-1080209**
State or other jurisdiction of incorporation or organization	**(I.R.S. Employer Identification No.)**
2100 Wharton Street, Suite 701 Pittsburgh, Pennsylvania	**15203**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code (412) 586-5830

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	KRYS	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 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	☐	Emerging growth company	☐
Non-accelerated filer	☐	Smaller reporting 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 ☒

The aggregate market value of common stock held by non-affiliates of the registrant, based on the closing sales price for such stock on June 30, 2025 as reported by The Nasdaq Stock Market, was $3.5 billion.

The number of shares of registrant's common stock outstanding as of February 11, 2026 was 29,232,189.

Portions of the registrant's definitive proxy statement relating to its 2026 Annual Meeting of Stockholders are incorporated by reference into Part III of this report where indicated. Such proxy statement will be filed with the U.S. Securities and Exchange Commission within 120 days after the end of the fiscal year to which this report relates.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or similar expressions and the negatives of those terms. These statements relate to future events or to our future operating 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, performances or achievements expressed or implied by the forward-looking statements.

Forward-looking statements appearing in a number of places throughout this Annual Report on Form 10-K include, but are not limited to, statements about the following, among other things:

- our commercialization plans in the United States, the European Union ("EU"), Japan, and elsewhere for our first commercial product, VYJUVEK® (beremagene geperpavec-svdt), which was approved by the United States Food and Drug Administration (the "FDA") in May 2023, the European Commission ("EC") in April 2025, and Japan's Ministry of Health, Labour and Welfare ("MHLW") in July 2025 for the treatment of dystrophic epidermolysis bullosa ("DEB");
- the initiation, timing, progress, and results of clinical trials for our product candidates, as well as expected timing of reporting of data readouts from our clinical trials;
- the timing, scope or results of regulatory filings and approvals of our product candidates;
- our estimates regarding the potential market opportunity for any of our product candidates;
- our research and development programs for our product candidates;
- our plans and ability to successfully identify, develop and commercialize our product candidates;
- our beliefs about our proprietary HSV-1 based vector platform;
- our commercialization, marketing and manufacturing capabilities and strategy;
- the scalability and commercial viability of our proprietary manufacturing methods and processes;
- our competitive position;
- our intellectual property position and our ability to protect and enforce our intellectual property;
- the impact of laws and regulations and potential changes thereto; and
- any statements regarding U.S. or global economic conditions and the impact on our business, or performance and any statement of assumptions underlying any of the foregoing.

Forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and elsewhere in this Annual Report on Form 10-K. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Annual Report on Form 10-K may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements. You should read this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect.

Forward-looking statements represent our management's beliefs and assumptions only as of the date of this Annual Report on Form 10-K. Except as required by law, we assume no obligation to update or revise these forward-looking statements publicly as a result of subsequent events, developments or otherwise, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

This Annual Report on Form 10-K also contains estimates and other statistical data made by independent parties and by the Company that involves a number of assumptions and limitations. Neither the Company nor any other person makes any representation as to the accuracy or completeness of such data or undertakes any obligation to update such data after the date of this Annual Report.

Other than VYJUVEK, all products described in this Annual Report on Form 10-K are investigational therapies. This Annual Report on Form 10-K contains references to details of our clinical trials that can be found at www.clinicaltrials.gov. Nothing included on www.clinicaltrials.gov shall be deemed incorporated by reference into this Annual Report on Form 10-K. The Company is using the Aerogen Solo® Nebulizer System and Aerogen® Ultra in its clinical trials evaluating KB407, KB408, and inhaled KB707.

Throughout this Annual Report on Form 10-K, unless the context requires otherwise, all references to "Krystal," "the Company," "we," "our," "us" or similar terms refer to Krystal Biotech, Inc., together with its consolidated subsidiaries.

Summary Risk Factors

Our business is subject to a number of risks, including risks that may prevent us from achieving our business objectives or may adversely affect our business, financial condition, results of operations, cash flows and prospects. These summary risks provide an overview of many of the risks we are exposed to in the normal course of our business and are discussed more fully in "Risk Factors" herein. These risks include, but are not limited to, the following:

- We are substantially dependent on the commercial success of VYJUVEK.
- If we are not successful in discovering, developing, and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives would be impaired.
- We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize and market our product candidates, if approved.
- If any product liability lawsuits are successfully brought against us, we may incur substantial liabilities and may be required to limit commercialization of VYJUVEK or our product candidates.
- Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.
- We are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.
- Our internal computer systems, or those of any third-party with whom we do business may fail or suffer a cyber-security incident, such as a data breach or computer virus, which could harm our business by damaging our reputation, exposing us to liability, adversely impacting our revenue, or materially disrupting our operations, including production of VYJUVEK or our product development programs.
- Our international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement and economic risks associated with doing business outside of the United States.
- If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.
- If we are unable to advance our product candidates through clinical trials, obtain regulatory approval and ultimately commercialize our product candidates, or if we experience significant delays in doing so, our business will be materially harmed.
- VYJUVEK or our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences before or following any potential marketing approval.
- We rely on third parties to conduct certain of our preclinical studies or aspects of our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for, or commercialize, our product candidates.
- We may encounter substantial delays in our clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- VYJUVEK and our product candidates that receive marketing approvals remain subject to regulatory oversight even after regulatory approval. We will continue to incur costs related to regulatory compliance and are subject to risks related to non-compliance with or changes to applicable laws and regulations, which could cause VYJUVEK or any of our product candidates that obtain regulatory approval to lose that approval.

- Delays in obtaining regulatory approvals of the process, or changes to the process, and facilities needed to manufacture VYJUVEK or our product candidates or disruptions in our manufacturing process may disrupt our production of VYJUVEK or delay or disrupt our development and commercialization efforts with respect to our product candidates.
- Although we have established our own manufacturing facilities for VYJUVEK and our product candidates, we may also utilize third parties to conduct our product manufacturing or components thereof. We are also dependent on a limited number of third-party suppliers for some of the components and materials used in manufacturing VYJUVEK and our product candidates and in commercially supplying VYJUVEK. Therefore, we are subject to the risk that these third parties may not perform satisfactorily.
- Any contamination in, or changes to, our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our ability to produce VYJUVEK or any other approved product for commercial supply or any product candidate for clinical development.
- We have limited experience as a commercial company and the sales, marketing, and distribution of VYJUVEK or any future approved products may be unsuccessful or less successful than anticipated.
- If we are unable to maintain our agreements with third parties to distribute VYJUVEK, our results of operations and business could be adversely affected.
- If we are unable to expand our medical affairs, marketing, market access, sales, and distribution capabilities or collaborate with third parties to market and sell our product candidates for which we obtain marketing approval, we may be unable to generate sufficient product revenue.
- If the market opportunities for VYJUVEK or our product candidates are smaller than we believe they are, our product revenue may be adversely impacted, and our business may suffer.
- Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for VYJUVEK and our product candidates, if approved, which would adversely affect our revenue and results of operations.
- The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for VYJUVEK or our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.
- If we are unable to obtain and maintain adequate United States and foreign patent protection for VYJUVEK, our current product candidates, and any future product candidates we may develop, and/or our vector platform, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technologies similar or identical to ours, and our ability to successfully commercialize VYJUVEK, our current product candidates, any future product candidates we may develop, and our platform technologies may be adversely affected.
- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

PART I

Item 1. Business.

Overview

We are a fully integrated, global, commercial-stage biotechnology company focused on the discovery, development, manufacturing and commercialization of genetic medicines to treat diseases with high unmet medical needs. Using our patented gene therapy technology platform that is based on engineered herpes simplex virus-1 ("HSV-1"), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell's own machinery then transcribes and translates the transgene to treat the disease. Our vectors are amenable to formulation for non-invasive or minimally invasive routes of administration at a healthcare professional's office or in the patient's home by a healthcare professional, caregiver, or directly by the patient themselves. Our innovative technology platform is supported by two in-house, commercial scale Current Good Manufacturing Practice ("CGMP") manufacturing facilities.

Our first commercial product, VYJUVEK®, is now approved in the United States, the European Union ("EU"), and Japan for the treatment of dystrophic epidermolysis bullosa ("DEB"). We launched VYJUVEK in the United States in 2023 and started launching VYJUVEK in Europe and Japan in 2025.

Our development pipeline includes multiple clinical stage product candidates for the treatment of rare and serious diseases, and we are investing in research and development to advance and grow this pipeline. We possess exclusive rights to develop, manufacture, and commercialize VYJUVEK and our pipeline product candidates throughout the world.

While our focus is on the development of gene therapies to treat patients with rare diseases with high unmet medical needs, we are also evaluating the potential of our platform to address more common severe or life-threatening diseases, such as non-small cell lung cancer ("NSCLC"), as well as aesthetic conditions via our wholly-owned subsidiary Jeune Aesthetics, Inc. ("Jeune Aesthetics"), which we incorporated in April 2019.

Our Redosable Gene Therapy Platform

We believe that certain inherent features of the HSV-1 virus, combined with the modifications we have made to the viral backbone provides our proprietary gene therapy platform with specific advantages over other viral and non-viral vector platforms and represents an opportunity to generate a portfolio of highly differentiated and potentially first-in-class or best-in-class genetic medicines. Advantages of our gene therapy technology platform include the following:

- **Repeat Administration**: One of the major challenges with many viral vector platforms is that the host immune system may recognize them as foreign agents and launch a robust immune response, resulting in toxicity and rapid removal of the virus. Wild-type HSV-1 is known to persist in the body by becoming latent and hiding from the immune system. We have harnessed the natural ability of HSV-1 to evade host-mediated immunogenicity, while removing specific viral elements that exacerbate host immunity, thus making our viral vector safer for repeat administration as needed to achieve durability of effect. The immune evasive properties of our vector also enable us to treat patients who may have baseline antibodies to HSV-1, ensuring that prior exposure to the wild-type virus will not limit the number of patients who may be amenable to treatment with VYJUVEK or our product candidates.
- **Non-Integrating Nature**: Upon entry into cells, the HSV-1 vector persists as an episomal unit in the nucleus, meaning it remains physically separate from the host cell chromosome. Certain other viral vectors currently being used in the development of gene therapy treatments, such as the lentiviral and retroviral vectors, integrate into the host cell DNA to achieve gene expression. Integration into the host cell DNA carries the risk of disrupting host genes. In contrast, a non-integrating vector such as our HSV-1-based vector does not carry the same risk of disrupting the expression of host cell genes.
- **Payload Capacity**: HSV-1 is a large virus, approximately 150 kilobases, or Kb, of DNA in size. We have made strategic deletions within this genome to remove critical "immediate early", or IE, genes. These IE genes are required for expression of most of the downstream genes that allow the HSV-1 virus to replicate and destroy host cells. Deletion of these IE genes inhibits expression of most of the viral proteins, making the resulting viral vector replication-deficient and non-toxic. These deletions also enable the vector to easily accommodate a payload of 35 Kb or greater without any significant impact on yield or titer. In VYJUVEK, we have successfully inserted two functional copies of the complete ~9 Kb human *COL7A1* gene. In contrast, packaging capacity for most other vectors being used is at or under ~10 Kb, which limits their ability to deliver large transgenes. In addition, we believe the high payload capacity of our viral vector will allow us to insert multiple and/or combinations of genes or effectors that could enable the treatment of non-monogenic conditions.
- **High Transduction Efficiency**: Poor transduction efficiency has remained a major hurdle for direct delivery of most vectors particularly in the epithelia of the skin and lung. HSV-1 has a natural affinity, or tropism, for

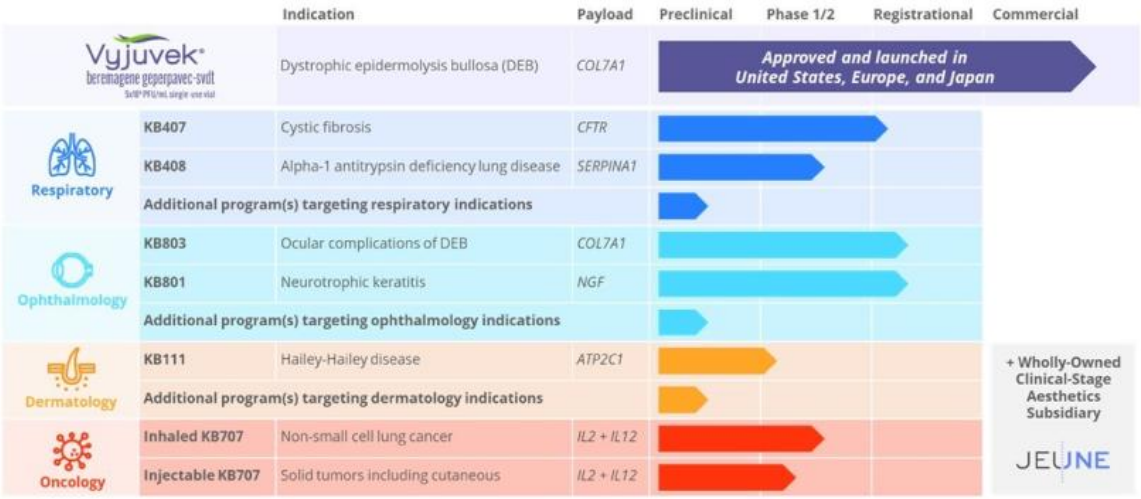
epithelial cells. Consequently, we believe our vector penetrates and delivers its payload much more efficiently than other vectors, resulting in transduction efficiencies or cell penetration as high as 95% in cell-based studies. The greater payload capacity of our vector and the high transduction efficiencies achieved allow us to deliver a full gene (or genes) directly to any patient's tissues for off-the-shelf, *in vivo* gene expression without additional manipulation.

- **Direct Delivery**: Our engineered HSV-1 vector allows for noninvasive or minimally invasive local gene delivery. The advantages of direct delivery are that our products can be administered in a doctor's office or the patient's home, requiring no hospitalization or expensive, invasive, and time-consuming procedures or sophisticated medical teams. Taking gene therapy to the patient minimizes patient travel and circumvents upfront logistical burdens typical of other gene therapy approaches.
- **Stability**: HSV-1 is extremely stable and resistant to degradation by physical shearing, solvents, and enzymes, facilitating purification and flexibility with final formulation of our product candidates. Our vectors are stable frozen for long-term storage, under refrigerated conditions for short-term storage and shipment, in addition to being stable over several freeze-thaw cycles. This facilitates our ability to ship VYJUVEK and our product candidates globally from our manufacturing facilities in Pennsylvania.
- **Reproducible and Scalable Manufacturing**: Successful production of viral vectors involves two steps: (i) the 'upstream' process, which yields a bulk virus harvest; and (ii) the 'downstream' process, which involves purification and concentration of the clinical product. Successful and reproducible execution of both processes is critical for commercial manufacturing. Our scientific team's collective decades of experience and expertise in HSV engineering and purification has allowed us to successfully optimize our engineered HSV-1 vector production process and develop in-house Chemistry, Manufacturing, and Controls ("CMC") capabilities.
- **Regulatory Precedent and Platform Recognition**: With the approval of our first platform product, VYJUVEK, in the United States, EU, and Japan, regulatory precedent for our proprietary HSV-1-based platform has been established, and regulator familiarity with HSV-1 and our platform continues to grow. Reflecting this increasing familiarity, in October 2025, the FDA granted platform technology designation to our genetically modified, non-replicating HSV-1 viral vector used in our product candidate, KB801 for the treatment of neurotrophic keratitis. The FDA's platform technology designation program is intended to provide efficiencies in drug development, manufacturing, and review processes for drug product applications that incorporate designated platform technologies. The designation recognizes the reproducibility and scalability of our platform and its potential to support the development of multiple biologic products without compromising quality, manufacturing, or safety.

The above listed benefits of our innovative platform, and compatibility with formulation for topical, injectable, and inhaled delivery, make it the ideal choice to treat diseases and conditions of the skin, lung, and eye.

Our Commercial Product and Pipeline

The following table summarizes information regarding our commercial product, VYJUVEK, and product candidates in various stages of clinical and preclinical development as of the date of this Annual Report:



Our Commercial Product

VYJUVEK (beremagene geperpavec-svdt, or B-VEC; referred to as B-VEC outside the United States, Europe, and Japan)

Disease Background

DEB is a rare and severe monogenic skin disease. DEB affects the skin and mucosal tissues and is caused by one or more mutations in a gene called *COL7A1*, which is responsible for the formation of the protein type VII collagen ("COL7"). COL7 forms anchoring fibrils that bind the dermis (inner layer of the skin) to the epidermis (outer layer of the skin). In DEB patients, the genetic defect in *COL7A1* results in loss or malfunctioning of these anchoring fibrils, leading to extremely fragile skin that blisters and tears from minor friction or trauma. Those who are born with DEB are sometimes called "butterfly children," because their skin is likened to be as fragile as the wings of a butterfly. DEB patients may suffer from open wounds, skin infections, fusion of fingers and toes, ocular complications that can result in severe vision loss, and gastrointestinal tract problems throughout their lifetime, and may eventually develop squamous cell carcinoma, a potentially fatal condition. We believe that there are, at present, over 3,000 DEB patients in the United States and over 9,000 worldwide. Prior to the approval of VYJUVEK, the standard of care for DEB patients had been limited to palliative measures that seek to provide relief from some of the symptoms of DEB but do not meaningfully impact disease outcomes.

VYJUVEK

VYJUVEK is a redosable, off-the-shelf gene therapy designed to deliver two copies of the *COL7A1* gene when applied topically, directly onto an open wound. Unlike the previous standard of care, VYJUVEK treats DEB at the molecular level by providing the patient's skin cells the template to make normal COL7 protein, thereby addressing the fundamental disease-causing mechanism. VYJUVEK was specifically designed to be easily administered in a doctor's office or at the patient's home. VYJUVEK was first approved by FDA in 2023 for sale in the United States and, in 2025, was approved by European and Japanese regulators for sale in the EU and Japan.

We believe our approach to treating DEB is positively differentiated relative to palliative approaches, which do not address the underlying genetic cause of DEB or impact the durability of wound closure, and corrective treatments that employ autologous approaches. Autologous treatments use a patient's own tissues and cells to manufacture an individualized therapy. Such therapies tend to be expensive, invasive and time consuming to use, and require extensive patient travel, extended hospital stays, and highly sophisticated medical teams and procedures.

Commercial Launches

We possess exclusive rights to commercialize VYJUVEK throughout the world. We are commercializing VYJUVEK directly in the United States, major European markets, and Japan.

We first launched VYJUVEK in the United States in 2023. We estimate that there are over 3,000 patients in the United States suffering from DEB, of which 1,200 were identified at launch through claims analytics and pre-launch patient identification activities conducted by our commercial field force.

In August 2025, we launched VYJUVEK in Germany, our first commercial launch outside the United States, and in October 2025, we launched VYJUVEK in France under the post-marketing authorization early reimbursed access Accès Précoce program. Over 1,000 DEB patients are already identified across both of these countries. Pricing negotiations are underway in both Germany and France and are expected to continue until at least the second half of 2026 in Germany and until 2027 in France.

We are advancing pricing discussions with Italian reimbursement authorities to enable a potential launch in Italy in the second half of 2026.

We are initiating pricing discussions with relevant authorities in other key Western European markets. The timing of additional European launches is uncertain and will depend on the cadence and outcomes of regulatory interaction and pricing negotiations.

In October 2025, we launched VYJUVEK in Japan following successful completion of pricing negotiations with Japan's Ministry of Health, Labour and Welfare ("MHLW").

We contract with specialty distributors to commercialize VYJUVEK in territories outside of the United States, major European markets, and Japan. To date, we have entered into distribution agreements with leading regional specialty distributors covering key markets in Central and Eastern Europe, the Middle East, and Turkey, with Israel representing our most recent addition in February 2026, and we expect to further expand our specialty distributor network during 2026.

Since our first commercial launch of VYJUVEK in the United States, we have reported $730.3 million in net product revenue.

Regulatory Status

On May 19, 2023, the FDA approved VYJUVEK, the first ever redosable gene therapy, for treating patients, six months of age or older, suffering from DEB. No clinical post-marketing commitments or Risk Evaluation and Mitigation Strategies program were required by the FDA. With the approval, the FDA issued a Rare Pediatric Disease Priority Review Voucher ("PRV"), which confers priority review to a subsequent drug application that would not otherwise qualify for priority review. We sold the PRV in the third quarter of 2023 for $100 million.

In September 2025, the FDA approved a label update for VYJUVEK that expanded the treatment eligible population to include DEB patients from birth and provided patients with greater dosing flexibility, including the option for VYJUVEK to be applied by a healthcare professional ("HCP"), caregiver, or directly by the patient themselves, either at home or in a healthcare setting.

The FDA previously granted Orphan Drug Designation ("ODD"), Fast Track Designation ("FTD"), Rare Pediatric Disease Designation ("RPDD"), and Regenerative Medicine Advanced Therapy ("RMAT") to B-VEC for the treatment of DEB.

On April 23, 2025, the European Commission (the "EC") granted marketing authorization to VYJUVEK for the treatment of wounds in patients with DEB starting from birth. VYJUVEK is the first corrective medicine approved in the EU for the treatment of DEB. The approval granted by the EC allows for flexible VYJUVEK dosing either at home or in healthcare setting, with the option for patient or caregiver administration if deemed appropriate by a HCP. The EC decision authorizes the marketing of VYJUVEK in all EU member states, as well as Iceland, Norway and Liechtenstein.

Previously, in September 2023, we received a positive opinion from the European Medicines Agency ("EMA") Pediatric Committee on the Pediatric Investigation Plan for B-VEC for the treatment of DEB. Based on this positive opinion, we expect to be eligible for up to an additional two years of marketing exclusivity in the EU, on top of the ten-year EU market exclusivity granted upon marketing authorization in the EU.

European regulatory authorities also previously granted Orphan Designation and PRIority MEdicines eligibility to B-VEC for the treatment of DEB.

On July 24, 2025, Japan's MHLW granted marketing authorization to VYJUVEK for the treatment of wounds in patients with DEB, starting from birth. VYJUVEK is the first genetic medicine approved in Japan for the treatment of DEB. The Japanese approval allows for dosing at home or in a healthcare setting, with the option for administration by patients or their family members. Genetic testing is not a requirement for treatment. The re-examination period for VYJUVEK in Japan is ten years.

Japanese regulatory authorities previously granted ODD status to B-VEC for the treatment of DEB, a designation which confers multiple benefits including extended registration validity.

Regulatory filings for the marketing authorization of B-VEC in additional global markets are planned or underway.

Clinical Development

We initiated Phase 1 testing of a topical formulation of B-VEC in May 2018 at Stanford University, and we announced positive interim results from this clinical study on two patients in October 2018. The Phase 2 portion of the trial commenced in December 2018 at Stanford University, and we announced positive interim results from this clinical study on June 24, 2019. In March 2022, results from the complete Phase 1/2 study of topical B-VEC for the treatment of DEB were published in *Nature Medicine.*

We initiated a pivotal Phase 3 trial (the "GEM-3 trial") in July 2020. The GEM-3 trial of topical B-VEC for the treatment of DEB was a randomized, double-blind, intra-patient placebo-controlled multicenter study designed to evaluate the efficacy and safety of B-VEC for patients suffering from both recessive and dominant forms of DEB. The trial enrolled 31 participants with DEB, aged 6 months or older at time of consent. In each patient, a primary wound pair was identified by the investigator; one wound was randomized to receive a weekly topical application of B-VEC and the other to receive placebo. These primary wounds were treated once weekly for six months until wound closure. If a wound re-opened at any point during the study, weekly dosage resumed until closure. The dose administered to each wound was dependent on the size of the wound. A maximum vector dose per patient per week was defined on the basis of preclinical and clinical safety data. In the event that the maximum dose per patient had not been reached based on dosing of the primary wounds, the study investigators and patients had the opportunity to select additional "secondary" wounds across which the remaining weekly dose was applied. We announced positive results from the GEM-3 trial in November 2021 and, in December 2022, full results from the GEM-3 trial were published in the *New England Journal of Medicine.*

Following completion of the GEM-3 trial, we initiated an open label extension ("OLE") study to provide extension of B-VEC treatment for participants who completed the GEM-3 trial ("rollover participants") and B-VEC treatment for newly

enrolling participants ("naïve participants") with DEB. The OLE was a multi-center, open-label study of B-VEC for the topical treatment of DEB wounds. The study enrolled 47 participants in total, comprising 24 rollover participants and 23 naïve participants, at five sites in the United States. In April 2022, following feedback from the FDA, we announced that patients enrolled in the OLE study would have the option to be dosed in their homes by a HCP. The primary study objective was the assessment of safety and tolerability of extended dosing with B-VEC in a broader patient population. Various quality of life and participant satisfaction metrics were also assessed. The OLE study was concluded in the third quarter of 2023, and the safety profile continued to support the overall benefit-risk of B-VEC, with no new safety concerns noted with extended duration of dosing of B-VEC. Full OLE study results were published in the *American Journal of Clinical Dermatology* in April 2025.

In July 2023, Japan's Pharmaceuticals and Medical Devices Agency (" PMDA") officially accepted our OLE study of B-VEC in Japanese patients (the "Japan OLE"). Following that acceptance, we initiated the Japan OLE study and completed study enrollment. In April 2024, the efficacy portion of the Japan OLE study was completed with results that closely mirrored those of our GEM-3 trial in the United States. B-VEC was well tolerated in the Japanese study population, with a safety profile consistent with previous studies, and all four patients that completed the study achieved the primary endpoint of complete wound closure at six months. Results of the Japan OLE study were published the *Journal of Dermatology* in July 2025.

Our Pipeline

Respiratory

KB407 for Cystic Fibrosis ("CF")

Disease Background

CF is the most common inherited genetic disorder in the United States and is caused by mutations in the cystic fibrosis transmembrane conductance regulator ("*CFTR*") gene. Lack of functional CFTR protein in secretory airway epithelia results in defective Cl-, bicarbonate, and thiocyanate secretion, coupled with enhanced Na+ absorption and mucus production, leading to dehydration and acidification of the airway surface liquid. CF is characterized by recurrent chest infections, increased airway secretions, and eventually, respiratory failure. While CF comprises a multiorgan pathology affecting the upper and lower airways, gastrointestinal and reproductive tracts, and the endocrine system, the primary cause of morbidity and mortality in CF is progressive lung destruction.

According to the Cystic Fibrosis Foundation ("CFF"), the median age at death for patients with CF in the United States was 36.9 years in 2023. Currently approved CFTR modulating therapies are limited to patients with specific genetic mutations and there is a significant unmet medical need for the approximately 10%-15% of patients with CF who have genetic mutations non-amenable to currently approved CFTR small molecule "modulators". The CFF estimates that there are close to 40,000 children and adults living with CF in the United States, and an estimated 105,000 people diagnosed with CF across 94 countries. People of every racial and ethnic group are affected by this debilitating disease.

KB407

KB407 is a redosable off the-shelf gene therapy designed to deliver two copies of the full-length *CFTR* transgene directly to the airway epithelia via inhaled (nebulized) administration. By enabling expression of full-length, normal CFTR protein in the lung, treatment with KB407 has potential to restore ion and water flow into and out of lung cells to correct the lung manifestations of the disease in patients regardless of their underlying genetic mutation. Preclinical efforts show that KB407 successfully transduces patient-derived epithelial cells and delivers functional CFTR *in vitro* in 2D and 3D organotypic systems, and is amendable to non-invasive inhaled administration *in vivo*, as indicated by successful delivery to the lungs through the use of a clinically relevant nebulizer in small animal models. Successful delivery and distribution throughout the lung also was observed in nonhuman primates.

The FDA and the EMA have granted KB407 ODD and Orphan Designation, respectively, for the treatment of CF, and the FDA has granted KB407 RPDD for the treatment of CF.

Clinical Development of KB407

In July 2023, we announced that we had dosed the first patient in our Phase 1 CORAL-1 study evaluating KB407, delivered via a nebulizer, for the treatment of patients with CF. The CORAL-1 study is a multi-center, dose-escalation trial of KB407 in patients with CF, regardless of their underlying genotype. In December 2024, we announced an interim safety data update for patients treated with KB407 in the first two dose escalation cohorts. KB407 was well tolerated with only mild to moderate and transient adverse events observed.

In January 2025, the CFF Therapeutic Development Network ("TDN") Clinical Research Executive Committee granted full sanctioning of our KB407 Phase 1 CORAL-1 study protocol.

In January 2026, we announced a positive interim clinical update from Cohort 3, the highest dose cohort of CORAL-1, confirming the successful lung delivery and expression of wild-type CFTR protein following inhaled administration of KB407.

KB407 transduction was observed in all Cohort 3 patients with successful bronchoscopies irrespective of modulator-status and genetic background, with broad airway distribution and transduction as assessed by CFTR or viral marker immunofluorescence, ranging from 29.4% to 42.1%. Consistent with the safety profile previously reported from Cohorts 1 and 2, inhaled KB407 continued to be well tolerated by patients treated with the highest dose in Cohort 3. All but one KB407-related adverse event were mild to moderate in severity and transient in nature. One serious adverse event ("SAE") of asthma exacerbation was reported 24 hours after completion of the bronchoscopy and was deemed procedure related, and not related to KB407, by the independent data monitoring committee. The SAE resolved in 5 days. Details of the CORAL-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05504837.

We are now working with the TDN on a repeat dosing study design, CORAL-3, which was submitted to the FDA in December 2025. CORAL-3 is designed to evaluate the safety and efficacy of repeat KB407 administration, including through regular assessments of lung function by spirometry, and to support potential registration. We expect to align on the CORAL-3 study design with the FDA and start enrollment in the potentially registrational CORAL-3 study in the first half of 2026. Additional details on the study design will be provided by the time of study initiation.

KB408 for Alpha-1 Antitrypsin Deficiency ("AATD") Lung Disease

Disease Background

AATD is a genetic condition caused by mutations that lead to decreased levels and/or decreased functionality of the alpha-1- antitrypsin ("AAT") protein. AATD lung disease is a consequence of diminished or absent functional protein in the lungs due to impaired transport into, and low concentrations in, patient plasma. Low AAT serum levels can result in life threatening, progressive pulmonary impairment and severe respiratory insufficiency, manifesting as chronic obstructive pulmonary disease and panacinar emphysema. The lung degeneration observed in AATD patients derives from unopposed, and therefore enhanced, neutrophil elastase ("NE") activity, leading to an excessive degradation of elastin, collagen, and fibronectin. The absence of proper NE inactivation by functional AAT ultimately results in lung tissue destruction, airway obstruction, and an increased inflammation state that compromises the integrity of the organ and contributes to an inadequate response to insults, including inefficient pulmonary bacterial clearance.

We estimate that there are over 60,000 patients in the United States and over 250,000 patients globally suffering from severe AATD. Currently, many AATD patients undergo "augmentation therapy" consisting of weekly intravenous ("IV") infusions of either plasma-purified AAT or recombinant AAT. This therapy requires burdensome weekly IV infusions and often includes the risk of exposure to bloodborne pathogens connected with the use of blood-derived products.

KB408

KB408 is an inhaled (nebulized) formulation of our proprietary vector, designed to deliver two copies of the *SERPINA1* transgene that encodes functional, full-length human AAT protein, for the treatment of AATD. Preclinical studies have shown that KB408 successfully transduces patient-derived lung epithelial cells *in vitro*, leading to production and secretion of full-length human AAT protein capable of irreversibly binding its cognate target NE. In small animal models, analysis of lung tissue biopsies, serum, and bronchoalveolar lavage fluid harvested 24 and 48 hours after inhalation of KB408 shows secretion of full-length AAT protein, with no evidence of significant or systemic toxicity.

The FDA has granted KB408 ODD for the treatment of AATD.

Clinical Development of KB408

In February 2024, we announced that we had dosed the first patient in our Phase 1 SERPENTINE-1 study evaluating KB408, delivered via a nebulizer, for the treatment of patients with AATD. SERPENTINE-1 is a Phase 1 open-label, single dose escalation study in adult patients with AATD with a Pi*ZZ or Pi*ZNull genotype.

In December 2024, we announced an interim clinical data update including safety data for seven patients enrolled in the first two dose escalation cohorts of SERPENTINE-1 as well as molecular data from two patients in the second cohort ("Cohort 2") that had consented to bronchoscopy. Clear evidence of successful gene delivery and AAT expression was observed in both patients that underwent bronchoscopies, with the proportion of conducting airway epithelial cells positive for AAT increasing from 0% to 39% in one patient and from 3% to 35% in the other. Secretion and functionality of encoded AAT was also demonstrated in the patient with available lavage samples, with AAT levels in epithelial lining fluid reaching 729 nM, and the proportion of free NE dropping from 97.2% to 40.2%, after a single KB408 dose. KB408 was also found to be well-tolerated at both tested dose levels, with only mild to moderate and transient adverse events observed.

In August 2025, we confirmed *SERPINA*1 delivery and functional AAT expression in a third patient dosed with KB408 in Cohort 2 and amended the SERPENTINE-1 protocol to investigate repeat dosing at the Cohort 2 dose level (the repeat dose cohort is referred to as "Cohort 2B"). The first patient in Cohort 2B was dosed in August 2025 and enrollment in this repeat dose cohort is ongoing. We expect to report interim safety and *SERPINA1* delivery data from the repeat dose Cohort 2B in 2026. Enrollment in single dose cohorts is now closed. We are working closely with the Alpha-1 Foundation and their

Therapeutic Development Network on the SERPENTINE-1 study. Details of the SERPENTINE-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06049082.

Ophthalmology

KB803 (Ophthalmic B-VEC) for Ocular Complications of DEB

Disease Background

DEB affects not only the skin, but also mucosal tissues that depend on COL7 anchoring fibrils to maintain epithelial lining integrity. This includes the eye, where COL7 anchors the corneal epithelium. For a meaningful proportion of DEB patients, the genetic defect in *COL7A1* results in loss or malfunctioning of these anchoring fibrils causing ocular complications, such as corneal erosions, abrasions, blistering, and scarring, that can lead to progressive vision loss.

Over 50% of patients with the recessive form of DEB and an estimated 10% of patients with the dominant form of DEB are thought to suffer from ocular complications. Correspondingly, we believe there are over 750 patients in the United States and over 2,000 worldwide that are affected. Disease management varies from supportive care and wound management to surgical interventions to remove scar tissue. No corrective or FDA approved therapies are presently available.

KB803 (Ophthalmic B-VEC)

KB803 is a redosable eye drop formulation of B-VEC, designed to deliver two copies of the *COL7A1* transgene to the epithelial cells in a patient's eye to produce COL7 protein. As with VYJUVEK, the goal of therapy with KB803 is to treat the disease locally, at the molecular level, by providing the patient's epithelial cells of the eye the template to make normal COL7 protein and thereby address the fundamental disease-causing mechanism. In preclinical studies, single and repeated topical B-VEC administration to the eye in a mouse corneal lesion model resulted in localized *COL7A1* expression with no adverse effects noted histologically.

B-VEC has been applied topically to the eye of one DEB patient under a compassionate use protocol. The clinical observations of this compassionate use case were published in the *New England Journal of Medicine* in February 2024. The patient presented with severe cicatrizing conjunctivitis secondary to DEB. Surgical symblepharon lysis of the patient's right eye with pannus removal was conducted and regular administration of B-VEC as an eye drop directly to the eye (5×10^9 PFU/mL) were added to routine post-surgical care, three times weekly for the first two weeks and then once weekly. B-VEC application frequency was further decreased to once monthly once the corneal epithelium was healed. B-VEC was well tolerated with no drug-related adverse events noted. Full corneal healing was observed at three months, as well as significant visual acuity improvement from hand motion to 20/25 by eight months.

Clinical Development of KB803

In June 2025, we announced that we dosed the first patient in IOLITE, an intra-patient, double-blind, placebo-controlled, decentralized, multicenter Phase 3 registrational study with a crossover design to evaluate KB803 for the treatment and prevention of corneal abrasions in DEB patients, six months of age or older. After observing a promising clinical safety profile in the initial patients treated with Krystal's eye drop gene therapies, we modified the KB803 dosing schedule to reduce the potential impact of human error in eye drop administration. Under the updated protocol, enrolled patients receive either a single eye drop of placebo or KB803, at a concentration of 10^9 PFU/mL, to each eye three times weekly for 12 weeks. Drug administration is in the home setting and may be performed by a HCP or by the patient or their caregiver after receiving training on appropriate administration technique. At the conclusion of the first 12 weeks, patients will be switched from placebo to KB803, or vice versa, and continue with three times weekly administration for a second 12 week period. We expect to enroll approximately 16 patients in the IOLITE study. The primary study endpoint is the change in the average number of days per month with corneal abrasion symptoms while receiving KB803 versus placebo. Safety and secondary efficacy data, including weekly assessments of eye pain and monthly Epidermolysis Bullosa Eye Disease Index questionnaires, will be collected through to the end of the 24-week study period. We continue to enroll patients in IOLITE and expect to complete enrollment in the first half of 2026 and report top-line results later in 2026. More details of the IOLITE study can be found at www.clinicaltrials.gov under NCT identifier NCT07016750.

Patients seeking to participate in IOLITE must first enroll in an ongoing natural history study and complete a 12-week run-in period, during which they report the number of days that they experience symptoms of corneal abrasions. Patients meeting the inclusion criteria following the 12-week run-in are eligible to participate in the IOLITE trial. The natural history study was initiated in August 2024 and remains open for enrollment. Details of the natural history study can be found at www.clinicaltrials.gov under NCT identifier NCT06563414.

KB801 for Neurotrophic Keratitis ("NK")

Disease Background

NK is a rare, degenerative corneal disease characterized by damage or loss of function in the neurons innervating the eye leading to corneal epithelial defects, ulcers, and perforation. Left untreated, NK can result in severe vision loss. Although NK is a rare disease with an estimated prevalence in the range of 10 to 50 cases per 100,000, claims data analyses suggest awareness and diagnosis rates are on the rise in the United States. Based on available claims data, an estimated 68,000 patients in the United States had a NK claim in 2024, up over 115% from 31,000 patients with a NK claim in 2020.

Recombinant nerve growth factor ("NGF") eye drops have been shown to significantly improve corneal healing and are approved for the treatment of NK in multiple jurisdictions worldwide including the United States, but the short half-life of recombinant protein results in rapid clearance from the eye, thereby necessitating burdensome administration six times a day, and may lead to suboptimal treatment outcomes. Eye pain during treatment is also frequently reported.

KB801

KB801 is an eye drop formulation of our novel HSV-1 based vector designed to deliver two transgene copies to the corneal epithelium for the sustained, localized expression and secretion of NGF and treatment of NK. In preclinical studies presented at the Association for Research in Vision and Ophthalmology 2025 Annual Meeting in May 2025, KB801 was shown to efficiently transduce corneal epithelial cells *in vitro* and *in vivo* leading to sustained NGF production in the front of the eye. By transducing the cells of the corneal epithelium to produce and secrete NGF, KB801 has the potential to significantly reduce the treatment burden for patients while also maintaining more consistent NGF levels in the front of the eye.

Clinical Development of KB801

In July 2025, we announced that we dosed the first patient in EMERALD-1, a Phase 1/2, randomized, double-masked, multicenter, placebo-controlled study evaluating KB801, administered as an eye drop, for the treatment of NK. In October 2025, the FDA granted platform technology designation to the engineered HSV-1 viral vector used in KB801, a designation which affords development and manufacturing efficiencies for the development of KB801. Following receipt of this designation, we amended the EMERALD-1 protocol to enable the study to serve as a registrational trial supporting the potential registration of KB801. Under the updated protocol, we expect to enroll approximately 60 adult patients with Stage 2 or Stage 3 NK, as defined by the Mackie criteria. Enrolled patients are randomized 1:1 to receive either KB801, at a concentration of 10^{10} PFU/mL, or placebo topically to the study eye daily for eight weeks. Drug administration may be performed either by a HCP or by the patient or their caregiver at home after receiving training on appropriate administration technique. The KB801 dosing schedule was modified taking into consideration the potential to reduce the impact of human error in eye drop administration at home and the promising clinical safety profile in the initial patients treated with KB801 and KB803. The primary objectives of EMERALD-1 are to evaluate the safety and efficacy of topical ocular administration of KB801 for the treatment of NK. The primary efficacy assessment is the proportion of patients with complete durable healing of corneal epithelium at eight weeks. Additional exploratory efficacy measures will include change in corneal lesion size from baseline, each assessed at weeks 4, 6, 8, and 10, as well as evaluations of corneal sensation and patient-reported symptom burden. Enrollment in EMERALD-1 is ongoing, and we expect to report top-line data in 2026. More details of the EMERALD-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06999733.

Dermatology

KB111 for Hailey-Hailey Disease ("HHD")

Disease Background

HHD is a serious and rare monogenic skin disorder characterized by painful rash and blistering in skin folds and linked to low expression of levels human calcium transporter ATPase type 2C member 1 ("ATP2C1") in keratinocytes. Patients with HHD report debilitating symptoms of pain, itch, burning, infections, and body odor, as well severe, negative impacts on quality of life and psychological distress.

The prevalence of HHD is not well characterized and is most commonly estimated at roughly 1 per 50,000, although underreporting is possible. Current disease management is supportive in nature and no specific therapy for HHD has been approved by the FDA or EMA.

KB111

KB111 is a topical gel formulation of our novel vector designed to deliver two copies of the full-length, wild-type *ATP2C1* transgene to skin cells for the treatment of HHD. In preclinical studies presented at the Society for Investigative Dermatology 2025 Annual Meeting in May 2025, KB111 was shown to efficiently deliver *ATP2C1* to keratinocytes *in vitro* and *in vivo* resulting in increased expression of functional ATP2C1. By increasing functional ATP2C1 levels in the skin, KB111 has the potential to accelerate lesion healing and meaningfully reduce disease burden for HHD patients.

In January 2026, the FDA granted KB111 FTD for the treatment of HHD.

Clinical Development of KB111

In October 2025, the FDA cleared our investigational new drug ("IND") application to evaluate KB111 in the clinic. We are currently developing an HHD-specific evaluation scale necessary for the clinical evaluation of KB111. We expect to complete development and validation of the scale in the first half of 2026 and initiate a registrational, intra-patient randomized, double-blind, placebo-controlled, multi-center study evaluating KB111 for the treatment of HHD in the second half of 2026.

Oncology

KB707 for Solid Tumors

Disease Background

Cancer is progressive and typically fatal disease for which adequate treatment options are lacking. Despite recent advancements, the treatment of locally advanced or metastatic solid tumors remains particularly difficult and long-term outcomes are poor. Standard of care treatments are rarely curative, therapeutic benefits are transient, and the majority of patients with locally advanced or metastatic solid tumors are either ineligible for, or will eventually exhaust, currently available therapies. Many available therapies are also highly toxic and poorly tolerated by patients.

Cancer imposes a heavy burden on patients worldwide. The World Health Organization lists cancer as a leading cause of death globally and estimates that the disease was responsible for nearly 10 million deaths in 2020. Of these, an estimated five million deaths were attributed to solid tumor malignancies of the lung, colon and rectum, liver, stomach, and breast alone. Solid tumor malignancies similarly impose a heavy burden on patients in the United States, with the National Cancer Institute estimating that hundreds of thousands of patients died from lung, colon and rectum, pancreas, breast, prostate, liver and bile duct, and melanoma of the skin cancers in 2025.

There is an especially urgent need for new therapies for the treatment of lung cancer, which is widely considered the deadliest cancer globally and in the United States. Even with currently available therapies, the National Cancer Institute estimates that over 124,000 patients died from lung cancer in 2025.

KB707

KB707 is a redosable, immunotherapy designed to deliver genes encoding both human interleukin-2 ("IL-2") and interleukin-12 ("IL-12") to the tumor microenvironment and promote systemic immune-mediated tumor clearance. Two formulations of KB707 are in development, a solution formulation for transcutaneous injection and an inhaled (nebulized) formulation for lung delivery. IL-2 and IL-12 are secreted cytokines with complementary functions promoting cell-mediated immunity in humans. Both IL-2 and IL-12 have been shown to elicit anti-tumor immune responses in preclinical models and/or in the clinic and have been extensively studied for their potential in cancer immunotherapy. Despite promising signs of efficacy, it has proven difficult to effectively harness IL-2 and IL-12 for therapeutic benefit, as systemic administration is often poorly tolerated, and the inherently short half-lives of these cytokines necessitate high dose levels and extremely frequent dose intervals. KB707 leverages our HSV-1 vector platform – and its ability to efficiently deliver a durable DNA payload without active replication and minimal cytotoxicity – to drive local and sustained cytokine expression within the tumor microenvironment and maximize the therapeutic window and benefit of IL-2 and IL-12.

In preclinical studies, KB707 has been shown to efficiently transduce mammalian cells *in vitro* leading to the secretion of bioactive IL-2 and IL-12 and drive localized, durable cytokine expression in mouse skin after intradermal injection. Furthermore, in stringent, checkpoint inhibitor refractory 'cold' syngeneic mouse models, HSV-1 vector based delivery of murine equivalent IL-2 and IL-12 elicited robust antitumor responses and survival benefits, including via intratumoral injection in single and dual flank B16F10 melanoma models, as well as via intratracheal delivery in a metastatic K7M2 osteosarcoma model, with evidence of protection from tumor rechallenge in both models suggestive of prolonged adaptive immunity.

In July 2023, the FDA granted intratumoral KB707 Fast Track Designation for the treatment of anti-programmed cell death protein-1 ("PD-1") relapsed/refractory locally advanced or metastatic melanoma and, in February 2024, the FDA granted inhaled KB707 Fast Track Designation for the treatment of patients with solid tumors with pulmonary metastases that are relapsed or refractory to standard of care therapy. Both intratumoral and inhaled KB707 have also been granted RPDD by the FDA, with intratumoral KB707 receiving RPDD for the treatment of rhabdomyosarcoma in August 2024 and inhaled KB707 receiving RPDD for the treatment of osteosarcoma in May 2024.

In February 2026, the FDA also granted RMAT designation to KB707 for the treatment of advanced or metastatic NSCLC.

We have prioritized development of the inhaled KB707 formulation based on early clinical evidence of efficacy for the treatment of NSCLC.

Clinical Development of Inhaled KB707

In January 2024, the FDA accepted an amendment to our KB707 IND application to evaluate inhaled KB707 in a clinical trial to treat patients with locally advanced or metastatic solid tumors of the lung. The study, KYANITE-1, is an open-label, multi-center, dose escalation and expansion Phase 1/2 study, evaluating inhaled KB707, as monotherapy or in combination, in patients with advanced solid tumor malignancies affecting the lungs, who relapsed or are refractory to standard of care. The primary objective of the study is to evaluate safety and tolerability of KB707 delivered via inhalation, alone or in combination. Efficacy is also being assessed by multiple measures, including objective response rate ("ORR"), as are the immune effects of KB707 monotherapy.

The first patient in KYANITE-1 was dosed in April 2024. In August 2024, the monotherapy dose escalation portion of the study was completed, and the monotherapy dose expansion cohort was opened.

In December 2024, we announced an initial clinical update and early evidence of monotherapy activity from the monotherapy dose escalation and expansion cohorts of KYANITE-1. The data update included safety data for 37 patients that had received at least one dose of inhaled KB707 and efficacy data from 11 patients with advanced NSCLC evaluable for response with at least one radiographic scan and RECIST v1.1 evaluation. Patients included in the efficacy analysis were heavily pre-treated with four median lines of prior therapy and all had received at least one line of prior immunotherapy. In this NSCLC patient cohort, as of data cut-off, an ORR of 27%, with three partial responses, was achieved. The disease control rate ("DCR") was 73% with 7 out of 11 patients still remaining on treatment. Duration of treatment for patients included in the analysis ranged from 10.3 to 33.3 weeks as of data cut-off. Inhaled KB707 was also found to be safe and generally well tolerated and amenable to administration in an outpatient setting. The majority of treatment-related adverse events were mild to moderate in severity and transient, with no Grade 4 or 5 adverse events observed. Based on these positive initial results, we also announced, in December 2024, the amendment of KYANITE-1 to include additional cohorts for the evaluation of inhaled KB707 in combination.

In June 2025, we disclosed a clinical update from the monotherapy dose escalation and expansion cohorts of KYANITE-1, including updated efficacy data from the previously disclosed cohort of 11 evaluable patients with heavily pre-treated advanced NSCLC. As of the updated data cutoff, the ORR in the NSCLC patient cohort was 36%. The DCR was 54%. Median duration of response and progression free survival were not reached. Inhaled KB707 was again found to be safe and generally well tolerated as monotherapy in the 39 patients included in the safety analysis, with the majority of treatment-related adverse events deemed mild to moderate in severity and transient and no Grade 4 or 5 adverse events observed.

In August 2025, we were granted an End of Phase 2 meeting with the FDA to discuss the inhaled KB707 program and, in November 2025, we announced that based on the FDA's feedback, we expect that a single Phase 3 registrational study, evaluating inhaled KB707 in combination with chemotherapy against chemotherapy alone in patients with advanced NSCLC, would be sufficient to support potential registration of inhaled KB707 in combination with chemotherapy as a second-line treatment for NSCLC. In support of this potential registrational pathway, we opened a new cohort in KYANITE-1 to evaluate inhaled KB707 in combination with chemotherapy in patients with advanced NSCLC.

Enrollment in the new cohort of KYANITE-1 evaluating inhaled KB707 in combination with chemotherapy is ongoing, and we expect to report interim efficacy data and potential registrational study plans in 2026. Details of the KYANITE-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06228326.

Clinical Development of Intratumoral KB707

In July 2023, we announced that the FDA had accepted our initial KB707 IND application to evaluate intratumoral KB707 in a clinical trial to treat patients with locally advanced or metastatic solid tumors. The study, OPAL-1, is an open-label, multi-center, dose escalation and expansion Phase 1/2 study, evaluating intratumoral KB707, as monotherapy or in combination, in patients with locally advanced or metastatic solid tumors, who relapsed or are refractory to standard of care, with at least one measurable and injectable tumor accessible by transcutaneous route. The primary objective of OPAL-1 is to evaluate safety and tolerability of KB707 alone and in combination. Efficacy is also being evaluated by multiple measures, including ORR, as are the immune effects of KB707 monotherapy. The first patient in OPAL-1 was dosed in October 2023 and, in May 2024, the third and final monotherapy dose escalation cohort was cleared. OPAL-1 was subsequently amended to add two dose expansion cohorts, in addition to the monotherapy dose expansion cohort, evaluating intratumoral KB707 in combination with anti-PD-1 and anti-lymphocyte activation gene 3 therapy or anti-PD-1 therapy alone, in patients with advanced melanoma that is relapsed or refractory to standard of care.

In August 2025, we announced that we were prioritizing the development of inhaled KB707 for the treatment of NSCLC and had paused enrollment in OPAL-1. We continue to follow patients enrolled in OPAL-1 and based on safety and efficacy results from the study, we may adjust development plans for intratumoral KB707. Details of the OPAL-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05970497.

Aesthetics

While our focus is on the development of gene therapies to treat patients with severe, life-threatening, or rare diseases with high unmet medical needs, we are also evaluating the potential of our platform to address aesthetic conditions. We incorporated Jeune Aesthetics, a wholly-owned subsidiary, for the purposes of undertaking preclinical and clinical studies for aesthetic skin conditions.

KB304 for Dynamic Wrinkles of the Décolleté

Background

The skin is largely composed of collagen-rich connective tissue, with dermal collagen, composed primarily of type 1 and 3 collagen fibrils ("COL1" and "COL3", respectively), representing over 90% of the dry weight of human skin. These fibrils provide strength to the skin and are critical for the maintenance of skin tissue architecture. In the skin, new collagen synthesis is affected by the deposition of, and complex interactions between, COL1 and COL3. COL3 appears early during collagen fibril formation and has been shown to both regulate the dimensions of COL1 fibers and enhance COL1 elasticity. Many characteristics of skin aging are largely due to aberrant collagen homeostasis, including reduced collagen biosynthesis, increased collagen fibril fragmentation, and progressive loss of dermal collagen culminating in a net collagen deficiency, resulting from both intrinsic (e.g., passage of time, genetics) and extrinsic (e.g., chronic light exposure, pollution) pressures. These factors together lead to cumulative structural and physiological alterations to the skin, ultimately leading to the onset and eventual worsening of skin wrinkles.

Elastin ("ELN") is the major constituent of elastic fibers which provides resilience and elasticity to tissues and organs and, as such, is another important determinant of skin tissue architecture. Although ELN represents only 2% of total dermal protein, it is roughly 1,000-fold more flexible than collagen, and thus, is the main component providing elasticity to skin. ELN synthesis occurs early in life and through childhood, after which very little turnover is observed unless the elastic fibers are subject to injury. Although elastin is a durable biopolymer that does not turn over appreciably in healthy tissue, aging of elastin is observed over time as damage to elastic fibers increases susceptibility to enzymatic degradation. Elastin aging is induced and accelerated by environmental influences, primarily UV radiation. UV-induced extrinsic aging leads to, among other defects, loss of elasticity.

Skin rejuvenation is the process of reversing or repairing irregularities in the skin and is achieved, in part, by the synthesis of new collagen and ELN. Significant consumer demand exists for fundamentally rejuvenative aesthetic products, including among younger consumers that are seeking more preventative interventions to maintain a natural-looking, youthful appearance. This demand is expected to grow driven by increasing aesthetic injectable adoption in emerging markets and shifting consumer attitudes about wellness, beauty, and healthy aging that have increased awareness and acceptance of aesthetic treatments among new consumer segments.

One area of the skin that can experience early aging is the female décolleté (upper chest). This skin is naturally thinner than other areas of the body and has fewer sebaceous glands, making it more susceptible to visible signs of aging. The female décolleté is considered an extension of the face and demand for aesthetic solutions is high, yet there are no FDA-approved aesthetic injectable products for the décolleté, and aesthetic procedures for the delicate skin of the décolleté are challenging and can cause hyperpigmented and hypopigmented skin.

KB304

KB304 leverages our clinical experience in delivering genes of interest to the skin and is designed to stimulate biorejuvenation of the skin via delivery of both *COL3A1* and *ELN* transgenes encoding full-length human COL3 and ELN. KB304 is administered via intradermal injection. We believe that our approach of directed expression of full-length human COL3 and ELN via intradermal application of KB304 provides a unique, comprehensive, and straightforward approach to restoring collagen homeostasis and skin elasticity, and by extension, reconstructing an optimal physiologic environment in the skin to treat wrinkles or other presentations of aged or damaged skin.

Clinical Development of KB304

In November 2024, we dosed the first subject in PEARL-2, a 2:1 randomized, double-blind, placebo-controlled Phase 1 study evaluating KB304, for the treatment of wrinkles of the décolleté, In July 2025, we announced positive safety and efficacy results from PEARL-2, including significant improvements in key skin aesthetic attributes such as wrinkles and elasticity. Meaningful aesthetic improvements in multiple skin attributes were reported by the investigator and subjects alike following KB304 treatment, with clear and statistically significant advantages over placebo. Improvements were reported not only for wrinkles but also multiple additional skin attributes, including elasticity, crepiness, hydration, and radiance. Increased subject satisfaction with wrinkle appearance was also reported, with clear separation from placebo. All adverse events were mild-to-moderate in severity and transient. The frequency and duration of adverse events also decreased with subsequent doses

of KB304. No serious or severe adverse events were reported. Details of the study can be found at www.clinicaltrials.gov under NCT identifier NCT06724900.

Based on the broad aesthetic improvements observed following KB304 treatment in PEARL-2, we are progressing KB304 into a Phase 2 study for the treatment of wrinkles of the décolleté. In support of the Phase 2 study, we developed and validated a décolleté-specific photonumeric scale ("JDWS") which was submitted to the FDA in the second half of 2025. We have aligned with the FDA on the JDWS and expect to initiate the Phase 2 study in 2027.

Additional Pipeline Product Candidates for Aesthetic Skin Conditions Including KB301

In addition to KB304, we are advancing a pipeline of aesthetic product candidates targeting the loss or reduced expression of individual structural proteins of the skin to address specific skin attributes and aesthetic skin conditions. Of these, the most advanced candidate is KB301, a solution formulation of our novel vector for intradermal injection designed to deliver two copies of the *COL3A1* transgene to address signs of aging or damaged skin caused by declining levels of, or damaged proteins within the extracellular matrix, including type III collagen.

Clinical Development of KB301

We initiated a Phase 1 clinical trial, the PEARL-1 trial, for the treatment of aesthetic skin conditions in August 2020. The Phase 1 dose-ranging trial evaluated the safety, tolerability, and initial efficacy of intradermal injections of KB301 in adult subjects aged 18-75. KB301 was well tolerated, and we were able to biopsy and demonstrate proof-of-mechanism. Complete results from Cohort 1 focused on safety were presented at the 2021 Society for Investigative Dermatology Annual Meeting.

In March 2022, we announced positive proof-of-concept efficacy and safety data from Cohort 2 of the PEARL-1 study of KB301 for the treatment of aesthetic skin indications. Cohort 2 was a randomized, double-blind, placebo-controlled clinical trial that evaluated the safety and efficacy of KB301 for the improvement of fine lines and skin texture in the lower and upper cheek and for improvement in skin thickness in the knee. Cohort 2 enrolled 27 subjects across two trial sites. Bilateral treatment areas included the neck behind the ear to assess initial safety and on the cheek below and above the zygomatic arch (lower and upper cheek), and around the knee. Subjects were randomized 2:1 to receive low dose KB301 or placebo in the upper cheek and knee as multiple micro depot injections over the selected treatment area with a 33 G needle. Subjects receiving KB301 in the lower check were randomized 2:1 to receive either low dose KB301, high dose KB301 or placebo. Four patients dropped out of the Cohort 2 study – one subject following the initial safety assessment behind the ear, two subjects for unspecified reasons, and one subject due to unevenness in face between active and placebo during the study.

A subset of subjects from the PEARL-1 Cohort 2 trial were enrolled into a durability trial to look for duration of effect, reduction of the unevenness in placebo treated sites, and for long term safety monitoring. Ten subjects were enrolled in the durability trial, an open-label extension study to assess duration of effect below the zygomatic arch (the lower cheek area). The durability trial enrolled subjects who had received the high dose regimen of KB301 during the PEARL-1 Cohort 2 trial in one or both of their lower cheeks. Subject Satisfaction Scores and Investigator Assessments were measured monthly for three consecutive visits that correspond to timepoints up to nine months following administration of the last dose of KB301. In addition, subjects with placebo-treated lower cheeks were dosed with KB301 during the durability trial to normalize their appearance. In November 2022, we announced nine-month durability of effect in Cohort 2 of the PEARL-1 study of KB301.

Building on the results from Cohort 2, we opened two additional open-label, single-arm PEARL-1 cohorts to evaluate KB301 in two potential target indications for a Phase 2 trial, lateral canthal lines at rest and dynamic wrinkles of the décolleté, referred to as Cohorts 3 and 4, respectively. In August 2024, we announced positive interim safety and efficacy results from both cohorts, assessed out to two months following KB301 injections. Meaningful and sustained improvements in multiple skin aesthetic attributes, including wrinkles, crepiness, hydration, and radiance, were reported by the study investigators and subjects alike in both the décolleté and lateral canthal regions. Increased subject satisfaction with wrinkle appearance was also reported, including among 94% of subjects treated in the décolleté region. Across both cohorts, the KB301 safety profile was consistent with prior clinical experience in Cohorts 1 and 2 and other injectable aesthetic products. Adverse events were primarily injection associated, mild-to-moderate, and transient. No drug related serious adverse events were reported. Details of the PEARL-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT04540900.

We are currently evaluating aesthetic indications most suitable for advanced clinical development of KB301.

Future Opportunities

In addition to the programs specified herein, we are also conducting exploratory preclinical research and development to expand potential applications of our proprietary HSV-1 based vector platform. Research focus areas include the development of new candidates for the treatment of additional monogenic rare diseases in the skin, lung, and eye, as well as exploration of new routes of administration to treat diseases in additional tissues. We also believe the ability to redose, as well as the large payload capacity of our proprietary vectors, will allow us to deliver multiple genes and other effectors, which could enable development of therapies for more common conditions that are not necessarily the result of an inherited genetic defect.

If we are able to successfully generate product candidates to treat these more common conditions, we intend to seek collaborative alliances towards the development and potential commercialization of these therapies.

Manufacturing

In-House CGMP Facilities

We have built in-house CGMP facilities in the United States to enable better quality control, shorten lead times, lower costs and strengthen command over our intellectual property. Our first facility, ANCORIS, a commercial-scale CGMP-compliant manufacturing facility, is producing VYJUVEK for commercial sales. In December 2022, the FDA completed a successful audit of our ANCORIS facility. In February 2024, the EMA completed inspection of our ANCORIS facility as part of the marketing authorization application review process and, in May 2024, European Union good manufacturing practice certification was granted by the EMA. ANCORIS has also passed inspection by Japanese regulatory authorities as part of the Japanese New Drug Application review process in 2025.

Our second commercial scale CGMP facility, ASTRA, was completed and qualified in 2023. It is a state-of-the-art CGMP manufacturing facility that, in addition to adding significant capacity to support our growing pipeline, also allows for in-house incorporation of raw material preparation, excipient manufacturing, testing, packaging, labeling and distribution, thereby fully integrating all components of the supply chain from starting materials to patient experience. We announced the ground breaking of ASTRA in January 2020 and began operational production in the third quarter of 2023.

Our proprietary manufacturing process which was initially developed for B-VEC and is now being used across our platform, was developed and optimized internally and involves both an upstream production process and downstream purification process. Recombinant viral vectors are rendered incapable of, or attenuated for, replication in human cells by removal of specific viral machinery, including packaging proteins. However, to produce the recombinant virus, these viral proteins have to be re-introduced into the virus production process so that the viral vector can be packaged. In most other viral vector production systems, the missing viral proteins are supplied in one or more individual helper plasmids, along with the base viral vector plasmid. All the plasmids are then co-transfected into a production cell line in the presence of a transfection agent to facilitate viral vector production and packaging. The difficulty of this approach is that it requires c-scale manufacturing and qualification of each of the packaging plasmids and optimization of the transfection method. Even with optimized reagents and methods, other viral vector production systems exhibit significant batch-to-batch variability in viral vector yield and titer that, we believe, drives up the cost of viral vector manufacturing and scale-up and increases the risk of failure during manufacturing.

Our proprietary upstream production process avoids the aforementioned issues. Our process requires three critical components:

- Production of a master virus seed stock (“MVSS”);
- Production of complementing master cell bank (“MCB”); and
- Optimized transduction parameters.

For VYJUVEK and each of our product candidates, we generate a MVSS which is scaled up from a single purified clone of the modified HSV-1 vector expressing the therapeutic effector. The MCB is a complementing cell line that stably expresses the HSV-1 viral proteins that are required for HSV-1 growth but have been deleted from the recombinant HSV-1 backbone. By introducing the deleted proteins into the MCB, as opposed to including them in the viral replication process via co-transfection of individual plasmids, we eliminate the need for multiple qualifications of the plasmids or variability in transfection efficiency from batch to batch, that other production processes face. Infection of the MCB with the MVSS at the optimal concentration results in production of the viral particle. Once the MCB, the MVSS, and the conditions of infection are established, virus production and resultant yield and titer are highly reproducible and scalable over multiple runs, and the risk of failure is minimal.

Optimization of MCB, MVSS and production methods requires extensive knowledge and technical experience with the HSV-1 genome and significant upfront effort to design and select the best virus seed stock and complementing cell line. We have screened hundreds of cell line clones to find the best complementing cell lines and designed and generated the optimal virus seed stocks for VYJUVEK and each of our product candidates. The viral seed stock expresses the therapeutic proteins under the control of strong constitutive or tissue-specific promoters and additional non-coding regulatory sequences have been included to optimize gene expression. We also have optimized the transduction conditions to reproducibly obtain high yields of the virus.

Unlike the upstream process, steps used to purify and concentrate the viral vector product are often common across different viral vector platforms and usually involve multiple stages of purification, clarification, concentration, and diafiltration, with the ultimate goal to remove contaminants and concentrate the product. We have developed a robust and reproducible

process for purifying our viral vector to required concentrations for commercial and clinical use, while successfully removing contaminants to meet FDA and other regulatory guidelines.

We believe that the MVSS and MCB are a vital part of the production of VYJUVEK and our product candidates, as they ensure the reproducible production of multiple commercial and clinical batches in a short six-week cycle time frame and in a cost-effective manner.

We have made significant investments in developing the most comprehensive and optimized manufacturing process for VYJUVEK and our product candidates, including:

- A proprietary vector manufacturing technique and a series of high-efficiency purification processes that produce highly purified therapeutic vectors and can be adapted for each product candidate; and
- A critical list of CGMP assays to accurately characterize our process and the HSV-1-based vectors we produce.

Competition

The biotechnology and pharmaceutical industries are highly competitive. In particular, the field of gene therapy is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. Some of our competitors have substantially greater financial resources and larger research and development organizations. In addition, our experience in clinical trials, obtaining FDA and other regulatory approvals, and manufacturing and commercialization of products may be more limited.

VYJUVEK / B-VEC

Dystrophic Epidermolysis Bullosa

A number of companies are developing or commercializing drug candidates for the treatment of DEB. VYJUVEK is the first corrective therapy for DEB approved worldwide. We believe our competitors fall into two broad categories:

- **Corrective Approaches:** We are aware of companies, Abeona Therapeutics Inc. and Castle Creek Biosciences, Inc., which are developing or have commercialized autologous or grafting gene therapy approaches to treating DEB. Abeona Therapeutics Inc.'s autologous gene therapy product Zevaskyn® (prademagene zamikeracel) was approved by the FDA in 2025. We are also aware of a recombinant-protein-based approach being developed by BridgeBio Pharma, Inc.'s affiliate company, Phoenix Tissue Repair.
- **Palliative Treatments:** We are aware of companies, such as Chiesi Farmaceutici S.p.A. and RHEACELL GmbH & Co., which are developing or have commercialized products taking a palliative approach to treating the disease. Chiesi Farmaceutici S.p.A.'s palliative treatment Filsuvez® (birch triterpenes) was approved by the FDA in 2023 and was previously approved by the EMA.

Respiratory

Cystic Fibrosis

There are no approved therapies for CF patients ineligible or intolerant to modulator regiments. We are aware of several preclinical or early clinical stage nucleic-acid-based programs for treatment of this patient population including programs at Vertex Pharmaceuticals Inc., ReCode Therapeutics, Inc., Spirovant Sciences, Inc., and 4D Molecular Therapeutics, Inc.

Alpha-1 Antitrypsin Deficiency

Currently approved treatments for AATD consist of IV administered AAT augmentation therapy, administered weekly. We are aware of at least three companies marketing augmentation therapies globally: CSL Limited, Takeda Pharmaceutical Company Limited., and Grifols, S.A. We are also aware of several preclinical or clinical stage programs in development for the treatment of various clinical manifestations of AATD. These can be generally classified into four broad categories:

- **Gene Silencing Approaches:** We are aware of two companies, Takeda Pharmaceutical Company Limited (in partnership with Arrowhead Pharmaceutical Inc.) and Novo Nordisk A/S (in partnership with Alnylam Pharmaceuticals, Inc.), which are developing interfering RNA medicines to treat the liver manifestations of AATD.
- **Alternate Augmentation Approaches:** We are aware of companies, such as Kamada Ltd. and Sanofi S.A., which are developing new augmentation treatments with modified frequency or routes of administration to treat the lung manifestations of AATD.

- **Direct Protease Inhibition:** We are aware of companies, such as Peak Bio, Inc. and Mereo BioPharma Group plc, which are developing protease inhibitors to treat the lung manifestations of AATD.
- **Gene Editing Approaches:** We are aware of companies, such as Wave Life Sciences Ltd. and Beam Therapeutics Inc., which are developing gene editing therapies inhibitors to treat both the lung and liver manifestations of AATD.

Ophthalmology

Ocular Complications of DEB

There are no approved therapies for ocular complications secondary to DEB at this time. We are aware of Eliksa Therapeutics' program evaluating amniotic fluid derived ELK-003 eye drops to treat corneal abrasions in individuals with epidermolysis bullosa.

Neurotrophic Keratitis

We are aware of companies, such as Dompé farmaceutici S.p.A., which are developing or have commercialized product candidates for the treatment of NK. Dompé farmaceutici S.p.A,'s Oxervate® (cenegermin) was approved by the FDA in 2018 and by the EMA in 2017.

Dermatology

Hailey-Hailey Disease

There are no approved therapies for HHD at this time. We are aware of an investigator-initiated study evaluating Johnson & Johnson's Tremfya® (guselkumab) for the treatment of HHD, and a previously completed investigator initiated study evaluating botulinum toxin for the treatment of HHD.

Oncology

Solid Tumors

A large number of companies are focused on the development and commercialization of new therapeutics for the treatment of locally advanced or metastatic tumors. We are aware of multiple programs across all stages of development as well as marketed products aiming to improve outcomes for patients with solid tumor malignancies. Some of the most established companies in the marketing and development of new cancer drugs include Merck & Co Inc., Bristol Myers Squibb Company, Johnson & Johnson, and Pfizer Inc.

Aesthetics

Aesthetic Skin Conditions

There are multiple approved therapies for aesthetic skin conditions, including hyaluronic acid and botulinum toxin based products marketed by AbbVie Inc., Revance Therapeutics, Inc., Merz Pharma GmbH & Co., KGaA, Galderma S.A., and others. We are also aware of multiple preclinical and clinical stage programs for improvement in aesthetic skin conditions, including those underway at AbbVie Inc. and Galderma S.A.

Intellectual Property and Proprietary Rights

Our success depends in part upon our ability to obtain and maintain exclusivity for our approved product, product candidates and platform technology. We typically rely on a combination of patent protection and regulatory exclusivity to maintain exclusivity for our approved product and product candidates, whereas exclusivity for our platform technology is generally based on patent protection and trade secret protection. However, trade secrets can be difficult to protect. In addition to patent protection, regulatory exclusivity, and trade secret protection, we also protect our approved product, product candidates and platform technology with trademarks and contractual protections. Additionally, we seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and intellectual property assignment agreements with our employees, consultants and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets, and know-how, including by implementing measures intended to maintain the physical and electronic security of our research and manufacturing facilities, as well as our information technology systems.

We actively seek patent protection for our product candidates and certain of our proprietary technologies by filing patent applications in the United States and other countries as appropriate. These patent applications are directed to various inventions. We do not have patents or patent applications in every jurisdiction where there is a potential commercial market for

our approved product or our product candidates. For each of our programs, our decision to seek patent protection in specific foreign markets, in addition to the United States, is based on many factors, including:

- our available resources;
- the number and types of patents already filed or pending;
- the likelihood of success of the product candidate;
- the size of the commercial market;
- the presence of a potential competitor in the market; and
- whether the legal authorities in the market effectively enforce patent rights.

We continually evaluate our patent portfolio and patent strategy and believe our patents and patent applications provide us with a competitive advantage; however, if markets where we do not have patents or patent applications become commercially important, our business may be adversely affected. A discussion of certain risks and uncertainties that may affect our freedom to operate, patent position, regulatory exclusivities, and other proprietary rights is set forth in Item 1A. Risk Factors included in this Annual Report on Form 10-K.

Certain of our product candidates are in therapeutic areas that have been the subject of many years of extensive research and development by academic organizations and third parties who may control patents or other intellectual property that they might assert against us, should one or more of our product candidates in these therapeutic areas succeed in obtaining regulatory approval and thereafter be commercialized. We continually evaluate the intellectual property rights of others in these areas in order to determine whether a claim of infringement may be made by others against us. Should we determine that a third party has intellectual property rights that could impact our ability to freely market a product candidate, we consider a number of factors in determining how best to prepare for the commercialization of any such product candidate. In making this determination we consider, among other things, the stage of development of our product candidate, the anticipated date of first regulatory approval, whether we believe the intellectual property rights of others are valid, whether we believe we infringe the intellectual property rights of others, whether a license is available upon commercially reasonable terms, whether we will seek to challenge the intellectual property rights of others, the term of the rights, and the likelihood of and liability resulting from an adverse outcome should we be found to infringe the intellectual property rights of others.

Currently, United States patents, as well as most foreign patents, are generally effective for 20 years from the date the earliest regular application was filed. In some countries, the patent term may be extended to recapture a portion of the term lost during regulatory review of the product candidate. For example, in the United States, under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly known as the Hatch-Waxman Act, a patent that covers an FDA-approved biologic may be eligible for patent term extension (for up to 5 years, but not beyond a total of 14 years from the date of product approval) as compensation for patent term lost during the FDA regulatory review process. The application for the extension must be submitted prior to the expiration of the patent and only one patent may be extended for any product based on FDA review delay. The United States Patent and Trademark Office ("USPTO"), in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In addition to patent term extension under the Hatch-Waxman Act, patents in the United States may be granted additional term due to delays at the USPTO during prosecution of a patent application. We actively strive to maximize the potential for patent protection for our product and product candidates in accordance with the law.

Patents

Our technology platform, VYJUVEK, and our product candidates are primarily protected by composition of matter and methods of use patents and patent applications. A summary of granted composition of matter and/or methods of use patents that we own, which cover our technology platform, VYJUVEK, and our product candidates in the United States and elsewhere, is provided below.

Our Technology Platform

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 10,441,614	United States	**Composition of Matter & Methods of Use** – Delivery platform for targeted therapeutics, as well as methods of its use for delivering any effector to the skin	12/28/2036	Krystal
U.S. 11,185,564	United States	**Methods of Use** – Methods of use of replication-defective HSV vectors for delivering any effector to skin-targeted therapeutics	12/28/2036	Krystal

U.S. 11,865,148	United States	**Methods of Use** – Methods of use of replication-defective HSV-1 for delivering any effector to the eye	12/28/2036	Krystal
JP 7,480,105	Japan	**Composition of Matter & Uses Thereof** – Delivery platform for targeted therapeutics, as well as uses thereof, including for delivery of any effector to the skin	12/28/2036	Krystal
AU 2019280069	Australia	**Composition of Matter & Methods of Use** – Delivery platform for targeted therapeutics, as well as methods of its use for delivering any effector to the skin	12/28/2036	Krystal
AU 2022204729	Australia	**Methods of Use** – Methods of use of replication-defective HSV vectors for delivering any effector to skin-targeted therapeutics	12/28/2036	Krystal
AU 2025200334	Australia	**Composition of Matter & Methods of Use** – Delivery platform for targeted therapeutics, as well as methods of its use for delivering any effector to the skin	12/28/2036	Krystal
NZ 778381	New Zealand	**Composition of Matter & Uses Thereof** – Delivery platform for targeted therapeutics, as well as uses thereof, including for delivery of any effector to the skin	12/28/2036	Krystal
NZ 783621	New Zealand	**Composition of Matter & Uses Thereof** – Delivery platform for targeted therapeutics, as well as uses thereof, including for delivery of any effector to the skin	12/28/2036	Krystal

VYJUVEK / B-VEC

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 9,877,990	United States	**Composition of Matter & Methods of Use** – Compositions comprising HSV vectors encoding certain effectors, including the effector encoded in B-VEC, and methods of using the same for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
U.S. 10,155,016	United States	**Composition of Matter** – Covers compositions containing B-VEC, formulated for alternate routes of administration	12/28/2036	Krystal
EP 3,377,637	Europe	**Composition of Matter** – Pharmaceutical compositions comprising B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
JP 6,970,086	Japan	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
AU 2016401692	Australia	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
CL 69.593	Chile	**Composition of Matter & Uses Thereof** – Compositions comprising replication-defective HSV vectors encoding certain effectors, including the effector encoded in B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
CL 70.567	Chile	**Composition of Matter & Uses Thereof** – Compositions comprising HSV vectors encoding certain effectors, including the effector encoded in B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
IN 498868	India	**Composition of Matter** – Compositions comprising replication-defective HSV vectors encoding certain effectors, including the effector encoded in B-VEC	12/28/2036	Krystal
MX 394867	Mexico	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
NZ 746213	New Zealand	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal
SG 11201808314Q	Singapore	**Composition of Matter & Uses Thereof** – Compositions comprising replication-defective HSV vectors encoding certain effectors, including the effector encoded in B-VEC, as well as uses thereof, including for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin	12/28/2036	Krystal

Respiratory

KB407

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 10,829,529	United States	**Methods of Use** – Methods of using herpes virus vectors encoding CFTR for the treatment of cystic fibrosis and other disease causing progressive	2/7/2040	Krystal
U.S. 12,522,636	United States	**Composition of Matter** – Pharmaceutical compositions comprising replication-defective HSV-1 vectors encoding CFTR	2/7/2040	Krystal
AU 2020219343	Australia	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising herpes virus vectors encoding CFTR, as well as uses thereof, including for the treatment of cystic fibrosis and other disease causing progressive lung destruction	2/7/2040	Krystal
CO 44406	Colombia	**Composition of Matter** – Pharmaceutical compositions comprising replication-defective HSV-1 vectors encoding CFTR	2/7/2040	Krystal
NZ 778665	New Zealand	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising herpes virus vectors encoding CFTR, as well as uses thereof, including for the treatment of cystic fibrosis and other disease causing progressive lung destruction	2/7/2040	Krystal
ZA 2022/05420	South Africa	**Methods of Use** – Methods of using herpes virus vectors encoding CFTR for the treatment of cystic fibrosis and other disease causing progressive	2/7/2040	Krystal

KB408

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
JP 7,773,468	Japan	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising HSV vectors encoding certain effectors, including the effector encoded in KB408, and methods of using the same for treating a disease affecting the airways and/or lungs	51488	Krystal

Oncology

KB707

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 11,779,660	United States	**Composition of Matter** – Pharmaceutical compositions comprising HSV vectors encoding IL-2 and IL-12	4/14/2042	Krystal
U.S. 11,918,660	United States	**Composition of Matter** – Pharmaceutical compositions comprising HSV-1 vectors encoding IL-2 and IL-12	4/14/2042	Krystal
U.S. 12,364,775	United States	**Methods of Use** - Methods of use of HSV vectors encoding IL-2 and IL-12 for providing therapeutic relief of one or more signs or symptoms of lung cancer	4/1/2042	Krystal

ZA 2025/02822	South Africa	**Composition of Matter** – Pharmaceutical compositions comprising HSV vectors encoding IL-2 and IL-12	4/1/2042	Krystal
ZA 2025/02821	South Africa	**Composition of Matter** – Pharmaceutical compositions comprising HSV-1 vectors encoding IL-2 and IL-12	4/1/2042	Krystal

Dermatology

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 10,525,090	United States	C**omposition of Matter & Methods of Use** – Pharmaceutical compositions comprising herpes virus vectors encoding TGM1, as well as methods of providing prophylactic, palliative, or therapeutic relief to TGM1-deficient ARCI	4/11/2039	Krystal
U.S. 11,717,547	United States	C**omposition of Matter & Methods of Use** – Pharmaceutical compositions comprising replication-defective HSV-1 vectors encoding TGM, as well as methods of delivering TGM to cells	4/11/2039	Krystal
AU 2019252658	Australia	C**omposition of Matter & Methods of Use** – Pharmaceutical compositions comprising herpes virus vectors encoding TGM1, as well as methods of providing prophylactic, palliative, or therapeutic relief to TGM1-deficient ARCI	4/11/2039	Krystal
AU 2023222939	Australia	**Composition of Matter & Methods of Use** – Pharmaceutical compositions comprising replication-defective HSV-1 vectors encoding TGM, as well as methods of delivering TGM to cells	4/11/2039	Krystal
U.S. 11,642,384	United States	**Composition of Matter** – Pharmaceutical compositions comprising eplication-defective HSV vectors encoding SPINK5	9/24/2039	Krystal
JP 7,562,515	Japan	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising herpes virus vectors encoding SPINK, as well as uses thereof, including for providing prophylactic, palliative, or therapeutic relief of one or more signs or symptoms of Netherton Syndrome and/or atopic dermatitis	9/24/2039	Krystal
AU 2019346549	Australia	**Compositions of Matter** - Pharmaceutical compositions comprising replication-defective HSV vectors encoding SPINK5	9/24/2039	Krystal
JP 7,560,449	Japan	**Composition of Matter & Uses Thereof** – Herpes virus vectors and pharmaceutical compositions encoding laminin, as well as uses thereof, including for treating one or more signs or symptoms of Junctional Epidermolysis Bullosa	9/25/2039	Krystal

Antibody

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
JP 7,749,055	Japan	**Composition of Matter & Uses Thereof** – Pharmaceutical compositions comprising replication-defective HSV vectors encoding one or more antibodies, and methods of using the same for treating a disease	6/28/2039	Krystal

Aesthetics

KB301

Patent Number	Country / Region*	Patent Type	Expiration Date **	Owner
U.S. 10,786,438	United States	**Composition of Matter & Methods of Use** – Pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as methods of their use for improving skin condition, quality, and/or appearance	4/26/2039	Krystal
U.S. 12,128,122	United States	**Composition of Matter & Methods of Use** – Compositions comprising replication-defective HSV-1 vectors encoding one or more cosmetic proteins, as well as methods of their use for improving skin condition, quality, and/or appearance, and for treating one or more signs or symptoms of dermatological aging	4/26/2039	Krystal
JP 7,602,999	Japan	**Compositions of Matter & Uses Thereof** – Cosmetic compositions comprising herpes virus vectors encoding one or more cosmetic proteins, as well as uses thereof, including for improving skin condition, quality, and/or appearance	4/26/2039	Krystal
AU 2019260757	Australia	**Composition of Matter & Methods of Use** – Pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as methods of their use for improving skin condition, quality, and/or appearance	4/26/2039	Krystal
NZ 769822	New Zealand	**Composition of Matter** –Compositions comprising HSV vectors encoding one or more cosmetic proteins	4/26/2039	Krystal
ZA 2023/06237	South Africa	**Composition of Matter & Uses thereof** – Pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as uses thereof, including for improving skin condition, quality, and/or appearance	4/26/2039	Krystal
ZA 2025/01799	South Africa	**Composition of Matter & Uses thereof** – Pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as uses thereof, including for improving skin condition, quality, and/or appearance	4/26/2039	Krystal

* Granted patents in the U.S. and elsewhere are shown. Additional patent protection in the United States, Europe or other countries or regions through pending or granted counterparts may be available.

** Stated expiration dates do not account for any patent term extension, supplemental protection certificate, or pediatric extensions that may be available.

Regulatory Exclusivity

The various types of regulatory exclusivity or designations for which VYJUVEK and our product candidates have been granted, or which our current or future product candidates may be eligible to receive are generally discussed above or below, under "Government Regulation and Product Approval".

Trademarks

Our trademarks are important to us and are generally filed to protect our corporate brand, our approved product, our product candidates, and our platform technology. We typically file trademark applications and pursue their registration in the United States, Europe and other markets in which we anticipate using such trademarks. We are the owner of several federal trademark registrations in the United States and have pending trademark applications and registrations in the United States and in major foreign markets. Trademark protection varies in accordance with local law and continues in some countries as long as the trademark is used and in other countries as long as the trademark is registered. Trademark registrations generally are for fixed but renewable terms.

Government Regulation and Product Approval

In the United States, the FDA regulates biologic products including gene therapy products under the Federal Food, Drug, and Cosmetic Act (the "FDCA"), the Public Health Service Act ("PHSA"), and regulations and guidance implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, importation, advertising and other promotional practices involving biologic products. Investigational new drug, or IND, applications to the FDA are required before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA, and in limited instances the National Institutes of Health (the "NIH"), through its Recombinant DNA Advisory Committee, or RAC. The FDA's authorization also must be obtained before marketing of biologic products. The process of obtaining regulatory approvals or licenses and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources, and we may not be able to obtain the required regulatory approvals to successfully develop and commercialize our product candidates.

Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within CBER, the review of gene therapy and related products is in the Office of Therapeutic Products ("OTP") and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. CBER works closely with the NIH and the RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA has provided guidance for the development of gene therapy products generally, including a growing body of guidance documents on CMC, clinical investigations, and other areas of gene therapy development, all of which are intended to facilitate the industry's development of gene therapy products.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

United States Biologic Products Development Process

The FDA must authorize the marketing of a product candidate in the United States. The process required by the FDA before a biologic product candidate may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and in vivo studies in accordance with the FDA's Current Good Laboratory Practice ("CGLP"), regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND application, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by each clinical trial site's Institutional Review Board ("IRB") and, if applicable, Institutional Biosafety Committee ("IBC"), before the clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's Current Good Clinical Practice ("CGCP") regulations and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product candidate for its intended use;
- preparation and submission to the FDA of an application for marketing approval that includes substantial evidence of safety, purity and potency from results of nonclinical testing and clinical trials;
- review of the product by an FDA advisory committee, if applicable;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with CGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality, potency and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the application; and
- payment of user fees and FDA review and marketing authorization.

Before testing any new biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as in vivo studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. The conduct of the preclinical tests must comply with federal regulations and requirements including CGLPs.

Concurrent with clinical trials, companies usually must complete some long-term preclinical testing, such as animal studies of reproductive adverse events and carcinogenicity and must also develop additional information about the chemistry and physical characteristics of the biological product and finalize a process for manufacturing the biological product in commercial quantities in accordance with CGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the biological product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final biological product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted, to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of an IND application. Some preclinical testing may continue even after the IND application is submitted. With gene therapy protocols, if the FDA allows the IND application to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND application review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND application for our product candidates will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Human Clinical Trials Under an IND

Clinical trials involve the administration of the biologic product candidate to healthy volunteers or patients under the supervision of qualified investigators who generally are physicians not employed by or under the control of the trial sponsor. Clinical trials are conducted under written study 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, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND application. An IND application becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold, including concerns that human research subjects will be exposed to unreasonable health risks. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Accordingly, submission of an IND application may or may not result in the FDA allowing clinical trials to commence.

Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising CGCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an IRB and, if applicable, IBC 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 items such 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 form and content of the informed consent that must be signed by each clinical trial subject, or their legal representative, reviews and approves the study protocol, and must monitor the clinical trial until completed. Clinical trials involving recombinant DNA at certain institutions also must be reviewed by an IBC, a local institutional committee that reviews and oversees basic and clinical research that utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials typically are conducted in three sequential phases that may overlap or be combined:

- Phase 1. The biologic product candidate initially is introduced into a small number of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients. Phase 1 clinical trials of gene therapies are typically conducted in patients rather than healthy volunteers.
- Phase 2. The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product candidate for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- Phase 3. Phase 3 clinical trials are commonly referred to as "pivotal" studies, which typically denotes studies that present the data the FDA or other relevant regulatory agencies will use to determine whether or not to approve a biologic product. In Phase 3 studies, the biologic product candidate is administered to an expanded patient population, generally at multiple geographically dispersed clinical trial sites in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.
- Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

Additional Regulation for Gene Therapy Clinical Trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND application or Biologics License Application ("BLA"); and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

United States Review and Approval Processes

The results of the preclinical tests and clinical trials, together with detailed information relating to the product's CMC and proposed labeling, among other things, are submitted to the FDA as part of a BLA or other submission requesting authorization to market the product for one or more indications. For gene therapies, selecting patients with applicable genetic defects is a necessary condition to effective treatment. Under the Prescription Drug User Fee Act ("PDUFA"), each BLA (or New Drug Application ("NDA") for some biologics) must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. The PDUFA also imposes an annual product fee for biologics and an annual establishment license fee on facilities used to manufacture prescription biologics. 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. Additionally, no user fees are assessed on BLAs or NDAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before it accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In that event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth, substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and potent, or effective, for its intended use, has an acceptable purity profile and whether the product candidate is being manufactured in accordance with CGMP to assure and preserve the product candidate's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that 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. During the product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy ("REMS") program is necessary to assure the safe use of the product candidate.

REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. 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 a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with CGMP requirements and adequate to assure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA typically will inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with the IND application trial requirements and CGCP requirements.

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter or license authorizes commercial marketing of the biologic product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post-marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biologic product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review standard BLAs in ten months after the FDA accepts the BLA for filing, and priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Fast Track Designation

Fast Track designation is granted to drugs being developed for the treatment of serious or life-threatening diseases or conditions where there is an unmet medical need. The purpose of the Fast Track designation provision is to help facilitate development and expedite the review and potential approval of drugs to treat serious and life-threatening conditions. Sponsors of drugs that receive Fast Track designation have the opportunity for more frequent interactions with the FDA review team throughout the development program. These can include meetings to discuss study design, data required to support approval, or other aspects of the clinical program. Additionally, products that have been granted Fast Track designation may be eligible for priority review of a BLA application and the FDA may consider reviewing portions of the submission before the sponsor submits the complete application, also known as a rolling review.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition, generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was

designated. Competitors, however, may receive approval of different products for the same indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Other benefits include reduced regulatory fees, protocol assistance and tax credits for certain clinical research costs.

Orphan medicinal product status in the EU and Japan have similar, but not identical benefits.

Breakthrough Therapy

A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval.

Regenerative Medicine Advanced Therapy ("RMAT") Designation

Established under the 21st Century Cures Act, RMAT designation is a program designed to expedite the development and approval of regenerative medicine products, including gene therapy products. An investigational therapy is eligible for the RMAT designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates a potential to address unmet medical needs for that disease or condition. The designation includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designations and enables the ability to work more closely and frequently with the FDA to discuss surrogate or intermediate endpoints to support the potential acceleration of approval and satisfy post-approval requirements.

PRIME Designation

The PRIority MEdicines ("PRIME") designation is awarded by the EMA to promising medicines that target an unmet medical need. These medicines are considered priority medicines by the EMA. To be eligible and accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data coupled with non-clinical data. Through PRIME, the EMA offers enhanced support to medicine developers including early interaction and dialogue, and a pathway for accelerated evaluation by the agency. The program is intended to optimize development plans and expedite the review and approval process so that these medicines may reach patients as early as possible.

Rare Pediatric Disease Priority Review Voucher

The FDA also offers a rare pediatric disease drug designation. If a drug receives the designation of a "rare pediatric disease" drug, it is eligible during the FDA marketing process to apply for a Rare Pediatric Disease Priority Review Voucher. According to the FDA website, under the Rare Pediatric Priority Review Voucher Program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

Post-Approval Requirements

Rigorous and extensive FDA regulation of biologic products continues after approval, particularly with respect to CGMP requirements. Manufacturers are required to comply with applicable requirements in the CGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biologic products include reporting of CGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product; recordkeeping requirements; reporting of adverse effects; reporting updated safety and efficacy information; and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA, together with a release protocol, showing a summary of the history of manufacture of the lot and the results of all tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biologic products. A sponsor also must comply with the FDA's advertising and promotion requirements, such as the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label promotion").

Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market, as well as possible civil or criminal sanctions. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented, and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

Government Regulation Outside of the United States

In addition to regulations in the United States, sponsors are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of biologic products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not a sponsor obtains FDA approval for a product, a sponsor must 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 application, prior to the commencement of human clinical trials. In the EU, for example, a request for a Clinical Trial Authorization ("CTA") must be submitted to the competent regulatory authorities and the competent Ethics Committees in the EU Member States in which the clinical trial takes place, much like FDA and the IRB, respectively. Once the CTA request is approved in accordance with the EU and the EU Member State's requirements, clinical trial development may proceed. The requirements and processes governing the conduct of clinical trials, product licensing, pricing and reimbursement in the EU vary from country to country. In all cases, the clinical trials are conducted in accordance with the applicable EU laws and regulatory requirements of the country or countries in which the clinical trial is performed, as well as the International Council for Harmonisation of Technical Requirements of Pharmaceuticals for Human Use's guidelines on good clinical practice and ethical principles that have their origin in the Declaration of Helsinki (whichever provides the greater protection to the clinical trial participants).

Failure to comply with applicable foreign regulatory requirements may result in, among other things, fines; suspension, variation or withdrawal of regulatory approvals; product recalls; seizure of products; operating restrictions; and criminal prosecution.

Other Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and use of pharmaceutical products that are granted marketing approval. Arrangements with third-party payors, existing or potential customers and referral sources are subject to broadly applicable fraud and abuse and other healthcare laws and regulations, and these laws and regulations may constrain the business or financial arrangements and relationships through which manufacturers market, sell and distribute the products for which they obtain marketing approval. Such restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or kind, in exchange for, or to induce, either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers, on the one hand, and prescribers, purchasers and formulary managers on the other. The Patient Protection and Affordable Care Act ("ACA") amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to commit a violation;
- the federal false claims and civil monetary penalties laws, including the civil False Claims Act ("FCA"), which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. Certain marketing practices, including off-label promotion, also may implicate the FCA. In addition, the ACA codified case law 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 FCA;
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services information related to payments and other transfers of value to physicians, certain other healthcare providers and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;
- the federal Health Care Fraud statute imposes criminal and civil liability for executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and its implementing regulations, and as amended again by the final HIPAA omnibus rule (together with HIPAA and HITECH, the "HIPAA

Rules") which imposes privacy, security, and breach obligations, including mandatory contractual terms, with respect to safeguarding the security and privacy of individually identifiable health information by entities subject to the HIPAA Rules, such as health plans, health care clearinghouses, and health care providers that engage in certain covered transactions, as well as their business associates;

- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for federally sponsored healthcare benefits, items or services; and
- state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and data security of personal information and health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Violation of the laws described above or any other governmental laws and regulations may result in penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, the exclusion from participation in federal and state healthcare programs, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities and health programs in the United States such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to incurring the costs required to obtain FDA approvals. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all FDA-approved drugs for a particular indication. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The United States 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. 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.

In the EU, pricing and reimbursement schemes vary widely from member state to member state. While a product may usually be legally placed on the market in the EU once a marketing authorization has been obtained, meaningful market access in many EU member states depends on the completion of national pricing and reimbursement procedures. Some member states may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states may approve a specific price for a product or may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations may not allow favorable reimbursement and pricing arrangements.

Health Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, for example, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected and continues to face major uncertainty due to the status of proposed and enacted legislative initiatives surrounding healthcare reform. On August 16, 2022, the Inflation Reduction Act ("IRA") was signed into law. The IRA includes several provisions to lower prescription drug costs for

people with Medicare and reduce drug spending by the federal government, including allowing Medicare to negotiate prices for certain prescription drugs, requiring drug manufacturers to pay a rebate to the federal government if prices for single-source drugs and biologicals covered under Medicare Part B and nearly all covered drugs under Part D increase faster than the rate of inflation (CPI-U), and limiting out of pocket spending for Medicare Part D enrollees.

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 Conservation and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling and disposal of various biologic, chemical and radioactive substances used in, and wastes generated by, 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. Equivalent laws have been adopted in other countries that impose similar obligations.

United States Foreign Corrupt Practices Act

The United States Foreign Corrupt Practices Act ("FCPA") prohibits United States corporations and individuals from engaging in certain activities to obtain or retain business abroad or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA includes interactions with certain healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

Human Capital

As of February 11, 2026, we had 295 full-time employees, primarily engaged in research and development, pre-clinical and clinical trials, manufacturing VYJUVEK and our pipeline product candidates, commercial activities for VYJUVEK in the United States, the European Union, and Japan, regulatory matters, strategic business development, finance and other technical matters, supply chain, and general and administrative services. None of our employees are represented by a labor union and we consider our employee relations to be good.

We believe our employees are among the most important assets to our company and are key to achieving our goals and expectations. Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, and incentivizing our existing and new employees. We offer robust compensation packages, including competitive base pay, incentive compensation and stock compensation programs, and provide a broad range of benefits. The principal purpose of our stock compensation program is to attract, retain and reward personnel through the granting of stock-based awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives. In addition, we are committed to the professional advancement of our employees and offer various training programs and career development opportunities.

Corporate Information

We commenced operations in April 2016. In March 2017, we converted from a California limited liability company to a Delaware C-corporation, and changed our name from Krystal Biotech LLC to Krystal Biotech, Inc. Our principal offices are located at 2100 Wharton Street, Suite 701, Pittsburgh, PA 15203, and our telephone number is 412-586-5830. In April 2019, we incorporated Jeune Aesthetics, Inc., a Delaware corporation and wholly-owned subsidiary, for the purpose of undertaking preclinical and clinical studies for aesthetic skin conditions. In January 2022, August 2022, December 2022, August 2023, March 2024, November 2024, December 2024 and July 2025 we incorporated wholly-owned subsidiaries in Switzerland, Netherlands, France, Germany, Japan, Italy, Spain, and the UK, respectively, for the purpose of establishing operations in Europe and Japan for the commercialization of VYJUVEK® and our product pipeline. Our website address is www.krystalbio.com. Our website and the information contained on, or that can be accessed through, the website are not incorporated by reference in, and are not considered part of, this Annual Report on Form 10-K. Access to our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and our Proxy Statements, and amendments to these reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Exchange Act are available free of charge on the investor relations section of our website as soon as reasonably practicable after we electronically file such material with, or furnish it to the Securities and Exchange Commission, or the SEC. The SEC also maintains a website that contains reports, proxy and information statements, and other information regarding the Company that we file electronically with the SEC. The address of the website is http://www.sec.gov. We may use our website as a means of disclosing material non-public information and for complying with our disclosure obligations under the Regulation FD. Accordingly, investors should monitor the investor relations section of our website, in addition to following the company's press releases, SEC filings, public conference calls and webcasts, and our social media accounts.

ITEM 1A. RISK FACTORS

Our business involves significant risks, some of which are 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 included in this Annual Report on Form 10-K. If any of the following risks actually occur, it could harm our business, prospects, operating results, 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 on Form 10-K.

Risks Related to Our Business and Industry

We are substantially dependent on the commercial success of VYJUVEK

To date, we have invested substantial efforts and financial resources in the research and development of VYJUVEK and our product candidates. Our near-term prospects, including our ability to develop our product candidates and generate revenue, and our future growth are substantially dependent on the commercial success of VYJUVEK.

Although we received approval from the U.S. Food and Drug Administration, or FDA, the European Commission, and Japan's Ministry of Health, Labour and Welfare, or MHLW, for VYJUVEK for the treatment of dystrophic epidermolysis bullosa, or DEB, we can provide no assurances that we will obtain regulatory approval in any other jurisdiction, which could have an adverse impact on our results of operations. In addition, the successful commercialization of VYJUVEK will depend on a number of factors and involves risk, including some of the risks identified in these "Risk Factors." One or more of these risks, many of which are beyond our control, could cause significant delays or an inability to successfully commercialize VYJUVEK.

We may not be successful in our efforts to identify, develop, and commercialize additional product candidates, which may impair our ability to expand our business and achieve our strategic objectives, and we may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success.

Although a substantial amount of our efforts are focused on the commercialization of VYJUVEK and the development and potential approval of our current product candidates, a key component of our strategy is to identify, develop and potentially commercialize a portfolio of genetic medicines. Research programs to identify new product candidates require substantial technical, financial, and human resources and may not be successful in identifying potential product candidates. Even if we identify product candidates that initially show promise, we may fail to successfully develop and commercialize such product candidates for many reasons, including the following:

- competitors may develop alternatives that render our product candidates obsolete;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth may be impaired.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have commercial potential. Our resource allocation decisions may cause us to fail to capitalize timely on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing, or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may

allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

VYJUVEK and, if approved, our investigational product candidates regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Patient Protection and Affordable Care Act ("ACA") includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 ("BPCIA"), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing product if the FDA approves a Biologics License Application, or BLA, for the competing product containing that company's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of that company's product. In addition, a competitor may choose to challenge our patent rights relating to the reference product by initiating litigation during the 12-year period of exclusivity. After the FDA approves the BLA for the competing product, the competitor may also bring a declaratory judgment action of non-infringement, invalidity, and/or unenforceability of our patent rights. The law is complex and is still being interpreted and implemented by the FDA, and recent FDA policies, described in draft guidance, could lower development burdens and accelerate the timing and volume of biosimilar competition. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

We believe that any of our product candidates approved 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 our investigational genetic medicines to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of our approved 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.

If competitors are able to obtain marketing approval for biosimilars referencing any of our approved products, our approved products may become subject to competition from such biosimilars, which would impair our ability to successfully commercialize and generate revenue from sales of such products.

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize and market our product candidates.

We are aware of several companies and institutions that have developed, or are currently developing, alternative autologous, or palliative gene therapy or other approaches for our targeted indications, including DEB. Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical, and other resources, such as larger research and development, clinical, marketing, and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunities could be reduced or eliminated if competitors commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than VYJUVEK or any product candidate that we may commercialize. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than we may obtain approval for our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render VYJUVEK or any of our product candidates uneconomical or obsolete, and we may not be successful in marketing VYJUVEK or any of our product candidates that obtain regulatory approval against such competitors.

Even if we commercialize a product candidate faster than our competitors, we could also face competition from lower cost biosimilars.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for VYJUVEK or any product candidate that we may develop and commercialize.

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

We face an inherent risk of product liability lawsuits related to the sale of VYJUVEK, use of VYJUVEK and our product candidates, and testing of our product candidates. Product liability claims may be brought against us by participants enrolled in our clinical trials, patients, health care providers, or others using or administering VYJUVEK and our product candidates. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities. Regardless of their merit or eventual outcome, liability claims may result in:

- decreased demand for VYJUVEK or any of our product candidates that are approved for commercial sale;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- increased regulatory scrutiny;
- significant litigation costs;
- substantial monetary awards to, or costly settlement with, claimants;
- product recalls for any approved products or a change in the indications for which they may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to successfully commercialize VYJUVEK or our product candidates, if approved.

With respect to VYJUVEK and any of our product candidates that are approved for commercial sale, we are, and will be, highly dependent upon physician and patient perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity. We could also be adversely affected if any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to patients. Because of our dependence upon consumer perceptions, any adverse publicity could have a material adverse impact on our financial condition or results of operations.

Our product liability insurance coverage may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage when we begin commercialization of our product candidates, if approved. Insurance coverage is becoming increasingly expensive. As a result, we may be unable to maintain or obtain sufficient insurance at a reasonable cost to protect us against losses that could have a material adverse effect on our business. A successful product liability claim, or series of claims brought against us, particularly if judgments exceed any insurance coverage we may have, could decrease our cash resources and adversely affect our business, financial condition, and results of operations.

Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product or product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology. Ethical, social, and legal concerns about gene therapy could result in additional regulations restricting or prohibiting VYJUVEK or our product candidates. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success depends upon physicians who specialize in the treatment of DEB or genetic diseases targeted by our product candidates prescribing VYJUVEK or treatments that involve the use of our product candidates that may be in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive 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 commercialization of VYJUVEK, regulatory approval of our product candidates, or demand for VYJUVEK or any product candidates that are approved for commercial sale. For example, other companies' gene therapies and earlier gene therapy trials using other vectors led to several well-publicized adverse events, including death. Serious adverse events in our clinical trials or other clinical trials involving gene therapy products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates,

stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates.

Our business operations may subject us to disputes, claims and lawsuits, which may be costly and time-consuming and could materially and adversely impact our financial position and results of operations.

From time to time, we may become involved in disputes, claims, and lawsuits relating to our business operations. For example, we may face or initiate claims related to intellectual property matters, employment matters, or commercial matters. Any dispute, claim, or lawsuit may divert management's attention away from our business, result in significant expenses to address or defend such matters, and require us to pay damage awards or settlements or become subject to equitable remedies, any of which could materially and adversely affect our operations and financial results. In addition, the uncertainty associated with litigation could lead to increased volatility in our stock price.

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

Social media is increasingly being used by us, our employees, or others to communicate about our business, VYJUVEK, our clinical development programs, DEB, and the diseases our product candidates are being developed to treat. We use social media in connection with our commercialization efforts of VYJUVEK and intend to use it in connection with our commercialization efforts of our product candidates, if approved. Social media practices in the biotechnology and biopharmaceutical industries continue to evolve, and regulations and regulatory guidance relating to such use are evolving and not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us, along with the potential for litigation and heightened scrutiny by the FDA, the European Medicines Agency, or EMA, the European Commission, the MHLW, the SEC, and other regulators. For example, patients may use social media to comment on their experience in an ongoing clinical trial of our product candidates, or to report an alleged adverse event. If such disclosures occur, there is a risk that clinical trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations, or that 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 product candidates. There is also a risk of inappropriate disclosure of sensitive information, loss of trade secrets or other intellectual property, public exposure of personal information of our employees, patients who use VYJUVEK, clinical trial patients, and others, or negative or inaccurate posts or comments about us on social media. In addition, we may encounter attacks on social media regarding our company, management, VYJUVEK, or our product candidates that seriously damage our reputation, brand image, and goodwill. 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 that could have a material adverse effect on our business, prospects, operating results, and financial condition, and could adversely affect the price of our common stock.

We have experienced significant growth in the number of employees and infrastructure and may experience difficulties in managing this growth. If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

We have experienced a period of significant expansion in personnel and of our facilities, infrastructure and overhead as we developed our own manufacturing facilities, built our sales, marketing, and distribution infrastructure that we believe is necessary to commercialize VYJUVEK, and increased our research and development efforts. The commercialization of VYJUVEK and our ongoing development of other product candidates will continue to impose significant capital requirements, as well as added responsibilities on members of management, including the need to identify, recruit, maintain, and integrate new personnel in the United States and abroad. Our future performance and our ability to compete effectively will depend, in part, on our ability to manage our growth effectively. If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial, and other systems and resources to manage our operations, continue our research and development activities, and build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. Depending on demand, we may need to scale up the manufacturing process for any approved product, which is subject to risks and uncertainties. Future growth would impose significant added responsibilities on members of management. Our management, finance, development personnel, and systems (including infrastructure such as IT and facilities) currently in place may not be adequate to support this expected future growth. Our need to effectively manage our operations, growth, and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain enough numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development, and growth goals.

Our future success depends on our ability to retain key employees and scientific advisors and to attract, retain, and motivate qualified personnel.

We are highly dependent on members of our management team, the loss of whose services may adversely impact the achievement of our objectives. Our employees and scientific advisors are at-will employees and consultants, and the loss of one or more of them might impede the achievement of our research, development, and commercialization objectives.

Recruiting and retaining qualified employees and scientific advisors for our business, including scientific and technical personnel, also will be critical to our success. Competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, or advisors, may impede the progress of our research, development, and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our employees, principal investigators and advisors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, and advisors. Misconduct by these parties could include intentional failures to comply with FDA regulations, regulations applicable in the EU, Japan, and other jurisdictions; to provide accurate information to the FDA, the European Commission, the EMA, the MHLW, and other regulatory authorities; to comply with healthcare fraud and abuse laws and regulations in the United States and abroad; to report financial information or data accurately; or to disclose unauthorized activities. Sales, marketing, and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing, and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or 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, financial condition, results of operations, and prospects, including the imposition of significant fines, criminal penalties, or other sanctions.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or regulators in other jurisdictions if we conduct clinical trials outside of the United States. The FDA or other applicable regulators may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the clinical trial data. The FDA or other applicable regulators may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or foreign regulators and may ultimately lead to the denial of marketing approval of our current and future product candidates.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell VYJUVEK and any product candidates for which we obtain marketing approval.

In the United States, there have been and continue to be a number of legislative efforts to contain healthcare costs. Any legislative changes that result in price controls, reduce access to and reimbursement for care, or add additional regulations may have an adverse effect on our financial condition and results of operations. Any changes that reduce, or impede the ability to obtain, reimbursement for VYJUVEK or our product candidates that we intend to commercialize in the United States could adversely affect successful commercialization of VYJUVEK and our plans to introduce our product candidates in the United States.

In August 2022, the Inflation Reduction Act ("IRA") was signed into law. The IRA includes several provisions to lower prescription drug costs for people with Medicare and reduce drug spending by the federal government. In relevant part, the IRA allows Medicare to negotiate prices for certain prescription drugs, requires drug manufacturers to pay a rebate to the federal government if prices for single-source drugs and biologicals covered under Medicare Part B and nearly all covered drugs under Part D increase faster than the rate of inflation, caps out of pocket spending for Medicare Part D enrollees, and makes other

benefit design changes to Medicare Part D intended to lower drug costs for enrollees and Medicare. Implementation of these changes began in 2023 and will continue to be implemented over the next several years. Beginning January 1, 2025, Medicare Part D enrollees now have a new annual out-of-pocket cap of $2,000 on prescription drugs. Other 2025 Medicare Part D changes include the elimination of the coverage gap phase and the replacement of the Coverage Gap Discount Program with the Manufacturer Discount Program, under which drug manufacturers are required provide a 10% discount for brand-name drugs and biologics during the initial coverage phase and a 20% discount during the catastrophic phase. Multiple pharmaceutical manufacturers have challenged the law in court, largely on constitutional grounds. To date, the majority of these challenges have been unsuccessful, with courts upholding the IRA. However, these suits will likely continue and the ultimate effects of such legal challenges are unclear. On April 15, 2025, President Trump issued an executive order directing the Secretary of the Department of Health and Human Services to take certain actions on drug pricing reform, including working with Congress on amendments to the IRA and rule making to establish new Medicare payment models for so-called "high-cost" prescription drugs and biological products. Subsequently, President Trump has issued additional executive orders directing additional actions designed to lower drug prices, including by tying the price of certain drugs to the price of those drugs in other countries. It is unclear whether the Trump administration executive orders will lead to actual legislative initiatives. At this time, we continue to evaluate the effect of the IRA and the Trump administration's executive actions on our business operations and financial condition and results as the full impact of the IRA and the executive actions remains uncertain.

On July 4, 2025, legislation commonly referred to as the One Big Beautiful Bill Act was signed into law, which reduces funding to federal healthcare programs and imposes additional requirements to be eligible for healthcare, which may result in decreased access to healthcare, particularly in Medicaid programs.

Further, there has been heightened governmental scrutiny in recent years over the manner in which manufacturers set prices for their marketed products and the cost of prescription drugs to consumers and government healthcare programs, which have resulted in several recent Congressional inquiries and proposed and enacted bills designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the United States 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 for branded prescription drugs to limit the growth of government paid health care costs. For example, the United States government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. Individual states in the United States have also increasingly enacted legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, required discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing.

Additional changes may affect our business, including those governing enrollment in federal healthcare programs, reimbursement changes, fraud and abuse enforcement, and expansion of new programs, such as Medicare payment for performance initiatives. Healthcare reform measures that may be adopted could result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. 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 could result in reduced demand for our product and product candidates that are approved for sale or additional pricing pressures and may adversely impact our ability to generate sufficient revenue, attain consistent profitability, or commercialize our product candidates, if approved.

We are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

With the FDA approval of VYJUVEK, our operations are directly, or indirectly through our prescribers, customers, and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, federal civil and criminal false claims laws, and the Physician Payments Sunshine Act and regulations. These laws impact, among other things, our sales, marketing, access assistance, sponsored genetic patient testing, and educational programs. In addition, we are subject to privacy and data security laws concerning personal information and health information imposed by both the federal government and the states in which we conduct our business, as well as by foreign jurisdictions. The laws that affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering, or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing, or furnishing of an item or service reimbursable under a federal healthcare

program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers, and formulary managers on the other. The ACA amended the intent requirement of the federal Anti-Kickback Statute to clarify that a person or entity does not have to have actual knowledge of this statute or specific intent to violate it;

- federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other government payors that are false or fraudulent. The ACA provides that a claim for items or services resulting from an Anti-Kickback Statute violation is a false claim under the False Claims Act ("FCA"). Cases against pharmaceutical manufacturers support the view that certain marketing practices, including off-label promotion, may implicate the FCA;
- the federal Health Care Fraud statute imposes criminal and civil liability for executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Health Insurance Portability and Accountability Act of 1996 Rules ("HIPAA Rules"), which impose certain requirements relating to the privacy, security, and transmission of individually identifiable health information by certain entities subject to the HIPAA Rules, such as health plans, health care clearinghouses, and health care providers that engage in certain covered transactions, known as covered entities, as well as their business associates that perform certain services that involve the use or disclosure of individually identifiable health information for or on behalf of covered entities;
- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the United States Centers for Medicare and Medicaid Services ("CMS") information related to: (i) payments or other "transfers of value" made to physicians and teaching hospitals, and (ii) ownership and investment interests held by physicians and their immediate family members;
- our operations in Europe and Japan may be directly or indirectly subject to European and Japanese law equivalents of each of the above U.S. federal laws, some of which may not have been applicable prior to the recent European Commission and MHLW approvals and commercial launch of VYJUVEK in Europe and Japan;
- U.S. state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and
- state and foreign laws governing data privacy and security of health information, many of which differ from each other and require attention to frequently changing regulatory requirements, thus complicating compliance efforts in certain circumstances and increasing exposure to liability.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Often, to avoid the threat of treble damages and penalties under the FCA, health care providers and drug manufacturers will resolve allegations in a settlement without admitting liability. Any such settlement could materially affect our business, financial operations, and reputation.

Efforts to ensure that our business arrangements with third parties comply with applicable healthcare laws and regulations involve substantial costs. 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.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against such action, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain a robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that we may run afoul of one or more of the requirements.

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 generation, handling, use, storage, treatment, manufacture, transportation, and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic materials. Our operations also 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. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Moreover, certain environmental laws may impose liability without regard to fault or legality of the action at the time of its occurrence. We also could incur significant costs associated with civil or criminal fines and penalties. We do not carry specific biological or hazardous waste insurance coverage. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount that could have a material adverse effect on our business, financial condition, results of operations, and prospects, and our clinical trials or regulatory approvals could be suspended.

Although we maintain workers' compensation insurance for certain costs and expenses that 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 also may incur substantial costs to comply with current or future environmental, health, and safety laws and regulations, which have tended to become more stringent over time. 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 or liabilities, which could materially adversely affect our business, financial condition, results of operations, and prospects.

We are subject to stringent and evolving U.S. and foreign laws, regulations, and other obligations related to privacy and data security. Our actual or perceived failure to comply with such obligations could lead to regulatory inquiries or actions, litigation, fines and penalties, disruptions to our business operations, reputational harm, loss of revenue, and other adverse business consequences.

Privacy and data security have become significant areas of legal and regulatory focus in the United States, European Union, Japan, and in many other jurisdictions where we conduct or may conduct our operations. In our ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, "process") personal information and other sensitive information, including, but not limited to, health information, individuals' financial information, as well as proprietary and confidential business data, including trade secrets, intellectual property, and sensitive third-party data (collectively, "sensitive data"). The legislative and regulatory landscape for privacy and data security continues to evolve, and there has been an increasing focus on privacy and data security issues, which may affect our business and is expected to increase our compliance costs and exposure to liability. Our data processing activities may subject us to numerous privacy and data security obligations, including, but not limited to, domestic and international laws, regulations, guidance, industry standards, external and internal privacy and security policies, and contractual requirements.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal information privacy laws, consumer protection laws, and other similar laws. Notably, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes requirements on covered entities, as well as their business associates regarding the privacy, security, and transmission of individually identifiable health information.

Further, states continue to adopt new laws or amend existing laws related to privacy and data security, requiring attention to frequently changing regulatory requirements. For example, the California Consumer Privacy Act of 2018 ("CCPA") requires businesses to provide specific disclosures in their privacy notices and honor residents' privacy rights. The CCPA provides for civil penalties of up to $7,500 per violation and allows private litigants affected by certain data breaches to recover

significant statutory damages. Although the CCPA does not apply to certain data that we process in the context of clinical trials, efforts to comply with the CCPA may increase our annual compliance costs and subject us to potential liability with respect to other personal information we may maintain about California residents. In addition, the California Privacy Rights Act of 2020 ("CPRA"), which came into effect on January 1, 2023, expanded the CCPA's requirements, extending it to cover personal information of business representatives and employees and the CPRA established a new regulatory agency to implement and enforce the law. Other states, such as Virginia, Nevada, Connecticut, Utah, Texas, Colorado, Oregon, Montana, Iowa, Indiana, Tennessee, Delaware, and New Jersey, have enacted comprehensive privacy laws that have taken effect or are scheduled to take effect, and similar laws are being considered in several other states, as well as at the federal and local levels, which impose similar obligations to those in the CCPA. Further, other states, such as Nevada and Washington, have enacted privacy laws specifically governing consumer health information, with Washington providing for a private right of action, and additional states are considering similar consumer health information-related laws. Although many of these laws currently exempt certain health-related information, the laws may increase our potential liability related to our data processing activities, complicate our compliance efforts, and increase both legal risk and compliance costs for us and the third parties upon whom we rely.

Outside of the United States, there are an increasing number of laws, regulations, and industry standards regarding privacy and data security. For example, the EU General Data Protection Regulation ("GDPR"), the Japanese Act on the Protection of Personal Information, and UK GDPR impose strict requirements on processing personal information. Companies that violate the GDPR may face temporary or permanent bans on certain data processing activities and may be subject to significant penalties, including fines of up to 20 million Euros under the EU GDPR or 17.5 million pounds sterling under the UK GDPR, or 4% of annual global revenue, whichever is greater. In addition, companies may be subject to private litigation relating to processing of personal information, including actions brought by brought by classes of data subjects or consumer protection organizations authorized to represent data subjects' interests.

In some circumstances, we may be unable to transfer personal information between certain jurisdictions due to data localization requirements or other limitations on cross-border data flows. The European Economic Area ("EEA"), the UK, Japan, and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal information to other countries. In particular, the EEA and the UK have significantly restricted the transfer of personal information to the United States and other countries whose privacy laws they consider inadequate. Although there are various mechanisms that may be used to transfer personal information from the EEA and UK to the United States in compliance with the law, such as the EEA and UK's standard contractual clauses, and other approved data transfer mechanisms, these mechanisms are subject to legal challenges, and we may be unable to rely on these measures to lawfully transfer personal information to the United States in all cases. If there is no lawful manner for us to transfer personal information from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally compliant transfer are too onerous, we could face significant adverse consequences, including increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors, and other third parties, and injunctions against our processing or transferring of personal information necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal information to recipients outside of the EEA for allegedly violating the EU GDPR's cross-border data transfer limitations. Additionally, companies that transfer personal information to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups.

Compliance with 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 new data protection rules. Failure to comply with any such laws or regulations puts us at risk of facing significant fines and penalties that could adversely affect our business, financial condition, reputation, and results of our operations. Furthermore, conflicting requirements across applicable privacy and data security laws would complicate our compliance efforts and increase both legal risk and compliance costs for us and the third parties upon whom we rely.

In addition to any applicable privacy and data security laws and regulations, we may be subject to industry standards adopted by industry groups or bound by other contractual obligations related to privacy and data security. We may publish privacy policies, marketing materials, and other statements, such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to regulatory inquiries, regulatory enforcement actions, and other adverse consequences.

Our obligations related to privacy and data security are quickly changing, becoming increasingly stringent, and creating regulatory uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent between jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties that process personal information or other sensitive data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our privacy and data security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties that process personal information or other sensitive data on our behalf fail, or are perceived to have failed, to address or comply with applicable privacy and data security obligations, we could face significant consequences, including but not limited to government enforcement actions (e.g., investigations, fines, penalties, audits, and inspections), litigation (including class-action claims), additional reporting requirements and/or oversight, bans on processing personal information, and orders to destroy or not use personal information. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to loss of customers, significant reputational harm, an inability to process personal information or to operate in certain jurisdictions, limited ability to commercialize VYJUVEK or develop and commercialize our product candidates, expenditures of time and resources to defend ourselves against claims or inquiries, adverse publicity, or substantial changes to our business model or operations.

Unfavorable global economic and geopolitical conditions could adversely affect our business, financial condition, or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets, including inflation and interest rates and concerns of a recession in the United States or other major markets due to a number of factors. In addition, the current geopolitical uncertainty may lead to prolonged, adverse impacts on global economic, sociopolitical, and market conditions. A severe or prolonged economic downturn could result in a variety of risks to our business, including our ability to raise additional capital if needed or on acceptable terms, if at all. A weak or declining economy, sanctions, trade restrictions, and other global conditions could also strain our suppliers, possibly resulting in supply delays or disruptions. In addition, we may be impacted by the imposition of tariffs, trade protection measures or other policies adopted by any jurisdiction that favors domestic companies and technologies over foreign competitors. Any of the foregoing could materially and adversely affect our business, and we cannot anticipate all the ways in which the current economic climate and financial market conditions could adversely impact our business, financial condition, results of operations, and prospects.

Our internal computer systems, or those of any third-party with whom we do business may fail or suffer a cyber-security incident, such as a data breach or computer virus, which could harm our business by damaging our reputation, exposing us to liability, adversely impacting our revenue, or materially disrupting our operations, including production of VYJUVEK or our product development programs.

We rely on our information technology systems and infrastructure to manage our business. In addition, we receive, process, store, and transmit, often electronically, confidential data of others, including the participants in our clinical trials. Unauthorized access to our (or any third party with whom we do business, such as suppliers, distributors, manufacturers, or vendors) computer systems or stored data could result in the theft or improper disclosure of personal or confidential information or other sensitive data, the deletion or modification of records, or could cause interruptions in our operations. Cybersecurity threats include, but are not limited to, ransomware attacks, phishing attempts, and the exploitation of software vulnerabilities to gain access to our information technology environment, and cyber-security risks increase when we transmit information from one location to another, including transmissions over the Internet or other electronic networks. Despite our robust security measures and our commitment to implementing and continually improving our cybersecurity posture to mitigate the risk of a cybersecurity incident, we cannot guarantee that such incidents will not occur to us or any third-party with whom we do business. For example, in 2024, our specialty pharmacy provider was affected by a cybersecurity incident that delayed reimbursement approvals and had a negative impact on our product revenue. A cybersecurity incident, even if promptly addressed, may harm our reputation, damage our brand, and erode trust. Our systems, and those of any third-party with whom we do business, may also be vulnerable to software viruses, stolen, misplaced, or lost data, programming and/or human errors, or other similar events which may disrupt our operations or expose personal and confidential information.

Moreover, in the event of a cybersecurity incident, we may face investigations, legal actions, including class action litigation, regulatory inquiries, and regulatory enforcement actions. We may also be subject to fines, consent orders, or mandated corrective actions that could have a material adverse impact on our operations and financial position. Furthermore, cybersecurity incidents and their legal consequences may impact investor confidence, potentially leading to a decrease in our stock price or limitations on our access to capital markets. If such an event were to occur and cause material interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information. For example, the loss of clinical trial data from completed or future 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 breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed.

Certain data breaches must be reported to affected individuals and various government and/or regulatory agencies, and in some cases to the media, under provisions of HIPAA, other U.S. federal and state laws, and requirements of non-U.S.

jurisdictions, including the EU GDPR and relevant member state law in the EU and other foreign laws. Although we maintain cyber-security and other customary insurance, our insurance policies may not be adequate to compensate us for the potential losses arising from breaches, failures, or disruptions of our infrastructure. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us and defending a suit, regardless of its merit, could be costly and divert management's attention. Any security breach involving the misappropriation, loss or other unauthorized disclosure or use of confidential information of others, whether by us or a third-party, could: (i) subject us to civil and criminal penalties; (ii) have a negative impact on our reputation; or (iii) expose us to liability to third parties or government authorities.

Notifications and follow-up actions related to a data security incident could impact our reputation and cause us to incur significant costs, including significant legal expenses and remediation costs. We expect to incur significant costs in an effort to detect and prevent security incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security incident. However, we cannot guarantee that we will be able to detect or prevent any such incidents, or that we can remediate any such incidents in an effective or timely manner. Our efforts to improve security and protect data from compromise may also identify previously undiscovered instances of data breaches or other cybersecurity incidents. To the extent that any data breach, disruption or security incident were to result in any loss, destruction, or alteration of, damage, unauthorized access to or inappropriate or unauthorized disclosure or dissemination of, our data, including personal data, or other information that is processed or maintained on our behalf, we could be exposed to litigation and governmental investigations and inquiries, the further development and commercialization of our product candidates could be delayed, and we could be subject to significant fines or penalties for any noncompliance with applicable state, federal, and foreign privacy and security laws, rules, regulations, and standards.

Artificial intelligence presents risks and challenges that could negatively impact our business.

Artificial intelligence ("AI")-based platforms and tools are increasingly being used in the biopharmaceutical industry, and we have adopted and integrated in limited situations artificial intelligence platforms for limited specific business uses and may adopt and integrate additional AI platforms and/or tools into our business. As with many technological innovations, AI presents opportunities, risks and challenges that could impact our business. Harnessing AI's potential may enable us to speed up the discovery and development of new product candidates, optimize our manufacturing processes, and drive efficiencies. However, AI may exacerbate existing risks, including risks associated with data privacy, cybersecurity, intellectual property, healthcare fraud and abuse, product development and manufacturing, and risks to subjects in clinical trials.

If the models underlying AI technologies that we use or may use are incorrectly designed, implemented, or trained; rely on incomplete, inadequate, biased, or otherwise poor-quality data; rely on data with respect to which we do not have sufficient rights or for which we or the data providers have not implemented appropriate legal compliance measures; are used without sufficient oversight and governance to ensure responsible use; are misused, or used outside the scope of applicable regulatory authorizations; or are adversely impacted by unforeseen defects, technical challenges, cybersecurity threats, or material performance issues, the performance of our products and business, as well as our reputation, could suffer. In addition, we could incur liability as a result of violations of applicable laws or contracts to which we are a party, regulatory enforcement actions, or civil claims.

Additionally, the use of AI solutions by us or third parties on which we rely could lead to (i) the public disclosure of confidential information (including personal data of our employees, clinical trial participants, or other third parties) in contravention of our internal policies, data protection laws, other applicable laws, or contractual requirements, and/or (ii) the loss of proprietary information, trade secrets, or other intellectual property. In addition to existing risks, AI also introduces new risks, due to the autonomous nature of the technology, which, in some cases, may be deployed to perform tasks, inform decisions, automate decisions, and make predictions. AI may amplify biased and discriminatory decision making, perform unreliably, malfunction, generate insights which are difficult to interpret and explain, and cause direct harm to individuals or groups. Our failure to use AI technologies in a way that maintains trust, quality and control in our business activities and to capitalize on opportunities presented by AI may place us at a competitive disadvantage. Furthermore, uncertainties regarding developing legal and regulatory requirements and standards may require significant resources to modify and maintain business practices to comply with such laws and regulations concerning the use of AI. Failure to address AI risks could reduce our ability to deliver on our strategic objectives, result in reputational harm, and have a material adverse effect on our business, prospects, operating results, and financial condition.

The regulatory framework for AI is rapidly evolving as many federal, state, and foreign government bodies and agencies have introduced or are currently considering additional laws and regulations, including the European Union's AI act. Evolving AI-related regulations, particularly in the United States, may impact our ability to develop, use, and commercialize AI technologies in the future. It is possible that further new laws and regulations will be adopted in the United States and in other non-U.S. jurisdictions, or that existing laws and regulations may be interpreted in ways that would limit our ability to use AI for our business, or require us to change the way we use AI in a manner that negatively affects the performance of our systems and business. We may need to expend resources to adjust our systems in certain jurisdictions if the laws, regulations, or decisions

are not consistent across jurisdictions. Further, the cost to comply with such laws, regulations or decisions and/or guidance interpreting existing laws, could be significant and could increase our operating expenses (such as by imposing additional reporting obligations regarding our use of AI). Such an increase in operating expenses, as well as any actual or perceived failure to comply with such laws and regulations, could materially and adversely affect our business, financial condition, results of operations, and prospects.

Our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural or technological disasters could severely disrupt our operations or the operations of third-party suppliers or service providers and have a material adverse effect on our business, financial condition, results of operations, and prospects. The severity and frequency of information technology system failures and cyberattacks continue to increase. Additionally, the severity and frequency of weather-related natural disasters have been amplified, and are expected to continue to be amplified, by global climate change. Such natural and technological disasters may cause damage to and/or disrupt our operations, which may result in a material adverse effect on our VYJUVEK sales, our product candidates, business, and results of operations. Moreover, climate change may also result in various chronic physical changes, such as changes in temperature or precipitation patterns or sea-level rise, that could have an adverse impact on our operations. Our suppliers, vendors, and business partners also face similar risks, and any disruption to their operations could have an adverse effect on our supply chain, manufacturing operations, or our commercial operations. If a natural disaster, power outage, or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our manufacturing facilities and IT systems, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans that we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. A significant portion of our current supply of materials necessary for production of VYJUVEK and our product candidates, as well as finished VYJUVEK and product candidates, is located at our manufacturing facilities in Pittsburgh, Pennsylvania. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement, and economic risks associated with doing business outside of the United States.

On April 23, 2025, the European Commission granted marketing authorization to VYJUVEK, and we commercially launched VYJUVEK in Germany in August 2025. On July 24, 2025, Japan's MHLW granted marketing authorization to VYJUVEK, and we commercially launched in Japan in October 2025. We currently have operations and employees located outside the United States and our business strategy incorporates potential additional international expansion to target patient populations outside the United States. Doing business internationally involves a number of risks, including, but not limited to:

- compliance with multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our product candidates in various countries;
- complexities and difficulties in obtaining protection and enforcing our intellectual property, and additional potentially relevant third-party patent rights;
- difficulties in staffing and managing foreign operations, including language barriers and translation;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, increased expenses for travel, translation, and insurance, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products that are approved for sale, and exposure to foreign currency exchange rate fluctuations;
- risks from or relating to natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions; and

- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our potential international expansion and operations and, consequently, our results of operations.

We are subject to U.S. and certain foreign export and import controls, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We could face criminal liability and other serious consequences for violations, which could harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting, or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We engage third parties to sell VYJUVEK and may engage third parties to sell our product candidates, if approved, abroad and/or to obtain necessary marketing authorizations, permits, licenses, patent registrations, and other regulatory approvals. We may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Furthermore, U.S. export control laws and economic sanctions prohibit the provision of certain products and services to countries, governments, and persons targeted by U.S. sanctions. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other adverse consequences.

The effect of pandemics, epidemics, outbreaks of infectious diseases, or similar public health crises on our operations and the operations of our customers, suppliers, third-party partners, and regulators could have an adverse impact on our business.

Pandemics, epidemics, outbreaks of infectious diseases, or similar public health crises could adversely disrupt or impact our operations or those of our customers, suppliers, third-party partners, and regulators. In response to a pandemic or public health crisis, authorities may impose, and businesses and individuals may implement, numerous measures to try to contain the pandemic or public health crisis or treat its impact, such as travel bans and restrictions, quarantines, shelter-in-place/stay-at-home and social distancing orders, shutdowns, and vaccine requirements. In the event that such measures or similar measures or restrictions are implemented as a result of a pandemic or public health crisis, our employees conducting research and development or manufacturing activities may not be able to access our laboratory or manufacturing spaces, and our core activities may be significantly limited or curtailed, possibly for an extended period of time. In addition, the operations of our customers, suppliers, third-party partners, and regulators could be significantly limited or curtailed. Timely initiation and completion of clinical trials are essential to our business and clinical trials are dependent upon the availability of clinical trial sites, researchers and investigators, regulatory agency personnel, and materials, any of which may be adversely affected by public health crises, such as pandemics. The extent to which a health crisis may impact our business, results of operations, and future growth prospects will depend on a variety of factors and future developments, which are highly uncertain and cannot be predicted with confidence, including the duration, scope, and severity of the public health crisis. A future public health crisis may have a material adverse effect on our business and results of operations.

Inadequate funding for the FDA and other government agencies, including from government shut downs, 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.

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. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which could adversely affect our business. For example, when the U.S. government has shut down in the past, certain regulatory agencies, such as the FDA and the United States Securities and Exchange Commission 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 review and process our

regulatory submissions in a timely manner, 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 capital, if necessary. In addition, government funding of government agencies on which our operations may rely is subject to the political process, which is inherently fluid and unpredictable.

There remains substantial uncertainty as to how the current U.S. administration will seek to or continue to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over VYJUVEK and our product candidates. This uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, if we are not able to maintain regulatory compliance, if we experience delays in obtaining approval, or if we fail to obtain approval of our product candidates, we may lose any marketing approval that we may have obtained, which would materially and adversely affect our business, financial condition, results of operations, and prospects, or the commercial prospects for our product candidates may be harmed and our ability to generate revenue could be materially impaired.

Risks Related to the Development, Regulatory Review, and Approval of Our Product Candidates

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

The development and commercialization of our product candidates are subject to many uncertainties, including the following:

- successful completion of preclinical studies, including animal studies, to determine the predicted safety and efficacy profile of our product candidates;
- successful enrollment and completion of clinical trials;
- positive results from our current and planned clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- successful development of our internal manufacturing processes on an ongoing basis, including any required or desired changes to our manufacturing processes, and maintenance of our existing arrangements with third-party suppliers or manufacturers for clinical supply;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others; and
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors

If we fail in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

Our gene therapy platform is based on novel technology, which makes it difficult to predict the time and cost of obtaining regulatory approvals for our product candidates.

The clinical trial requirements of the FDA, EMA, European Commission, MHLW, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use, and market of such product candidates. The regulatory approval process for novel product candidates such as ours, including approvals of or changes to manufacturing processes, can be more expensive and take longer than for other, better known or more extensively studied product candidates. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union, Japan, or elsewhere, or how long it will take to commercialize our product candidates. Approvals by the European Commission, MHLW, or other foreign regulators may not be indicative of what the FDA may require for approval and approval by the FDA may not be indicative of what the European Commission, MHLW, or other foreign regulators would require for approval.

Regulatory requirements and policy governing gene and cell therapy products have changed frequently and may continue to change in the future. In 2016, the FDA established the Office of Tissues and Advanced Therapies ("OTAT") within its Center for Biologics Evaluation and Research to consolidate the review of gene therapy and related products, and has

established the Cellular, Tissue and Gene Therapies Advisory Committee, among others, to advise this 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. If we engage a National Institutes of Health funded institution to conduct a clinical trial, that institution's Institutional Biosafety Committee as well as its Institutional Review Board would need to review the proposed clinical trial to assess the safety and ethics of the trial. Further, the FDA continues to update policies relating to clinical trial design and data collection which may impact compliance requirements. Similarly, the EMA, the European Commission, MHLW, or other foreign regulators may issue new or revised guidelines concerning the development and marketing authorization of gene therapy medicinal products.

These regulatory review committees and advisory groups and the new guidelines they promulgate 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. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations, and prospects would be materially and adversely affected.

Our product or product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences before or following any potential marketing approval.

There have been several significant adverse side effects in gene therapy trials using other vectors in the past. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

In addition to side effects caused by our product or product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that such adverse events were caused by the administration process or related procedures and not by our product candidates, the FDA, the European Commission, or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we can demonstrate that any serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend, or terminate any clinical trial of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenue from the product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop product candidates, and may harm our business, financial condition, and prospects significantly.

Additionally, if a product candidate receives marketing approval, the FDA or a foreign regulatory authority could require us to adopt a post-approval safety monitoring program to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by VYJUVEK or our product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of VYJUVEK or our product candidates that may be approved;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way VYJUVEK or a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of VYJUVEK or our product candidates and could significantly harm our business, financial condition, results of operations, and prospects.

Our product candidates that are in preclinical stages of development may never advance to clinical development and for those product candidates that do progress to clinical development, we may encounter substantial delays in our clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Preclinical studies, which are also known as nonclinical studies, refer to a stage of research that begins before clinical trials (testing in humans) can begin, and during which important feasibility, iterative testing, and safety data are collected.

Because of their early nature, preclinical product candidates tend to carry a higher risk of failure as compared with clinical-stage assets. Preclinical product candidates must generate sufficient safety and efficacy data through in vitro studies and a variety of tests before they can be considered appropriate for testing in humans. The development risks, timeline, and cost of preclinical assets can be high because of the unknowns and absence of data. It can be difficult to identify relevant tests and animal models for preclinical studies. If preclinical studies of our product candidates do not generate strong data, our preclinical stage programs may never progress to clinical development and may prove to be worthless. In addition, the results of preclinical studies may not be predictive of the results of clinical trials. For example, we utilize animal models to ascertain the predicted safety and efficacy profile of our product candidates, but animal models are imperfect predictors of a product candidate's effect/interactions in humans. As such, positive data from animal models may not be predictive of positive human results, patients may have side effects that were not observed in animals, and a product candidate may pose significant and unexpected safety risks to humans.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. Obtaining marketing approval is an extensive, lengthy, expensive, and inherently uncertain process, and regulatory authorities may delay, limit, or deny approval of our product candidates for many reasons. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical trials include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in opening sites and recruiting a sufficient number and diversity of suitable study subjects to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or concerns with a class of product candidates, or after an inspection of our clinical trial operations or trial sites;
- delays in having study subjects complete participation in a trial or return for post-treatment follow-up;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

The results of nonclinical studies and early clinical trials may not be predictive of the results of later-stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. In some instances, there can be significant variability in safety or efficacy results between different 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 study subject populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. In addition, preclinical and clinical data are often susceptible to various interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials.

If we make manufacturing processes or formulation changes to our product or product candidates, we may need to conduct additional studies to bridge our modified product or product candidate to earlier versions and obtain regulatory approvals. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our approved products or allow our competitors to bring products to market before we do, which could limit our potential revenue or impair our ability to successfully commercialize our approved products and may harm our business, financial condition, results of operations, and prospects. Any delays, setbacks or failures in our clinical trials could materially and adversely affect our business, financial condition, results of operations, and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval, if at all, or be required to conduct additional confirmatory safety and/or efficacy studies;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, precautions, or contraindications;

- obtain approval without labeling claims that are necessary or desirable for the successful commercialization of our product candidates;
- be subject to additional and costly post-marketing testing requirements or clinical trials;
- be required to perform additional clinical trials to support approval;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution;
- be sued; or
- experience damage to our reputation.

Our product development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an Institutional Review Board, may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's Current Good Clinical Practice, or CGCP, regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our investigational new drug, or IND, applications or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be negatively impacted, and our ability to generate revenue from our product candidates may be eliminated or delayed.

We rely on third parties to conduct certain of our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for, or commercialize, our product candidates.

We depend upon third parties to conduct certain of our preclinical studies and depend on third parties, including independent principal investigators, to conduct our clinical trials under agreements with universities, medical institutions, and others. We negotiate budgets and contracts with such third parties, which may result in delays to our development timelines and increased costs.

We rely on third party contract research organizations ("CROs") to conduct our preclinical animal studies to support product candidate development and related regulatory submissions. Use of CROs for our animal studies subjects us to a number of risks, including a CRO going out of business, failing to meet deadlines or appropriately performing an animal study, or altering their internal practices (e.g., changing the types or biosafety levels of materials they are willing to handle) that prevent future collaboration. In addition, our use of CROs located outside of the United States is subject to changes in import/export regulations that could delay or prevent timely shipping/receiving of our product candidates for animal studies. If any such adverse events occur, the development of our product candidates or the acceptability of nonclinical data packages for regulatory agencies could be significantly and adversely impacted.

We rely on third parties over the course of our clinical trials, and, as a result, may have limited control over the clinical principal investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements, 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 CGCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these CGCP requirements through periodic inspections of clinical trial sponsors, clinical investigators, and clinical trial sites. If we or any of these third parties fail to comply with applicable CGCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these clinical trials or perform additional preclinical studies or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with CGCP requirements. In addition, our later-stage clinical trials must be conducted with product produced under Current Good Manufacturing Practice, or CGMP, requirements (and comparable quality regulations in foreign countries) and may require a large number of study subjects.

Our failure or any failure by these third parties to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be adversely affected if any of these third

parties violate federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws. Any third parties conducting our preclinical studies or our clinical trials are not our employees and, except for remedies that may be available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our preclinical studies 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 trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully perform their contractual duties or meet expected deadlines, need to be replaced, or fail to adhere to our protocols or applicable regulatory requirements, or if the quality or accuracy of the preclinical or clinical data they generate is otherwise compromised, our development timelines, including clinical development timelines, may be extended, delayed or terminated, and we may be unable to complete development of, obtain regulatory approval for, 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 or precluded entirely. Though we carefully manage our relationships with principal investigators and other third parties, there can be no assurance that we will not encounter challenges or delays or that these delays or challenges will not have a material adverse impact on our business, financial condition, and prospects.

Interim, "top-line," and preliminary data from our clinical trials that we announce or publish from time to time may change as more data becomes available or as additional analyses are conducted, and as the data are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim, "top-line," or preliminary data from our clinical trials. Interim data from clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as study subject enrollment continues and more data becomes available. Preliminary or "top-line" 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, interim and preliminary data should be viewed with caution until the final data are available. Material adverse changes between preliminary, "top-line," or interim data and final data could significantly harm our business, financial condition, results of operations, and prospects.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for them outside of the United States, which would limit our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of VYJUVEK or our product candidates, if approved, outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidate in those countries and the process for obtaining such approval may be lengthy and expensive. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our product candidates, if approved, is also subject to approval. Obtaining a Marketing Authorization Application ("MAA") from the European Commission following the opinion of the EMA and obtaining marketing authorization from the MHLW in Japan are lengthy and expensive processes. Even if a product candidate is approved, the FDA, the European Commission, or the MHLW, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States, the European Union, and Japan also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties, and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations, and prospects will be adversely affected.

VYJUVEK and our product candidates that receive marketing approvals remain subject to regulatory oversight even after regulatory approval. We will continue to incur costs related to regulatory compliance and are subject to risks related to non-compliance with or changes to applicable laws and regulations, which could cause VYJUVEK or any of our product candidates that obtain regulatory approval to lose that approval.

VYJUVEK, our first FDA, European Commission, and MHLW-approved product, and any other product candidates that obtain regulatory approval in the future, will remain subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to a post-approval safety monitoring program, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety, and efficacy of the product. For example, the holder of an approved Biologics License Application, or BLA, is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling, or manufacturing process. For example, if demand for an approved product increases more than we previously estimated, we may need or desire to scale up an existing FDA-approved manufacturing process and the scaled-up manufacturing process would be subject to FDA review and approval. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal, state, and foreign laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with CGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with an approved product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or a regulatory authority disagrees with the promotion, marketing, or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility, or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements, a regulatory authority may, among other actions:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil, or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners, if any;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our approved product, VYJUVEK, and any product candidates that obtain regulatory approval and adversely affect our business, financial condition, results of operations, and prospects.

The FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could negatively impact the existing marketing approval for VYJUVEK and prevent, limit, or delay regulatory approval of our product candidates.

While we have obtained orphan drug exclusivity for VYJUVEK in the United States, and orphan drug designation for certain product candidates in the United States and other jurisdictions, it may not effectively protect us from competition, and we may be unable to obtain orphan drug designation for other product candidates. If our competitors are able to obtain orphan drug exclusivity before us, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States, the European Union, and Japan may designate drugs for relatively small patient populations as orphan drugs.

Under the Orphan Drug Act of 1983, as amended, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States of such drug. Orphan drug designation itself does not convey any advantage in or shorten the duration of the regulatory review and approval process, but it can lead to financial incentives, such as opportunities for grant funding toward clinical trial costs, tax advantages, and user-fee waivers. In addition, if a product candidate that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the drug is entitled to orphan drug marketing exclusivity for a period of seven years. Orphan drug marketing exclusivity generally prevents the FDA from approving another application to market the same drug or biological product for the same disease or condition for seven years, except in limited circumstances, including if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. A designated orphan drug may not receive orphan drug marketing exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Orphan drug marketing exclusivity rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

In the European Union, the European Commission, upon a recommendation from the EMA's Committee for Orphan Medicinal Products, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the EU. Additionally, orphan designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating, or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product. In the European Union, orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but orphan drug designation may entitle an applicant to financial incentives such as reduction of fees or fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Upon grant of a marketing authorization, orphan products are entitled to ten years of market exclusivity for the approved therapeutic indication, which means that the EMA and European Commission cannot accept another marketing authorization application, grant a marketing authorization, or accept an application to extend a marketing authorization for a similar product for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed Pediatric Investigation Plan. The ten-year market exclusivity in the European Union may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for which it received orphan designation, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity, or where the prevalence of the condition has increased above the threshold. Additionally granting of an authorization for another similar orphan medicinal product where another product has market exclusivity can happen at any time: (i) that the second applicant can establish that its product, although similar, is safer, more effective, or otherwise clinically superior, (ii) that the first applicant cannot supply enough orphan medicinal product, or (iii) where the first applicant consents to a second orphan medicinal product application. Pursuant to a recent legislative reform initiative of the pharmaceutical framework, for which a provisional political agreement has been reached between the European Parliament and the Council in December of 2025, exclusivity for new orphan medicinal products will likely run nine years (as opposed to the current ten years), while "breakthrough" orphan medicinal products addressing diseases with no existing treatments can benefit from up to 11 years of market exclusivity. The final legislative text has not yet been published, however, and remains subject to formal adoption, entry into force, and transitional arrangements.

The orphan drug designation system in Japan aims to support the development of drugs for diseases that affect fewer than 50,000 patients in Japan, for which significant unmet medical need exists. An investigational therapy may be eligible for orphan drug designation in Japan if there is no approved alternative treatment option or if the therapy is expected to demonstrate superior efficacy or safety compared to existing treatment options. Specific measures to support the development of orphan drugs in Japan include subsidies for research and development expenditures, prioritized consultation regarding clinical development, reduced consultation fees, tax incentives, priority review of applications, reduced application fees, and extended registration validity period. Up to ten years of orphan exclusivity, known as the re-examination period, is granted for the product after approval. The orphan drug exclusivity may be rescinded by the Japanese government in certain circumstances.

Even though we have obtained orphan drug exclusivity for VYJUVEK in the United States, EU, and Japan and obtained orphan drug designation for certain product candidates in the United States and the European Union, we may not be able to maintain orphan drug exclusivity, and if we are able to maintain the orphan drug exclusivity, the exclusivity may not effectively protect the product or product candidate from competition because different drugs can be approved for the same condition. Further, we cannot assure you that any of our other product candidates will be approved for any orphan-designated use in any jurisdiction, in a timely manner, or at all, or that a competitor will not obtain orphan drug exclusivity that could block the regulatory approval of any of our product candidates for several years. If we are unable to maintain or obtain orphan drug exclusivity, our ability to generate sufficient revenue may be negatively affected. If a competitor is able to obtain orphan drug exclusivity that would block our product candidates' regulatory approval, our ability to generate revenue could be significantly reduced, which would harm our business prospects, financial condition, and results of operations. We do not know if, when, or how the FDA or other regulators may change the applicable orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes may be made to orphan drug regulations and policies, our business could be adversely impacted.

Accelerated approval by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek approval of our current or future product candidates using the FDA's accelerated approval pathway. This pathway may not lead to a faster development, regulatory review, or approval process and does not increase the likelihood that our product candidates will receive marketing approval. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint or an intermediate clinical endpoint that is reasonably likely to predict clinical benefit. As a condition of approval, the FDA may require that a sponsor of a product receiving accelerated approval perform adequate and well-controlled post-marketing confirmatory clinical trials. These confirmatory trials must be completed with due diligence. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is permitted to require, as appropriate, that a post-approval confirmatory trial or trials be underway prior to approval or within a specified time after the date accelerated approval was granted. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such studies, including progress toward enrollment targets, and the FDA must promptly post this information publicly. The FDA has also continued to update its accelerated approval policies, including expectations for clinical trial endpoints. Furthermore, under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory trial or submit timely reports to the agency on their progress. In addition, for products under consideration for accelerated approval, the FDA currently requires, unless otherwise requested by the agency, pre-approval of promotional materials intended for dissemination or publication within 120 days of marketing approval be submitted to the agency for review during the review period, which could adversely impact the timing of the commercial launch of the product. There can be no assurance that the FDA would allow any of our product candidates to proceed on an accelerated approval pathway, and even if the FDA did allow such pathway, there can be no assurance that any expedited development, review, or approval will be granted on a timely basis, or at all.

Breakthrough Therapy Designation, Fast Track Designation, Regenerative Medicine Advanced Therapy ("RMAT") Designation, or Priority Review by the FDA, or PRIority MEdicines ("PRIME") Scheme by the EMA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review, or approval process, and such designations may not increase the likelihood that any of our product candidates will receive marketing approval.

We may seek a Breakthrough Therapy Designation for some of our product candidates. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review, or approval compared to therapies considered for approval under conventional FDA procedures and does not ensure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time for FDA review or approval will not be shortened.

We have obtained and may seek Fast Track Designation for some of our product candidates. For instance, VYJUVEK and KB707 (intratumoral and inhaled) were granted Fast Track Designation by the FDA. If a therapy is intended for the treatment of a serious or life-threatening condition and the therapy demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review, or approval compared to conventional FDA procedures. For product candidates that receive Fast Track Designation, sponsors may have greater interactions with the FDA, and the FDA may initiate review of sections of the marketing application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a Fast Track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing an application does not begin until the last section of the application is submitted. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from clinical programs. Many biologics that have received Fast Track Designation have failed to obtain marketing approval. Fast Track Designation alone does not guarantee qualification for the FDA's priority review procedures.

We were granted RMAT designation for B-VEC from the FDA, and we may seek RMAT designation for some of our product candidates. In 2017, the FDA established the RMAT designation as part of its implementation of the 21st Century Cures Act to expedite review of any drug that meets the following criteria: it qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. Like Breakthrough Therapy Designation, RMAT designation provides potential benefits that include more frequent meetings with the FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval based on surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT-designated products that receive accelerated approval may, as appropriate, fulfill their post-approval requirements through the submission of clinical evidence, clinical trials, patient registries, or other sources of real-world evidence, such as electronic health records; through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy. There is no assurance that we will be able to obtain RMAT designation for our product candidates. RMAT designation does not change the FDA's standards for product approval, and there is no assurance that such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily result in an expedited regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle, or at all.

We have obtained and may seek to qualify our product candidates under the PRIME scheme from the EMA. For instance, B-VEC was granted PRIME designation. The PRIME scheme is open to medicines under development and for which the applicant intends to apply for an initial MAA through the centralized procedure. Eligible products must target conditions for which there is an unmet medical need (there is no satisfactory method of diagnosis, prevention, or treatment in the European Union or, if there is, the new medicine will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods or therapy or improving existing ones. There is no assurance that we will be able to obtain PRIME qualification for our product candidates. PRIME does not change the standards for product approval, and there is no assurance that such qualification will result in expedited review or approval. Moreover, where, during the course of development, a product no longer meets the eligibility criteria, support under the PRIME scheme may be withdrawn.

We have obtained a rare pediatric disease designation for certain of our product candidates; however, there is no guarantee that FDA approval will result in issuance of a priority review voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor that receives an approval for a drug

or biologic for a "rare pediatric disease" that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the United States within one year following the date of approval. We received rare pediatric disease designation for VYJUVEK and were awarded a priority review voucher following FDA approval of VYJUVEK in May 2023. The priority review voucher was sold in August 2023. We have also obtained a rare pediatric disease designation for certain of our product candidates, including KB407, inhaled KB707, and intratumoral KB707. However, there is no guarantee that we will be able to obtain a priority review voucher if these product candidates are approved by the FDA. Congress included a sunset provision in the statute authorizing the rare pediatric disease priority review voucher program. Under current law, the FDA's authority to award rare pediatric disease priority review vouchers is scheduled to sunset after September 30, 2029. If the program is not extended, modified, or reauthorized prior to that date, we may be unable to obtain a priority review voucher for one or more of our product candidates, even if such product candidates otherwise satisfy the applicable criteria. Separately, in June 2025, the FDA announced the Commissioner's National Priority Voucher pilot program, which is distinct from the priority review voucher program and is intended to expedite review of certain applications aligned with national health priorities.

We have received designation for our platform technology as a designated platform technology, but such designation may not lead to a faster development or regulatory review or approval process.

Under FDORA, a platform technology incorporated within or utilized by a drug or biologic is eligible for designation as a designated platform technology if (1) the platform technology is incorporated in, or utilized by, a product approved under a New Drug Application, or NDA, or BLA; (2) preliminary evidence submitted by the sponsor of the approved product, or a sponsor that has been granted a right of reference to data submitted in the application for such product, demonstrates that the platform technology has the potential to be incorporated in, or utilized by, more than one product without an adverse effect on quality, manufacturing, or safety; and (3) data or information submitted by the sponsor indicates that incorporation or utilization of the platform technology has a reasonable likelihood to bring significant efficiencies to the product development or manufacturing process and to the review process. A sponsor may request the FDA to designate a platform technology as a designated platform technology concurrently with, or at any time after, submission of an IND application for a product that incorporates or utilizes the platform technology that is the subject of the request. If so designated, the FDA may expedite the development and review of any subsequent NDA or BLA for a product that uses or incorporates the platform technology. In 2025, the FDA granted platform technology designation to our genetically modified, non-replicating herpes simplex virus type 1 (HSV-1) viral vector used in our redosable eye drop gene therapy KB801 that is currently under evaluation in a clinical trial for the treatment of neurotrophic keratitis. However, the receipt of such designation does not ensure that our applicable product candidates will be developed more quickly or receive a faster FDA review process or ultimate FDA approval. Moreover, the FDA may revoke a designation if the FDA determines that a designated platform technology no longer meets the criteria for such designation.

Risks Related to Manufacturing

Delays in obtaining regulatory approvals of the process, or changes to the process, and facilities needed to manufacture VYJUVEK or our product candidates or disruptions in our manufacturing process may disrupt our production of VYJUVEK or delay or disrupt our development and commercialization efforts with respect to our product candidates.

Before we can begin to commercially manufacture our product candidates, we must pass a pre-approval inspection of our manufacturing facilities by the FDA. A manufacturing authorization may also be required from the appropriate regulatory authorities outside of the United States. The timeframe required for us to obtain such approvals is uncertain. To obtain approval, we need to ensure that all our processes, methods, and equipment are compliant with CGMP, and perform extensive audits of vendors, contract laboratories, and suppliers. If any of our vendors, contract laboratories, or suppliers are found to be out of compliance with CGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories, or suppliers. The CGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with CGMP, we are obligated to expend time, money, and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we will be subject to possible regulatory action and may not be permitted to sell any approved product.

In addition, the manufacturing process used to produce VYJUVEK and our product candidates is complex and novel and demand for an approved product may require us to change the manufacturing process, which may require regulatory approval before we are able to sell the product manufactured by the changed process. The production of VYJUVEK and our product candidates require processing steps that are more complex than those required for most pharmaceuticals. Moreover, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the

finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and that VYJUVEK and our product candidates are made strictly and consistently in compliance with the process. Problems with an approved manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, or insufficient inventory. In addition, changes to an approved manufacturing process may result in problems with the process design, process reproducibility, stability, or batch consistency, and may require regulatory approval before we are permitted to sell products manufactured with the changed manufacturing process, which could potentially delay commercial availability of an approved product. We may encounter problems achieving adequate quantities and quality of materials that meet FDA or other applicable standards or specifications with consistent and acceptable production yields and costs, which could materially and adversely affect our business, financial condition, results of operations, and prospects.

Although we have established our own manufacturing facilities for VYJUVEK and our product candidates, we may also utilize third parties to conduct our product manufacturing or components thereof. We are also dependent on a limited number of third-party suppliers for some of the components and materials used in manufacturing VYJUVEK and our product candidates and in commercially supplying VYJUVEK. Therefore, we are subject to the risk that these third parties may not perform satisfactorily.

We may maintain third-party manufacturing capabilities in order to provide multiple sources of supply of VYJUVEK or a product candidate that is approved for sale. In addition, we may utilize third parties to manufacture components of VYJUVEK or our product candidates. For example, we use a third-party to manufacture the sterile gel that is mixed with our in-house produced vector for VYJUVEK. Our ability to commercially supply VYJUVEK depends, in part, on the ability of third parties to supply and manufacture the raw materials and other important components related to our manufacture of VYJUVEK. Potential changes in export/import and trade laws, regulations, and policies of the United States and other countries, including any increased trade restrictions or tariffs, may impact prices and availability of raw material and other components used in the manufacture of VYJUVEK, cause supply chain volatility, and harm the development of our product candidates, any of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. For some materials and components related to our manufacture of VYJUVEK and our product candidates, there are, in general, relatively few alternative sources of supply. Our use of a limited number of suppliers for some of the components and materials used in manufacturing VYJUVEK and our product candidates and commercially supplying VYJUVEK exposes us to several risks, including disruptions in supply, price increases, late deliveries, and an inability to meet demand. If we fail to develop and maintain supply relationships with these third parties, we may be unable to successfully commercialize VYJUVEK or any approved product candidate. Any of our existing suppliers may:

- fail to supply us on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities or equipment or otherwise;
- fail to increase manufacturing capacity and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs;
- be unable to meet our production demands due to issues related to their reliance on sole-source suppliers and manufacturers;
- supply us with materials that fail to meet regulatory requirements;
- become unavailable through business interruption or financial insolvency;
- lose regulatory status as an approved supply source;
- be unable or unwilling to (i) honor current supply agreements or (ii) renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or
- discontinue production or manufacturing of materials that we acquire through such third-party supplier.

In the event of any of the foregoing, if we do not have an alternative supplier or manufacturer in place, we may not be able to manufacture our products for commercial, regulatory, or clinical purposes and would be required to expend substantial management time and expense to identify, qualify, and transfer to alternative suppliers or manufacturers. There can be no assurance that replacements would be available to us on a timely basis, on acceptable terms, or at all. Any need to find and qualify new suppliers or manufacturers could significantly delay production of VYJUVEK or any product candidate, if approved, adversely impact our ability to market VYJUVEK or any product candidate, if approved, and have a material adverse effect on our business, financial condition, results of operations, and prospects.

If we or a third-party supplier or manufacturer fails to comply with applicable CGMP regulations, the FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending marketing application for a product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations, and prospects to be materially harmed.

Any contamination in, or changes to, our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our ability to produce VYJUVEK or any other approved product for commercial supply or any product candidate for clinical development.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our ability to produce VYJUVEK or our product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of VYJUVEK or our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines and our business, financial condition, results of operations, and prospects.

Failure to increase manufacturing capacity and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs could result in delays in commercial availability of an approved product. If a manufacturing process is approved by the FDA or other regulator, implementing a new or changed manufacturing processes is difficult, time consuming, and would require regulatory approvals, which could potentially delay commercial availability of an approved product, which, in turn, could harm our results of operations and cause reputational damage.

Our failure to maintain or continuously improve our quality management program could have an adverse effect upon our business, subject us to regulatory actions, and cause patients to lose confidence in us or our products, among other negative consequences.

Quality management plays an essential role in the manufacturing of VYJUVEK and our product candidates, conducting clinical trials, preventing defects, improving our product candidates, and assuring the safety and efficacy of VYJUVEK and our product candidates. We seek to maintain a robust quality management program which includes the following broad pillars of quality:

- monitoring and assuring regulatory compliance for clinical trials, manufacturing, and testing of good applicable practice ("GxP") for regulated products (e.g., CGCP and CGMP);
- monitoring and providing oversight of all GxP suppliers;
- establishing and maintaining an integrated, robust quality management system for clinical, manufacturing, supply chain, and distribution operations; and
- cultivating a proactive, preventative quality culture and employee and supplier training to ensure quality.

Our success depends on our ability to maintain and continuously improve our quality management program. Any change to an approved manufacturing process will put strain on our quality management program. A quality or safety issue may result in adverse inspection reports, warning letters, monetary sanctions, injunctions to halt manufacture and distribution of VYJUVEK or our product candidates, civil or criminal sanctions, costly litigation, refusal of a government regulator to grant approvals and licenses, restrictions on operations, or withdrawal, suspension or variation of existing approvals and licenses. An inability to address a quality or safety issue in an effective and timely manner may also cause negative publicity, or a loss of patient confidence in us or VYJUVEK or our product candidates, which may result in difficulty in successfully launching products and the loss of potential future sales, which could have an adverse effect on our business, financial condition, and results of operations.

If demand for VYJUVEK or any product for which we obtain marketing approval increases more than previously estimated or we wish to improve manufacturing efficiencies to lower cost of production, we may need or choose to scale up or change a current FDA-approved commercial manufacturing process, which is subject to risks and uncertainties and could require us to submit a Prior Approval Supplement ("PAS") to the FDA and obtain the agency's approval for the manufacturing process changes before they can be implemented.

We may desire or need to make manufacturing process changes to scale-up manufacturing to meet increased demand, to improve efficiencies or costs, or otherwise. Scaling up a manufacturing process carries regulatory, financial, and operational risks, which could potentially impact product availability. For example, as a result of the strong and increasing commercial demand for VYJUVEK, we designed a revised commercial manufacturing process that should more than quadruple the output

of each production batch. We validated the revised commercial manufacturing process and submitted a PAS to the FDA, which was approved by the FDA. Receipt of FDA approval was required before we could commercially sell VYJUVEK manufactured with the revised commercial manufacturing process. In addition to requiring FDA approval of a PAS, there are risks associated with scaling up a manufacturing process, including, among others, cost overruns, potential problems with the process scale-up design, process reproducibility, stability issues, and batch consistency. Furthermore, studies or tests to demonstrate comparability of product manufactured under an existing and under a revised manufacturing process, or any other studies on a revised process, such as validation studies, may uncover findings that result in the FDA delaying or refusing to approve a revised process. We cannot guarantee if or when the FDA may approve a PAS for any manufacturing process change(s), and failure to obtain FDA approval or other risks associated with manufacturing processes changes could impact commercial availability of VYJUVEK or an approved product candidate, which could result in the loss of sales and adversely affect our business, results of operations, and financial condition.

We may need or desire to transfer VYJUVEK or an approved product candidate manufacturing from ANCORIS, our commercial scale CGMP-compliant manufacturing facility where VYJUVEK is currently manufactured to ASTRA, our recently completed and qualified state-of-the-art CGMP manufacturing facility, or transfer an approved product manufacturing from ASTRA to ANCORIS, and technical transfer of a manufacturing process is subject to risks and uncertainties and requires FDA inspection and approval of the facility where manufacturing is planned to be transferred.

We plan to complete a technical transfer process to allow us to commercially manufacture VYJUVEK at ASTRA, our recently completed and qualified state-of-the-art CGMP manufacturing facility, in addition to ANCORIS, where VYJUVEK is currently manufactured. This process may be time consuming and will require an FDA inspection of ASTRA. We cannot provide any assurance of the timing of such FDA approval, or if the FDA will approve commercial manufacturing of VYJUVEK at ASTRA. We have never completed a technical transfer process to an in-house manufacturing facility, and there is no guarantee that we will be successful doing so. Failure or delay in technical transfer of VYJUVEK or another approved product from ANCORIS to ASTRA, or from ASTRA to ANCORIS, could impair our ability to supply sufficient product to meet commercial demand and successfully commercialize and generate revenue from sales of approved products, which could adversely affect our business, financial condition, and results of operations.

Risks Related to Commercialization of VYJUVEK and Our Product Candidates

We have limited experience as a commercial company and the sales, marketing, and distribution of VYJUVEK or any future approved products may be unsuccessful or less successful than anticipated.

We received FDA approval of VYJUVEK in May 2023, European Commission approval of VYJUVEK in April 2025, and MHLW approval in July 2025, and initiated a commercial launch of VYJUVEK in the United States in the second quarter of 2023, in Germany in August 2025, and in Japan in October 2025. As a company, we have no prior experience commercializing a biologic. The success of our commercialization efforts is difficult to predict and subject to the effective execution of our business plan, including, among other things, the continued development of our internal sales, marketing, manufacturing, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management of such capabilities. For example, our commercial launch of VYJUVEK in the United States, the European Union, or Japan may not develop as planned or anticipated, which may require us to, among others, adjust or amend our business plan and incur significant expenses. Further, given our lack of experience commercializing products, we do not have a track record of successfully executing a commercial launch. There is a risk that we underestimate the level of demand for a product, which could require us to change a manufacturing process to increase production yields and changes to a manufacturing process are time consuming and subject to regulatory, financial, and operational risks. If we are unsuccessful in accomplishing our objectives and executing on our business plan, or if our commercialization efforts do not develop as planned, we may not be able to successfully commercialize VYJUVEK and any future approved products, we may require significant additional capital and financial resources, we may not be profitable on a consistent basis, and we may not be able to compete against more established companies in our industry.

If we are unable to maintain our agreements with third parties to distribute VYJUVEK to patients in the United States, our results of operations and business could be adversely affected.

We rely on a small number of third parties to commercially distribute VYJUVEK to patients in the United States. We have contracted with a third-party packaging company to package VYJUVEK, a third-party logistics company to warehouse, process, and ship VYJUVEK to a limited number of specialty pharmacies that mix the medication and may administer it to patients in the patient's home by a healthcare professional and to a limited number of hospitals and distributors where patients are administered the medication in a hospital or clinic. This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from VYJUVEK. If we are unable to effectively manage the distribution process, the sales of VYJUVEK could be compromised and our results of operations may be harmed.

If the third parties involved in the commercial distribution of VYJUVEK in the United States, European Union, or Japan do not fulfill their contractual obligations or refuse or fail to adequately or to properly distribute VYJUVEK and serve patients, or the agreements with them are terminated without adequate notice, shipments of VYJUVEK, and associated revenue, could be adversely affected. In addition, if we were required to replace such third parties, it could take time to locate an appropriate replacement third-party on acceptable terms, which could cause delays in our distribution network and increased expenses, and thereby adversely impact our commercial sales of VYJUVEK and result in a material adverse effect on our business, financial condition, results of operations, and prospects.

Using specialty distributors to market and sell VYJUVEK in certain jurisdictions outside of the United States, the United Kingdom, certain EU countries, and Japan subject us to certain risks.

Outside of the United States, major European markets, and Japan, we have entered into distribution agreements with specialty distributors to commercialize VYJUVEK. We have entered into agreements with leading regional specialty distributors to cover key markets in central and eastern Europe and the middle east, and we expect to further expand our specialty distributor network. We may be unable to enter into appropriate specialty distribution arrangements on favorable terms, if at all. Our use of distributors in these markets to market and sell VYJUVEK involves certain risks, including, but not limited to, risks that these organizations will not comply with applicable laws and regulations, not effectively sell or support VYJUVEK or reduce or discontinue their efforts to sell or support VYJUVEK, not devote the resources necessary to market and sell VYJUVEK in the volumes and within the time frame we expect, not be able to satisfy financial obligations to us or others, not provide us with accurate or timely information regarding their inventories of VYJUVEK or the number of patients who are using VYJUVEK, or not provide us with accurate or timely information regarding serious adverse events and/or product complaints. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of VYJUVEK in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

In connection with the commercial launch of VYJUVEK in the United States, Europe, and Japan, we have recruited a sales force and established marketing, market access, and medical affairs teams and distribution capabilities and if the commercial launch of VYJUVEK is not successful for any reason, we could incur substantial costs and our investment would be lost if we cannot retain or reposition our sales, marketing, market access, and medical affairs personnel.

To achieve commercial success for VYJUVEK, we have devoted and anticipate that we will continue to devote significant resources to support our sales force, marketing, market access, and medical affairs teams and distribution capabilities. There are risks involved with establishing our own sales, marketing, distribution, training, and support capabilities. For example, recruiting and training sales and marketing personnel is expensive and time-consuming and could delay our ability to focus on other priorities. If the commercial launch of VYJUVEK in the United States, European Union, or Japan is not successful for any reason, this would be costly, and our investment would be lost if we cannot retain or reposition our sales, marketing, market access, and medical affairs personnel or terminate on favorable terms any agreements entered into with third parties to support our commercialization efforts.

Factors that may inhibit our efforts to commercialize VYJUVEK or any other product candidates, if approved, on our own in the United States, European Union, Japan, or elsewhere include:

- our inability to train and retain adequate numbers of effective sales, marketing, training, and support personnel;
- the inability of sales personnel to obtain access to physicians, including key opinion leaders, or to educate an adequate number of physicians of the benefits of VYJUVEK or any approved product candidate;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive or integrated product offerings; and
- unforeseen costs and expenses associated with establishing and maintaining an independent sales, marketing, training, and support organization.

If our sales force, marketing, market access, and medical affairs teams and distribution capabilities fail, or are otherwise unsuccessful, it would materially adversely impact the commercial launch of VYJUVEK in the United States, Europe, or Japan, impact our ability to generate revenue, and harm our business.

If we are unable to expand our medical affairs, marketing, market access, sales, and distribution capabilities or collaborate with third parties to market and sell our product candidates for which we obtain marketing approval, we may be unable to generate sufficient product revenue.

To successfully commercialize any products for which we obtain marketing approvals, we will need to expand our sales force, marketing, market access, medical affairs teams, and distribution capabilities, either on our own or in collaboration with others. The development of a sales force, marketing, market access, and medical affairs teams and distribution capabilities effort is expensive and time-consuming, and our expenses associated with maintaining our sales force may be disproportional compared to the revenue we may be able to generate on sales of VYJUVEK and future products for which we obtain marketing authorization. We cannot be certain that we will be able to internally develop this capability successfully. We may enter into collaborations with other entities to utilize their established marketing and distribution capabilities. However, we may be unable to enter into such agreements on favorable terms, if at all.

We compete with many companies that currently have extensive, experienced, and well-funded medical affairs, marketing, market access, distribution, and sales operations to recruit, hire, train and retain personnel, and we may not be able to hire or retain such talent on commercially reasonable terms, if at all. We also face competition in our search for third parties to assist us with the sales and marketing efforts. If any future collaborators do not commit sufficient resources to commercialize our product candidates, if approved, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business.

Our efforts to educate the medical community and third-party payors on the benefits of VYJUVEK or our product candidates, if approved, may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our products. If VYJUVEK or any of our product candidates that are approved fails to achieve market acceptance among physicians, patients, or third-party payors, we will not be able to generate significant revenue from such product, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If the market opportunities for VYJUVEK or our product candidates are smaller than we believe they are, our product revenue may be adversely impacted, and our business may suffer.

We focus our research and product development primarily on genetic medicines to treat diseases with high unmet medical needs. We base our market opportunity estimates on a variety of factors, including our estimates of the number of people who have these diseases, the potential scope of our approved product labels, the subset of people with these diseases who have the potential to benefit from treatment with VYJUVEK or our product candidates, various pricing scenarios, and our understanding of reimbursement policies in particular countries. These estimates are based on many assumptions and may prove incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for rare indications, as epidemiological data is often more limited than for more prevalent indications and can require additional assumptions to assess potential patient populations. For example, as we commercialize VYJUVEK in the United States and abroad and learn more about market dynamics and engage with regulators on additional potential marketing approvals, our view of VYJUVEK's initial potential market opportunity will become more refined. The addressable patient population in the United States and internationally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with VYJUVEK or our product candidates, if approved, or may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations, and prospects. If we are unable to successfully commercialize VYJUVEK or any future product candidates with attractive market opportunities, our future product revenue may be smaller than anticipated, and our business may suffer.

Further, there are several factors that could contribute to making the actual number of patients who receive VYJUVEK less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment may diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

In addition to determining market opportunities for our products, we need to accurately forecast demand and the timing of the demand, which is difficult. Incorrect demand estimates could adversely impact our business, financial condition, results of operations, and prospects. For example, if product demand is higher than we initially estimate, we may need to spend time and money increasing our manufacturing capabilities and/or changing our manufacturing processes. This could require greater capital expenditures than initially forecasted and potentially delay commercial availability of an approved product, which could adversely affect our business, financial condition, and results of operations.

The commercial success of VYJUVEK and our product candidates will depend upon their degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the requisite approvals from the FDA in the United States, from the European Commission in the European Union, and the MHLW in Japan, and other regulatory authorities internationally (and potential approvals of any of our product candidates by regulatory authorities), the commercial success of VYJUVEK and our product candidates will depend, in part, on the acceptance of physicians, patients, and health care payors of gene therapy products in general, and VYJUVEK and our product candidates, in particular, as medically necessary, cost-effective, and safe. VYJUVEK and any product candidate that we commercialize may not gain acceptance by physicians, patients, health care payors, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become consistently profitable. The degree of market acceptance of VYJUVEK and our genetic medicine product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of VYJUVEK and our product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of VYJUVEK and our product candidates over alternative treatments, if available;
- the cost of VYJUVEK and our product candidates relative to alternative treatments if any are available;
- the clinical indications for which VYJUVEK and our product candidates are approved by the FDA and other regulatory authorities;
- identification of patients and the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the European Commission, the MHLW, or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products and their ability to meet market demand;
- publicity concerning VYJUVEK and our product candidates or competing products and treatments;
- any restrictions on the use of VYJUVEK and our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

Even if a product candidate displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for VYJUVEK and our product candidates, if approved, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement of pharmaceuticals may be increasingly restricted both in the United States and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Drug pricing by pharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the United States and abroad. Government and private third-party payors have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the United States. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payors, which may render VYJUVEK or our product candidates, if approved, not commercially viable or may adversely affect our anticipated future revenue and gross margins.

We cannot predict the extent to which our business may be affected by proposed health care reforms and cost reductions or potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of drugs or biologics generally could restrict the amount that we are able to charge for VYJUVEK or our product candidates, if approved, which would adversely affect our anticipated revenue and results of operations.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for VYJUVEK or our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford our approved genetic medicine products. Accordingly, sales of VYJUVEK and our product candidates, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of our product or product candidates will be paid by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a 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.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical, and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates, if approved. Even if coverage is provided, the coverage may be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property protection, manufacture, sale, and distribution expenses, and therefore, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved drug products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as our product candidates. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations. Increasing emphasis on cost-containment initiatives in the European Union and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. For example, the European Union member states operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product to currently available therapies. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many counties outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. Some countries outside the United States allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our approved products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for VYJUVEK or any of our product candidates.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, such as Average Sales Price, Average Manufacturer Price, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover product candidates that we are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, additional legislative changes, statements by elected officials, and administrative changes. The downward pressure on healthcare costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

Ethical, legal, and social issues related to genetic testing may reduce demand for our product candidates, if approved.

Prior to receiving VYJUVEK, patients are required to undergo genetic testing, and we anticipate that prior to receiving certain of our other product candidates, if approved, patients may be required to undergo genetic testing. Genetic testing has raised concerns regarding the appropriate utilization and the confidentiality of information provided by genetic testing. Genetic tests for assessing a person's likelihood of developing a chronic disease have focused public attention on the need to protect the privacy of genetic information. For example, concerns have been expressed that insurance carriers and employers may use these tests to discriminate based on genetic information, resulting in barriers to the acceptance of genetic tests by consumers. Concerns have also been raised about the accuracy of genetic testing. This could lead to governmental authorities restricting genetic testing or calling for additional regulation of genetic testing, particularly for diseases for which there is no known cure. Any of these scenarios could decrease demand for VYJUVEK and our product candidates, if approved.

Increasing demand for compassionate use or expanded access of our unapproved therapies could negatively affect our reputation and harm our business.

We are developing our product candidates principally for illnesses for which there are currently limited or no available therapeutic options. At least one other company has been the target of disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide our product candidates under an expanded access corporate policy, our reputation may be negatively affected, and our business may be harmed. In 2018, the Right to Try Law was enacted, allowing eligible patients to request access to certain investigational drugs, including biologics, that have not been FDA approved. New and emerging legislation regarding expanded access to unapproved drugs for life-threatening illnesses could negatively impact our business in the future.

A possible consequence of both activism and legislation in this area is the need for us to initiate an unanticipated expanded access program or to make our product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and have exhausted all other available therapies. The risk for serious adverse events in this patient population is high which could have a negative impact on the safety profile of our product candidates if we were to provide them to these patients in accordance with our expanded access corporate policy, which could cause significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business. If we were to provide patients with our product candidates under our expanded access corporate policy, we may in the future need to restructure or pause ongoing compassionate use and/or expanded access programs in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of our product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain adequate United States and foreign patent protection for VYJUVEK, our current product candidates, and any future product candidates we may develop, and/or our vector platform, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technologies similar or identical to ours, and our ability to successfully commercialize VYJUVEK, our current product candidates, any future product candidates we may develop, and our platform technologies may be adversely affected.

Our success depends, in large part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our approved product, current product candidates, additional product candidates in our pipeline,

and current and future innovations related to our vector platform. The patent prosecution process is expensive, time-consuming, and complex; we may not be able to file, prosecute, maintain, and/or enforce all necessary or desirable patent applications and issued patents at a reasonable cost or in a timely manner.

Even if we are granted the patents we are currently pursuing, they may not issue in a form that will provide us with the full scope of protection we desire, they may not prevent competitors or other third parties from competing with us, and/or they may not otherwise provide us with a competitive advantage. Our competitors, or other third parties, may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Moreover, our patent estate does not preclude third parties from having intellectual property rights that could interfere with our freedom to use our platform, including for our intended indications. Even assuming patents issue from our pending and future patent applications, changes in either the patent laws or interpretation of the patent laws in the United States and foreign jurisdictions may diminish the value of our patents or narrow their scope of protection.

We also may not be aware of all third-party intellectual property rights potentially relating to technologies similar to our own. Publications of discoveries in the scientific literature often lag their actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after earliest priority date or, in some cases, not at all until patents are issued. Therefore, it is impossible to be certain that we were the first to develop the specific technologies as claimed in any owned patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

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

Filing, prosecuting, and defending patents on VYJUVEK, our product candidates, and current and future innovations related to our vector platform, in all countries throughout the world would be prohibitively expensive, and intellectual property rights in some countries outside the United States may differ in scope from those eventually granted in the United States. Thus, in some cases, we may not have the opportunity to obtain patent protection for certain technologies in some jurisdictions outside the United States. 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. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we do pursue patent protection. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with VYJUVEK and our product candidates that are approved, and our patents 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. Such challenges in enforcing rights in these countries could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our current and future patent rights in foreign jurisdictions could result in substantial costs and may divert our efforts and attention from other aspects of our business; could put our patents at risk of being invalidated or interpreted narrowly; could put any future patent applications, including continuation and divisional applications, at risk of not issuing; and could provoke third parties to assert claims against us. We may not prevail in any lawsuits, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce any intellectual property rights around the world stemming from intellectual property that we develop may be inadequate to obtain a significant commercial advantage in these foreign jurisdictions.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability (and the ability of any potential future collaborators) to market and sell VYJUVEK and to develop, manufacture, market, and sell our product candidates for which marketing approvals are granted, and to freely use our proprietary technologies without infringing the rights and intellectual property of others. Many companies and institutions have filed, and continue to file, patent applications related to various aspects of gene therapy. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing, and can be revised before issuance, there may be applications now pending which may later result in issued patents that a third-party asserts are infringed by the manufacture, use, sale, or importation of VYJUVEK or any of our product candidates, if approved. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to VYJUVEK, our product candidates, or related technologies, including, for example, interference proceedings, post grant review challenges, and *inter partes* review before The United States Patent and

Trademark Office. Our competitors or other third parties may assert infringement claims against us, alleging that our products, manufacturing methods, formulations or administration methods are covered by their patents. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue, and against whom our patent portfolio may therefore have no deterrent effect.

There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patents or other intellectual property rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize VYJUVEK or any of our product candidates, if approved. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. In such a hypothetical situation, there is no assurance that a court of competent jurisdiction would find that our product, product candidates, or technologies do not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcomes are uncertain. If we are found, or believe there is a risk that we may be found, to infringe a third-party's valid and enforceable intellectual property rights, we could be required (or may choose) to obtain a license from such a third-party to continue developing, manufacturing and marketing our approved product, product candidates, and technologies. However, we may not be able to obtain any required license on commercially reasonable terms, if at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and further, it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing product or technologies. We also could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property rights. A finding of infringement could prevent us from manufacturing and commercializing our products and technologies or force us to cease some or all our business operations. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations, and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming. Competitors may infringe our current or future patents, should such patents issue, or we may be required to defend against claims of infringement or other unauthorized use of intellectual property. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially and adversely impact our financial results and reduce the resources available for development activities or any future sales, marketing, or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing, misappropriating, or successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

We have been subject to claims asserting that we, our employees, or our advisors have wrongfully used or disclosed alleged trade secrets of other parties, and we may face such claims in the future or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including potential competitors, and we have and may in the future enter into agreements providing us with rights to intellectual property of third parties for limited purposes. Although we endeavor to observe the terms of agreements under which we obtain access to third-party intellectual property and to ensure that our employees and advisors do not use the proprietary information or know-how of others in their work for us, we have been in the past, and may be again in the future, subject to claims that these individuals, or we, have used or disclosed intellectual property, including trade secrets or other proprietary information, of third parties or the current or former employers of employees or

advisors. If we fail to successfully defend any such claims, in addition to paying monetary damages, we may be subject to an injunction and may lose valuable intellectual property rights or personnel. Moreover, any such litigation, or the threat thereof, may adversely affect our ability to hire new employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize VYJUVEK or our product candidates, if approved, which could have an adverse effect on our business, results of operations, and financial condition. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

While it is our policy to require our employees and contractors who may be involved in the conception of intellectual property to execute agreements assigning such intellectual property rights to us, unforeseen complications may arise when fully and adequately executing such an agreement with each party who, in fact, conceives of intellectual property that we regard as our own. Examples of such complications may include, for example, when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached. Such complications may lead to us being forced to bring claims against third parties or current and former employees, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing obligations to a third-party, such as an academic institution, and thus an agreement with us may be insufficient in fully perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property may have a material adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect VYJUVEK or our product candidates.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. For example, in September 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act included several significant changes to U.S. patent law, including provisions that affected the way patent applications are prosecuted, and altered strategies regarding patent litigation. These provisions also switched the United States from a "first-to-invent" system to a "first-to-file" system, allowed third-party submissions of prior art to the United States Patent and Trademark Office ("USPTO") during patent prosecution, and set forth additional procedures to attack the validity of a patent through various post grant proceedings administered by the USPTO. As patent reform legislation can inject serious uncertainty into the patent prosecution and litigation processes, it is not clear what impact future patent reform legislation will have on the operation of our business. However, such future legislation, and its implementation, could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Moreover, the patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain given the ever evolving and constantly shifting nature of precedential patent cases decided by both the U.S. Court of Appeals for the Federal Circuit and the U.S. Supreme Court. We cannot assure you that our efforts to seek patent protection for our technology, commercial product, and product candidates will not be negatively impacted by future court decisions or changes in guidance or procedures issued by the USPTO. These decisions, and any guidance issued by the USPTO (or changes thereto), could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property rights in the future.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Although we have registered certain of our trademarks and trade names, they may be challenged, infringed, circumvented, or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which are important for building name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. There also could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trade names that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to patents, trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.

The degree of current and 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 gene therapy products that are similar to our approved product or any of our product candidates but that are not covered by the claims of our current patents, or of patents that we may own or license in the future;
- we, or any license partners or collaborators, may not have been the first to file patent applications covering certain aspects of the relevant products or technologies;
- others may independently develop similar or alternative technologies, or duplicate any of our technologies, that may fall outside the scope of our current or future issued patent claims and therefore may not infringe our intellectual property rights;
- it is possible that our pending or future patent applications may not result in issued patents;
- issued patents to which we currently hold rights or to which we may hold rights in the future may be held invalid or unenforceable, including as a result of legal challenges by third parties or our competitors;
- others may have access to any future intellectual property rights licensed to us on a non-exclusive basis;
- our competitors might conduct research and development activities in countries where we do not have or pursue 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 or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent application covering certain of our trade secrets or know-how, and a third-party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations, and prospects.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred net losses in the past and may not sustain profitability.

We have generated net income for each of the previous three years starting with the year ended December 31, 2023; however, we previously incurred recurring losses and negative cash flows from operations since our inception. Our transition to consistent operating profitability depends on our ability to (i) successfully commercialize VYJUVEK in the United States and abroad, and (ii) complete the development of, and obtain the regulatory approvals necessary to successfully commercialize our product candidates with significant market potential. We have devoted substantially all our efforts to date to (i) research and development of our gene therapy platform, product candidates and our manufacturing infrastructure, and, more recently, (ii) commercializing VYJUVEK in the United States, European Union, and Japan. We expect to continue to incur significant expenses for the foreseeable future and our operating results may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- manufacture, market, and sell our lead product, VYJUVEK, in the United States and market and sell VYJUVEK in the EU and Japan;
- continue our research, preclinical studies, and the clinical development of our current product candidates, including our current clinical trials and planned clinical trials;
- initiate preclinical studies and clinical trials for any additional product candidates;
- prepare for regulatory approvals for our product candidates in the United States, EU, Japan, and in other key geographies;
- continue to operate our in-house commercial-scale CGMP manufacturing facilities, ANCORIS and ASTRA, and as we seek to obtain FDA approval for commercial manufacture of VYJUVEK at ASTRA, which approval may not be granted;

- manufacture material for commercial sales of VYJUVEK and clinical trials or potential commercial sales of our product candidates;
- further develop our gene therapy platform;
- further establish our sales, marketing, and distribution infrastructure to commercialize VYJUVEK and product candidates for which we may obtain marketing approval;
- develop, maintain, expand, and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

To sustain profitability over the long term, we must be successful in a range of challenging activities, including designing, initiating, and completing clinical trials for our product candidates, developing, validating, and maintaining commercial scale manufacturing processes, obtaining marketing approvals, manufacturing, marketing, and selling VYJUVEK and any product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. If we are required to discontinue the development of any of our product candidates, if VYJUVEK does not receive regulatory approval outside the United States, the EU, or Japan, if any of our product candidates do not receive regulatory approvals, or if VYJUVEK or any of our product candidates, if approved, fail to achieve sufficient market acceptance, our ability to remain profitable and our business prospects and financial condition could be materially adversely affected.

We currently have one commercial product, VYJUVEK, approved by the FDA, the European Commission, and MHLW and several product candidates in the clinical trials stages. However, we may never develop, acquire or in-license additional product candidates. We may never generate revenue from any of our product candidates. Even with sustained net income in recent periods, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to sustain profitability would decrease the value of our company and could impair our ability to maintain our research and development efforts, expand our business, raise capital, or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment.

Because of the numerous risks and uncertainties associated with development of our product candidates, we are unable to accurately predict the timing or amount of increased expenses. If we are required by the FDA, the EMA, the European Commission, the MHLW, or other regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of our product candidates, our expenses could increase and the timing or amount of revenue from product candidates in development could be adversely affected.

We may need to raise additional funding to maintain and expand our commercialization capabilities and to complete the development of, and obtain the regulatory approvals necessary to, commercialize our product candidates. Such funding may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate certain of our product development efforts or other operations.

To complete the process of obtaining regulatory approval for our product candidates and to continue building the manufacturing, sales, marketing, and distribution infrastructure that we believe is or will be necessary to successfully commercialize VYJUVEK and our product candidates, if approved, we may require substantial additional funding. We expect to continue to incur significant expenses related to sales, medical affairs, marketing, manufacturing, and distribution of VYJUVEK in the United States and abroad. In addition, if we obtain marketing approval for our product candidates, we expect to incur significant additional expenses related to product sales, medical affairs, marketing, manufacturing, and distribution. We may need additional funding to complete the development of our product candidates and to commercialize any such approved products. Our future capital requirements will depend on many factors, including:

- the ability of VYJUVEK to generate sufficient revenue;
- the costs of product sales, medical affairs, marketing, manufacturing, and distribution for VYJUVEK;
- the progress, timing, results, and costs of our current and planned clinical trials;
- the continued development and the filing of IND applications for our product candidates;
- the initiation, scope, progress, timing, costs, and results of drug discovery, laboratory testing, manufacturing, preclinical studies, and clinical trials for any product candidates that we may pursue in the future;
- the costs of maintaining our own commercial-scale CGMP manufacturing facilities;
- the outcome, timing, and costs of seeking regulatory approvals for any of our product candidates;

- the costs associated with the manufacturing process development and evaluation of third-party suppliers or manufacturers, if necessary;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, in the event we receive marketing approval for any of our current and future product candidates;
- the extent to which the costs of our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers, and other third-party payors;
- subject to receipt of marketing approval, if any, revenue received from commercial sales of our current and future product candidates;
- the terms and timing of any current or future collaborations, distribution, licensing, consulting, or other arrangements;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense, and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements, if any;
- the terms of our license agreements, if any, and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Identifying product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales for our product candidates currently in development or future product candidates. Revenue will be derived from VYJUVEK until we have another product candidate receive marketing approval. Accordingly, we may need to rely on additional financing to achieve our business objectives. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance may cause the market price of our common stock to decline. The sale of additional equity or convertible securities would dilute all of our existing stockholders. Existing stockholders may not agree with our financing plans or the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all. The terms of additional financing may be impacted by, among other things, general market conditions, the market's perception of our approved product, VYJUVEK, and product candidates, our growth potential, and the market price per share of our common stock. See "Raising additional capital could cause the price of our common stock to decline and cause dilution to our stockholders, restrict our operations or require us to relinquish rights." Failure to obtain necessary capital when needed could force us to delay, limit, or terminate certain of our product development efforts or other operations, which could significantly harm our business, financial condition, results of operations, and prospects.

Changes in tax law may adversely affect our business and financial condition

We are subject to evolving and complex tax laws in the United States and the foreign jurisdictions in which we operate. New income, sales, use or other tax laws, statutes, rules, regulations, or ordinances could be enacted at any time, or interpreted, changed, modified, or applied adversely to us, any of which could adversely affect our business operations and financial performance. Changes to tax laws (which could apply retroactively) could adversely affect us and our stockholders. In recent years, such changes have been made and changes are likely to occur in the future, which could have a material adverse effect on our business, cash flow, financial condition, and results of operations.

Our ability to use our net operating loss carryforwards and certain tax credit carryforwards may be subject to limitation.

The Company fully utilized its federal net operating losses in 2025. We have state net operating loss carryforwards, which are available to reduce future taxable income. These state net operating losses will begin to expire in 2037. We also have federal research and development tax credits and federal orphan drug tax credits which may be used to offset future tax liabilities and expire beginning in 2042.

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, changes in our ownership may limit the amount of our net operating loss carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire and could have a material adverse effect on our results of operations in future years.

Risks Related to Ownership of Our Common Stock

Our Chief Executive Officer and Chairman of the Board of Directors and our Founder, President, Research & Development and Director have the ability to substantially influence all matters submitted to stockholders for approval.

Krish S. Krishnan and Suma M. Krishnan, our Chief Executive Officer and Chairman of the Board and our Founder, President, Research & Development and Director, respectively, in the aggregate, collectively beneficially own over 10% of our outstanding common stock. As a result, they will be able to substantially influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, they could influence the election of directors and approval of any merger, consolidation, or sale of all or substantially all our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in the management of our company by persons with whom our other stockholders disagree.

If securities analysts publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us or our business. If securities analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Raising additional capital could cause the price of our common stock to decline and cause dilution to our stockholders, restrict our operations, or require us to relinquish rights.

In the future, we may need to finance our cash needs through a combination of private and public equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We may issue additional common stock or restricted securities as part of such financing activities and any such issuances may have a dilutive effect on our then-existing stockholders. Sales of substantial amounts of our common stock in the open market, or the availability of such shares for sale, could adversely affect the price of our common stock.

The incurrence of indebtedness would result in fixed payment obligations and a portion of our operating cash flows being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell, or license intellectual property rights, and other operating restrictions that could adversely impact our ability to conduct our business.

If we are unable to raise additional funds through equity or debt financings when needed, and instead raise additional capital through marketing and distribution agreements or other collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our current and future product candidates, technologies, future revenue streams, or discovery programs or grant licenses on terms that may not be favorable to us.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

The price of our common stock has been and is likely to continue to be volatile. The stock market in general and the market for biotechnology and pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of such companies. As a result of this volatility, a stockholder may not be able to sell their common stock at or above the price that they paid for it. The market price of our common stock may be influenced by many factors, including:

- our ability to continue to successfully sell VYJUVEK in the United States and internationally;
- our ability to successfully proceed to and conduct clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- our ability to obtain regulatory approval for our product candidates and our ability to successfully commercialize any of our approved product candidates;

- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to VYJUVEK and any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire, or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines, or recommendations by securities analysts;
- our inability to manufacture adequate product supply for VYJUVEK and any other approved product or inability to do so at acceptable costs;
- disputes or other developments relating to proprietary rights, including patent applications, and issued patents;
- our ability to obtain patent protection for our product candidates and technologies;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry, and market conditions; and
- the other factors described in this "Risk Factors" section.

If we fail to maintain effective internal control over financial reporting, we may not be able to accurately report our financial results, which may adversely affect investor confidence in our company and, as a result, the value of our common stock.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting and is required to have an independent auditor assess the effectiveness of our internal control over financial reporting, pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, as amended (the "Sarbanes-Oxley Act"). We cannot provide any assurance that material weaknesses will not be identified in connection with our compliance with the provisions of Section 404 of the Sarbanes-Oxley Act. The existence of any material weakness would preclude a conclusion by management and our independent auditors that we maintained effective internal control over financial reporting. Our management may be required to devote significant time and expense to remediate any material weaknesses that may be discovered and may not be able to remediate any material weakness in a timely manner. The existence of any material weakness in our internal control over financial reporting could also result in errors in our financial statements that could require us to restate our financial statements, cause us to fail to meet our reporting obligations, and cause investors to lose confidence in our reported financial information, all of which could lead to a decline in the market price of our common stock.

Provisions in our certificate of incorporation and bylaws, as well as provisions of Delaware law, could make an acquisition of our company that may be beneficial to our stockholders more difficult and could prevent or deter our stockholders from replacing or removing our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay, or prevent a merger, acquisition, or other change in control of us that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 80% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

We have broad discretion in the use of our cash, cash equivalents, and marketable securities and may not use them effectively.

Our management has broad discretion in the application of our cash, cash equivalents, and marketable securities and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline, and delay the development of our product candidates. Pending their use, we may invest our cash and cash equivalents in a manner that does not produce income or that results in a loss of value.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid cash dividends on our common stock. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Issuing additional shares of our common stock could cause the price of our common stock to decline and cause dilution to our stockholders.

To the extent we raise additional capital by issuing additional shares of our common stock, or securities convertible into or exchangeable or exercisable for common stock, our existing stockholders may experience substantial dilution. Additionally, if we issue additional shares of our common stock or instruments convertible into our common stock, the trading price of our common stock could decline. We cannot predict whether we will raise additional capital by issuing shares of our common stock, or securities convertible into or exchangeable or exercisable for common stock, the size of any future issuances, or the effect, if any, that they may have on the market price for our common stock. We also have stock options, restricted stock units, and performance-based restricted stock units outstanding, and we expect to issue additional equity awards to directors and employees. The issuance of restricted common stock, common stock upon exercise of outstanding options, common stock upon vesting of restricted stock units, or common stock upon vesting of performance-based restricted stock units would be dilutive and may cause the market price for our common stock to decline. If we issue preferred stock in the future, the holders of that preferred stock could gain rights superior to our holders of common stock, such as liquidation and other preferences, or the market price of our common stock could be adversely affected.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity.

Cybersecurity represents a critical component of our overall approach to risk management. Our cybersecurity policies, standards, and practices are integrated into our enterprise risk management approach, and cybersecurity risks are among the core enterprise risks that are subject to oversight by our Board of Directors (the "Board"). We generally approach cybersecurity threats through a cross-functional, multilayered approach, with specific goals of: (i) identifying, preventing and mitigating cybersecurity threats to the Company; (ii) preserving the confidentiality, security and availability of the information that we collect and store; (iii) protecting the Company's intellectual property; (iv) maintaining the confidence of our employees, patients, HCPs, suppliers, and other third parties; and (v) providing appropriate public disclosure of cybersecurity risks and incidents when required. Our cybersecurity program is developed using industry standards and best practices as a guide, including the National Institute of Standards and Technology ("NIST") Cybersecurity Framework. The NIST framework provides us with a common language and structure for identifying, assessing, and managing cybersecurity risks across our organization.

Cyber Security Risk Management and Strategy

Our cybersecurity program focuses on the following areas:

- Vigilance: Our cybersecurity threat operations function 24/7 with the specific goal of identifying, preventing and mitigating cybersecurity threats and responding to cybersecurity incidents in accordance with our established incident response and recovery plans.
- Systems Safeguards: We deploy systems safeguards that are designed to protect the Company's information systems from cybersecurity threats, including firewalls, intrusion prevention and detection systems, anti-malware functionality and access controls, which are evaluated and improved through ongoing vulnerability assessments and cybersecurity threat intelligence.
- Third-Party Risk Management: We maintain a comprehensive, risk-based approach to identifying and overseeing cybersecurity risks presented by third parties, including vendors, service providers and other external users of the Company's systems, as well as the systems of third parties that could adversely impact our business in the event of a cybersecurity incident affecting those third-party systems.
- Training: We provide periodic mandatory training for personnel regarding cybersecurity threats, which reinforces the Company's information security policies, standards and practices.
- Incident Response and Recovery Planning: We have established and maintain incident response and recovery plans that address the Company's response to a cybersecurity incident and the recovery from a cybersecurity incident, and such plans are tested and evaluated on an regular basis.
- Communication, Coordination and Disclosure: We utilize a cross-functional approach to address the risk from cybersecurity threats, involving management personnel from the Company's technology, operations, legal, financial, and other key business functions, as well as the members of the Board in an ongoing dialogue regarding cybersecurity threats and incidents, while also implementing controls and procedures so that decisions regarding the disclosure and reporting of such incidents can be made by management in a timely manner.

A part of our strategy for managing risks from cybersecurity threats is assessment and testing of the effectiveness of our cybersecurity measures. We engage third parties to perform assessments on our cybersecurity measures, including annual penetration testing, and we adjust our cybersecurity measures as necessary based on the information provided by the assessments.

Governance

The Board oversees the management of risks from cybersecurity threats. The Board receives regular presentations and reports on cybersecurity, which address a wide range of topics and also receives prompt and timely information regarding any cybersecurity incident that is or may become material, as well as ongoing updates regarding such incident until it has been addressed. At least once each year, the Board discusses the Company's approach to cybersecurity risk management with the Company's Executive Director of Information Technology Operations and Information Security, who is the member of management that is principally responsible for overseeing cybersecurity at the Company, in partnership with other business leaders across the Company, including our Chief Executive Officer, Chief Accounting Officer, General Counsel, and Human Resources leader. Our Senior Director of Cyber Security has served in various roles in information technology and information security for over 20 years, including serving as Information Security Officer for a global medical device manufacturer. He holds degrees in Global Business Management and Computer Science.

Cybersecurity threats, including as a result of any previous cybersecurity incidents, have not materially affected or are reasonably likely to affect the Company, including its business strategy, results of operations, or financial condition.

Item 2. Properties.

As of December 31, 2025, we leased approximately 67,000 square feet of combined laboratory and office space in Pittsburgh, Pennsylvania that we use for our research, development and manufacturing efforts. The lease for combined laboratory and office space expires in October 2031.

In 2021, we completed the acquisition of the building shell for ASTRA, our second commercial scale CGMP facility located outside of Pittsburgh, PA, and entered into an associated ground lease. Astra is an approximately 155,000 sq. ft., state-of-the-art CGMP manufacturing facility that, in addition to adding significant capacity to support our growing pipeline, also allows for in-house incorporation of raw material preparation, excipient manufacturing, testing, packaging, labeling and distribution, thereby fully integrating all components of the supply chain from starting materials to patient experience. ASTRA was completed and validated in 2023.

As of December 31, 2025, we also leased international office space in Switzerland, Netherlands, France, Germany, Japan, Italy, and Spain.

Item 3. Legal Proceedings.

The information set forth in Note 7 of the Notes to the Consolidated Financial Statements included in Part II Item 8 of this Annual Report on Form 10-K is incorporated by reference into this Item 3.

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.

Market Information

Our common stock trades on the Nasdaq Global Select Market under the symbol "KRYS."

Holders of Record

As of February 11, 2026, there were four stockholders of record of our common stock. The actual number of stockholders 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 and other nominees. This 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 cash dividends on our capital stock. We currently intend to retain any future earnings for use in the operation and growth of our business and do not intend to declare or pay any cash dividends in the foreseeable future. Any further determination to pay dividends on our capital stock will be at the discretion of our Board, subject to applicable laws, and will depend on our financial condition, results of operations, capital requirements, general business conditions and other factors that our Board considers relevant.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about securities authorized for issuance under existing equity compensation plans is incorporated by reference from Part III, Item 12 of this Annual Report on Form 10-K.

Purchases of Equity Securities by the Issuer or Affiliated Purchasers

There were no repurchases of shares of common stock made during the three months ended December 31, 2025.

Sales of Unregistered Securities

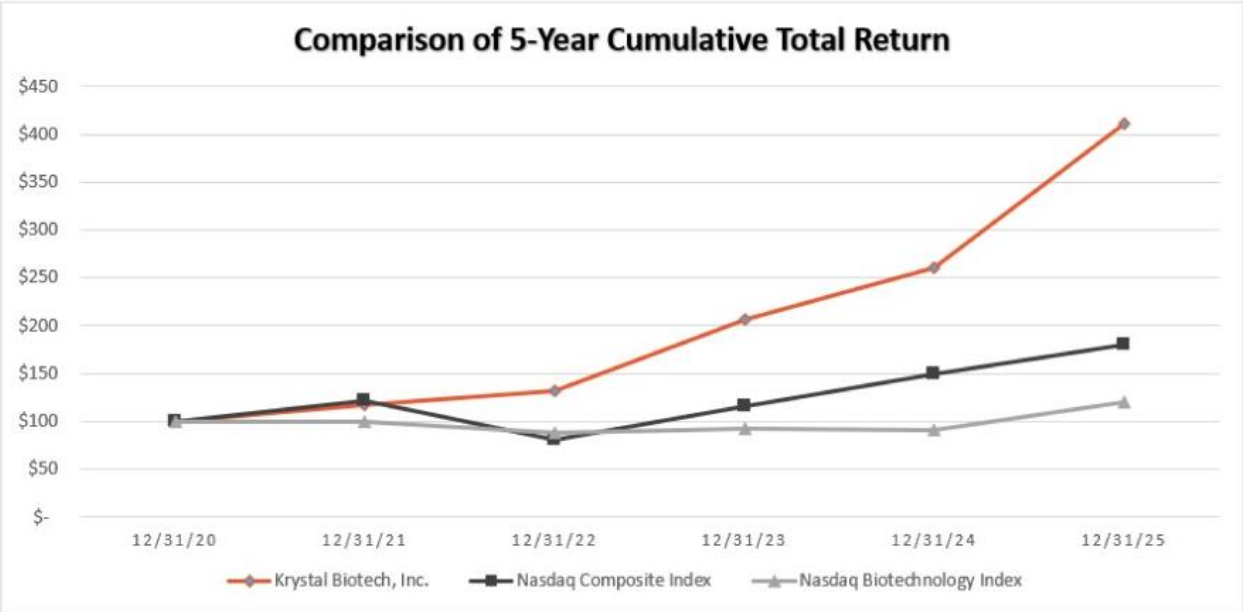
There have been no sales of unregistered securities by us during the past three years except as previously disclosed on prior Quarterly Reports on Form 10-Q and Current Reports on Form 8-K.

Stock Performance Graph

Set forth below is a graph comparing the cumulative total return on an indexed basis of a $100 investment in the Company's common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index commencing on December 31, 2020 and continuing through December 31, 2025. The graph assumes our closing sale price on December 31, 2020 of $60.00 per share as the initial value of our common stock for indexing purposes. Points on the graph represent the performance as of the last business day of each of the months indicated.

The comparisons shown in the graph below are based upon historical data. We caution that the stock price performance shown in the graph below is not necessarily indicative of, nor is it intended to forecast, the potential future performance of our common stock.

This performance graph shall not be deemed "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Exchange Act or incorporated by reference into any filing of Krystal Biotech, Inc. under the Securities Act or the Exchange Act, except to the extent we specifically incorporate it by reference into such filing. The past performance of our common stock is no indication of future performance.


Comparison of 5-Year Cumulative Total Return
$450
$400
$350
$300
$250
$200
$150
$100
$50
$-
12/31/20
12/31/21
12/31/22
12/31/23
12/31/24
12/31/25
Krystal Biotech, Inc.
Nasdaq Composite Index
Nasdaq Biotechnology Index

Item 6. [Reserved]

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

The following information should be read in conjunction with the consolidated financial statements and related notes thereto included in this Annual Report on Form 10-K. In addition to historical information, this report contains forward-looking statements that involve risks and uncertainties. We encourage you to review the risks and uncertainties discussed in the sections entitled Item 1A. "Risk Factors" and "Forward-Looking Statements" included at the beginning of this Annual Report on Form 10-K. The risks and uncertainties can cause actual results to differ materially from those forecast in forward-looking statements or implied in historical results and trends. We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the U.S. Securities and Exchange Commission, or SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

This section of this Annual Report on Form 10-K generally discusses 2025, 2024 and 2023 items and year-to-year comparisons between 2025 and 2024, and 2024 and 2023 of the Company's results of operations and cash flows.

Overview

We are a fully integrated, commercial-stage, global biotechnology company focused on the discovery, development, manufacturing, and commercialization of genetic medicines to treat diseases with high unmet medical needs. Using our patented gene therapy technology platform that is based on engineered herpes simplex virus-1 ("HSV-1"), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell's own machinery then transcribes and translates the transgene to treat the disease. Our vectors are amenable to formulation for non-invasive or minimally invasive routes of administration at a healthcare professional's office or in the patient's home. Our innovative technology platform is supported by two in-house, commercial scale Current Good Manufacturing Practice ("CGMP") manufacturing facilities.. Refer to *Part I, Item 1 - Business* for more information about our commercial product, VYJUVEK®, clinical development pipeline and research programs, and the status of our product candidates.

Our Commercial Product

VYJUVEK (beremagene geperpavec-svdt or B-VEC)

VYJUVEK is a non-invasive, topical, redosable gene therapy approved in the United States, Europe, and Japan for the treatment of DEB, a rare and severe monogenic disease that affects the skin and mucosal tissues and is caused by one or more mutations in a gene called *COL7A1*. VYJUVEK is designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds, providing the patient's skin cells the template to make normal type VII collagen protein and thereby addressing the fundamental disease-causing mechanism.

We are commercializing VYJUVEK directly in the United States, major European markets, and Japan. We launched VYJUVEK in the United States in 2023, in Germany in August 2025, and in France and Japan in October 2025. The launch in France is under the post-marketing authorization early reimbursed access Accès Précoce program.

Pricing negotiations are underway in both Germany and France and are expected to continue until at least the second half of 2026 in Germany and 2027 in France. Pricing negotiations were successfully completed in Japan prior to launch.

We are advancing pricing discussions with Italian reimbursement authorities to enable a potential launch in Italy in the second half of 2026. We are also preparing regulatory filings for the United Kingdom and Switzerland, as well as initiating pricing discussions with relevant authorities in other key Western European markets. The timing of additional European launches will depend on the cadence and outcomes of regulatory interaction and pricing negotiations.

We continue to expand our specialty distributor network to support the commercialization of VYJUVEK in territories outside of the United States, major European markets, and Japan.

Net VYJUVEK product revenue was $389.1 million for the year ended December 31, 2025. Since launch in August 2023, we have reported cumulative net product revenue of $730.3 million.

Gross margin for the year ended December 31, 2025 was 94%. We define gross margin as product revenue, net less cost of goods sold expressed as a percentage of product revenue, net.

Pipeline

Respiratory

KB407 for Cystic Fibrosis ("CF")

KB407 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the full-length cystic fibrosis transmembrane conductance regulator ("CFTR") transgene for the treatment of CF, a serious rare lung disease caused by missing or mutated CFTR protein. In January 2026, we announced a positive interim clinical update from Cohort 3, the highest dose cohort of CORAL-1, our Phase 1 study evaluating KB407 for the treatment of patients with CF, regardless of their underlying genotype. We are working on the study design for CORAL-3, a clinical study intended to evaluate the safety and efficacy of repeat KB407 administration, including through regular assessments of lung function by spirometry, and to support potential registration. We expect to align on the CORAL-3 study design with the FDA and start enrollment in the potentially registrational CORAL-3 study in the first half of 2026. Additional details on the study design will be provided by the time of study initiation.

KB408 for Alpha-1 Antitrypsin Deficiency ("AATD") Lung Disease

KB408 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the *SERPINA1* transgene, that encodes for normal human alpha-1- antitrypsin ("AAT") protein, for the treatment of AATD, a serious rare lung disease. We are currently running a a Phase 1 study, SERPENTINE-1, evaluating KB408 for the treatment of AATD in adult patients with AATD with a Pi*ZZ or a Pi*ZNull genotype. We expect to report interim safety and *SERPINA1* transgene delivery data from the repeat dose cohort of SERPENTINE-1 in 2026.

Ophthalmology

KB803 (Ophthalmic B-VEC) for Ocular Complications of DEB

KB803 is a redosable eye drop formulation of B-VEC, designed for the treatment of ocular complications in patients with DEB. These complications, which include corneal erosions, abrasions, blistering and scarring, can lead to progressive vision loss. There is currently no corrective therapy available. We are currently running a Phase 3 registrational study, IOLITE, evaluating KB803 for the treatment of and prevention of corneal abrasions in DEB patients six months of age or older. We expect to complete enrollment in IOLITE in the first half of 2026 and report top-line results later in 2026.

KB801 for Neurotrophic Keratitis ("NK")

KB801 is an eye drop formulation of our novel HSV-1 based vector designed to deliver two transgene copies to the corneal epithelium for the sustained, localized expression and secretion of nerve growth factor and the treatment of NK, a rare, degenerative corneal disease caused by nerve damage in the eye that leads to corneal epithelial defects, ulcers, and perforation. Our engineered HSV-1 viral vector used in KB801 was granted platform technology designation by the FDA in October 2025. We are currently running a registrational, randomized, double-masked, multicenter, placebo-controlled study, EMERALD-1, evaluating KB801 for the treatment of NK. We expect to enroll approximately 60 adult patients with Stage 2 or Stage 3 NK in EMERALD-1. Enrollment is ongoing, and we expect to report top-line data in 2026.

Dermatology

KB111 for Hailey-Hailey Disease ("HHD")

KB111 is a topical gel formulation of our novel vector designed to deliver two copies of the *ATP2C1* transgene encoding the human calcium transporter ATPase type 2C member 1 ("ATP2C1") for the treatment of HHD, a serious and rare monogenic skin disorder characterized by painful rash and blistering in skin folds and linked to low ATP2C1 expression levels in keratinocytes. We are currently developing an HHD-specific evaluation scale necessary for the clinical evaluation of KB111. We expect to complete development and validation of the scale in the first half of 2026 and initiate a registrational study evaluating KB111 for the treatment of HHD in the second half of 2026.

Oncology

KB707 for Solid Tumors

KB707 is a redosable, cancer immunotherapy designed to deliver genes encoding both human interleukin-2 and interleukin-12 to the tumor microenvironment and promote systemic immune-mediated tumor clearance. Two formulations of KB707 are in development, a solution formulation for transcutaneous injection and an inhaled (nebulized) formulation for lung delivery. We have prioritized development of the inhaled KB707 formulation for the treatment of non-small cell lung cancer ("NSCLC") based on early evidence of efficacy from KYANITE-1, our ongoing, Phase 1/2 study evaluating inhaled KB707, as monotherapy or in combination to treat patients with locally advanced or metastatic solid tumors of the lung. We opened a new cohort in KYANITE-1 in late 2025 to evaluate inhaled KB707 in combination with chemotherapy in patients with advanced NSCLC. Enrollment in this cohort is ongoing, and we expect to report interim efficacy data and potential registrational study plans in 2026.

We continue to follow patients enrolled in OPAL-1, our Phase 1/2 study, evaluating intratumoral KB707, as monotherapy or in combination, for the treatment of locally advanced or metastatic solid tumors.

Aesthetics

In addition to focusing on genetic medicines to treat patients with diseases with high unmet medical needs, we are leveraging the ability of our platform to deliver proteins of interest to cells in the skin in the context of aesthetic medicine via our wholly-owned subsidiary, Jeune Aesthetics. Based on the broad aesthetic improvements observed following KB304 treatment in PEARL-2, our Phase 1 study evaluating KB304 for the treatment of wrinkles of the décolleté, we are progressing KB304 into Phase 2 study for the treatment of wrinkles of the décolleté. We have aligned with the FDA on our validated décolleté-specific photonumeric scale, and we now expect to initiate the Phase 2 study in 2027.

Jeune Aesthetics has several other aesthetic medicine product candidates in various stages of development, including our clinical-stage product candidate KB301 that is designed to deliver two copies of the *COL3A1* transgene for the treatment of aesthetic skin conditions. We are currently evaluating aesthetic indications most suitable for advanced clinical development of KB301.

Financial Overview

Product Revenue, Net

After FDA approval of VYJUVEK in May 2023, we launched VYJUVEK in the United States in 2023, in Germany in August 2025, and in France and Japan in October 2025. Our future revenue will fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any such sales.

The transaction price that we recognize as revenue for VYJUVEK sales includes an estimate of variable consideration, which may include discounts, returns, and rebates that are offered within contracts. Refer to Note 2 of the notes to the consolidated financial statements included in this Form 10-K for additional information.

Cost of Goods Sold

Cost of goods sold includes direct and indirect costs related to the manufacturing of VYJUVEK. These costs consist of manufacturing costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Cost of goods sold may also include period costs related to certain manufacturing services and inventory adjustment charges.

Prior to receiving FDA approval in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expenses.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred to advance our preclinical and clinical development programs and the development and manufacturing of our product candidates, which include:

- agreements with contract research organizations, consultants and other third parties that conduct preclinical activities, clinical trials and other research and development activities on our behalf;
- costs of acquiring, developing and manufacturing product candidates and clinical trial materials, lab supplies and consumables;
- facility costs, depreciation and other related expenses, which include expenses for rent and the maintenance of our facilities;
- other testing and support costs and supplies; and
- payroll related expenses, including stock-based compensation expense.

We expense research and development costs to operations as incurred.

We expect our research and development expenses will increase as we continue the manufacturing of preclinical and clinical materials, manage the clinical trials of and seek regulatory approval for our product candidates and as we expand our product portfolio. Due to the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration, costs and timing of clinical trials, and as a result, the actual costs to complete clinical trials may exceed the expected costs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist principally of salaries and other related costs, including stock-based compensation for personnel in our executive, finance, legal, commercial, business development, information technology and other general and administrative functions and are expensed as incurred. Selling, general and administrative expenses also include professional fees associated with corporate and intellectual property-related legal expenses, consulting and accounting services, insurance, facility-related costs and expenses associated with obtaining and maintaining patents. Other selling, general

and administrative costs include travel expenses, patient access program costs, management service fees, marketing expenses, and selling expenses which include transportation, shipping and handling fees.

We anticipate that our selling, general and administrative expenses will increase in the future relating to our commercialization efforts and to support the development of our product candidates. These increases will likely include increased costs for insurance, costs related to the hiring of additional personnel and payments to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate that we will continue to increase our salary and personnel costs and other expenses to support Vyjuvek commercialization globally.

ASTRA Capital Expenditures

In March 2021, we closed on the purchase of the building that was constructed to house our second commercial scale CGMP facility, ASTRA. In March 2023, we received the permanent occupancy permit for ASTRA which allowed the Company to begin utilizing certain parts of the building for research and development operations once qualification was completed and a portion of the assets were placed into service throughout 2023 and 2024. We incurred significant capital expenditures related to the construction of ASTRA in 2023 and expect to continue to incur capital expenditures related to ASTRA throughout the operational life of the facility.

Gains from Sale of Priority Review Voucher ("PRV")

Gain from sale of priority review voucher relates to proceeds from sale of the rare pediatric PRV we received in connection with the FDA's approval of VYJUVEK.

Interest and Other Income, Net

Interest and other income, net consists primarily of income earned from our cash, cash equivalents and investments and gains and losses on foreign currency transactions.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial position and results of operations is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, we evaluate estimates which include, but are not limited to, variable consideration associated with revenue recognition, stock-based compensation expense, accrued expenses, and the valuation allowance included as part of the net deferred tax assets calculation during the period. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances. Actual results may differ materially from those estimates or assumptions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Revenue Recognition

After FDA approval of VYJUVEK in May 2023, we began commercial marketing and made our first product sales in 3Q 2023. Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC"), Topic 606, *Revenue from Contracts with Customers* ("ASC 606") requires us to make estimates of variable consideration, included in our contracts, to be included in the transaction price.

Revenue is recognized when the Company satisfies a performance obligation by transferring control of the promised good to the customer. The only performance obligation in the Company's contracts with customers is the timely delivery of the product to the customer's designated location.

The Company sells VYJUVEK to a limited number of specialty pharmacy ("SPs") providers that mix the medication to be administered at a healthcare professional's office or in the patient's home and to a limited number of hospitals or specialty distributors ("SDs") who deliver to hospitals where patients are administered the medication in a healthcare setting. Revenue is recognized when the customer obtains control of the product, which occurs at a point in time, upon delivery to the customer.

Revenue is measured as the amount of consideration the Company expects to receive in exchange for transferring VYJUVEK and is generally based upon a list price and is recorded at the net sales price upon delivery and transfer of control to the customer, and includes an estimate of variable consideration, which results from discounts, rebates, copay assistance, and returns that are offered within contracts between the Company and its customers. These reserves, representing the Company's best estimates of the amount of consideration to which the Company is entitled, are based on the terms of the contract.

Variable consideration reduces the transaction price to reflect the Company's best estimate of the amount of consideration to which the Company is entitled based on the terms of the contracts and is recorded in the same period the

related product revenue is recognized. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, the Company will adjust these estimates in the period these variances become known.

- *Prompt Pay Discounts*: As an incentive for prompt payment, the Company may offer cash discounts to its counterparties. The Company estimates accrued prompt pay discounts using the most likely amount method. The Company expects that all eligible counterparties will comply with the contractual terms to earn the discount. The Company records the discount as a reduction of revenue on the consolidated statements of operations and as an allowance against accounts receivable, net on the consolidated balance sheets.
- *Government Rebates*: The Company participates in certain government rebate programs including Medicaid, Medicare and Tricare. For Medicare, the Company estimates the accrued liability based on the estimated number of patients in the prescription drug coverage gap under the Medicare Part D program. The Company also estimates accrued government rebates using the expected value method based on estimated percentages of VYJUVEK that will be prescribed to qualified patients, estimated rebate percentages and estimated levels of inventory in the distribution channel that will be prescribed to qualified patients and records the rebates as a reduction of revenue on the consolidated statements of operations and accrued rebates and other long-term liabilities on the consolidated balance sheets.
- *Commercial Rebates:* The Company participates in certain commercial rebate programs. Under these rebate programs, the Company pays a rebate to the commercial entity or third-party administrator of the program. Accrued commercial rebates are estimated using the expected value method based on estimated percentages of VYJUVEK that will be prescribed to qualified patients and estimated levels of inventory in the distribution channel. Accrued commercial rebates are recorded as a reduction of revenue on the consolidated statements of operations and are included in accrued rebates on the consolidated balance sheets
- *Product Returns:* The Company offers limited return rights relating only to product damage or defects identified upon receipt, and therefore the Company expects minimal returns. Returns are estimated taking into consideration several factors including these limited product return rights, historical return activity, and other relevant factors. The Company has not experienced significant product returns to date, and accordingly no allowance for returns was recorded for the year ended December 31, 2025.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and commitments, communicating with our personnel to identify services that have been performed for us and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued research and development expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. Examples of accrued research and development expenses include costs associated with services for preclinical development, manufacturing of our product candidates and the conduct of clinical trials. If actual results in the future vary from our estimates, we will adjust these estimates in the period these variances become known.

Stock-Based Compensation

We have applied the fair value recognition provisions of Financial Accounting Standards Board Accounting Standards Codification, or ASC, Topic 718, *Compensation—Stock Compensation* ("ASC 718"), to account for stock-based compensation. We recognize compensation costs related to stock-based awards granted based on the estimated fair value of the awards on the date of grant.

ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations and comprehensive income based on their grant-date fair values. Compensation expense for stock options, restricted stock awards and restricted stock units is recognized on a straight-line basis based on the grant-date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense for performance-based restricted stock units is recognized for the awards that are probable of vesting over the service period of the award. On a quarterly basis, management estimates the probable number of performance-based restricted stock units that would vest until such time that the ultimate achievement of the performance criteria are known.

Determining the amount of stock-based compensation to be recorded requires us to develop estimates of the fair value of stock-based awards as of their measurement date. We recognize stock-based compensation expense over the requisite service period, which is the vesting period of the award. Calculating the fair value of stock-based awards requires that we make

assumptions. We estimate the fair value of its stock options using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including: (i) the expected stock price volatility; (ii) the expected term of the award; (iii) the risk-free interest rate; and (iv) expected dividends.

We estimate the expected term of stock options using the "simplified" method as prescribed by SEC Staff Accounting Bulletin No. 107, *Share-Based Payments*, whereby the expected term equals the arithmetic mean of the vesting term and the original contractual term of the option. The risk-free interest rates are based on US Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid and does not expect to pay dividends in the foreseeable future. The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which service conditions are expected to be satisfied.

Income Taxes

The Company is subject to tax in the United States and numerous foreign jurisdictions. Significant judgments and estimates are required in the determination of consolidated income tax expense. Income tax expense reflects the Company's best estimate of current and future taxes to be paid. The Company records deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities using enacted tax rates expected to be in effect when the differences are expected to reverse. Valuation allowances are provided when necessary to reduce net deferred tax assets to the amount that is more likely than not to be realized. The Company weighs all available evidence, including cumulative income and forecasted future income in order to assess the realizability of deferred tax assets. The assumptions about future taxable income require the use of significant judgment and are consistent with the plans and estimates the Company is using to manage the underlying business. In the event the Company determines that it will be able to realize deferred tax assets for which a valuation allowance was used to reduce their carrying value, the adjustment to the valuation allowance will be recorded as a reduction to the provision for income taxes in the period such determination is made.

The calculation of tax liabilities involves dealing with uncertainties in the application of complex tax laws and regulations in a multitude of jurisdictions across global operations. Significant judgment is required in the identification and measurement of uncertain tax positions. The liability for unrecognized tax benefits contains uncertainties because the Company is required to make assumptions and to apply judgment to estimate the exposures associated with our various filing positions. The Company adjusts the liabilities when judgment changes as a result of new information not previously available.

Results of Operations

Years Ended December 31, 2025, 2024 and 2023

	Years Ended December 31,			Change	
(in thousands)	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Product revenue, net	$ 389,130	$ 290,515	$ 50,699	$ 98,615	$ 239,816
Operating expenses					
Cost of goods sold	23,049	20,061	3,094	2,988	16,967
Research and development	58,045	53,580	46,433	4,465	7,147
Selling, general and administrative	146,741	113,626	98,289	33,115	15,337
Litigation settlement	—	37,500	12,500	(37,500)	25,000
Total operating expenses	227,835	224,767	160,316	3,068	64,451
Income (loss) from operations	161,295	65,748	(109,617)	95,547	175,365
Other income					
Gain from sale of priority review voucher	—	—	100,000	—	(100,000)
Interest and other income, net	28,176	29,608	22,514	(1,432)	7,094
Income before income taxes	189,471	95,356	12,897	94,115	82,459
Income tax benefit (expense)	15,360	(6,197)	(1,965)	21,557	(4,232)
Net income	$ 204,831	$ 89,159	$ 10,932	$ 115,672	$ 78,227

Product Revenue, Net

Product revenue, net was $389.1 million for the year ended December 31, 2025 as compared to $290.5 million for the year ended December 31, 2024 and $50.7 million for the year ended December 31, 2023 due to sales of VYJUVEK after FDA

approval was obtained on May 19, 2023. The increase in product revenue, net from 2024 to 2025 was driven by an increase in VYJUVEK sales as compared to the prior year.

Cost of Goods Sold

Cost of goods sold was $23.0 million for the year ended December 31, 2025 as compared to $20.1 million for the year ended December 31, 2024 and $3.1 million for the year ended December 31, 2023 due to initial sales of VYJUVEK after FDA approval was obtained on May 19, 2023. The increase in cost of goods sold from 2024 to 2025 was driven by an increase in VYJUVEK sales partially offset by a decrease primarily due to manufacturing process optimizations that resulted in lower average costs per unit.

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the years ended December 31, 2025, 2024 and 2023:

	Years Ended December 31,			Change	
(in thousands)	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
B-VEC	$ 6,690	$ 8,760	$ 9,039	$ (2,070)	$ (279)
KB111	1,837	—	—	$ 1,837	$ —
KB301	184	635	485	(451)	150
KB304	960	1,342	66	(382)	1,276
KB407	1,805	1,877	1,668	(72)	209
KB408	882	1,630	1,043	(748)	587
KB707	10,856	8,677	3,828	2,179	4,849
KB801	2,175	1,314	—	861	1,314
KB803	2,564	604	—	1,960	604
Other dermatology programs	12	935	284	(923)	651
Other ophthalmology programs	44	554	71	(510)	483
Other programs	2,493	2,098	1,506	395	592
Stock-based compensation	10,375	9,237	10,051	1,138	(814)
Other unallocated expenses[(1)]	17,168	15,917	18,392	1,251	(2,475)
Research and development expense	$ 58,045	$ 53,580	$ 46,433	$ 4,465	$ 7,147

(1) Other unallocated expenses consist of shared pre-commercial manufacturing costs, primarily relating to certain raw materials, process development, quality control and quality assurance activities, as well as other manufacturing and facility related costs including rent, storage and depreciation which support the development of multiple product candidates.

Research and development expenses increased $4.5 million for the year ended December 31, 2025 compared to the year ended December 31, 2024. The increase was primarily driven by the following:

- an increase of $2.1 million in payroll related expenses, inclusive of $1.1 million in stock-based compensation, to support our research and development primarily due to an increase in KB111, KB801, KB803 and other research & development programs partially offset by a decrease in B-VEC and KB304; and
- a net increase of $2.3 million in clinical development costs, primarily due to an increase in KB707 costs related to our Phase 1/2 clinical trials for inhaled KB707.

Research and development expenses increased $7.1 million in the year ended December 31, 2024 compared to the year ended December 31, 2023. The increase was primarily driven by the following:

- an aggregated increase of $4.9 million related to KB304 costs, KB408 costs, KB803 costs, other dermatology programs, other ophthalmology programs and other aesthetics programs all related to increases in manufacturing expenses, payroll costs and professional services related to pre-clinical contracts;
- an increase of $4.8 million in KB707 costs following the expansion of our research and development pipeline to oncology consisting of an increase in payroll related costs to support our research, an increase in contract research expenses in preparation for clinical trials, and an increase in clinical trial costs associated with our Phase 1/2 clinical trial of KB707 that commenced in 2024; and
- an increase of $0.7 million in other research programs.

The increases were partially offset by:

- a net decrease of $2.5 million in other unallocated expenses primarily due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and cost of goods sold, partially offset by increases related to (1) depreciation due to the Company's second CGMP facility being placed into service throughout 2023 and 2024 partially offset by the capitalization of depreciation associated with increased commercial batches of VYJUVEK and (2) other facilities and equipment related costs; and
- a decrease of $0.8 million in stock-based compensation due to the allocation of labor costs related to work performed to manufacture VYJUVEK to inventory.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased $33.1 million for the year ended December 31, 2025 compared to the year ended December 31, 2024. The increase was primarily driven by the following:

- an increase of $9.2 million of payroll costs, including stock-based compensation;
- an increase of $5.5 million in other general and administrative costs mainly related to $1.5 million in charitable contributions, $1.1 million in taxes and $1.1 million in subscriptions;
- an increase of $11.1 million related to professional services, including legal and consulting services; and
- an increase of $6.7 million in marketing costs to support commercial sales of VYJUVEK.

Selling, general and administrative expenses increased $15.3 million for the year ended December 31, 2024 compared to the year ended December 31, 2023. The increase was primarily driven by the following:

- an increase of $10.0 million in stock-based compensation;
- an increase of $3.7 million in selling expenses related to the commercial launch of VYJUVEK, which includes $1.2 million related to our patient access program; and
- an increase of $3.3 million related to professional services incurred to support our commercial growth.

The increases were partially offset by:

- a decrease of $2.0 million in marketing costs due to the timing of marketing activities ahead of the VYJUVEK commercial launch.

Litigation Settlement

Litigation settlement for the years ended December 31, 2025, 2024 and 2023 was zero, $37.5 million and $12.5 million, respectively, and consisted of amounts related to the settlement of litigation with PeriphaGen. See "Legal Proceedings" in Note 7 of the notes to consolidated financial statements included in this Annual Report on Form 10-K for more information.

Gain from Sale of Priority Review Voucher

Gain from sale of priority review voucher for the year ended December 31, 2023 was $100.0 million and was related to the sale of our rare pediatric PRV, which was awarded to the Company in connection with the FDA's approval of VYJUVEK.

Interest and Other Income, Net

Interest and other income, net for the years ended December 31, 2025, 2024 and 2023 was $28.2 million, $29.6 million and $22.5 million, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments as well as the effects of foreign exchange rates. The decrease in interest and other income for the year ended December 31, 2025 compared to the year ended December 31, 2024 is primarily the result of the effects of foreign exchange rates. The increase in interest and other income for the years ended December 31, 2024 compared to the year ended December 31, 2023 is the result of increased investment activity and more favorable interest rates as compared to the prior period and an increase in our balance of cash, cash equivalents, and investments.

Income Tax Benefit (Expense)

Income tax benefit for the year ended December 31, 2025 was $15.4 million. Income tax expense for the years ended December 31, 2024 and 2023 was $6.2 million, and $2.0 million, respectively. In 2023 and 2024, income tax expense related to state, federal and foreign income taxes. In 2025, we determined that it was more likely than not that the benefit from certain of our deferred tax assets will be realized. Accordingly, the related valuation allowance was released and a one-time benefit was recognized. See Note 11 of the notes to consolidated financial statements included in this Form 10-K for more information.

Liquidity and Capital Resources

Overview

As of December 31, 2025, our cash, cash equivalents and short-term investments balance was approximately $827.8 million. As of December 31, 2025, we had a retained earnings balance of $24.2 million. We believe that our cash, cash equivalents and short-term investments will be sufficient to allow us to fund our operations for at least 12 months from the filing date of this Annual Report on Form 10-K.

Costs related to clinical trials can be unpredictable and, therefore, there can be no guarantee that we will have sufficient capital to fund the continued or planned pre-clinical and clinical studies for our product candidates, or our operations. Further, we expect future revenue to fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any product sales. While we are in the process of building out our internal vector manufacturing capacity, some of our manufacturing activities will be contracted out to third parties. Additionally, we currently utilize third-party contract research organizations to carry out some of our clinical development activities. As we seek to obtain regulatory approval for our product candidates and prepare for product sales, marketing, commercial manufacturing, packaging, labeling and distribution, we expect to continue to incur significant manufacturing and commercialization expenses. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercially launch our product candidates. Accordingly, to obtain marketing approval for and to commercialize these or any other product candidates, we may be required to obtain further funding through public or private equity offerings, debt financings, collaboration and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, if at all. Our failure to raise capital when needed could have a negative effect on our financial condition and our ability to pursue our business strategy.

Operating Capital Requirements

Our primary uses of capital are, and we expect will continue to be for the near future, compensation and related expenses, manufacturing costs for preclinical and clinical materials, regulatory expenses, third-party clinical trial research and development services, laboratory and related supplies, selling expenses, costs to manufacture our commercial product, legal expenses and general overhead costs. In order to complete the process of obtaining regulatory approval for any of our product candidates and to build the sales, manufacturing, marketing and distribution infrastructure that we believe will be necessary to commercialize our product candidates, if approved, we may require substantial additional funding.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect, and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development, manufacturing and commercialization of genetic medicines, 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 costs needed to commercialize and market our lead product, VYJUVEK;
- the progress, timing and costs of clinical trials of our current product candidates;
- the progress, timing and cost of manufacturing VYJUVEK and revenue received from commercial sale of VYJUVEK;
- the continued development and the filing of an IND application for current and future product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies and clinical trials for any product candidates that we may pursue in the future, if any;
- the costs of maintaining our own commercial-scale CGMP manufacturing facilities;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs associated with the manufacturing process development and evaluation of third-party manufacturers;
- the extent to which the costs of VYJUVEK and our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- the costs of commercialization activities for our current and future product candidates if we receive marketing approval for such product candidates, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;

- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements;
- our current license agreements remaining in effect and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

We may need to obtain substantial additional funding in order to receive regulatory approval and to commercialize our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely affect our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves.

Contractual Obligations

Operating Leases

Operating lease payments represent our commitments for future minimum rent made under non-cancelable leases for our corporate headquarters in Pittsburgh, Pennsylvania and our global office locations, and for the ground lease associated with our second CGMP manufacturing facility, ASTRA. The total future payments for our operating lease obligations that had commenced as of December 31, 2025 are $17.0 million, of which $1.9 million is due in the next twelve months and the remaining payments are due over the terms of the respective leases. For additional details regarding our leases, see Note 8 to our consolidated financial statements included in this Annual Report on Form 10-K.

Clinical Supply and Product Manufacturing Agreements

We enter into various agreements in the normal course of business with Contract Research Organizations, Contract Manufacturing Organizations and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. We are obligated to make milestone payments under certain of these agreements. The estimated remaining commitment expected to be due in the next twelve months as of December 31, 2025 under these agreements is immaterial.

Sources and Uses of Cash

The following table summarizes our sources and uses of cash:

	Years Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Net cash provided by (used in) operating activities	$ 200,865	$ 123,420	$ (88,804)
Net cash (used in) provided by investing activities	(58,417)	(163,439)	82,638
Net cash provided by financing activities	8,705	27,014	202,750
Effect of exchange rate changes on cash and cash equivalents	286	(458)	(156)
Net increase (decrease) in cash	$ 151,439	$ (13,463)	$ 196,428

Operating Activities

Net cash provided by operating activities for the year ended December 31, 2025 was $200.9 million and consisted primarily of net income of $204.8 million adjusted for $32.5 million of non-cash items and a $36.4 million decrease in cash due to an increase in net working capital. Non-cash adjustments included depreciation of $5.7 million, amortization of operating lease right-of-use assets of $0.8 million, stock-based compensation expense, net of $54.5 million, and other adjustments of

$0.7 million offset by a change in our deferred income taxes of $22.8 million, realized gain on investments of $6.0 million and accretion on marketable securities of $0.5 million.

Net cash used by operating activities for the year ended December 31, 2024 was $123.4 million and consisted primarily of net income of $89.2 million adjusted for $48.7 million of non-cash items and a $14.5 million decrease in cash due to an increase in net working capital. Non-cash adjustments included depreciation of $6.0 million, amortization of operating lease right-of-use assets of $0.7 million, stock-based compensation expense, net of $49.1 million, and other adjustments of $0.7 million offset by realized gain on investments of $6.1 million and accretion on marketable securities of $1.7 million.

Net cash used in operating activities for the year December 31, 2023 was $88.8 million and consisted primarily of net income of $10.9 million adjusted for $61.2 million of non-cash items and a $38.5 million decrease in cash due to an increase in net working capital. Non-cash adjustments included gain on sale of priority review voucher of $100.0 million, which is classified as an investing activity, realized gain on investments of $5.1 million and accretion of marketable securities of $2.2 million, partially offset by stock-based compensation expense, net of $39.9 million, depreciation of $5.0 million, amortization of operating lease right-of-use assets of $0.9 million and other adjustments of $0.2 million.

Investing Activities

Net cash used in investing activities for the year ended December 31, 2025 was $58.4 million and consisted of $422.5 million in purchases of short-term and long-term investments and $12.0 million in purchases of property and equipment, partially offset by $375.6 million received from the maturities of investments and $0.4 million received in proceeds from disposal of assets.

Net cash used in investing activities for the year ended December 31, 2024 was $163.4 million and consisted of $457.7 million in purchases of short-term and long-term investments and $4.2 million in purchases of property and equipment, partially offset by $298.5 million received from the maturities of investments.

Net cash provided by investing activities for the year ended December 31, 2023 was $82.6 million and consisted of $503.2 million received from the maturities of investments and $100.0 million in proceeds from the sale of priority review voucher, partially offset by $508.8 million in purchases of short-term and long-term investments and $11.8 million in purchases of property and equipment.

Financing Activities

Net cash provided by financing activities for the year ended December 31, 2025 was $8.7 million and consisted of proceeds of $22.7 million from exercises of stock options, partially offset by $12.1 million used for employee tax withholding payments related to vested restricted stock units and $1.8 million used for employee tax withholding payments for settlement of vested restricted stock awards.

Net cash provided by financing activities for the year ended December 31, 2024 was $27.0 million and consisted of proceeds of $32.4 million from exercises of stock options, partially offset by $4.2 million used for employee tax withholding.

Net cash provided by financing activities for the year ended December 31, 2023 was $202.8 million and consisted of proceeds of $159.7 million from issuance of common stock, net of offering costs and proceeds of $43.8 million from exercises of stock options, partially offset by $0.7 million used for employee tax withholding payments for settlement of restricted stock awards.

Recent Accounting Pronouncements

See Note 2 to our consolidated financial statements.

Item 7A. Qualitative and Quantitative Disclosures About Market Risk

We had cash, cash equivalents and short-term investments of approximately $827.8 million as of December 31, 2025, which consisted primarily of money market funds, commercial paper, corporate bonds and U.S. government agency securities. The investments in these financial instruments are made in accordance with an investment policy which specifies the categories, allocations and ratings of securities we may consider for investment. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk. This means that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the prevailing interest rate later rises, the value of that security will probably decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and short-term investment securities available-for-sale in a variety of securities which may include money market funds, government and non-government debt securities and commercial paper, all with various maturity dates. Based on our current investment portfolio, we do not believe that our financial position would be materially affected by an immediate change of 1% in interest rates.

We also have established operations in Europe and Japan and hold cash in Swiss Francs, Euros and Japanese Yen. We are subject to foreign exchange rate risk arising from transactions conducted in the aforementioned foreign currencies, however, our foreign operations are not currently material to our business. We do not believe that our results of operations or our financial position would be materially affected by an immediate change of 10% in foreign currency exchange rates

We do not hold or issue derivatives, derivative commodity instruments or other financial instruments for speculative trading purposes. Further, we do not believe our cash, cash equivalents and short-term investments have significant risk of default or illiquidity. While we believe our cash, cash equivalents and short-term investments do not contain excessive risk, we cannot provide absolute assurance that any investments we make in the future will not be subject to adverse changes in market value. Our cash, cash equivalents and short-term investments are recorded at fair value.

Item 8. Financial Statements and Supplementary Data.

INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Krystal Biotech, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Krystal Biotech, Inc. and subsidiaries (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations and comprehensive income, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2025, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission, and our report dated February 17, 2026 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We are a public accounting firm registered with the 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 consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated 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 consolidated 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 consolidated 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 consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of a 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 accounts or disclosures to which it relates.

Evaluation of the sufficiency of audit evidence over the Medicaid rebate accrual

As discussed in Notes 2 and 3 to the consolidated financial statements, the Company recognizes product revenue on sales of VYJUVEK when the customer obtains control of the product, which occurs at a point in time, upon delivery to the customer. Product revenue, net is recorded at the net sales price, or transaction price, and includes an estimate of variable consideration, which results from discounts, rebates and returns that are offered within the Company's contracts. Government rebates, which include Medicaid, are accrued based on estimated percentages of VYJUVEK that will be prescribed to qualified patients, estimated rebate percentages and estimated levels of inventory in the distribution channel that will be prescribed to qualified patients and are recorded as a reduction of revenue. Rebates were $61.9 million as of December 31, 2025, of which a portion relates to the Medicaid rebate accrual.

We identified the evaluation of the sufficiency of audit evidence over the Medicaid rebate accrual as a critical audit matter. Subjective auditor judgment was required to evaluate the Medicaid rebate accrual because of the audit effort involved in determining the nature and extent of procedures to be performed.

The following are the primary procedures we performed to address the critical audit matter. We applied auditor judgment to determine the nature and extent of procedures to be performed over the Medicaid rebate accrual. We evaluated the design and tested the operating effectiveness of certain internal controls over the Company's Medicaid rebate accrual process.

We evaluated the relevance and reliability of the historical customer data used by the Company in developing the estimate of the Medicaid rebate accrual by performing a trend analytic over the estimated percentages of VYJUVEK that will be prescribed to qualified patients. We evaluated the Company's ability to accurately estimate the Medicaid rebate accrual by comparing the estimated Medicaid rebate accrual to the actual invoiced amounts that were paid by the Company throughout the period. We developed an independent estimate of the Medicaid rebate accrual by confirming the year-to-date units delivered to the customer and vouching payments made during the year, and compared the result to the Company's estimated Medicaid rebate accrual. We assessed the sufficiency of evidence obtained over the Medicaid rebate accrual by assessing the results of procedures performed, including the appropriateness of the nature and extent of audit effort.

/s/ KPMG LLP

We have served as the Company's auditor since 2022.

Pittsburgh, Pennsylvania
February 17, 2026

Krystal Biotech, Inc.
Consolidated Balance Sheets

(in thousands, except par value)	December 31, 2025	December 31, 2024
Assets		
Current assets		
Cash and cash equivalents	$ 496,304	$ 344,865
Short-term investments	331,487	252,652
Accounts receivable, net	127,425	104,746
Inventory	40,475	26,508
Prepaid taxes	14,006	1,617
Prepaid expenses and other current assets	14,905	11,657
Total current assets	1,024,602	742,045
Property and equipment, net	150,776	155,168
Long-term investments	128,066	152,114
Right-of-use assets	7,239	6,280
Deferred tax asset, net of valuation allowance	22,824	—
Other non-current assets	287	231
Total assets	$ 1,333,794	$ 1,055,838
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 3,238	$ 4,985
Current portion of lease liability	1,771	1,217
Accrued rebates	58,181	36,804
Accrued expenses and other current liabilities	39,752	58,989
Total current liabilities	102,942	101,995
Lease liability	7,568	6,044
Other long-term liabilities	3,724	1,419
Total liabilities	114,234	109,458
Commitments and contingencies (see note 7)		
Stockholders' equity		
Common stock; $0.00001 par value; 80,000 shares authorized as of December 31, 2025 and 2024; 29,192 and 28,794 shares issued and outstanding as of December 31, 2025 and 2024, respectively.	—	—
Additional paid-in capital	1,194,261	1,127,238
Accumulated other comprehensive income (loss)	1,136	(190)
Retained earnings (accumulated deficit)	24,163	(180,668)
Total stockholders' equity	1,219,560	946,380
Total liabilities and stockholders' equity	$ 1,333,794	$ 1,055,838

The accompanying notes are an integral part of these consolidated financial statements.

Krystal Biotech, Inc.
Consolidated Statements of Operations and Comprehensive Income

(in thousands, except per share data)	Years Ended December 31, 2025	2024	2023
Product revenue, net	$ 389,130	$ 290,515	$ 50,699
Operating expenses			
Cost of goods sold	23,049	20,061	3,094
Research and development	58,045	53,580	46,433
Selling, general and administrative	146,741	113,626	98,289
Litigation settlement	—	37,500	12,500
Total operating expenses	227,835	224,767	160,316
Income (loss) from operations	161,295	65,748	(109,617)
Other income			
Gain from sale of priority review voucher	—	—	100,000
Interest and other income, net	28,176	29,608	22,514
Income before income taxes	189,471	95,356	12,897
Income tax benefit (expense)	15,360	(6,197)	(1,965)
Net income	204,831	89,159	10,932
Unrealized gain (loss) on available-for-sale securities, net of tax	642	(440)	1,432
Foreign currency translation	684	(388)	(66)
Comprehensive income	$ 206,157	$ 88,331	$ 12,298
Net income per common share:			
Basic	$ 7.08	$ 3.12	$ 0.40
Diluted	$ 6.84	$ 3.00	$ 0.39
Weighted-average common shares outstanding:			
Basic	28,944	28,592	27,154
Diluted	29,951	29,740	27,752

The accompanying notes are an integral part of these consolidated financial statements.

Krystal Biotech, Inc.
Consolidated Statements of Stockholders' Equity

(in thousands)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings (Accumulated Deficit)	Total Stockholders' Equity
Balances as of January 1, 2023	25,764	$ —	$ 803,718	$ (728)	$ (280,759)	$ 522,231
Issuance of common stock pursuant to ATM Program, net of offering costs	1,730	—	159,909	—	—	159,909
Issuance of common stock upon exercise of stock options	753	—	43,773	—	—	43,773
Shares of restricted stock awards surrendered for taxes	(10)	—	(749)	—	—	(749)
Stock-based compensation	—	—	41,179	—	—	41,179
Unrealized gain on investments	—	—	—	1,432	—	1,432
Foreign currency translation	—	—	—	(66)	—	(66)
Net income	—	—	—	—	10,932	10,932
Balances as of December 31, 2023	28,237	$ —	$ 1,047,830	$ 638	$ (269,827)	$ 778,641
Issuance of common stock in private placement offering, net of offering costs	526	—	32,400	—	—	32,400
Issuance of common stock upon exercise of stock options	39	—	(4,181)	—	—	(4,181)
Shares of restricted stock awards surrendered for taxes	(8)	—	(1,205)	—	—	(1,205)
Stock-based compensation	—	—	52,394	—	—	52,394
Unrealized (loss) on investments	—	—	—	(440)	—	(440)
Foreign currency translation	—	—	—	(388)	—	(388)
Net income	—	—	—	—	89,159	89,159
Balances as of December 31, 2024	28,794	$ —	$ 1,127,238	$ (190)	$ (180,668)	$ 946,380
Issuance of common stock upon exercise of stock options	310	—	22,661	—	—	22,661
Vesting of restricted stock units, net of shares withheld for taxes	98	—	(12,144)	—	—	(12,144)
Shares of restricted stock awards surrendered for taxes	(10)	—	(1,812)	—	—	(1,812)
Stock-based compensation	—	—	58,318	—	—	58,318
Unrealized gain on investments, net of tax	—	—	—	642	—	642
Foreign currency translation	—	—	—	684	—	684
Net income	—	—	—	—	204,831	204,831
Balances as of December 31, 2025	29,192	$ —	$ 1,194,261	$ 1,136	$ 24,163	$ 1,219,560

The accompanying notes are an integral part of these consolidated financial statements.

Krystal Biotech, Inc.
Consolidated Statements of Cash Flows

(in thousands)	Years Ended December 31, 2025	2024	2023
Operating Activities			
Net income	$ 204,831	$ 89,159	$ 10,932
Adjustments to reconcile net income to net cash used in operating activities			
Gain from sale of priority review voucher	—	—	(100,000)
Deferred tax asset	(22,845)	—	—
Depreciation	5,724	5,967	5,007
Accretion of marketable securities	(498)	(1,706)	(2,183)
Amortization of operating lease right-of-use assets	827	747	904
Stock-based compensation expense, net	54,514	49,127	39,933
Realized gain on investments	(5,953)	(6,069)	(5,092)
Other, net	684	652	217
Changes in operating assets and liabilities			
Accounts receivable, net	(22,124)	(62,706)	(42,040)
Inventory	(6,286)	(11,907)	(4,475)
Prepaid taxes	(12,389)	(1,617)	—
Prepaid expenses and other assets	(2,270)	(7,292)	(1,612)
Lease liability	(578)	(833)	(829)
Other long-term liabilities	2,280	1,419	—
Accounts payable	826	1,011	(101)
Accrued rebates	21,268	30,827	5,977
Accrued expenses and other current liabilities	14,104	5,391	4,558
Accrued legal settlement	(31,250)	31,250	—
Net cash provided by (used in) operating activities	200,865	123,420	(88,804)
Investing Activities			
Proceeds from disposal of assets	435	—	—
Proceeds from sale of priority review voucher	—	—	100,000
Purchases of property and equipment	(11,951)	(4,238)	(11,799)
Purchases of investments	(422,540)	(457,740)	(508,776)
Maturities of investments	375,639	298,539	503,213
Net cash (used in) provided by investing activities	(58,417)	(163,439)	82,638
Financing Activities			
Proceeds from issuance of common stock, net	—	—	159,726
Proceeds from exercise of stock options	22,661	32,400	43,773
Taxes paid for employee tax withholding related to restricted stock units	(12,144)	(4,181)	—
Taxes paid related to settlement of restricted stock awards	(1,812)	(1,205)	(749)
Net cash provided by financing activities	8,705	27,014	202,750
Effect of exchange rate changes on cash and cash equivalents	286	(458)	(156)
Net change in cash and cash equivalents	151,439	(13,463)	196,428
Cash and cash equivalents at beginning of year	344,865	358,328	161,900
Cash and cash equivalents at end of year	$ 496,304	$ 344,865	$ 358,328
Supplemental Disclosures of Non-Cash Investing and Financing Activities			
Unpaid purchases of property and equipment	$ 3,067	$ 8,497	$ 8,602
Initial recognition of right-of-use assets	$ 1,786	$ —	$ —
Supplemental Disclosure of Cash Flow Information			
Income taxes paid	$ 15,673	$ 5,665	$ —

The accompanying notes are an integral part of these consolidated financial statements.

1. Organization

Krystal Biotech, Inc. (the "Company," or "we" or other similar pronouns) commenced operations in April 2016. In March 2017, we converted from a California limited liability company to a Delaware C-corporation, and changed our name from Krystal Biotech LLC to Krystal Biotech, Inc. In April 2019, we incorporated Jeune Aesthetics, Inc. ("Jeune Aesthetics"), a wholly-owned subsidiary, in Delaware, for the purpose of undertaking preclinical and clinical studies for aesthetic skin conditions. In January 2022, August 2022, December 2022, August 2023, March 2024, November 2024, December 2024 and July 2025 we incorporated wholly-owned subsidiaries in Switzerland, Netherlands, France, Germany, Japan, Italy, Spain, and the UK, respectively, for the purpose of establishing operations in Europe and Japan for the commercialization of VYJUVEK® and our product pipeline.

We are a fully integrated, commercial-stage, global biotechnology company focused on the discovery, development, manufacturing, and commercialization of genetic medicines to treat diseases with high unmet medical needs. Using our patented gene therapy technology platform that is based on engineered herpes simplex virus-1 ("HSV-1"), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell's own machinery then transcribes and translates the transgene to treat the disease. Our vectors are amenable to formulation for non-invasive or minimally invasive routes of administration at a healthcare professional's office or in the patient's home. Our innovative technology platform is supported by two in-house, commercial scale Current Good Manufacturing Practice ("CGMP") manufacturing facilities.

Liquidity

As of December 31, 2025, the Company had a retained earnings balance of $24.2 million. Our operating profitability is dependent upon the continued successful commercialization of VYJUVEK, our U.S. Food and Drug Administration ("FDA"), European Commission ("EC"), and Japan's Ministry of Health, Labour, and Welfare ("MHLW") approved product, as well as successful development, approval and commercialization of our product candidates. Management intends to fund future operations through its on hand cash and cash equivalents and revenue generated from the sale of VYJUVEK, and may also seek additional capital through arrangements with strategic partners, the sale of equity, debt financings or other sources.

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to the failure of product candidates in clinical and preclinical studies, the development of competing product candidates or other technological innovations by competitors, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to commercialize product candidates. The Company expects to incur significant costs in connection with, among other things, advancing its product pipeline, expanding its commercialization capabilities, and complying with EU post-authorization regulatory requirements and EU member state–specific pricing, reimbursement, and market access activities. The Company believes that its cash, cash equivalents and short-term investments of approximately $827.8 million as of December 31, 2025 will be sufficient to allow the Company to fund its planned operations for at least the next 12 months from the date of this Annual Report on Form 10-K.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). All intercompany balances and transactions have been eliminated in consolidation. Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassified amounts have no impact on the Company's previously reported financial position or results of operations.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the consolidated financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates in the period these variances

become known. Estimates are used in the following areas, among others: variable consideration associated with revenue recognition, stock-based compensation expense, accrued expenses, and income taxes.

Segment and Geographical Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company operates as one operating segment, which is focused on the discovery, development, manufacturing and commercialization of pharmaceutical products. See Note 13 to these consolidated financial statements for additional discussion.

Cash, Cash Equivalents and Investments

Cash and cash equivalents consist of money market funds and bank deposits. Cash equivalents are defined as short-term, highly liquid investments with original maturities of 90 days or less at the date of purchase.

Investments with maturities of less than one year are classified as short-term investments on the consolidated balance sheets and consist of commercial paper, corporate bonds and U.S. government agency securities and treasuries. Investments with maturities of greater than one year are classified as long-term investments on the consolidated balance sheets and consist of corporate bonds and U.S. government agency securities and treasuries. Accrued interest on investments is also classified as short-term investments on the consolidated balance sheets.

As the Company's entire investment portfolio is considered available for use in current operations, it classifies all investments as available-for-sale securities. Available-for-sale securities are carried at fair value, with unrealized gains and losses reported in accumulated other comprehensive income, which is a separate component of stockholders' equity on the consolidated balance sheets. Any premium arising at purchase is amortized to the earliest call date and any discount arising at purchase is accreted to maturity. Amortization and accretion of premiums and discounts are recorded in interest and other income, net on the consolidated statements of operations and comprehensive income.The Company evaluates its available-for-sale debt securities on a quarterly basis to determine whether a decline in fair value below amortized cost is attributable to credit-related factors. During the years ended December 31, 2025, 2024 and 2023, the Company concluded that no allowances for credit losses on investments were required.

Fair Value of Financial Instruments

Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. There is a three-level hierarchy that prioritizes the inputs used in determining fair value by their reliability and preferred use, as follows:

- *Level 1*—Valuations based on quoted prices in active markets for identical assets or liabilities.
- *Level 2*—Valuations based on quoted prices in active markets for similar assets and liabilities, quoted prices for identical or similar assets and liabilities in inactive markets, or other inputs that are observable, or can be corroborated by observable market data.
- *Level 3*—Valuations based on inputs that are both significant to the fair value measurement and unobservable.

To the extent that a valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized within Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

There have been no (1) significant changes to the valuation methods utilized by the Company or (2) transfers between Level 1, Level 2 and Level 3 during the years ended December 31, 2025, 2024 and 2023.

The carrying amounts of financial instruments consisting of cash and cash equivalents, investments, accounts receivable, net, prepaid expenses and other current assets, accounts payable, accrued expenses, accrued rebates, and other current liabilities included in the Company's consolidated balance sheets, are reasonable estimates of fair value, primarily due to their short maturities.

Our available-for-sale, short-term and long-term investments, which consist of commercial paper, corporate bonds, and U.S. government agency securities and treasuries are considered to be Level 2 financial instruments. The fair value of Level 2 financial assets is determined using inputs that are observable in the market or can be derived principally from or corroborated by observable market data such as pricing for similar securities, recently executed transactions, cash flow models with yield curves, and benchmark securities. In addition, Level 2 financial instruments are valued using comparisons to like-kind financial instruments and models that use readily observable market data as their basis.

Revenue Recognition

The Company recognizes product revenue in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC"), *Topic 606, Revenue from Contracts with Customers* ("ASC 606"). Under ASC 606, the Company is required to complete the following five steps:

(i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price, including variable consideration, if any; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation.

Revenue is recognized when the Company satisfies a performance obligation by transferring control of the promised good to the customer. The only performance obligation in the Company's contracts with customers is the timely delivery of the product to the customer's designated location.

The Company sells VYJUVEK to a limited number of specialty pharmacy ("SPs") providers that mix the medication to be administered at a healthcare professional's office or in the patient's home and to a limited number of hospitals or specialty distributors ("SDs") who deliver to hospitals where patients are administered the medication in a healthcare setting. Revenue is recognized when the customer obtains control of the product, which occurs at a point in time, upon delivery to the customer.

Revenue is measured as the amount of consideration the Company expects to receive in exchange for transferring VYJUVEK and is generally based upon a list price and is recorded at the net sales price upon delivery and transfer of control to the customer, and includes an estimate of variable consideration, which results from discounts, rebates, copay assistance, and returns that are offered within contracts between the Company and its customers. These reserves, representing the Company's best estimates of the amount of consideration to which the Company is entitled, are based on the terms of the contract.

Variable Consideration

Variable consideration reduces the transaction price to reflect the Company's best estimate of the amount of consideration to which the Company is entitled based on the terms of the contracts and is recorded in the same period the related product revenue is recognized. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, the Company will adjust these estimates in the period these variances become known.

The following are the Company's significant categories of variable consideration:

- *Prompt Pay Discounts*: As an incentive for prompt payment, the Company may offer cash discounts to its counterparties. The Company estimates accrued prompt pay discounts using the most likely amount method. The Company expects that all eligible counterparties will comply with the contractual terms to earn the discount. The Company records the discount as a reduction of revenue on the consolidated statements of operations and as an allowance against accounts receivable, net on the consolidated balance sheets.
- *Government Rebates*: The Company participates in certain government rebate programs including Medicaid, Medicare and Tricare. For Medicare, the Company estimates the accrued liability based on the estimated number of patients in the prescription drug coverage gap under the Medicare Part D program. The Company also estimates accrued government rebates using the expected value method based on estimated percentages of VYJUVEK that will be prescribed to qualified patients, estimated rebate percentages and estimated levels of inventory in the distribution channel that will be prescribed to qualified patients and records the rebates as a reduction of revenue on the consolidated statements of operations and accrued rebates and other long-term liabilities on the consolidated balance sheets.
- *Commercial Rebates:* The Company participates in certain commercial rebate programs. Under these rebate programs, the Company pays a rebate to the commercial entity or third-party administrator of the program. Accrued commercial rebates are estimated using the expected value method based on estimated percentages of VYJUVEK that will be prescribed to qualified patients and estimated levels of inventory in the distribution channel. Accrued commercial rebates are recorded as a reduction of revenue on the consolidated statements of operations and are included in accrued rebates on the consolidated balance sheets
- *Product Returns:* The Company offers limited return rights relating only to product damage or defects identified upon receipt, and therefore the Company expects minimal returns. Returns are estimated taking into consideration several factors including these limited product return rights, historical return activity, and other relevant factors. The Company has not experienced significant product returns to date, and accordingly no allowance for returns was recorded for the year ended December 31, 2025.

Cost of Goods Sold

Cost of goods sold includes direct and indirect costs related to the manufacturing of VYJUVEK. These costs consist of manufacturing costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Cost of goods sold may also include period costs related to certain manufacturing services and inventory adjustment charges.

Prior to receiving FDA approval in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expenses.

Accounts Receivable

Accounts receivable represents amounts arising from product sales and is recorded net of allowances for prompt payment discounts, returns, and credit losses. The Company estimates an allowance for credit losses by considering factors such as credit quality, the age of the accounts receivable balances, and current economic conditions that may affect a customer's ability to pay. The Company has no historical write-offs of its accounts receivable and its payment terms are generally 90 days or less from the invoice date. The Company evaluates the creditworthiness of each counterparty on a regular basis. As of December 31, 2025, the credit profiles for these counterparties were deemed to be in good standing and, as such, an allowance for credit losses was not recorded.

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents, short-term investments, long-term investments, and accounts receivable, net. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company is exposed to credit risk in the event of default by the financial institutions to the extent amounts recorded on the consolidated balance sheets are in excess of insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds. The Company's marketable securities, which primarily consist of U.S. government agency securities and treasuries, corporate bonds and commercial paper, potentially subject the Company to concentrations of credit risk. The Company has no financial instruments with off-balance sheet risk of loss.

Inventories

The Company capitalizes inventory costs associated with products when future economic benefit is expected to be realized. These costs consist of raw materials, manufacturing-related costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Prior to receiving FDA approval for VYJUVEK in May 2023, the Company expensed costs related to inventory for clinical and pre-commercial purposes directly to research and development expense. Following the FDA's approval of VYJUVEK, the Company began capitalizing inventory related to commercialized products held for sale, in-process of production for sale, and raw materials to be used in the manufacturing of inventory.

The Company values its inventories at the lower-of-cost and net realizable value, on a first-in, first-out basis. The Company adjusts the net realizable value of any excess, obsolete or unsalable inventories in the period in which they are identified. For the years ended December 31, 2025, 2024 and 2023, there were no inventory write-downs. See Note 6 to these consolidated financial statements for additional discussion.

Property and Equipment, Net

Property and equipment, net, is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred, while costs of major additions and betterments are capitalized. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss is included in the results of operations. Depreciation is recorded using the straight-line method over the estimated useful lives of the respective assets, which are as follows:

Buildings and building improvements	7 - 47 years
Computer equipment and software	3 - 7 years
Manufacturing equipment	3 - 30 years
Laboratory equipment	3 - 15 years
Furniture and fixtures	3 - 7 years
Leasehold improvements	lesser of remaining useful life or remaining life of lease

The Company reviews the estimated useful lives of its property and equipment on a continuing basis. In evaluating the useful lives, the Company considers how long assets will remain functionally effective, whether the technology continues to be relevant and considers other competitive and economic factors. If the assessment indicates that the assets will be used for a

shorter or longer period than previously anticipated, the useful life of the assets is adjusted, resulting in a change in estimate. Changes in estimates are accounted for on a prospective basis by depreciating the current carrying values of the assets over their revised remaining useful lives.

Construction in progress is not depreciated until the asset is placed in service.

Impairment of Long-Lived Assets

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. The Company reviews the recoverability of the net book value of long-lived assets whenever events and circumstances indicate ("triggering events") that the net book value of an asset may not be recoverable from the estimated undiscounted future cash flows expected to result from its use and eventual disposition. In cases where a triggering event occurs and undiscounted expected future cash flows are less than the net book value, the Company recognizes an impairment loss equal to an amount by which the net book value exceeds the fair value of the asset. Long-lived assets to be disposed of are reported at the lower of carrying amount or fair value less cost to sell. The Company has not experienced any triggering events or recognized any impairment losses for the years ended December 31, 2025, 2024 and 2023.

Leases

The Company accounts for its lease agreements in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 842, *Leases*. Right-of-use lease assets represent the right to use an underlying asset during the lease term and the lease liabilities represent the commitment to make lease payments arising from the lease. Right-of-use lease assets and obligations are recognized based on the present value of remaining lease payments over the lease term. As the Company's existing lease agreements do not provide an implicit rate and as the Company does not have any external borrowings, the Company has used an estimated incremental borrowing rate based on the information available at lease commencement in determining the present value of lease payments. Operating lease expense is recognized on a straight-line basis over the lease term. Variable lease expense is recognized in the period in which the obligation for the payment is incurred. In addition, the Company also has made an accounting policy election to exclude leases with an initial term of twelve months or less from its consolidated balance sheets and to account for lease and non-lease components of its operating leases as a single component.

Research and Development Expenses

Research and development costs are charged to expense as incurred in performing research and development activities. These costs include employee compensation costs, facilities and overhead, preclinical and clinical activities, contract research and manufacturing expenses.

The Company estimates contract research and manufacturing expenses based on the services performed pursuant to contracts with research organizations in the Company's ongoing preclinical and clinical studies. Non-refundable advanced payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. These estimates are based on communications with third-party service providers and the Company's estimates of accrued expenses using information available at each balance sheet date. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly.

Stock-Based Compensation Expense

The Company has applied the fair value recognition provisions of Financial Accounting Standards Board Accounting Standards Codification, or ASC, Topic 718, *Compensation—Stock Compensation* ("ASC 718"), to account for stock-based compensation. The Company recognizes compensation costs related to stock-based awards based on the estimated fair value of the awards on the date of grant.

ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations and comprehensive income based on their grant-date fair values. Compensation expense for stock options, restricted stock awards and restricted stock units is recognized on a straight-line basis based on the grant-date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense for performance-based restricted stock units is recognized for the awards that are probable of vesting over the service period of the award. On a quarterly basis, management estimates the probable number of performance-based restricted stock units that would vest until such time that the ultimate achievement of the performance criteria are known.

The Company estimates the fair value of its stock options using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including: (i) the expected stock price volatility; (ii) the expected term of the award; (iii) the risk-free interest rate; and (iv) expected dividends.

The Company estimates the expected term of its stock options using the "simplified" method, whereby the expected term equals the arithmetic mean of the vesting term and the original contractual term of the option. The risk-free interest rates are based on US Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid and does not expect to pay dividends in the foreseeable future. The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which service conditions are expected to be satisfied.

Foreign Currency Transaction Gain (Loss)

Gains and losses arising from transactions denominated in currencies other than U.S. dollars are recorded in interest and other income, net on the statement of operations and comprehensive income. The Company recorded losses of $1.2 million for the year ended December 31, 2025, and immaterial losses for the years ended December 31, 2024 and 2023.

Income Taxes

The Company accounts for income taxes in accordance with FASB ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. Under this method, the Company records deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities using enacted tax rates expected to be in effect when the differences are expected to reverse. Valuation allowances are provided when necessary to reduce net deferred tax assets to the amount that is more likely than not to be realized. As of December 31, 2025, after weighing all available evidence, including three-year cumulative income and forecasted future income, the Company is able to support the realizability of its deferred tax assets, except certain state tax attributes. Accordingly, except for certain state net operating loss and tax credits, the Company recorded no valuation allowance as of December 31, 2025 attributable to its deferred tax assets. The Company recorded a full valuation allowance as of December 31, 2024. The Company intends to continue assessing the realizability of the remaining valuation allowance until sufficient evidence exists to support its reversal.

The Company accounts for unrecognized tax benefits in accordance with the provisions of ASC 740. When unrecognized tax benefits exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances.

See Note 11 to these consolidated financial statements for additional discussion.

Comprehensive Income

Comprehensive income is defined as the change in equity during a period from transactions from non-owner sources. Unrealized gains or losses on available-for-sale securities is a component of other comprehensive gains or losses and is presented net of taxes. The Company records reclassifications from other comprehensive gains or losses to interest and other income, net on the consolidated statements of operations and comprehensive income related to realized gains on sales of available-for-sale securities.

Recently Issued Accounting Pronouncements, Not Yet Adopted

In November 2024, the FASB issued *ASU 2024-03 Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses.* This standard calls for 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, with the option to apply it retrospectively. Early adoption is allowed. Currently, the Company is assessing the potential impact of this guidance on its consolidated financial statement disclosures.

In December 2025, the FASB issued *ASU 2025-11 Interim Reporting (Topic 270): Narrow-Scope Improvements.* This standard clarifies current interim reporting requirements on Topic 270 and introduces a disclosure principle requiring entities to disclose events since the end of the last annual reporting period that have a material impact on the entity. This standard will be effective for fiscal years beginning after December 15, 2027, with the option to apply it retrospectively. Early adoption is allowed. Currently, the Company is assessing the potential impact of this guidance on its consolidated financial statement disclosures.

Recently Adopted Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09 *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. The purpose of this guidance is to enhance the transparency and usefulness of income tax disclosures and provide comprehensive income tax information, particularly in relation to rate reconciliation and income taxes paid in the U.S. and foreign jurisdictions. The Company adopted ASU 2023-09 during the year ended December 31, 2025. See Note 11 to these consolidated financial statements for additional discussion.

3. Product Revenue, Accounts Receivable and Reserves for Product Sales

Following FDA approval in May 2023, the Company began commercial marketing and sales of VYJUVEK and began recognizing revenue in the third quarter of 2023. The Company's product revenue, net of sales discounts and allowances totaled $389.1 million, $290.5 million and $50.7 million for the years ended December 31, 2025, 2024 and 2023, respectively. For the years ended December 31, 2025, 2024, and 2023, approximately 75%, 87% and 100%, respectively, of the Company's product revenue, net was generated from a single customer in the U.S. No other customer exceeded 10% of the Company's product revenue, net.

The Company's accounts receivable, net balance relating to VYJUVEK sales was $127.4 million and $104.7 million as of December 31, 2025 and 2024, respectively. Net product revenue receivable from the Company's customers who individually accounted for 10% or more of net product revenue receivable consisted of the following:

	Percent of Net Product Revenue Receivable	
	Year Ended December 31,	
	2025	2024
Customer A	66 %	81 %
Customer B	14 %	13 %

The following table summarizes changes in allowances and discounts:

(in thousands)	Rebates	Prompt Pay	Other Accruals	Total
Balance as of December 31, 2023	$ 5,977	$ 858	$ 279	$ 7,114
Provision	45,853	9,212	420	55,485
Payments/Credits	(13,607)	(7,500)	(373)	(21,480)
Balance as of December 31, 2024	$ 38,223	$ 2,570	$ 326	$ 41,119
Provision	65,273	13,478	507	79,258
Payments/Credits	(41,591)	(10,214)	(455)	(52,260)
Balance as of December 31, 2025	$ 61,905	$ 5,834	$ 378	$ 68,117

Rebates are included in accrued rebates and other long-term liabilities on the consolidated balance sheets. Prompt pay discount is recorded as an allowance against accounts receivable, net on the consolidated balance sheets. Other long-term liabilities include $3.7 million of long-term accrued rebates. Other accruals are included in accrued expenses and other current liabilities on the consolidated balance sheets. Provisions for rebates, prompt pay discount and other accruals are recorded as reductions to product revenue, net on the consolidated statements of operations and comprehensive income.

4. Net Income Per Share Attributable to Common Stockholders

Basic net income per share attributable to common stockholders is calculated by dividing net income attributable to common stockholders by the weighted-average shares outstanding during the period, without consideration for common stock equivalents. Diluted net income per share attributable to common stockholders is computed by dividing the net income by the weighted-average number of shares of common stock and common stock equivalents outstanding for the period. Common stock equivalents consist of common stock issuable upon (1) exercise of stock options and (2) vesting of restricted stock awards, restricted stock units and performance-based restricted stock units (collectively, "restricted stock").

For the years ended December 31, 2025, 2024 and 2023, there were (1) 571 thousand, 236 thousand and 897 thousand, respectively, common stock equivalents outstanding in the form of stock options, and (2) 1 thousand, 1 thousand and zero, respectively, in unvested restricted stock that have been excluded from the calculation of diluted net income per common share as their effect would be anti-dilutive.

(in thousands, except per share data)	Years Ended December 31, 2025	2024	2023
Numerator:			
Net income	$ 204,831	$ 89,159	$ 10,932
Denominator:			
Weighted-average basic common shares	28,944	28,592	27,154
Dilutive effect of stock options and unvested restricted stock	1,007	1,148	598
Weighted-average diluted common shares	29,951	29,740	27,752
Net income per common share—basic	$ 7.08	$ 3.12	$ 0.40
Net income per common share—diluted	$ 6.84	$ 3.00	$ 0.39

5. Fair Value Instruments

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of December 31, 2025 and 2024:

	December 31, 2025						
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities[1]	Long-term Marketable Securities[2]
Level 1:							
Cash and cash equivalents	$ 496,304	$ —	$ —	$ 496,304	$ 496,304	$ —	$ —
Subtotal	496,304	—	—	496,304	496,304	—	—
Level 2:							
Commercial paper	12,887	2	(1)	12,888		12,888	—
Corporate bonds	211,268	535	(6)	211,797		142,801	68,996
U.S government agency securities and treasuries	234,216	653	(1)	234,868		175,798	59,070
Subtotal	458,371	1,190	(8)	459,553	—	331,487	128,066
Total	$ 954,675	$ 1,190	$ (8)	$ 955,857	$ 496,304	$ 331,487	$ 128,066

	December 31, 2024						
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities[1]	Long-term Marketable Securities[2]
Level 1:							
Cash and cash equivalents	$ 344,865	$ —	$ —	$ 344,865	$ 344,865	$ —	$ —
Subtotal	344,865	—	—	344,865	344,865	—	—
Level 2:							
Commercial paper	15,373	4	(8)	15,369	—	15,369	—
Corporate bonds	177,771	423	(225)	177,969	—	86,693	91,276
U.S government agency securities and treasuries	211,283	318	(173)	211,428	—	150,590	60,838
Subtotal	404,427	745	(406)	404,766	—	252,652	152,114
Total	$ 749,292	$ 745	$ (406)	$ 749,631	$ 344,865	$ 252,652	$ 152,114

(1) The Company's short-term marketable securities mature in one year or less.
(2) The Company's long-term marketable securities mature between one year and two years.

See Note 2 to these consolidated financial statements for additional discussion regarding the Company's fair value measurements.

6. Balance Sheet Components

Inventory

Inventory consisted of the following:

	December 31,	
(in thousands)	**2025**	**2024**
Raw materials	$ 15,938	$ 13,639
Work-in-process	15,224	10,743
Finished goods	9,313	2,126
Inventory	$ 40,475	$ 26,508

Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31,	
(in thousands)	**2025**	**2024**
Building and building improvements	109,242	111,444
Manufacturing equipment	29,279	27,161
Leasehold improvements	27,227	25,673
Construction in progress	8,108	5,778
Laboratory equipment	3,490	3,183
Computer equipment and software	2,559	2,032
Furniture and fixtures	2,152	1,816
Total property and equipment	182,057	177,087
Accumulated depreciation	(31,281)	(21,919)
Property and equipment, net	$ 150,776	$ 155,168

Depreciation expense was $5.7 million, $6.0 million and $5.0 million for the years ended December 31, 2025, 2024 and 2023, respectively. Depreciation expense capitalized into inventory was $4.1 million, $3.5 million and $1.1 million for the years ended December 31, 2025, 2024 and 2023, respectively.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
(in thousands)	**2025**	**2024**
Accrued payroll and benefits	11,457	9,558
Accrued taxes	10,919	4,288
Accrued professional fees	5,936	2,659
Accrued preclinical and clinical expenses	4,667	2,537
Other current liabilities	3,579	2,403
Accrued construction in progress	2,189	5,077
Accrued inventory	1,005	1,217
Accrued litigation settlement	—	31,250
Total	$ 39,752	$ 58,989

7. Commitments and Contingencies

Agreements with Contract Manufacturing Organizations and Contract Research Organizations

The Company enters into various agreements in the normal course of business with Contract Manufacturing Organizations ("CMOs"), Contract Research Organizations ("CROs") and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. The agreements with CMOs primarily relate to the manufacturing of our sterile gel that is mixed with in-house produced vectors as part of the final drug product for VYJUVEK. Agreements with third

parties may also include research and development consulting activities, clinical-trial agreements, testing of our clinical-stage, pre-commercial and commercial stage products and/or storage, packaging and labeling. The Company is obligated to make milestone payments under certain of these contracts. The Company has incurred research and development expenses related to commitments under these agreements of $9.3 million, $7.1 million and $5.2 million for the years ended December 31, 2025, 2024 and 2023, respectively.

Legal Proceedings

In the ordinary course of business, the Company may be subject from time to time to various proceedings, lawsuits, disputes, or claims. In accordance with FASB ASC Topic 450, Contingencies ("ASC 450"), the Company accrues a liability for legal contingencies when it is probable that a liability has been incurred, and the amount of the loss can be reasonably estimated. If there is at least a reasonable possibility that a loss may be incurred, ASC 450 requires disclosure of a loss contingency. For the year ended December 31, 2025, no loss contingency exists.

In May 2020, PeriphaGen, Inc. ("PeriphaGen") commenced litigation against the Company alleging breach of contract and misappropriation of trade secrets. In April 2022, the Company and PeriphaGen entered into a final settlement agreement. In exchange for an upfront payment of $25.0 million and four contingent milestone payments of $12.5 million each, PeriphaGen (i) released all claims in the litigation; (ii) transferred certain assets to the Company and (iii) granted the Company a license for dermatological applications.

During the year ended December 31, 2025, the Company paid $31.25 million, and together with the $43.75 million paid prior to 2025, has fully paid the $75.0 million of total consideration in connection with the settlement of the PeriphaGen litigation. The Company recorded litigation settlement expense for the contingent milestone payments of zero, $37.5 million and $12.5 million for the years ended December 31, 2025, 2024, and 2023, respectively, on the consolidated statements of operations and comprehensive income.

In the first quarter of 2025, the Company and certain of its employees received subpoenas from the U.S. Department of Justice requesting that the Company produce certain documents regarding its sponsored genetic testing program relating to VYJUVEK and commercial practices relating thereto. The Company is cooperating and providing information in response to the subpoenas. It is not possible to estimate the amount of any loss or range of possible loss that might result from this inquiry, and because the final outcome cannot be predicted with certainty, unfavorable or unexpected developments or outcomes could result in a material impact to the Company's results of operations.

On September 18, 2025, a stockholder filed a derivative complaint in the Court of Chancery of the state of Delaware naming the Company's directors as defendants and the Company as a nominal defendant. The complaint alleges claims for breach of fiduciary duty, unjust enrichment, and waste of corporate assets based on allegedly excessive non-employee director compensation in each of 2021 through 2024. The complaint seeks unspecified damages in favor of the Company, restitution of compensation and other benefits from the individual defendants, reforms and improvements to the Company's corporate governance and internal procedures, and the award of costs and disbursements of the complaint, including reasonable attorneys' fees. The parties have reached an agreement in principle on settlement terms but must still negotiate and execute a definitive settlement agreement, which will be filed with the Delaware Court of Chancery and is subject to court approval. If approved, the Company will adopt, implement, and maintain certain corporate governance reforms for a period of five (5) years. At this time, the Company cannot reasonably estimate the likelihood of an unfavorable outcome or estimate the potential loss, if any.

8. Leases

Lease Agreements

The Company's operating leases primarily consist of leased office, manufacturing and laboratory space. The Company has an operating lease for laboratory and office space in Pittsburgh, Pennsylvania that commenced in June 2016 (the "Wharton Lease"). The Wharton Lease currently consists of approximately 67,000 square feet of office, lab, manufacturing, and warehouse space, including our commercial scale CGMP-compliant manufacturing facility ("ANCORIS") for a term ending on October 31, 2031.

In January 2021, in connection with the Company's second commercial gene therapy manufacturing facility ("ASTRA") in the Pittsburgh, Pennsylvania area, the Company entered into a ground lease with a term ending on January 31, 2071.

As of December 31, 2025, future minimum commitments under the Company's operating leases were as follows:

(in thousands)	December 31, 2025
2026	$ 1,864
2027	1,919
2028	1,954
2029	1,990
2030	2,016
Thereafter	7,279
Future minimum operating lease payments	17,021
Less: Interest	(7,682)
Present value of lease liability	$ 9,339

As of December 31, 2025 and 2024, the Company's weighted-average remaining lease term for operating leases was 10.1 years and 12.2 years, respectively, and the Company's weighted-average discount rate for operating leases was 9.7% and 9.5% as of December 31, 2025 and 2024, respectively.

The components of the Company's lease expense are as follows:

	Years Ended December 31,		
(in thousands)	2025	2024	2023
Lease cost:			
Operating lease expense	$ 1,594	$ 1,215	$ 1,596
Variable lease expense	226	210	203
Total lease expense	$ 1,820	$ 1,425	$ 1,799

9. Capitalization

ATM Program

The Company has an effective shelf registration statement on Form S-3 that expires on April 6, 2026. The Company has a $150 million at-the-market offering ("ATM") program under that shelf registration statement that has never been utilized. The Company does not currently intend to renew the ATM program after the shelf registration statement expires.

2023 Private Placement Offering

In May 2023, the Company sold an aggregate of 1,729,729 shares of our common stock in private placements to certain institutional investors at a price of $92.50 per share for aggregate net proceeds of $160.0 million.

10. Stock-Based Compensation

In 2017, the Company adopted the 2017 IPO Stock Incentive Plan ("Plan"), which governs the issuance of equity awards to employees, certain non-employee consultants, and directors. Initially, the Company reserved 900 thousand shares for issuance under the Plan with an initial sublimit for incentive stock options of 900 thousand shares. On an annual basis, the amount of shares available for issuance under the Plan increases by an amount equal to four percent of the total outstanding shares of common stock as of the last day of the preceding calendar year. The sublimit of incentive stock options is not subject to the increase. The Company has historically granted stock options, restricted stock awards ("RSAs"), restricted stock units ("RSUs") and performance-based restricted stock units ("PSUs") to certain employees.

Shares remaining available for grant under the Plan were 2.0 million at December 31, 2025

Stock Options

Options granted to employees and non-employees vest ratably over a four-year period and stock options granted to directors of the company vest ratably over one-year or three-year periods. Stock options have a life of ten years.

The following table summarizes the Company's stock option activity for the years ended December 31, 2025 and 2024:

	Stock Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value[(1)] *(in thousands)*
Balance as of January 1, 2024	2,606,592	$ 66.39	7.9	$ 150,405
Granted	348,642	$ 168.67		
Exercised	(525,857)	$ 61.62		
Cancelled or forfeited	(380,314)	$ 78.98		
Balance as of December 31, 2024	2,049,063	$ 82.69	7.3	$ 156,404
Granted	395,903	$ 168.08		
Exercised	(310,323)	$ 73.02		
Cancelled or forfeited	(104,226)	$ 109.35		
Balance as of December 31, 2025	2,030,417	$ 99.45	6.8	$ 298,662
Exercisable as of December 31, 2025	1,140,898	$ 73.28	5.8	$ 197,676

(1) Aggregate intrinsic value represents the difference between the closing stock price of our common stock on December 31, 2025, 2024 and 2023, respectively, and the exercise price of outstanding in-the-money options on the respective date.

The total intrinsic value (the amount by which the fair market value exceeded the exercise price) of stock options exercised during the years ended December 31, 2025, 2024 and 2023 was $35.4 million, $54.8 million and $43.8 million, respectively.

The weighted-average grant-date fair value per share of options granted to employees, non-employees and directors during the years ended December 31, 2025, 2024 and 2023 was $110.50, $114.31 and $63.38, respectively.

There was $58.6 million of unrecognized stock-based compensation expense related to employees', non-employees' and directors' options that is expected to be recognized over a weighted-average period of 2.7 years as of December 31, 2025.

The fair value of options granted was estimated at the date of grant using the Black-Scholes valuation model with the following weighted-average assumptions for the years ended December 31, 2025, 2024 and 2023:

	Years Ended December 31,		
	2025	2024	2023
Expected stock price volatility	69 %	73 %	73 %
Expected term of the award (years)	6.2	6.1	6.0
Risk-free interest rate	4.06 %	4.21 %	3.96 %
Weighted-average exercise price	$ 168.08	$ 168.67	$ 92.14
Dividend Yield	— %	— %	— %

Restricted Stock Awards

The following table summarizes the Company's RSA activity for the years ended December 31, 2025 and 2024:

	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSAs as of January 1, 2024	44,400	$ 78.89
Vested	(14,523)	$ 78.89
Surrendered for taxes	(7,677)	$ 78.89
Non-vested RSAs as of December 31, 2024	22,200	$ 78.89
Vested	(11,925)	$ 78.89
Surrendered for taxes	(10,275)	$ 78.89
Non-vested RSAs as of December 31, 2025	—	$ —

Restricted Stock Units

RSUs granted to employees vest ratably over a four-year period. The following table summarizes the Company's RSU activity for the years ended December 31, 2025 and 2024:

	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSUs as of January 1, 2024	160,900	$ 81.91
Granted	230,403	$ 160.15
Vested	(40,084)	$ 81.93
Forfeited	(43,123)	$ 119.02
Non-vested RSUs as of December 31, 2024	308,096	$ 135.22
Granted	139,256	$ 178.51
Vested	(84,524)	$ 129.82
Forfeited	(31,254)	$ 154.42
Non-vested RSUs as of December 31, 2025	331,574	$ 152.97

There was $37.7 million of unrecognized stock-based compensation expense related to employees' RSU awards that is expected to be recognized over a weighted-average period of 2.6 years as of December 31, 2025.

Performance-Based Restricted Stock Units

PSUs granted to employees vest ratably over two years based upon continued service through the vesting date and the achievement of specific regulatory and commercial performance criteria as determined by the Compensation Committee of the Company's Board of Directors. The performance criteria are to be completed by the end of the year in which the PSU awards were granted. Each PSU represents the right to receive one share of the Company's common stock upon vesting. The Company recognizes stock-based compensation expense for the fair value of the PSU awards relating to the portion of the awards that are probable of vesting over the service period.

The following table summarizes the Company's PSU activity for the years ended December 31, 2025 and 2024:

	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested PSUs as of January 1, 2024	50,000	$ 81.91
Granted	112,500	$ 159.47
Forfeited	—	$ —
Vested	(25,000)	$ 81.91
Non-vested PSUs as of December 31, 2024	137,500	$ 145.37
Granted	—	$ —
Forfeited	—	$ —
Vested	(81,250)	$ 135.61
Non-vested PSUs as of December 31, 2025	56,250	$ 159.47

There was $1.4 million of unrecognized stock-based compensation expense related to employees' PSU awards that is expected to be recognized over a weighted-average period of two months as of December 31, 2025.

Stock-Based Compensation Expense, Net

The Company recorded stock-based compensation expense, net related to stock options, RSAs, RSUs and PSUs in the consolidated statements of operations and comprehensive income for the years ended December 31, 2025, 2024 and 2023 as follows:

	Years Ended December 31,		
(in thousands)	2025	2024	2023
Research and development	$ 10,375	$ 9,237	$ 10,054
Selling, general and administrative	44,139	39,890	29,879
Total stock-based compensation	$ 54,514	$ 49,127	$ 39,933

After the FDA approval of VYJUVEK in May 2023, the Company began capitalizing stock-based compensation associated with the allocation of labor costs related to work performed to manufacture VYJUVEK. For the years ended December 31, 2025, 2024, and 2023 the Company capitalized $3.8 million, $3.3 million, and $1.1 million, respectively, into inventory.

Historically, the Company also capitalized the portion of stock-based compensation related to work performed on the construction of our manufacturing facilities. For the years ended December 31, 2025, 2024 and 2023, the Company capitalized zero, zero and $0.2 million, respectively, into property, plant and equipment.

11. Income Taxes

Income before income tax benefit (expense) by jurisdiction consisted of the following:

	Years Ended December 31,		
(in thousands)	2025	2024	2023
U.S.	$ 187,938	$ 93,808	$ 7,795
Foreign	1,533	1,548	5,102
Income before income taxes	$ 189,471	$ 95,356	$ 12,897

The benefit (expense) for income taxes consists of the following:

	Years Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Current:			
Federal	$ (2,987)	$ (1,445)	$ (125)
State	(8,257)	(4,599)	(1,702)
Foreign	(671)	(153)	(138)
Total current tax (expense)	$ (11,915)	$ (6,197)	$ (1,965)
Deferred:			
Federal	$ 19,637	$ —	$ —
State	7,188	—	—
Foreign	450	—	—
Total deferred tax benefit	$ 27,275	$ —	$ —
Total:			
Federal	$ 16,650	$ (1,445)	$ (125)
State	(1,069)	(4,599)	(1,702)
Foreign	(221)	(153)	(138)
Total benefit (expense) for income taxes	$ 15,360	$ (6,197)	$ (1,965)

Income taxes paid, net of refunds, consisted of the following:

	Years Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Federal	$ 12,210	$ 1,740	$ —
State:			
Kentucky	3,055	1,158	—
Pennsylvania	(880)	880	—
California	—	276	—
Other States	149	1,611	—
Total State	2,324	3,925	—
Foreign	259	—	—
Total Taxes Paid (Net of Refunds)	$ 14,793	$ 5,665	$ —

A reconciliation of income tax (benefit) expense computed at the statutory federal and state income tax rate for the year to income tax expense as reflected in our financial statements for years ended December 31, 2025, 2024 and 2023 are as follows:

(in thousands, except percentages)	Years Ended December 31,					
	2025		2024		2023	
	Amount	Percent	Amount	Percent	Amount	Percent
Federal Income Tax Expense at Statutory Rate	$ 39,791	21.0 %	$ 20,025	21.0 %	$ 2,708	21.0 %
State and Local Income Taxes, Net of Federal Income Tax Effect [(a)]	(5,227)	(2.8)	(547)	(0.6)	1,340	10.4
Change in Valuation Allowance	(51,231)	(27.1)	(8,833)	(9.3)	1,258	9.8
Nontaxable or Nondeductible Items						
Stock compensation	(2,721)	(1.4)	(4,793)	(5.0)	(1,715)	(13.3)
Executive compensation	4,570	2.4	2,645	2.8	2,675	20.7
Other nontaxable or nondeductible items	81	—	71	0.1	95	0.7
Effect of Cross Border Tax Laws						
Global Intangible Low-taxed Income (GILTI)	1	—	(220)	(0.2)	623	4.8
Foreign Derived Intangible Income (FDII)	(1,178)	(0.6)	(635)	(0.7)	—	—
Tax Credits						
R&D tax credits	(3,596)	(1.9)	(3,150)	(3.3)	(2,782)	(21.6)
Orphan drug credits	(931)	(0.5)	(1,416)	(1.5)	(1,570)	(12.2)
Other credits	—	—	16	—	(106)	(0.8)
Change in Unrecognized Tax Benefits	5,227	2.8	3,316	3.5	—	—
Other Adjustments						
Other comprehensive income	—	—	(114)	(0.1)	389	3.0
Other adjustments	(45)	—	5	—	(16)	(0.1)
Foreign Tax Effects						
Australia						
Other nondeductible expenses	—	—	(137)	(0.1)	137	1.1
Valuation allowance	—	—	(52)	(0.1)	(140)	(1.1)
Other Australia	—	—	57	0.1	11	0.1
Switzerland						
Foreign rate differential	(69)	—	(62)	(0.1)	(470)	(3.6)
Valuation allowance	—	—	—	—	(475)	(3.7)
Other Switzerland	31	—	(2)	—	—	—
Other Foreign Jurisdictions	(63)	—	23	—	3	—
Total Tax (Benefit) Expense	$ (15,360)	(8.1)%	$ 6,197	6.5 %	$ 1,965	15.2 %

[(a)] In 2025 and 2024, state and local income taxes in Kentucky comprised the majority of the state and local income taxes, net of federal effect category. In 2023, state and local income taxes in Kentucky and Pennsylvania comprised the majority of the state and local income taxes, net of federal category.

The significant components of the Company's deferred tax assets and liabilities as of December 31, 2025 and 2024 are as follows:

	December 31,	
(in thousands)	**2025**	**2024**
Deferred tax assets:		
Net operating loss carryforwards	$ 13,245	14,539
Stock compensation	11,432	9,090
Lease liability	2,205	1,784
Accrued expenses	2,419	1,929
Section 174 R&D capitalization	4,382	27,230
Intangible assets	15,946	17,632
Credits	1,855	10,376
Inventory	1,018	558
Other	16	—
Deferred tax assets	52,518	83,138
Valuation allowance	(14,214)	(68,094)
Deferred tax assets	38,304	15,044
Deferred tax liabilities:		
Depreciation	(11,761)	(11,771)
Right-of-use assets	(1,750)	(1,543)
Prepaid expenses	(1,388)	(1,647)
Unrealized gain on marketable securities	(581)	(83)
Total deferred tax liabilities	(15,480)	(15,044)
Net deferred tax assets	$ 22,824	$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its net U.S. deferred tax assets. Under the applicable accounting standards, management has considered the Company's history of operating losses and the uncertainty around any sustained future profitability. The Company has concluded that it is more likely than not that the Company will realize the benefits of its net deferred tax assets. Accordingly, the Company has decreased the valuation allowance for deferred tax assets from $68.0 million as of December 31, 2024 to $14.2 million as of December 31, 2025.

As of December 31, 2025 and 2024, the Company had federal research and development credit carryforwards of $0.5 million and $6.6 million, respectively. The federal tax credit carryforwards will begin to expire in 2042 if not utilized.

As of December 31, 2025 and 2024, the Company also had orphan drug tax credit carryforwards of $0.1 million and $3.8 million, respectively. The orphan drug tax credit carryforwards will begin to expire in 2042 if not utilized.

As of December 31, 2025 and 2024, the Company had state research and development credit carryforwards of $1 million and $0.7 million respectively. The state research and development credit carryforwards will begin to expire in 2038 if not utilized.

As of December 31, 2025, the Company had cumulative U.S. state net operating loss carryforwards of $166.2 million. The state net operating losses are available to offset future state income tax liabilities and will begin to expire in 2037.

Under the provisions of the Internal Revenue Code, the net operating loss carryforwards and tax credits utilized during the year are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Internal Revenue Code Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities.

At December 31, 2025, deferred tax assets have been recognized on our consolidated balance sheets related to federal research and development credits and orphan drug credits. The Company fully utilized its federal net operating losses in 2025. If we have previously had, or have in the future, one or more Section 382 or 383 "ownership changes," including in connection with our initial public offering or another offering, or if we do not generate sufficient taxable income, we may not be able to utilize a material portion of our federal tax credits.

The "One Big Beautiful Bill Act" (OBBBA) enacted on July 4, 2025, introduced notable changes to the U.S. Internal Revenue Code, including immediate expensing of domestic Section 174 costs. Section 174 costs are expenditures which represent research and development costs that are incident to the development or improvement of a product, process, formula, invention, computer software, or technique. As previously required under the Tax Cuts and Jobs Act, we capitalized research and development expenditures in the years ended December 31, 2022 through December 31, 2024. With the enactment of OBBBA, we began deducting 2025 and cumulative domestic Section 174 costs.

As of December 31, 2025, we have a deferred tax asset of $4.4 million related to capitalized Section 174 expenditures.

The Company files income tax returns in the United States at the federal and state level and in foreign jurisdictions in which the Company conducts business activities. The federal and state income tax returns are subject to tax examinations for the tax years ended December 31, 2022 through December 31, 2025. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Federal or state tax authorities to the extent utilized in a future period. Additionally, the Company is subject to tax examinations by taxing authorities in foreign jurisdictions where it has business operations. At this time, the Company is not undergoing examination by the Internal Revenue Service or any state or foreign taxing authorities.

The Company is subject to income taxes in U.S. federal, various state, and foreign jurisdictions. Significant judgment is required in evaluating the Company's tax positions and determining the provision for income taxes. During the ordinary course of business, there are many transactions and calculations for which the ultimate tax determination is uncertain. The Company establishes reserves for tax-related uncertainties based on estimates of whether, and the extent to which, additional taxes will be due. These reserves are established when the Company believes that certain positions might be challenged despite the belief that the tax return positions are fully supportable. The Company adjusts these reserves in light of changing facts and circumstances. The provision for income taxes includes the impact of reserve provisions and changes to reserves that are considered appropriate. We do not anticipate significant increases or decreases to the amount of unrecognized tax benefits within the next twelve months.

As of December 31, 2025, 2024, and 2023 the Company had unrecognized tax benefits of $10.0 million, $4.2 million and zero, respectively, of which $8.0 million, $3.3 million, and zero, respectively, if fully recognized would decrease the Company's effective tax rate. A reconciliation of unrecognized tax benefits for the years ended December 31, 2025, 2024, and 2023 are as follows:

	Years Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Unrecognized tax benefits - January 1	$ 4,152	$ —	$ —
Gross increases to tax positions in prior periods	34		
Gross increases to current period tax positions	5,863	4,152	—
Settlements with tax authorities	—	—	—
Lapse in statute of limitations	$ —	$ —	$ —
Unrecognized tax benefits - December 31	$ 10,049	$ 4,152	$ —

As of December 31, 2025, 2024, and 2023 the Company had accrued interest and penalties related to unrecognized tax benefits of $0.8 million, $0.2 million, and zero, respectively. The Company recognizes interest expense and any related penalties from unrecognized tax benefits in income tax expense.

The Company is also subject to taxation in various states and other foreign jurisdictions including Switzerland, Netherlands, France, Germany, Japan, United Kingdom, Italy and Spain.

12. Gain on Sale of Priority Review Voucher

In August 2023, the Company entered into an agreement to sell the rare pediatric disease priority review voucher ("PRV"), which was awarded to the Company in connection with the FDA's approval of VYJUVEK. The transaction closed in August 2023 and was not subject to any commissions or closing costs. The proceeds of $100.0 million from the sale of the PRV were recorded as a gain from sale of priority review voucher on the Company's consolidated statement of operations and comprehensive income as it did not have a carrying value at the time of the sale.

13. Segment Information

The Company operates as one operating segment, which is focused on the discovery, development, manufacturing and commercialization of genetic medicines to treat diseases with high unmet medical needs. The Company's chief operating decision maker ("CODM"), its chief executive officer, utilizes financial information presented on a consolidated basis to

manage and allocate resources. The CODM uses consolidated gross margin, operating margin, net income and total research and development expenses by product candidate or program to assess performance, forecast future financial results and allocate resources.

The following table presents selected financial information with respect to the Company's single operating segment for the years ended December 31, 2025, 2024, and 2023:

	Years Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Product revenues, net	$ 389,130	$ 290,515	$ 50,699
Less:			
Cost of goods sold	23,049	20,061	3,094
Gross margin	366,081	270,454	47,605
Gross margin percentage	94 %	93 %	94 %
B-VEC	6,690	8,760	9,039
KB111	1,837	—	—
KB301	184	635	485
KB304	960	1,342	66
KB407	1,805	1,877	1,668
KB408	882	1,630	1,043
KB707	10,856	8,677	3,828
KB801	2,175	1,314	—
KB803	2,564	604	—
Other dermatology programs	12	935	284
Other ophthalmology programs	44	554	71
Other programs	2,493	2,098	1,506
Other research and development costs [1]	27,543	25,154	28,443
Research and development	58,045	53,580	46,433
Selling, general and administrative	146,741	113,626	98,289
Litigation settlement	—	37,500	12,500
Operating income (expense)	$ 161,295	$ 65,748	$ (109,617)
Other income			
Gain from sale of priority review voucher	—	—	100,000
Interest and other income, net	28,176	29,608	22,514
Income before income taxes	189,471	95,356	12,897
Income tax benefit (expense)	15,360	(6,197)	(1,965)
Net income	204,831	89,159	10,932

(1) Includes stock-based compensation, other manufacturing expenses related to our product candidates and other unallocated expenses which largely relates to depreciation and other facilities and equipment related costs

14. Subsequent Events

The Company evaluates events or transactions that occur after the balance sheet date, but prior to the issuance of the financial statements, to identify matters that require disclosure. The Company concluded that no subsequent events have occurred that would require recognition or disclosure in the consolidated financial statements.

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

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Under the supervision of our Chief Executive Officer and Chief Accounting Officer, we evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) as of December 31, 2025. Based on that evaluation, our Chief Executive Officer and Chief Accounting Officer have concluded that our disclosure controls and procedures were effective as of December 31, 2025 to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Accounting Officer, as appropriate to allow timely discussion regarding required disclosures. In designing and evaluating our disclosure controls and procedures, management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. 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 its judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f). Our management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2025 based on the criteria established in *Internal Control - Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission.

Based on the results of its evaluation, management concluded that our internal control over financial reporting was effective as of December 31, 2025. The effectiveness of our internal control over financial reporting as of December 31, 2025 has been audited by KPMG, an independent registered public accounting firm, as stated in their report which is included herein.

Inherent Limitations on Controls and Procedures

Our management, including the Chief Executive Officer and Chief Accounting Officer, do not expect that our disclosure controls and procedures and our internal controls will prevent all error and all fraud. A control system, no matter how well designed and operated, can only provide reasonable assurances that the objectives of the control system are met. The design of a control system reflects resource constraints; the benefits of controls must be considered relative to their costs. Because there are inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within the Company have been or will be detected. As these inherent limitations are known features of the financial reporting process, it is possible to design into the process safeguards to reduce, though not eliminate, these risks. These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns occur because of simple error or mistake. Controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls is based in part upon certain assumptions about the likelihood of future events. While our disclosure controls and procedures are designed to provide reasonable assurance of achieving their objectives, there can be no assurance that any design will succeed in achieving its stated goals under all future conditions. Over time, controls may become inadequate because of changes in conditions or deterioration in the degree of compliance with the policies or procedures. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

We intend to review and evaluate the design and effectiveness of our disclosure controls and procedures on an ongoing basis and to improve our controls and procedures over time and to correct any deficiencies that we may discover in the future. While our Chief Executive Officer and Chief Accounting Officer have concluded that, as of December 31, 2025, the design of our disclosure controls and procedures, as defined in Rule 13a-15(e) under the Exchange Act, was effective, future events affecting our business may cause us to significantly modify our disclosure controls and procedures.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) or 15d-15(d) of the Exchange Act that occurred during the three months ended December 31, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors

Krystal Biotech, Inc.:

Opinion on Internal Control Over Financial Reporting

We have audited Krystal Biotech, Inc. and subsidiaries' (the Company) internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2025 and 2024, the related consolidated statements of operations and comprehensive income, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2025, and the related notes (collectively, the consolidated financial statements), and our report dated February 17, 2026 expressed an unqualified opinion on those consolidated financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's 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 for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of 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 or procedures may deteriorate.

/s/ KPMG LLP

Pittsburgh, Pennsylvania
February 17, 2026

Item 9B. Other Information.

Insider Trading Arrangements

On November 6, 2025, Kathryn Romano, our Chief Accounting Officer, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 20,000 shares of the Company's common stock and a gift of up to 750 shares of the Company's common stock. The Rule 10b5-1 trading arrangement will continue until August 31, 2026, subject to early termination in accordance with the terms of the Rule 10b5-1 trading arrangement, including upon completion of the sale of all of the shares of the Company's common stock subject to the Rule 10b5-1 trading arrangement.

On November 25, 2025, Daniel Janney, a member of the Company's Board of Directors, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 90,000 shares of the Company's common stock, a portion of which will be issued upon exercise of stock options that if not exercised will expire in 2026. The Rule 10b5-1 trading arrangement will continue until November 5, 2026, subject to early termination in accordance with the terms of the Rule 10b5-1 trading arrangement, including upon completion of the sale of all of the shares of the Company's common stock subject to the Rule 10b5-1 trading arrangement.

During the three months ended December 31, 2025, other than as disclosed above, none of our directors or officers (as that term is defined by the SEC in Rule 16a-1(f) under the Exchange Act) adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement.

Item 9C. Disclosure Regarding Foreign Jurisdictions That Prevent Inspections.

Not Applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Information required by this Item is hereby incorporated by reference to our 2026 Definitive Proxy Statement, which will be filed prior to April 30, 2026.

We maintain a Code of Business Conduct and Ethics for our employees, officers, and directors, including our principal executive officer, principal financial officer, principal accounting officer or controller and other persons performing similar functions. To view this code of ethics free of charge, please visit the investors section of our website at www.krystalbio.com. (The website address is not intended to function as a hyperlink, and the information contained in our website is not intended to be a part of this filing.) We intend to satisfy the disclosure requirements under Item 5.05 of Form 8-K regarding an amendment to or a waiver from a provision of this Code of Business Conduct and Ethics, if any, by posting such information on our website as set forth above.

Item 11. Executive Compensation.

Information required by this Item is hereby incorporated by reference to our 2026 Definitive Proxy Statement, which will be filed prior to April 30, 2026.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

Information required by this Item is hereby incorporated by reference to our 2026 Definitive Proxy Statement, which will be filed prior to April 30, 2026.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

Information required by this Item is hereby incorporated by reference to our 2026 Definitive Proxy Statement, which will be filed prior to April 30, 2026.

Item 14. Principal Accountant Fees and Services.

Information required by this Item is hereby incorporated by reference to our 2026 Definitive Proxy Statement, which will be filed prior to April 30, 2026.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(a) List the following documents filed as a part of the report:

(1) Financial statements

The response to this portion of Item 15 is set forth under Item 8 above.

(2) Financial statement schedule.

All schedules have been omitted because they are not required or because the required information is given in the financial statements or notes thereto set forth under Item 8 above.

(3) Exhibits.

A list of exhibits filed with this report or incorporated herein by reference can be found in the Exhibit Index of this Report.

Exhibit Index

Exhibit Number	Description
3.1	Second Amended and Restated Certificate of Incorporation of Krystal Biotech, Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, as filed with the SEC on September 25, 2017)
3.2	Amended and Restated Bylaws of Krystal Biotech, Inc. (incorporate by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, as filed with the SEC on September 25, 2017
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)
4.2	Form of Indenture (including form of Debt Securities) (incorporated by reference to Exhibit 4.5 to the Company's Registration Statement on Form S-3 (Reg. No. 333-237983), as filed with the SEC on May 4, 2020)
4.3	Description of Common Stock (incorporated by reference to Exhibit 4.3 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 27, 2023)
10.1#	Form of Indemnification Agreement by and between Krystal Biotech, Inc. and each of its directors and executive officers (incorporated by reference to Exhibit 10.1 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)
10.2#	Executive Employment Agreement, effective July 1, 2017, by and between Krystal Biotech, Inc. and Krish S. Krishnan (incorporated by reference to Exhibit 10.2 to the Company's Amendment No. 1 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 7, 2017)
10.3#	Executive Employment Agreement, effective May 1, 2017, by and between Krystal Biotech, Inc. and Suma M. Krishnan (incorporated by reference to Exhibit 10.3 to the Company's Amendment No. 1 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 7, 2017)
10.4#	Executive Employment Agreement, effective January 20, 2020, by and between Krystal Biotech, Inc. and Kathryn A. Romano (incorporated by reference to Exhibit 10.4 to the Company's Annual Report on Form 10-K, as filed with the SEC on March 1, 2021)
10.5#	Krystal Biotech, Inc. 2017 Stock Incentive Plan (incorporated by reference to Exhibit 10.6 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)
10.6#	Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan (incorporated by reference to Exhibit 10.7 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)
10.7#	Form of Krystal Biotech, Inc. 2017 Stock Incentive Plan Notice of Stock Option Award (incorporated by reference to Exhibit 10.8 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)
10.8#	Form of Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan Notice of Stock Option Award (incorporated by reference to Exhibit 10.9 to the Company's Amendment No. 2 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 14, 2017)

Exhibit Number	Description
10.9	Lease Agreement, dated as of May 26, 2016, by and between Wharton Lender Associates, L.P. and Krystal Biotech, LLC (incorporated by reference to Exhibit 10.10 to the Company's Amendment No. 1 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 7, 2017)
10.10	Second Amendment to Lease Agreement, dated as of February 27, 2017, by and between Wharton Lender Associates, L.P. and Krystal Biotech, LLC (incorporated by reference to Exhibit 10.11 to the Company's Amendment No. 1 to the Company's Registration Statement on Form S-1 (Reg. No. 333-220085), as filed with the SEC on September 7, 2017)
10.11	Third amendment to Lease Agreement, dated as of May 31, 2018, by and between Wharton Lender Associate, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.13 to the Company's Annual Report on Form 10-K, as filed with the SEC on March 1, 2021)
10.12	Fourth amendment to Lease Agreement, dated as of October 22, 2018, by and between Wharton Lender Associate, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.14 to the Company's Annual Report on Form 10-K, as filed with the SEC on March 1, 2021)
10.13	Fifth amendment to Lease Agreement, dated as of December 10, 2018, by and between Wharton Lender Associate, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.15 to the Company's Annual Report on Form 10-K, as filed with the SEC on March 1, 2021)
10.14	Sixth amendment to Lease Agreement and first amendment to storage space agreement, dated as of January 13, 2021, by and between Wharton Lender Associates, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.17 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 28, 2022)
10.15	Seventh amendment to Lease Agreement, dated as of May 11, 2021, by and between Wharton Lender Associates, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.18 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 28, 2022)
10.16	Eighth amendment to Lease Agreement, dated as of July 21, 2021, by and between Wharton Lender Associates, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.19 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 28, 2022)
10.17	Ninth amendment to Lease Agreement, dated as of January 4, 2022, by and between Wharton Lender Associates, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.20 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 28, 2022)
10.18	Purchase and Sale Agreement, dated January 29, 2021, by and between Krystal Biotech, Inc. and Northfield I, LLC. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, as filed with the SEC on February 2, 2021)
10.19#	Form of Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan Notice of Restricted Stock Award and Restricted Stock Award Agreement (incorporated by reference to Exhibit 10.23 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 27, 2023)
10.20#	Form of Time-Based Restricted Stock Unit Award Agreement under the 2017 IPO Stock Incentive Plan (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, as filed with the SEC on May 8, 2023)
10.21#	Form of Performance-Based Restricted Stock Unit Award Agreement under the 2017 IPO Stock Incentive Plan (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, as filed with the SEC on May 8, 2023)
10.22#	Krystal Biotech, Inc. Executive Change in Control Severance Plan, with an effective date of August 2, 2024 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, as filed with the SEC on August 5, 2024)
10.23#	First Amendment to the Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan, made and entered into effective as of August 2, 2024 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, as filed with the SEC on August 5, 2024)
10.24	Twelfth amendment to Lease Agreement, dated as of September 27, 2024, by and between Wharton Lender Associates, L.P. and Krystal Biotech, Inc. (incorporated by reference to Exhibit 10.29 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 19, 2025)
19.1	Krystal Biotech, Inc. Insider Trading Policy and Guidelines for Disclosure of Material Non-Public Information (incorporated by reference to Exhibit 19.1 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 19, 2025)
21.1*	Subsidiaries of Krystal Biotech, Inc.
23.1*	Consent of KPMG LLP

Exhibit Number	Description
24.1	Power of Attorney (included as part of signature page)
31.1*	Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Chief Executive Officer and Chief Accounting Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1	Executive Incentive Compensation Recoupment Policy, adopted August 4, 2023 (incorporated by reference to Exhibit 97.1 to the Company's Annual Report on Form 10-K, as filed with the SEC on February 26, 2024)
101	(i) XBRL Instance Document, (ii) XBRL Taxonomy Extension Schema Document, (iii) XBRL Taxonomy Extension Calculation Linkbase Document, (iv) XBRL Taxonomy Extension Definition Linkbase Document, (v) XBRL Taxonomy Extension Label Linkbase Document, (vi) XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101).

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* Filed herewith.

\# Indicates a management contract or compensatory plan or arrangement.

Item 16. Form 10-K Summary.

The Company has elected to not include a summary.

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 report to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Pittsburgh, Commonwealth of Pennsylvania, on February 17, 2026.

KRYSTAL BIOTECH, INC.

By: /s/ Krish S. Krishnan
Krish S. Krishnan
President and Chief Executive Officer

By: /s/ Kathryn A. Romano
Kathryn A. Romano
Chief Accounting Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Krish S. Krishnan and/or Kathryn A. Romano as his or her true and lawful attorney-in-fact and agent, with the full power of substitution, for him or her and in his or her name, place or stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that said attorneys-in-fact, or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Krish S. Krishnan Krish S. Krishnan	President and Chief Executive Officer and Director (Principal Executive Officer)	February 17, 2026
/s/ Kathryn A. Romano Kathryn A. Romano	Chief Accounting Officer (Principal Financial Officer)	February 17, 2026
/s/ Suma M. Krishnan Suma M. Krishnan	President, R&D and Director	February 17, 2026
/s/ Daniel S. Janney Daniel S. Janney	Director	February 17, 2026
/s/ Dino A. Rossi Dino A. Rossi	Director	February 17, 2026
/s/ Julian Gangolli Julian Gangolli	Director	February 17, 2026
/s/ Chris Mason Chris Mason	Director	February 17, 2026
/s/ E. Rand Sutherland E. Rand Sutherland	Director	February 17, 2026
/s/ Catherine Mazzacco Catherine Mazzacco	Director	February 17, 2026