

**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, 2024**

**or**

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

**For the transition period from                      to  
Commission file number: 000-30171**

**SANGAMO THERAPEUTICS, INC.**

**(Exact name of registrant as specified in its charter)**

**Delaware**  
**(State or other jurisdiction of  
incorporation or organization)**  
  
**501 Canal Blvd.**  
**Richmond, California**  
**(Address of principal executive offices)**

**68-0359556**  
**(I.R.S. Employer  
Identification No.)**  
  
**94804**  
**(Zip Code)**

**(510) 970-6000**  
**(Registrant's telephone number, including area code)**

**Not Applicable**  
**(Former name, former address and former fiscal year, if changed since last report)**

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

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common Stock, par value \$0.01 per share	SGMO	Nasdaq Capital Market

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

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

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Exchange 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 definition of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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 the common stock held by non-affiliates of the registrant based upon the closing sale price of the common stock on June 28, 2024 (the last business day of the registrant's most recently completed second fiscal quarter), as reported on the Nasdaq Global Select Market was \$74,246,155. For purposes of this calculation, directors and executive officers of the registrant have been deemed affiliates. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 13, 2025, a total of 224,710,019 shares of common stock, \$0.01 par value per share were outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Certain information required by Part III, Items 10-14 of this Form 10-K is incorporated by reference to the registrant's definitive Proxy Statement for the 2025 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Form 10-K, provided that if such Proxy Statement is not filed within such period, such information will be included in an amendment to this Form 10-K to be filed within such 120-day period.



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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

Some statements contained in this report are “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. These statements relate to our future events, including our anticipated operations, research, development, manufacturing and commercialization activities, clinical trials, operating results and financial condition. These forward-looking statements 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 may include, but are not limited to, statements about:

- our estimates regarding the sufficiency of our cash resources and our expenses, capital requirements and need for substantial additional financing, and our ability to obtain additional financing;
- our ability to continue to operate as a going concern, including our estimate that our available cash and cash equivalents as of December 31, 2024 will not be sufficient to fund our planned operations for one year from the issuance date of the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.
- our projected operating and financial performance;
- our plans for advancing our development programs and the plans of any collaboration partners for advancing partnered programs;
- anticipated research and development of product candidates and potential commercialization of any resulting approved products;
- the initiation, scope, rate of progress, enrollment, dosing, anticipated results and timing of our preclinical studies and clinical trials and those of our collaborators or strategic partners;
- the therapeutic and commercial potential of our product candidates, including the durability of therapeutic effects;
- the therapeutic and commercial potential of technologies used by us in our product candidates, including our gene therapy and gene editing technologies, zinc finger, or ZF, technology platform, and zinc finger transcriptional regulators, or ZF-transcriptional regulators, which include zinc finger repressors, or ZFRs;
- our ability to realize the expected benefits of our license agreements with Genentech, Inc., a member of the Roche group, or Genentech, and Astellas Gene Therapies, Inc., or Astellas, the potential for these licensees to complete clinical development, regulatory interactions, manufacturing and global commercialization of any resulting products, and the potential for us to receive milestone payments and/or additional fees and royalties from these licensees;
- anticipated investigational new drug, or IND, and clinical trial authorisation, or CTA, submissions and potential acceptance thereof by the U.S. Food and Drug Administration, or FDA, and regulatory authorities outside the United States;
- the potential for isaralgagene civaparvovec to obtain Accelerated Approval from the FDA, including the adequacy of data generated in the Phase 1/2 STAAR study to support any such approval, and our expectations concerning the availability of additional data to support a potential Biologics License Application, or BLA, submission for isaralgagene civaparvovec and the timing of such submission;
- our ability to establish and maintain collaborations and strategic partnerships and realize the expected benefits of such arrangements, including our ability to find a commercialization partner for our Fabry disease program and to enter into new collaborations with respect to our STAC-BBB capsid, epigenetic regulation capabilities and hemophilia A program;
- anticipated revenues from existing and new collaborations and the timing thereof;
- our and our collaborators’ anticipated plans and timelines in conducting our ongoing and potential future clinical trials and presenting clinical data from such clinical trials, and the anticipated advancement of our product candidates to late-stage development;
- our estimates regarding the impact of the macroeconomic environment on our business and operations and the business and operations of our collaborators, including preclinical studies, clinical trials and manufacturing, and our ability to manage such impacts;
- our research and development and other expenses;



- our ability to obtain adequate preclinical and clinical supplies of our product candidates from current and potential new suppliers and manufacturers;
- our ability, and the ability of our collaborators and strategic partners, to obtain and maintain regulatory approvals for product candidates and the timing and costs associated with obtaining regulatory approvals;
- our ability to comply with, and the impact of, regulatory requirements, obligations and restrictions on our business and operations;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others, including our ability to obtain and maintain rights to the technologies required to develop and commercialize our product candidates;
- competitive developments, including the impact on our competitive position of rival products and product candidates and our ability to meet such competition;
- our operational and legal risks; and
- our plans, objectives, expectations and intentions and any other statements that are not historical facts.

In some cases, you can identify forward-looking statements by use of future dates or by terms such as: “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “potential,” “seeks,” “should,” “will” and similar expressions intended to identify forward-looking statements. These statements reflect our current views with respect to future events, are based on assumptions and are subject to risks and uncertainties. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. We discuss many of these risks in greater detail under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in this Annual Report on Form 10-K. Except as required by law, we undertake no obligation to update or revise any forward-looking statements to reflect new information or future events or developments. Readers are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this Annual Report on Form 10-K.



## SUMMARY OF RISK FACTORS

Our business involves significant risks. Below is a summary of the material risks that our business faces, which makes an investment in our common stock speculative and risky. This summary does not address all these risks. These risks are more fully described below under the heading “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K. Before making investment decisions regarding our common stock, you should carefully consider these risks. The occurrence of any of the events or developments described below could have a material adverse effect on our business, results of operations, financial condition and prospects. In such event, the market price of our common stock could decline, and you could lose all or part of your investment. There are also additional risks not described below that are either not presently known to us or that we currently deem immaterial, and these additional risks could also materially impair our business, operations or market price of our common stock.

- There is substantial doubt about our ability to continue to operate as a going concern. We need substantial additional funding to execute our operating plan and to continue to operate as a going concern. If adequate funds are not available to us on a timely basis, or at all, we will be required to take additional actions to address our liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering our research and development activities, which would have a material adverse effect on our business and prospects, or we may be required to cease operations entirely, liquidate all or a portion of our assets, and/or seek protection under the U.S. Bankruptcy Code, and you may lose all or part of your investment. Future sales and issuances of equity securities would also result in substantial dilution to our stockholders.
- Our ability to continue funding our operations, advance development of our product candidates and ultimately commercialize our technologies depends on our ability to secure collaboration partners for our programs. If we are not able to find collaborators, or if our collaborators do not diligently pursue product development efforts, we may not be able to secure sufficient capital to continue to operate as a going concern or to develop our technologies or product candidates.
- We are a biotechnology company with no approved products or product revenues. Our success depends substantially on results of preclinical studies and clinical trials demonstrating safety and efficacy of our product candidates to the satisfaction of applicable regulatory authorities. Obtaining positive clinical trial results and regulatory approvals is expensive, lengthy, challenging and unpredictable and may never occur for any product candidates.
- Our core preclinical neurology programs, which are the current focus of our research and development efforts, are in the early stages. We may encounter difficulties in advancing product candidates from research programs to preclinical and clinical development and may fail to capitalize on product candidates with a greater commercial opportunity or for which there is a greater likelihood of success.
- Success in research and preclinical studies or early clinical trial results may not be indicative of results obtained in later trials. Likewise, preliminary, initial or interim data from clinical trials may be materially different from final data.
- We have historically incurred significant operating losses since inception and anticipate continued losses for the foreseeable future. We may never become profitable.
- Biotechnology and genomic medicine are highly competitive businesses. Our competitors may develop rival technologies and products that are superior to or are commercialized more quickly than our technologies and product candidates.
- The manufacture, storage and transport of our product candidates is complex, expensive, highly regulated and risky, which could hamper their commercial viability.
- Even if our product development efforts are successful and even if the requisite regulatory approvals are obtained, our products may not gain market acceptance among physicians, patients, healthcare payors and the medical community.
- Because it is difficult, time consuming and costly to obtain, maintain and enforce patent protections for our technologies and product candidates, and because third parties may have made inventions that are similar to ours, we may not be able to secure optimal patent protections of our technologies and product candidates.
- We may be involved in patent or intellectual property lawsuits or similar disputes involving patents under our control or patents of third parties claiming infringement, which lawsuits could be expensive, time-consuming and impair or prevent development and commercialization activities.



- We have experienced and may continue to experience difficulties in hiring, integrating and retaining qualified skilled employees.
- Unfavorable global economic conditions could have a negative impact on our operations, which could materially and adversely affect our ability to continue to operate as a going concern and otherwise have a material adverse effect on our business, financial condition, results of operations, prospects and market price of our common stock.
- We have in the past, and may in the future, be unable to comply with the listing standards of the Nasdaq Stock Market LLC. If we fail to comply with such listing standards, our common stock may be delisted. Delisting could adversely affect the liquidity of our common stock and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue to operate as a going concern would be substantially impaired.
- Our stock price has been volatile and will likely continue to be volatile, which could result in substantial losses for investors and potentially class action securities litigation against us, and could be influenced by public perception of genomic medicines and the biotechnology sector.
- We have fully impaired our goodwill and indefinite-lived intangible assets, have recorded significant impairment of our long-lived assets, and may be required to record significant additional charges if our long-lived assets become further impaired in the future.



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## PART I

### ITEM 1 – BUSINESS

#### OVERVIEW

We are a genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases. We believe our zinc finger epigenetic regulators are ideally suited to potentially address devastating neurology disorders and our capsid engineering platform has demonstrated the ability to expand delivery beyond currently available intrathecal delivery capsids, including in the central nervous system, or CNS, in preclinical studies.

##### *Our Core Neurology Programs*

Our neurology development is focused on two innovative areas: (i) development of epigenetic regulation therapies to treat serious neurological diseases and (ii) development of novel engineered adeno-associated virus, or AAV, capsids to deliver our therapies to the intended neurological targets.

Initial indications for our wholly owned preclinical programs include idiopathic small fiber neuropathy, or iSFN, a type of chronic neuropathic pain, and prion disease. Following clearance from the U.S. Food and Drug Administration, or FDA, of the investigational new drug application, or IND, for ST-503 for the treatment of iSFN in November 2024. We expect to commence enrollment and dosing of patients in the Phase 1/2 study in mid-2025 and anticipate submitting the clinical trial authorisation, or CTA, for our product candidate to treat prion disease in the first quarter of 2026. These timelines are each subject to our ability to secure adequate additional funding.

In addition, we are party to collaboration and license arrangements pursuant to which our collaborators are developing preclinical product candidates in indications such as tauopathies, amyotrophic lateral sclerosis, or ALS, and Huntington's disease. These partners include Genentech, Inc., a member of the Roche Group, or Genentech, Alexion Pharmaceuticals, Inc., or Alexion, and Takeda Pharmaceutical Company Limited, or Takeda.

We also continue to advance development of novel engineered AAV capsids enhanced for delivery to neurological targets and have identified a proprietary engineered neurotropic AAV capsid variant, STAC-BBB, that has demonstrated an ability to cross the blood-brain barrier, or BBB, in nonhuman primates, or NHPs, and mediated robust transduction, transgene expression, and targeted epigenetic repression throughout the brain and spinal cord after intravenous, or IV, administration. We believe this novel capsid has the potential to unlock multiple wholly owned neurology epigenetic regulation programs, and is already the subject of multiple license agreements, including with Genentech and Astellas Gene Therapies, Inc., or Astellas.

##### *Other Clinical Programs*

Other clinical-stage product candidates include:

- Isaralgagene civaparvovec, also known as ST-920, our wholly owned gene therapy product candidate for the treatment of Fabry disease, is currently being evaluated in our Phase 1/2 STAAR clinical study and has a clear regulatory pathway to Accelerated Approval from the FDA, in which we expect to use data from the ongoing Phase 1/2 STAAR study.
- Giroctocogene fitelparvovec, also known as SB-525, is a gene therapy product candidate for the treatment of moderately severe to severe hemophilia A, that we have co-developed with, and licensed to Pfizer Inc., or Pfizer, and to which we are scheduled to regain development and commercialization rights on April 21, 2025, following a decision by Pfizer in December 2024 not to proceed with the Biologics License Application, or BLA, and Marketing Authorization Application, or MAA, submissions for, or to pursue commercialization of, giroctocogene fitelparvovec.

##### *Our Novel Science and Technologies*

We are a leader in the research and development of zinc finger proteins, or ZFPs, which are abundantly occurring human proteins that have evolved to regulate the genome through interactions with DNA and regulatory proteins. Our strategy is to translate our differentiated and versatile zinc finger, or ZF, technology platform to create product candidates with best- or first-in-class clinical potential. We believe that the versatility and flexibility of our technology platforms enable us to design therapeutic approaches to resolve the underlying genetic or cellular causes of disease, using whichever technology is best suited to deliver that treatment. Our current area of focus is developing epigenetic regulation therapies with our ZF technology platform for serious neurological diseases.



We are also evaluating several potential routes of administration for our neurology-targeted investigational therapies, as delivery of genomic medicines to the CNS is a significant obstacle to developing therapies treating neurological disorders. We have developed a proprietary AAV capsid engineering platform, Selecting In vivo For Transduction and Expression of RNA, or SIFTER, with the aim of engineering capsids with improved CNS transduction and have presented results from capsids for both IV and cerebrospinal fluid, or CSF, administration.

We are also developing next generation modular integrase technology, engineered to enable large-scale genome editing. Building on our deep expertise in protein-DNA interactions derived from our zinc finger platform, the Modular Integrase, or MINT, platform is a versatile, protein-guided genome editing method designed to integrate large sequences of DNA into the genome to potentially treat – with a single medicine – patients who have unique mutations in the same gene.

In the process of developing these technologies, we have additionally accrued significant scientific and development capabilities, as well as manufacturing know-how, that are broadly applicable to the field of gene therapy, which we have used to develop our genomic medicine product candidates.

#### *Manufacturing*

We expect to be substantially reliant on external partners to manufacture clinical supply for our neurology portfolio. We retain our in-house analytical and process development capabilities.

#### *Collaborations and Licenses*

Our collaborations with biopharmaceutical companies bring us important financial and strategic benefits and reinforce the potential of our research and development efforts and our ZF technology platform. They leverage our collaborators' therapeutic and clinical expertise and commercial resources with the goal of bringing our medicines more rapidly to patients. We believe these collaborations reflect the value of our technology and will potentially expand the addressable markets of our product candidates. To date, we have received approximately \$887.0 million in upfront license fees, milestone payments and proceeds from sale of our common stock to collaborators and have the opportunity to earn up to \$4.7 billion in potential future milestone payments and additional licensed target fees from our ongoing collaborations and licenses to our capsid technology, in addition to potential product royalties.



## Product Pipeline

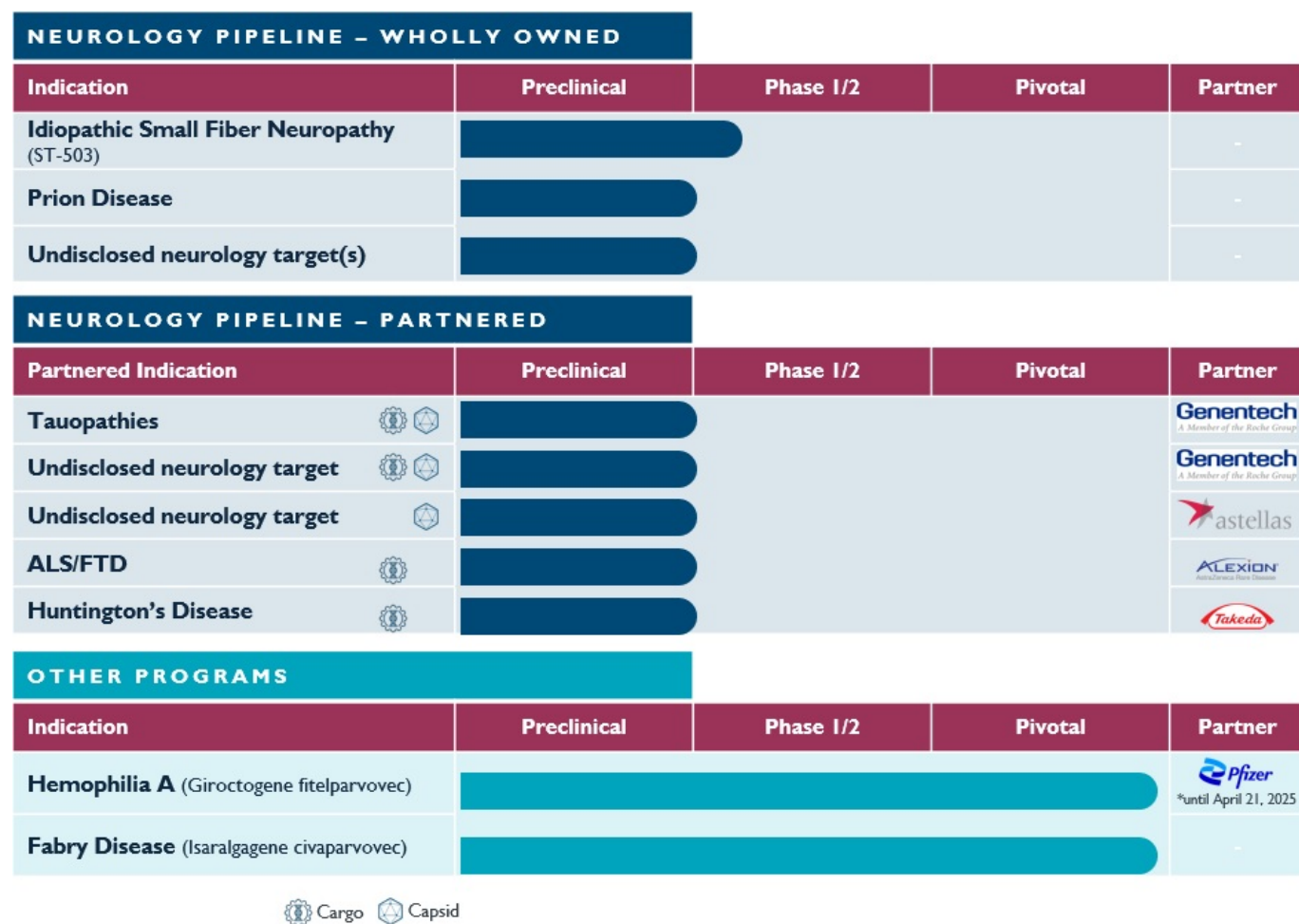


Figure 1: Our pipeline, subject to adequate additional funding

### Core Neurology Pipeline

#### *Chronic Neuropathic Pain – ST-503*

We continue to advance ST-503, an investigational epigenetic regulator for the treatment of intractable pain due to iSFN, a type of chronic neuropathic pain.

Neuropathic pain can be caused by a broad array of pathologies impacting the central or peripheral nervous systems, such as surgical trauma, spinal cord injury, nerve compression, neurological and infectious diseases, or metabolic and hereditary syndromes. ST-503 is not intended for sporadic or acute pain, but for chronic, intractable pain that completely dominates and often destroys the lives of patients over many years.

Following FDA clearance of the IND for iSFN in November 2024, we are planning to initiate enrollment and dosing of patients in the Phase 1/2 study for ST-503 in mid-2025, subject to our ability to secure adequate additional funding, with preliminary proof of efficacy data expected in the fourth quarter of 2026.







This misfolding of the prion protein is acutely toxic to neurons, and our aim is to remove a portion of prion protein from neurons to protect them from the toxicity of the misfolded prion protein. We think that this may prevent the spread and propagation of misfolded prion, and may therefore slow or halt neurodegeneration and disease progression.

At least 1,300 new cases of prion disease are identified each year in the United States and in Europe, with a similar presence globally.

We expect a CTA submission in the U.K. for the prion disease program in the first quarter of 2026, subject to our ability to secure adequate additional funding. We plan to begin clinical trial enrollment and dosing in mid-2026 and anticipate preliminary clinical data in the fourth quarter of 2026.

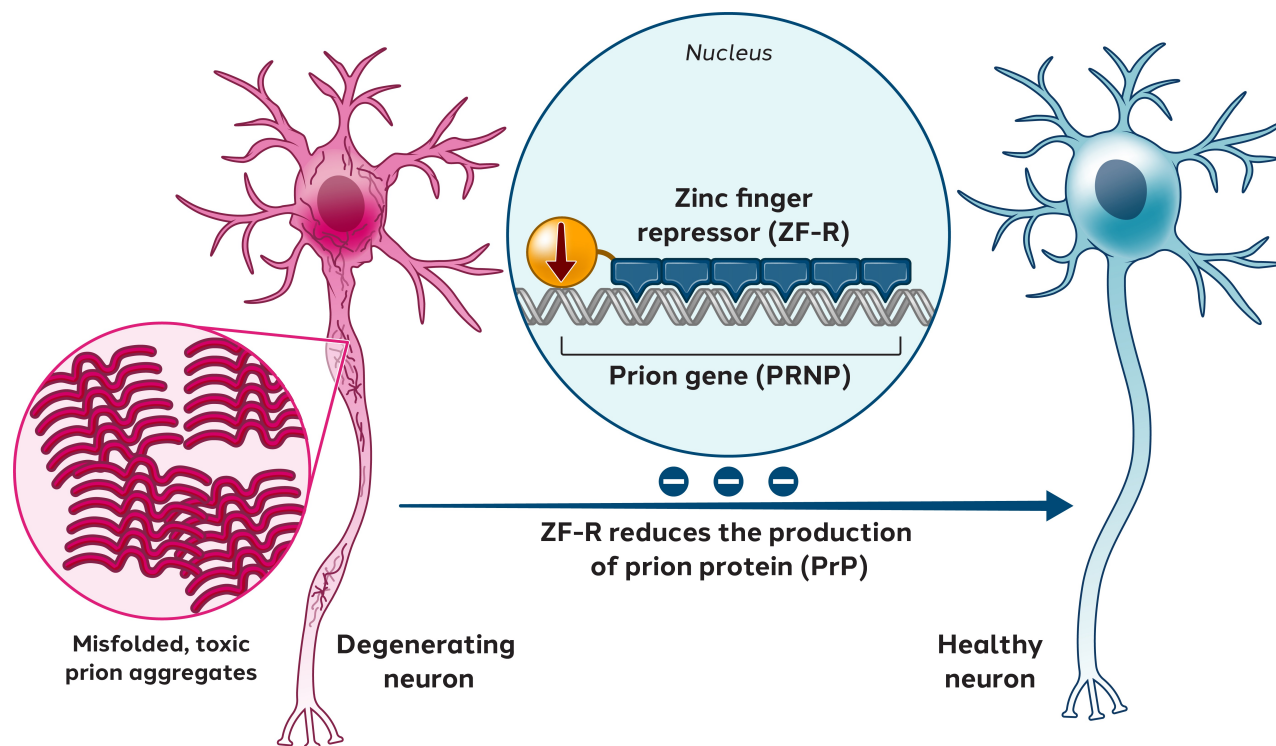


Figure 3: Our approach in prion disease using ZFRs

To address prion disease, we are developing ZFRs that target PRNP, to be delivered by STAC-BBB, our IV-administered neurotropic capsid. We presented updated preclinical data from this program at the Prion 2024 Conference in October 2024, showing the potency of Sangamo's ZFR in a disease mouse model at multiple dose levels. The ZFR significantly reduced expression of prion mRNA and protein in the brain, extended mouse survival and limited the formation of toxic prion aggregates. Additionally, we presented NHP data at the Prion 2024 Conference, showing that a single intravenous administration of the prion ZFR, delivered via STAC-BBB, resulted in potent and widespread repression of the prion gene in transduced neurons.

The clinical study is expected to be a Bayesian optimal interval design, to assess safety and efficacy, while potentially enabling rapid escalation to the maximum tolerated dose. The clinical study will use the Medical Research Council prion disease rating scale to assess the efficacy of the ZFR and compare to matched historic controls. The aim of the planned clinical study is to delay the progression of prion disease, offering potential for a meaningful extension of survival in prion patients.

### Clinical Programs

#### *Isaralgagene civaparvovec – Fabry Disease*

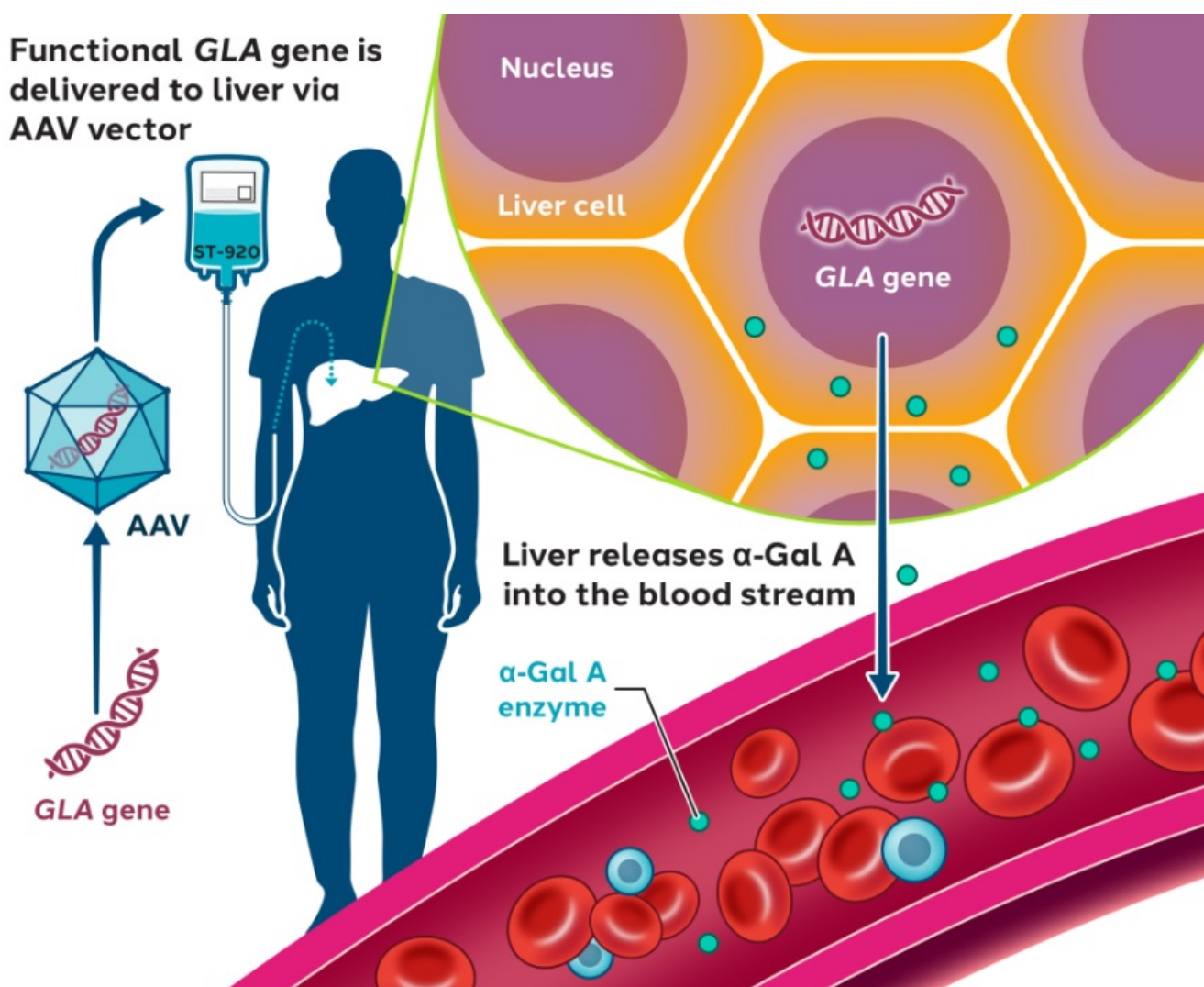
Isaralgagene civaparvovec, or ST-920, is our wholly owned gene therapy product candidate for the treatment of Fabry disease, a rare inherited metabolic disease. Isaralgagene civaparvovec is currently being evaluated in our Phase 1/2 STAAR clinical study and, as described below, has a clear regulatory pathway to Accelerated Approval from the FDA, in which we expect to use data from the ongoing Phase 1/2 STAAR study, with a potential BLA submission in the second half of 2025.

STAAR is an ongoing Phase 1/2 multicenter, open-label, dose-ranging clinical study designed to assess a single infusion of isaralgagene civaparvovec in symptomatic Fabry disease patients  $\geq 18$  years of age. Patients are infused intravenously with a single dose and followed for 52 weeks. A separate long term follow-up study is underway to monitor the patients treated in this study for up to five years following treatment to further assess safety, durability and efficacy. Patients



who are on stable Enzyme Replacement Therapy, or ERT, may withdraw ERT after treatment in a controlled and monitored fashion at the discretion of the patient and the investigator.

The dose escalation phase included males with classic Fabry disease. The study was subsequently expanded to enroll both males and females, including patients with Fabry-associated cardiac or renal disease. The study's primary endpoint is the incidence of treatment-emergent adverse events, or AEs. Secondary endpoints include change from baseline at specific time points over the one-year study period in alpha-galactosidase A, or  $\alpha$ -Gal A, activity, globotriaosylceramide, or Gb3, and lyso-Gb3 levels in plasma; frequency of ERT infusion; changes in renal function and cardiac function (left ventricular mass) measured by cardiac magnetic resonance imaging, or MRI, and rAAV2/6 vector clearance. Key exploratory endpoints include change from baseline in disease severity (Fabry Outcome Survey adaptation of the Mainz Severity Score Index, or FOS-MSSI, score), quality of life, or QoL, gastrointestinal, or GI, symptoms and neuropathic pain scores; and immune response to AAV6 capsid and  $\alpha$ -Gal A.



*Figure 4: Our approach in Fabry disease*

The goal of the study is to abrogate the need for ERT with a recombinant AAV2/6 vector encoding cDNA for human  $\alpha$ -Gal A, resulting in long-term expression of  $\alpha$ -Gal A. As a liver-directed gene therapy, isargalgene civaparvovec is designed to be delivered by a one-time IV infusion that does not require any preconditioning regimen for patients.

The FDA has granted Orphan Drug, Fast Track and regenerative medicine advanced therapy, or RMAT, designations to isargalgene civaparvovec, which has also received Orphan Medicinal Product designation and PRIME eligibility from the European Medicines Agency, or EMA, and Innovative Licensing and Access Pathway, or ILAP, from the U.K. Medicines and Healthcare products Regulatory Agency, or MHRA.

In October 2024, we announced the outcome of a successful interaction with the FDA that provided a clear regulatory pathway to Accelerated Approval for isargalgene civaparvovec. The FDA agreed in a Type B interaction that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using



estimated glomerular filtration rate, or eGFR, slope at 52 weeks across all patients as an intermediate clinical endpoint. We engaged with the FDA on alternative pathways to potential approval following analysis of clinical data from the Phase 1/2 STAAR study showing encouraging safety and efficacy data, including promising preliminary evidence of improved kidney function. The data showed that as of the February 29, 2024 data cutoff date, in the 18 male and female patients treated with isarlalgene civaparvovec with more than one year of follow-up data, a statistically significant positive mean annualized eGFR slope was observed. Based on these latest data, the FDA agreed that eGFR slope at 52 weeks can serve as an intermediate clinical endpoint to support a potential Accelerated Approval. The FDA also advised that eGFR slope at 104 weeks may be assessed to verify clinical benefit.

The complete dataset to support an Accelerated Approval pathway, including eGFR slope at 52 weeks across all dosed patients, will be available in the first half of 2025. This approach enables a potential BLA submission in the second half of 2025, three years ahead of previous estimates, and avoids the requirement for an additional, costly registrational study to establish clinical efficacy.

Dosing was completed in the Phase 1/2 STAAR study in April 2024, with 33 patients dosed in the study. The longest treated patient recently achieved four years of follow-up. In September 2024, the 18<sup>th</sup> and final patient who started the study on ERT was withdrawn from ERT. All 18 patients remain off ERT as of March 17, 2025.

We announced updated preliminary clinical data from our Phase 1/2 STAAR study via a platform presentation at the 21<sup>st</sup> Annual *WORLD Symposium* on February 6, 2025. A summary of the data is below.

We are executing BLA readiness activities for isarlalgene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

We continue to engage with the EMA on the proposed pathway to potential approval for isarlalgene civaparvovec in Europe.

Summary of Updated Preliminary Clinical Data from Phase 1/2 STAAR Study of Isarlalgene Civaparvovec Announced in Advance of Presentation at 21<sup>st</sup> Annual *WORLD Symposium* on February 6, 2025

- The STAAR study is an ongoing Phase 1/2 multicenter, open-label, dose-ranging clinical study designed to assess a single infusion of isarlalgene civaparvovec in Fabry disease patients  $\geq 18$  years of age. Patients are infused intravenously with a single dose and followed for 52 weeks. A separate long term follow-up study is underway to monitor the patients treated in this study for up to five years following treatment. Patients who are on stable ERT may withdraw ERT after treatment in a controlled and monitored fashion at the discretion of the patient and the investigator.
- The dose escalation phase included males with classic Fabry disease. The subsequent study expansion phase, which commenced in the second half of 2022, treated females, as well as patients with more severe Fabry-associated cardiac or renal disease. The study's primary endpoint is the incidence of treatment-emergent AEs. Additional safety evaluations include routine hematology, chemistry, and liver tests; vital sign monitoring; electrocardiogram; echocardiogram; serial alpha-fetoprotein testing and MRI of the liver to monitor for potential formation of any liver mass. Secondary endpoints include change from baseline at specific time points in  $\alpha$ -Gal A activity, Gb3 and lyso-Gb3 levels in plasma; frequency of ERT infusion; and changes in renal function and cardiac function (left ventricular mass) measured by cardiac MRI and rAAV2/6 vector clearance. Key exploratory endpoints include quality of life, Fabry symptoms and neuropathic pain scores; GI symptoms, and immune response to AAV6 capsid and  $\alpha$ -Gal A.
- As of the September 12, 2024 data cutoff date, 33 patients ranging in age from 18 to 67 years were treated with isarlalgene civaparvovec, nine in the dose escalation phase and 24 in the expansion phase of the study. Baseline characteristics of these 33 dosed patients are shown in Table 5 below. In the dose escalation phase, two patients were dosed in Cohort 1 at the dose of  $0.26 \times 10^{13}$  vg/kg, two patients were dosed in Cohort 2 at the dose of  $0.53 \times 10^{13}$  vg/kg, three patients were dosed in Cohort 3 at the dose of  $1.58 \times 10^{13}$  vg/kg, and two patients were dosed in Cohort 4 at the dose of  $2.63 \times 10^{13}$  vg/kg. In the expansion phase, 24 patients were dosed at the dose of  $2.63 \times 10^{13}$  vg/kg. As of the September 12, 2024 data cutoff date, the first dosed patient had been followed for at least 47.3 months post dosing, and the most recently dosed patient had been followed for 20 weeks post dosing, as shown in Figure 6 below.
- As of the September 12, 2024 data cutoff date, isarlalgene civaparvovec continued to be generally well-tolerated across all the dose cohorts, with the majority of AEs being graded as mild (Grade 1) or moderate (Grade 2) in nature. No liver function test elevations post-dosing requiring steroids occurred. A summary of the treatment-related AEs reported as of the September 12, 2024 cutoff date is shown in Table 7 below. Treatment-emergent serious AEs were reported in four patients: left arm pain; non-cardiac chest pain, sepsis, stroke and shoulder enthesopathy. No AEs led to study discontinuation. No deaths were reported.
- As of the September 12, 2024 data cutoff date, all ERT naïve or pseudo-naïve patients showed sustained elevated  $\alpha$ -Gal A activity up to 42 months for the longest treated patient, and up to 27 months for the longest treated patient receiving the



highest dose level ( $2.63 \times 10^{13}$  vg/kg), as shown in Figure 8 below. Sustained elevated expression of  $\alpha$ -Gal A activity was accompanied by the reduction and/or long-term stabilization of lyso-Gb3 levels, with the largest reductions in plasma lyso-Gb3 seen in patients with the highest levels at baseline, as shown in Figure 9 below.

- 18 patients began the study on ERT. 17 out of 18 patients had been withdrawn from ERT as of the data cutoff date. The remaining patient was withdrawn from ERT after the data cutoff date. All 18 patients remain off ERT as of March 17, 2025. Timing of ERT withdrawal was at the discretion of the investigator, to occur no earlier than eight weeks post dosing with isaralgagene civaparvovec. All patients who began the study on ERT showed sustained elevated  $\alpha$ -Gal A activity, as outlined in Figure 10 below, except for one patient who exhibited levels below the limit of detection at the time of the data cutoff date. Plasma lyso-Gb3 activity levels remained stable following ERT withdrawal for up to 33 months for the longest treated ERT patient, and up to 19 months for the longest treated ERT patient receiving the highest dose level ( $2.63 \times 10^{13}$  vg/kg), as outlined in Figure 11 below.
- For the 23 patients with at least 12-months of follow up or longer as of the September 12, 2024 data cutoff date, a mean eGFR slope of 3.061 mL/min/1.73m<sup>2</sup>/year (95% CI: 0.863, 5.258) was observed, indicating a potential improvement in kidney function.
- For the 23 patients with at least 12-months of follow-up, significant improvements in disease severity, QoL, and GI symptoms were observed. As of the September 12, 2024 data cutoff date, 68% of patients improved their total FOS-MSSI score and seven patients improved their FOS-MSSI category compared to baseline, as shown in Table 12 below. Statistically significant improvements in the short form-36, or SF-36, QoL scores were reported, with a mean change in the General Health score of 10.6 (p=0.0020) at week 52. For context, a 3- to 5-point change on any SF-36 score is the minimally clinically important difference. Significant improvements in physical component, bodily pain, physical, vitality, social function, and emotional SF-36 scores were also observed. The GI symptom rating scale, or GSRS, also demonstrated significant improvements, including the Abdominal Pain, Indigestion, Diarrhea and Constipation Scores.
- Immunogenicity remains an issue with ERT, leading to continued organ impairment. Ten patients had measurable titers of total antibodies, or Ab, or neutralizing antibodies, or Nab, against  $\alpha$ -Gal A associated with ERT at baseline. As shown in Table 13 below, following dosing, total Ab or Nab titers decreased markedly in nine patients and became undetectable in seven, or 70% of patients. Treatment did not induce anti- $\alpha$ -Gal A antibodies in seronegative patients.

*Table 5: Baseline characteristics: Dose escalation and dose expansion phases*

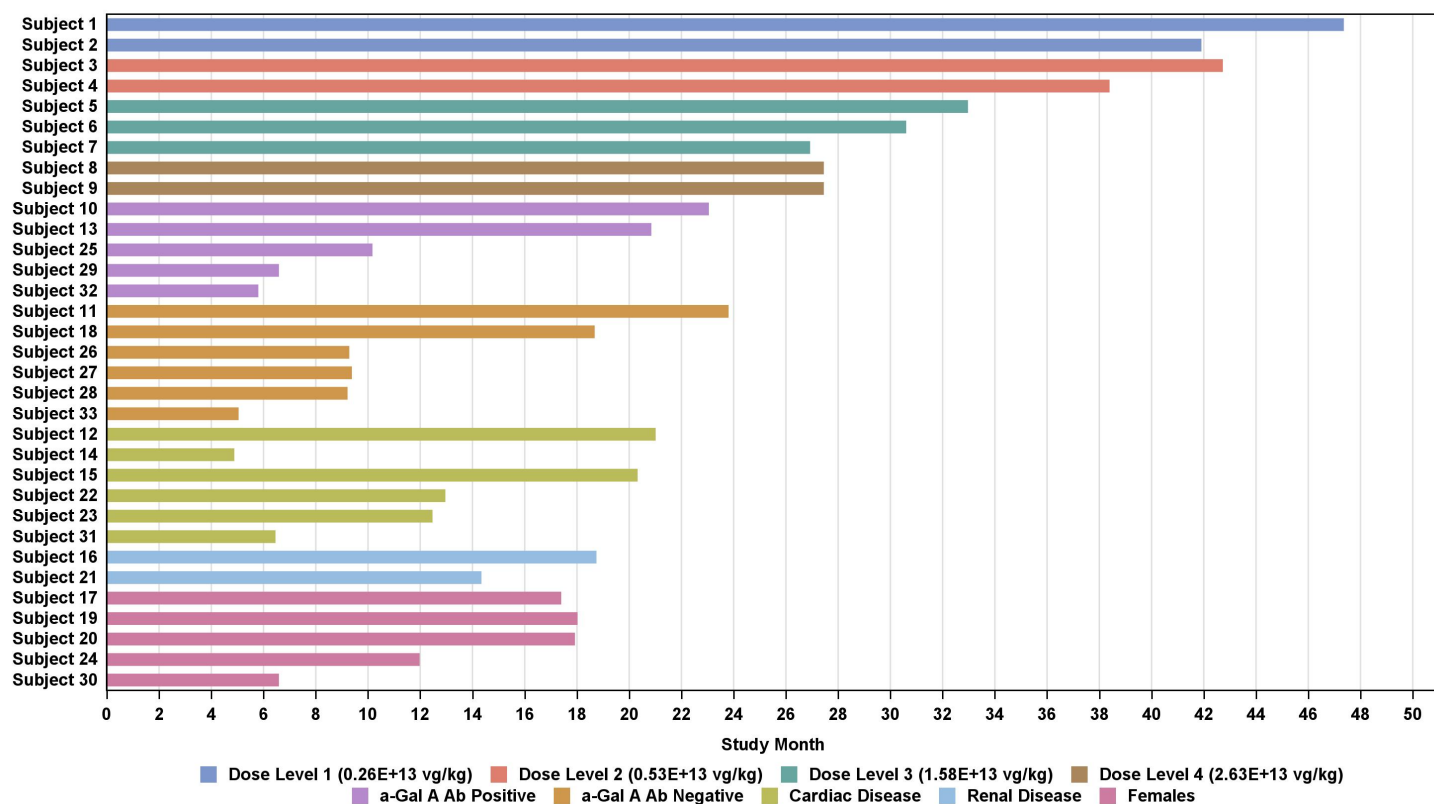
	Dose escalation (n=9)	Dose expansion (n=24)	All (n=33)
Age, median (range)	42 (22-50)	41.5 (18-67)	42 (18-67)
Sex (M:F)	9:0	14:10	23:10
<b>ERT status (n,%):</b>			
• Naïve	2 (22%)	7 (29%)	9 (27%)
• Pseudo-naïve	2 (22%)	4 (17%)	6 (18%)
• On ERT	5 (56%)	13 (54%)	18 (55%)
<b>Baseline Fabry symptoms (n,%):</b>			
• Cornea verticillata	4 (44%)	15 (63%)	20 (61%)
• Paresthesia	3 (33%)	7 (29%)	10 (30%)
• Anhidrosis	1 (11%)	5 (21%)	6 (18%)
• Angiokeratoma	2 (22%)	9 (38%)	11 (33%)
<b>eGFR<sub>CKD-EPI</sub> category, n (%)</b>			
• >90 mL/min/1.73 m <sup>2</sup>	4 (44%)	15 (63%)	19 (58%)
• 60-90 mL/min/1.73 m <sup>2</sup>	4 (44%)	6 (25%)	10 (30%)
• 40-<60 mL/min/1.73 m <sup>2</sup>	1 (11%)	3 (13%)	4 (12%)

Data cutoff date: September 12, 2024

eGFR, estimated glomerular filtration rate (mL/min/1.73m<sup>2</sup>); ERT, enzyme replacement therapy; N, number, M, male; F, female



Figure 6: Duration of follow-up



Data cutoff date: September 12, 2024

vg/kg, vector genomes per kilogram of total body weight

Subject 14 withdrew Day 148



*Table 7: Summary of treatment-emergent AEs in >3 patients of 33 treated patients*

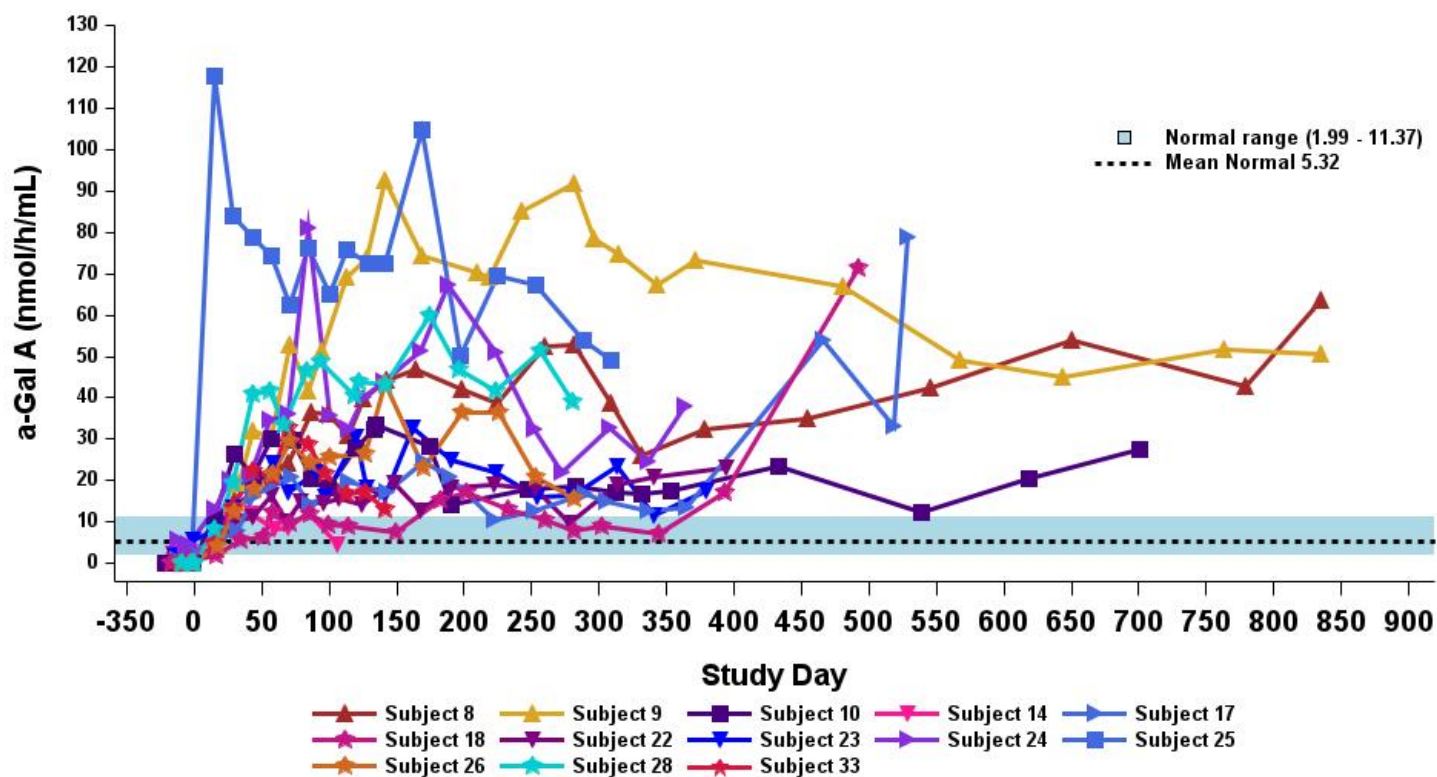
AE by preferred term	Treated subjects (n=33)	
	All grades	Grade 3-4
<b>Pyrexia</b>	20 (60.6%)	1 (3.0%) (G3)
<b>COVID-19</b>	12 (36.4%)	0
<b>Nasopharyngitis</b>	11 (33.3%)	0
<b>Headache</b>	12 (36.4%)	0
<b>Fatigue</b>	9 (27.3%)	0
<b>Nausea</b>	9 (27.3%)	0
<b>Cough</b>	5 (15.2%)	0
<b>Diarrhea</b>	5 (15.2%)	0
<b>Myalgia</b>	5 (15.2%)	1 (3.0%) (G3)
<b>Hypotension</b>	4 (12.1%)	0
<b>Urinary tract infection</b>	4 (12.1%)	0
<b>Paresthesia</b>	4 (12.1%)	0
<b>Chills</b>	4 (12.1%)	0

Data cutoff date: September 12, 2024

AE, adverse event



Figure 8: Plasma  $\alpha$ -Gal A in ERT naïve/pseudo-naïve subjects receiving  $2.63 \times 10^{13}$  vg/kg (n=13)



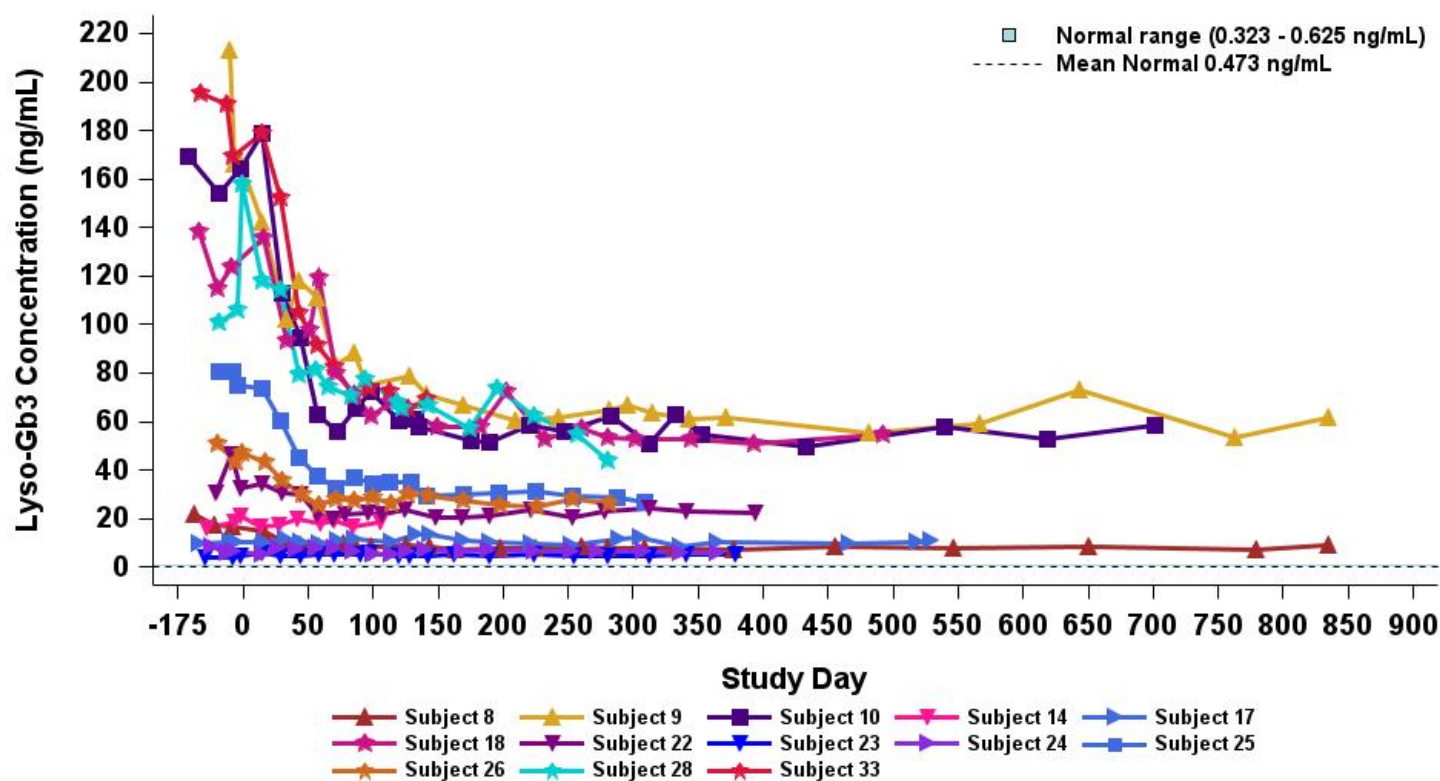
Data cut-off date: September 12, 2024

$\alpha$ -Gal A activity measured using 3-hour reaction time. Normal range determined in healthy males and females. Long Term Follow-Up Data: Data points > Study Day 365.

$\alpha$ -Gal A, alpha-galactosidase A; ERT, enzyme replacement therapy; vg/kg, vector genomes per kilogram of total body weight (as assessed by ddPCR)



Figure 9: Lyso-Gb3 in ERT naïve/pseudo-naïve subjects receiving  $2.63 \times 10^{13}$  vg/kg (n=13)



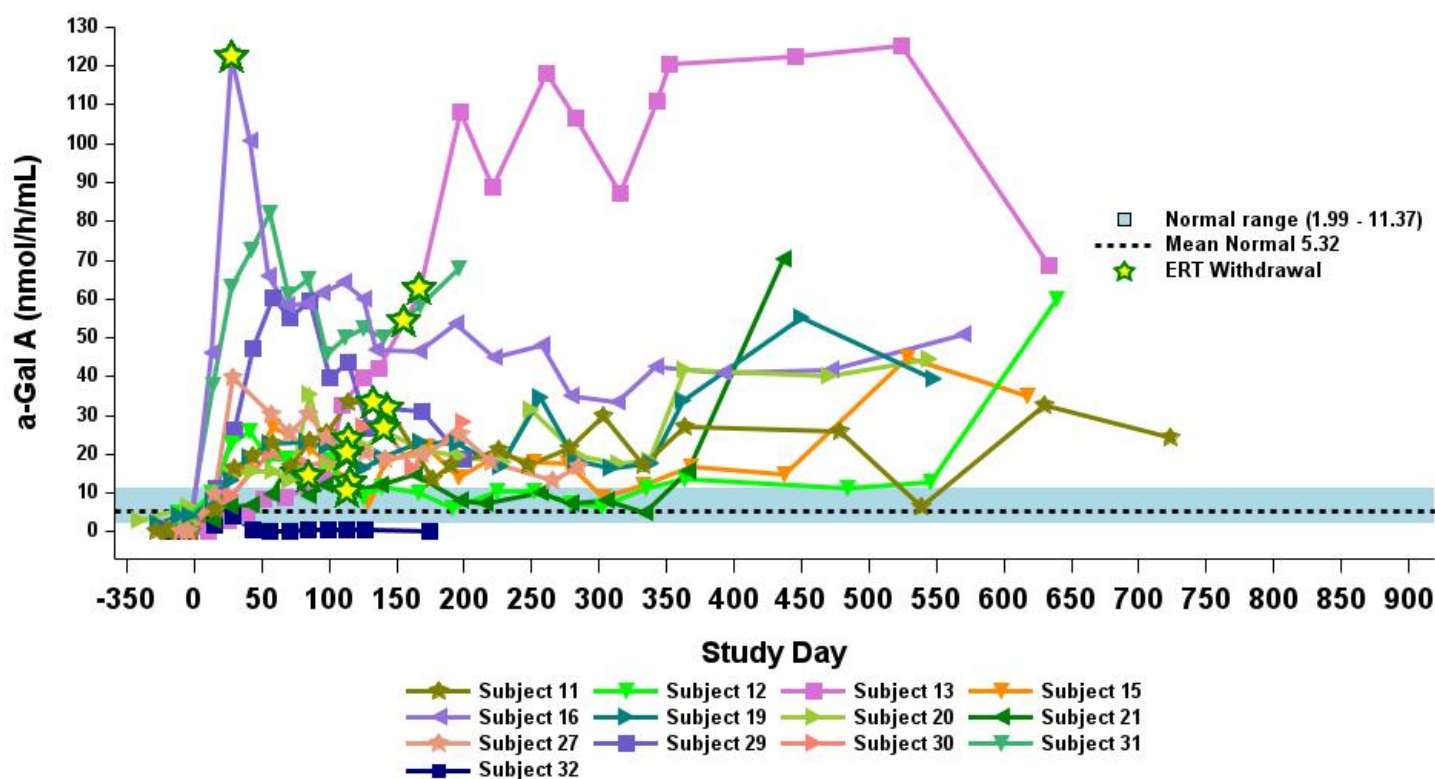
Data cut-off date: September 12, 2024

$\alpha$ -Gal A activity measured using 3-hour reaction time. Normal range determined in healthy males and females. Long Term Follow-Up Data: Data points > Study Day 365.

ERT, enzyme replacement therapy; vg/kg, vector genomes per kilogram of total body weight (as assessed by ddPCR)



Figure 10: Plasma  $\alpha$ -Gal A in ERT-treated patients receiving  $2.63 \times 10^{13}$  vg/kg (n=13)



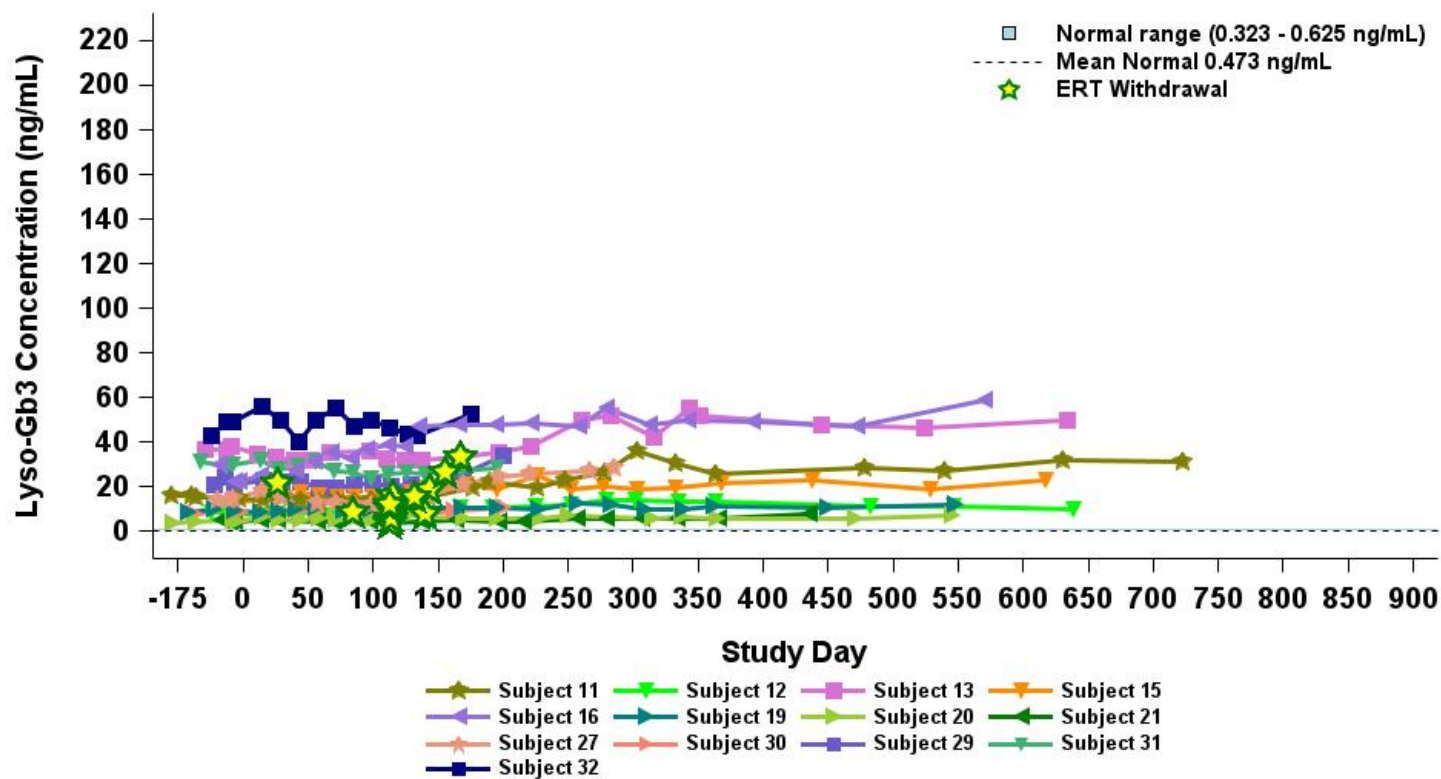
Data cut-off date: September 12, 2024

$\alpha$ -Gal A activity measured using 3-hour reaction time. Normal range determined in healthy males and females.

$\alpha$ -Gal A, alpha-galactosidase A; ERT, enzyme replacement therapy; vg/kg, vector genomes per kilogram of total body weight (as assessed by ddPCR)



Figure 11: Lyso-Gb3 in ERT-treated patients receiving  $2.63 \times 10^{13}$  vg/kg (n=13)



Data cut-off date: September 12, 2024

$\alpha$ -Gal A activity measured using 3-hour reaction time. Normal range determined in healthy males and females.

ERT, enzyme replacement therapy; lyso-Gb3, globotriaosylsphingosine; vg/kg, vector genomes per kilogram of total body weight (as assessed by ddPCR)



Table 12: Comparison of disease severity, quality of life and GI symptoms in 23 treated patients with ≥12 months follow-up

Subject	ERT status at Baseline	FOS-MSSI category Baseline	FOS-MSSI category Week 52	FOS-MSSI category Week 104	FOS-MSSI category Year 3	FOS-MSSI category Year 4
1	ERT	Moderate	Moderate		Moderate	Mild
2	Pseudo-naive	Mild	Mild	Mild	Mild	
3	Pseudo-naive	Moderate	Moderate	Mild	Mild	
4	ERT	Mild	Mild	Mild	Mild	
5	ERT	Moderate	Mild	Moderate		
6	ERT	Moderate	Mild	Mild	Mild	
7	ERT	Severe	Moderate	Moderate		
8	Naive	Moderate	Mild	Mild		
9	Naive	Moderate		Moderate		
10	Pseudo-naive	Moderate	Moderate	Moderate		
11	ERT	Moderate	Moderate	Moderate		
12	ERT	Mild	Mild	Mild		
13	ERT	Mild	Mild			
15	ERT	Mild	Mild			
16	ERT	Moderate	Moderate			
17	Naive	Moderate	Mild			
18	Naive	Moderate	Moderate			
19	ERT	Mild	Moderate			
20	ERT	Moderate	Moderate			
21	ERT	Moderate	Mild			
22	Naive	Mild	Mild			
23	Naive	Mild	Mild			
24	Pseudo-naive	Moderate	Moderate			

Data cut-off date: September 12, 2024

Analysis of ST-920 treated subjects with ≥12 m follow-up (n=22). “Month 12” is Week 52 study timepoint. All p-values are unadjusted nominal p-values. ERT, enzyme replacement therapy; GI, gastrointestinal; FOS-MSSI, Fabry Outcome Survey adaptation of the Mainz Severity Score Index; SF-36, Short Form-36 (a 3-to-5-point change on any SF-36 score is the minimally clinically important difference)



Table 13: Reduction or elimination of antibodies against  $\alpha$ -Gal A

	Anti- $\alpha$ -GalA Total Ab titer		Anti- $\alpha$ -GalA NAb titer	
	Baseline	On-study	Baseline	On-study
Subject 1	1280	160	160	Undetectable (W36)
Subject 3	160	Undetectable (W24)	0	-
Subject 4	160	Undetectable (W52)	0	-
Subject 5	10240	1280	320	160
Subject 10	80	Undetectable (W4)	10	Undetectable (W4)
Subject 13	5120	320	160	10
Subject 16	2560	640	40	Undetectable (W52)
Subject 25	160	Undetectable (W4)	160	Undetectable (W4)
Subject 31	80	Undetectable (W12)	10	Undetectable (W4)
Subject 32	20480	20480	640	320

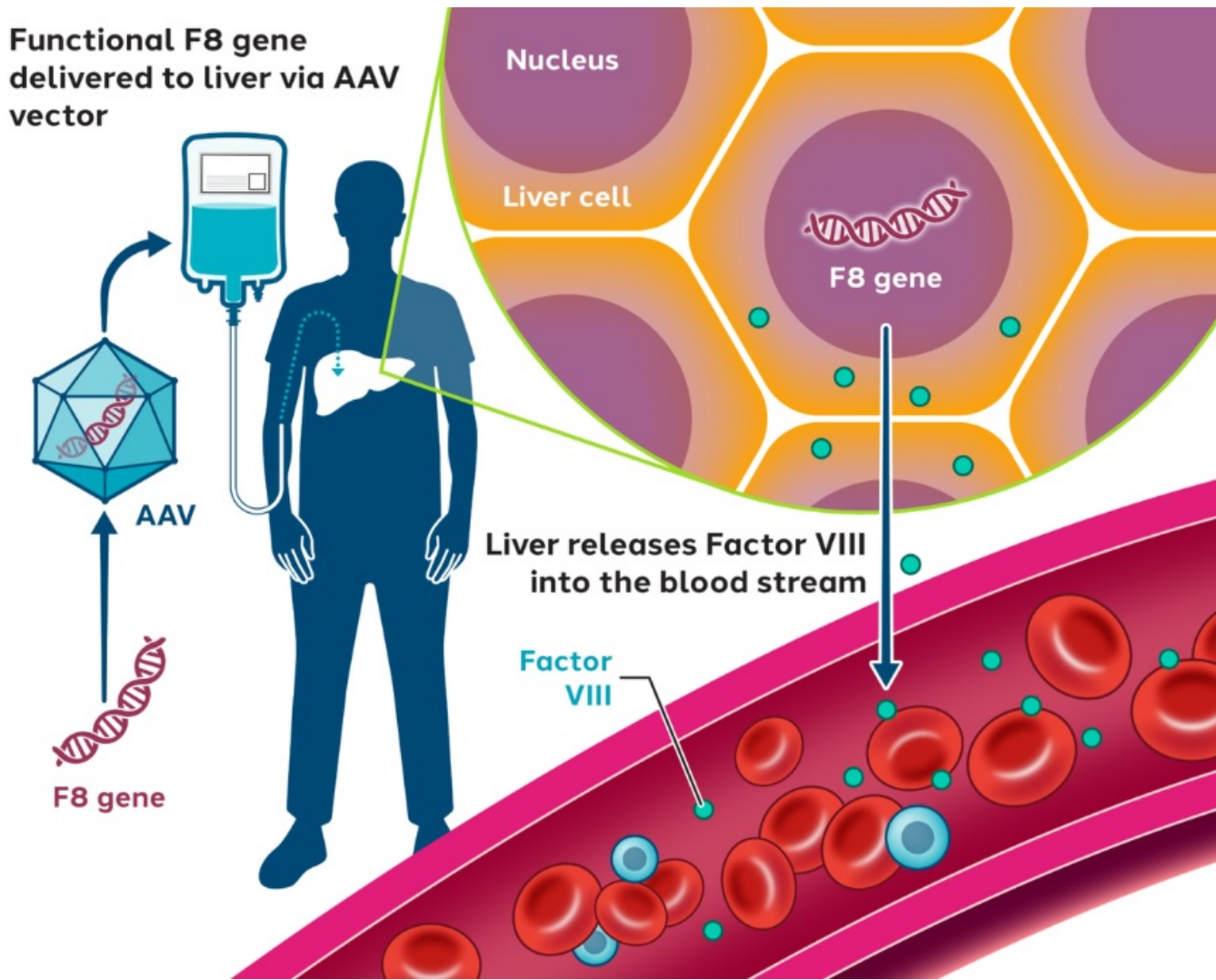
Data cut-off date: September 12, 2024

$\alpha$ -Gal A, alpha-galactosidase A; Ab, antibody; NAb, neutralizing antibody; W, week; (-) denotes NAb testing not done when total Ab titer is 0

#### *Giroctocogene Fitelparvovec – Hemophilia A*

AFFINE is a global Phase 3, open-label, multicenter, single arm trial evaluating the efficacy and safety of a single infusion of giroctocogene fitelparvovec, also known as SB-525, an investigational gene therapy that we have co-developed with and licensed to Pfizer for the treatment of adults with moderately severe to severe hemophilia A. The primary endpoint is impact on annualized bleeding rate, or ABR, through 12 months following treatment with giroctocogene fitelparvovec, compared to ABR on factor VIII, or FVIII, replacement therapy collected in the Phase 3 lead-in study period.





*Figure 14: Approach to Hemophilia A*

In July 2024, Pfizer reported that the AFFINE trial achieved its primary objective of non-inferiority, as well as superiority, of total ABR from Week 12 through at least 15 months of follow up post-infusion compared with routine FVIII replacement prophylaxis treatment. Following a single 3e13 vg/kg dose, giroctocogene fitelparvovec demonstrated a statistically significant reduction in mean total ABR compared to the pre-infusion period. Key secondary endpoints as defined by the trial protocol were met and also demonstrated superiority compared to prophylaxis. In the AFFINE trial, giroctocogene fitelparvovec was generally well tolerated.

In December 2024, Pfizer presented detailed data from the Phase 3 AFFINE trial in an oral presentation at the 66<sup>th</sup> American Society for Hematology Annual Meeting and Exposition and in a poster presentation. A summary of the data is below.

In December 2024, we announced that we are scheduled to regain development and commercialization rights to giroctocogene fitelparvovec following a decision by Pfizer to terminate the Collaboration and License Agreement dated May 10, 2017, or the Collaboration Agreement. Pfizer has indicated to us that the termination relates to its decision not to submit a BLA or MAA for, or pursue commercialization of, giroctocogene fitelparvovec. The Collaboration Agreement with Pfizer will terminate effective April 21, 2025, or the Termination Date, at which time Pfizer is scheduled to transition the giroctocogene fitelparvovec program back to us.

As of the Termination Date, the Collaboration Agreement will be terminated in its entirety, and we will be entitled to receive an exclusive, worldwide, royalty-bearing, sublicensable license from Pfizer to use Pfizer's relevant intellectual property to continue developing, manufacturing and commercializing giroctocogene fitelparvovec. In return, Pfizer would be eligible to receive low single digit royalties on net sales of giroctocogene fitelparvovec. In addition, at our request and expense following the Termination Date, Pfizer is required to transition the SB-525 program back to us and provide certain transition-related



services. We continue to explore how to maximize the value of the SB-525 program, including a search for a potential new collaboration partner.

#### Summary of Efficacy and Safety of Giroctocogene Fitelparvovec in Adults With Moderately Severe to Severe Hemophilia A: Primary Analysis Results From the Phase 3 AFFINE Gene Therapy Trial

- AFFINE is a global Phase 3, open-label, multicenter, single arm trial evaluating the efficacy and safety of a single infusion of giroctocogene fitelparvovec in 75 adult (ages 18-64 years) male patients with moderately severe to severe hemophilia A.
- The trial enrolled male participants with hemophilia A who had completed a lead-in study while on exogenous FVIII prophylaxis therapy prior to administration of a single infusion of 3e13 vg/kg giroctocogene fitelparvovec. The lead-in trial was initiated to establish prospective bleeding and infusion rates while on FVIII prophylaxis replacement therapy in the usual care setting of participants with hemophilia A. Primary and secondary endpoints were assessed in the efficacy population corresponding to participants with  $\geq 15$  months follow-up post-infusion and at least six months follow-up in the lead-in study, as shown in Figure 15 below.
- As of the cutoff date of June 2024, 75 participants (median age, 30 [range 19–59] years) were dosed with giroctocogene fitelparvovec. Of those 75 participants, 50 were included in the efficacy population. See Figure 16 below for baseline patient demographics.
- The primary endpoint was ABR for total (treated and untreated) bleeds from Week 12 (onset of clinically meaningful transgene-derived FVIII levels) through  $\geq 15$  months post-infusion compared to the pre-infusion prophylaxis period. Within this efficacy population, the study met its primary endpoint with a statistically significant decrease in total ABR from Week 12 through  $\geq 15$  months post-infusion compared to pre-infusion prophylaxis (mean total ABR, 1.24 vs 4.73). 64.0% (32/50) of participants experienced no bleeding events (median duration of follow-up, 33.6 months [range 14.5–44.4]) and 88.0% (44/50) of participants had no treated bleeds (median duration of follow-up, 33.6 months [range 14.5–44.4]). One participant in the efficacy population experienced a high number of bleeds (126, total ABR = 47.4) starting at Month 18 post infusion, while maintaining FVIII activity levels  $>150\%$  through the data cutoff. Post hoc sensitivity analysis excluding that one participant (n=49) demonstrated superiority vs FVIII prophylaxis (mean total ABR, 0.26 vs 4.65) as shown in Figure 17 below.
- Treated ABR during Week 12 through  $\geq 15$  months post-infusion was significantly reduced compared to prophylaxis (mean treated ABR, 0.07 vs 4.08), also demonstrating superiority, as shown in Figure 18 below. AIR post-infusion was reduced by 99.8% compared to the pre-infusion period (mean AIR, 0.21 vs 124.39). As of the June 2024 cutoff date, one (1.3%) dosed participant had resumed prophylaxis (at 16.07 months post-infusion).
- At Month 15, 84% of participants had FVIII activity  $>5\%$  via chromogenic assay, as shown in Figure 19 below. Participants continued to maintain FVIII activity  $>5\%$ , with 82.8% of participants [n=29] continuing to maintain FVIII activity  $>5\%$  at 2-years post-infusion and 63% of participants [n=8] at 3-years post-infusion respectively, as shown in Figure 20 below.
- A total of 624 AEs, mostly mild or moderate, were reported in 74 (98.7%) participants, as shown in Figure 21 below. There were 26 serious AEs, or SAEs, in 15 (20%) participants. The most common treatment-related AEs were pyrexia (54.7% of participants), alanine aminotransferase, or ALT, increased (46.7%), and headache (38.7%). There have been no study discontinuations. As of the data cutoff, there were no reported FVIII inhibitors and no malignancies related to the study drug. One thrombotic event was observed in a participant with a major protocol deviation (prior history of deep vein thrombosis, or DVT, and pulmonary embolism, or PE) and multiple thrombotic risk factors. Post-infusion, 62.7% of participants received at least one dose of corticosteroids due to ALT elevations or decreases in FVIII activity (median time to initiation, 84 [range 7–193] days; mean total time on corticosteroids, 114.6 [11–296] days), as shown in Figure 22 below. Overall corticosteroids were well tolerated with AEs related to corticosteroids reported in 19 (25.3%) participants.
- Transient FVIII activity  $>150\%$  (defined as  $\geq 1$  central chromogenic assay measurement  $>150\%$ ) was reached in 37 (49.3%) participants, with 23 (30.7%) treated with prophylactic direct oral anticoagulants based on protocol and investigator's recommendation, which was well tolerated, as shown in Figure 23 below.
- In summary, giroctocogene fitelparvovec yielded endogenous FVIII expression in the mild to normal range in most participants, resulting in superior bleed protection versus routine FVIII prophylaxis and significant reductions in bleeding. A single infusion was well tolerated and demonstrated durable efficacy on all primary and key secondary endpoints.



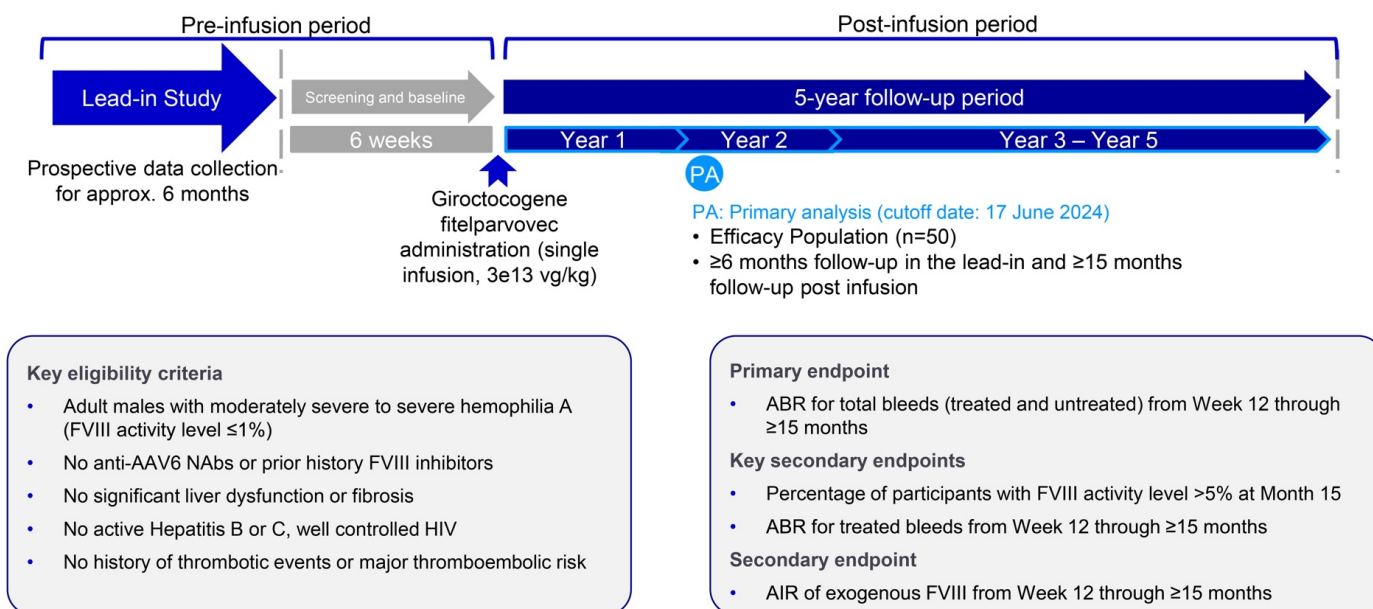


Figure 15: AFFINE study design

AAV-6=adeno-associated virus serotype 6; ABR=annualized bleeding rate; AIR=annualized infusion rate; FVIII=factor VIII; NAb=neutralizing antibody; vg=vector genome

n (%) <sup>a</sup>	N=75	n (%) <sup>a</sup>	N=75
Age (range), y	32.3 (19–59)	Region	
BMI ± SD, kg/m <sup>2</sup>	26.1 ± 5.1	North America	12 (16.0)
Male	75 (100)	Europe	19 (25.3)
Race		Middle East	30 (40.0)
White	56 (74.7)	Asia Pacific	10 (13.3)
Asian	14 (18.7)	South America	3 (4.0)
Black	5 (6.7)	Australia	1 (1.3)
Ethnicity		Ongoing controlled HIV	6 (8.0)
Non-Hispanic	59 (78.7)	History of hepatitis B	11 (14.7)
Hispanic	3 (4.0)	History of hepatitis C	19 (25.3)
Not reported	13 (17.3)	Target joints at baseline	25 (33.3)

Figure 16: Baseline demographics and characteristics

a n (%) unless otherwise noted. BMI=body mass index



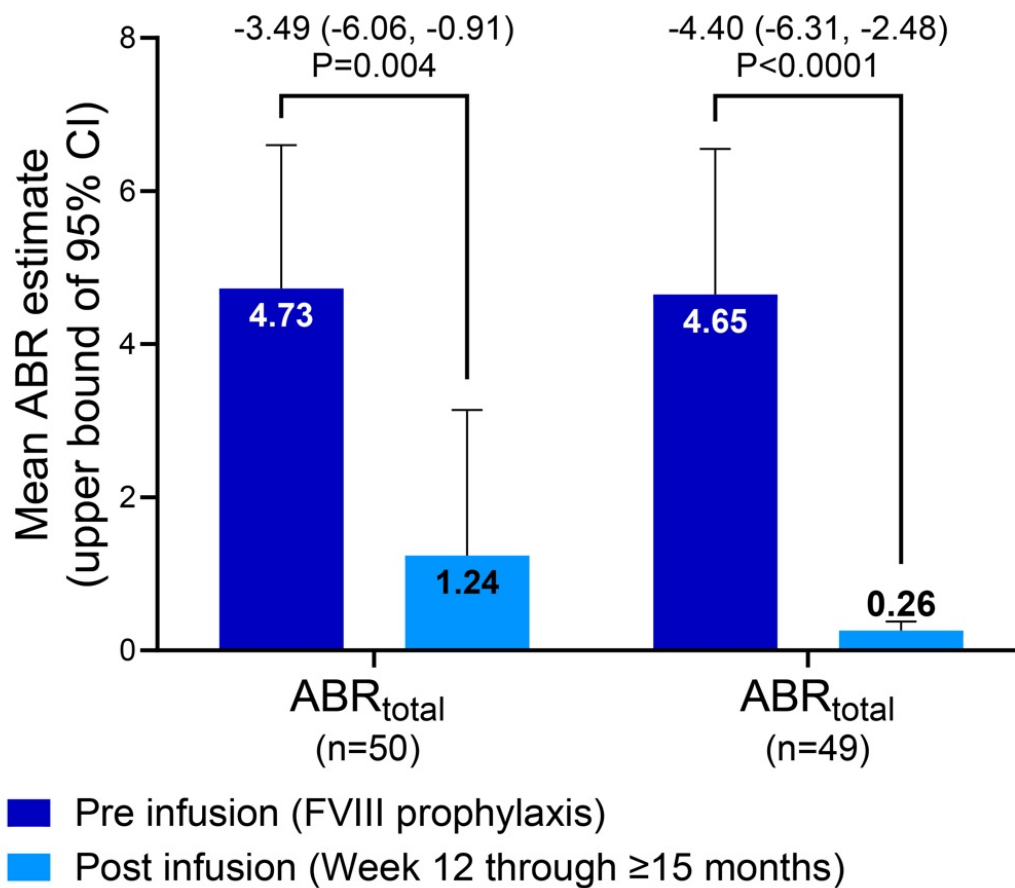


Figure 17: Annualized bleeding rate: Total (treated and untreated) bleeds

Numbers above graph represent treatment difference and 95% CI. Estimates and 1-sided P-value were obtained from a repeated measures generalized linear model with negative binomial distribution and identity link function with participant as a random effect and treatment and duration of follow-up (in years) as fixed effects.

ABR<sub>total</sub>=annualized bleeding rate for total (treated and untreated) bleeds; CI=confidence interval; FVIII=factor VIII



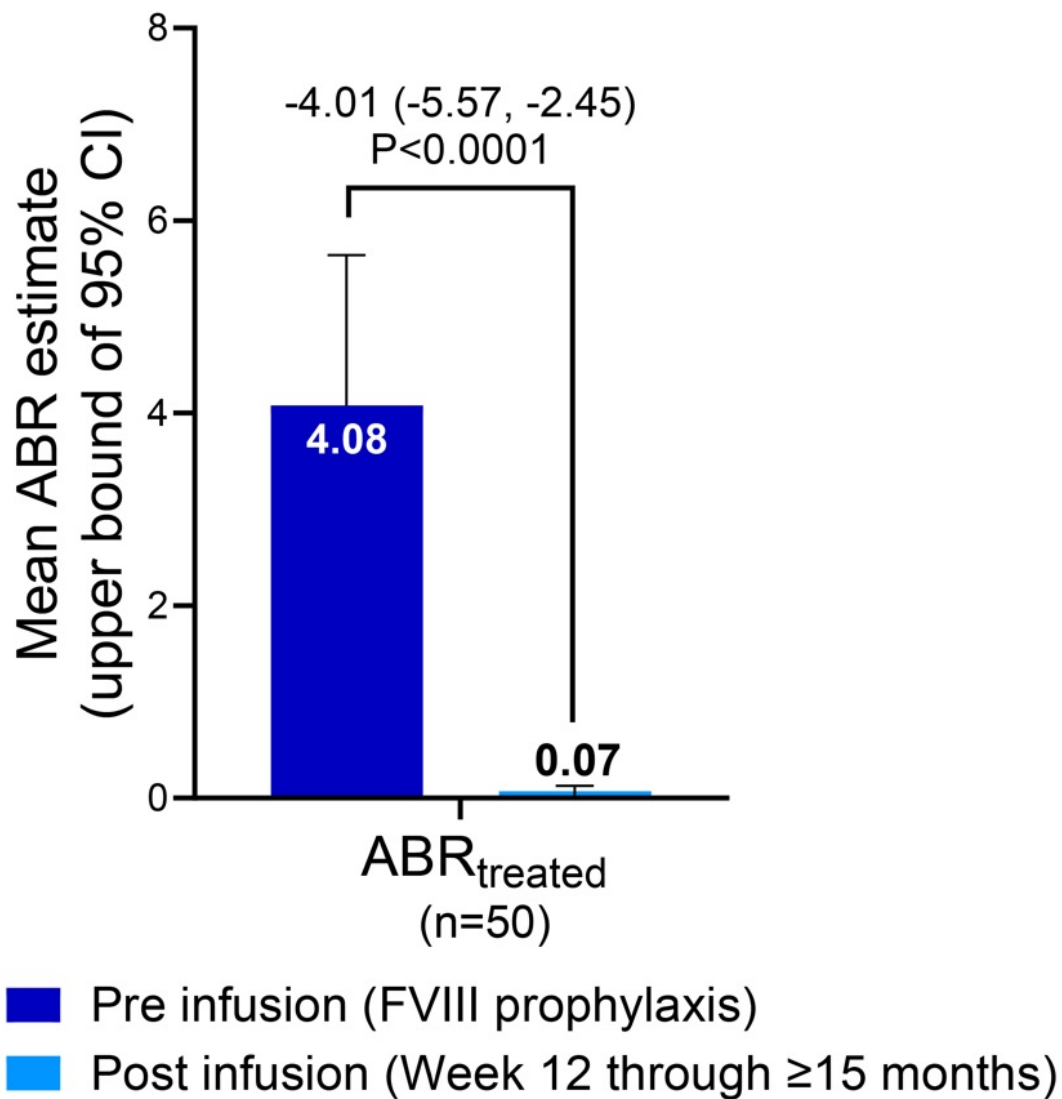


Figure 18: Annualized bleeding rate: Treated bleeds

Numbers above graph represent treatment difference and 95% CI. Estimates and 1-sided P-value were obtained from a repeated measures generalized linear model with negative binomial distribution and identity link function with participant as a random effect and treatment and duration of follow-up (in years) as fixed effects.

ABR<sub>treated</sub>=annualized bleeding rate for treated bleeds; CI=confidence interval; FVIII=factor VIII



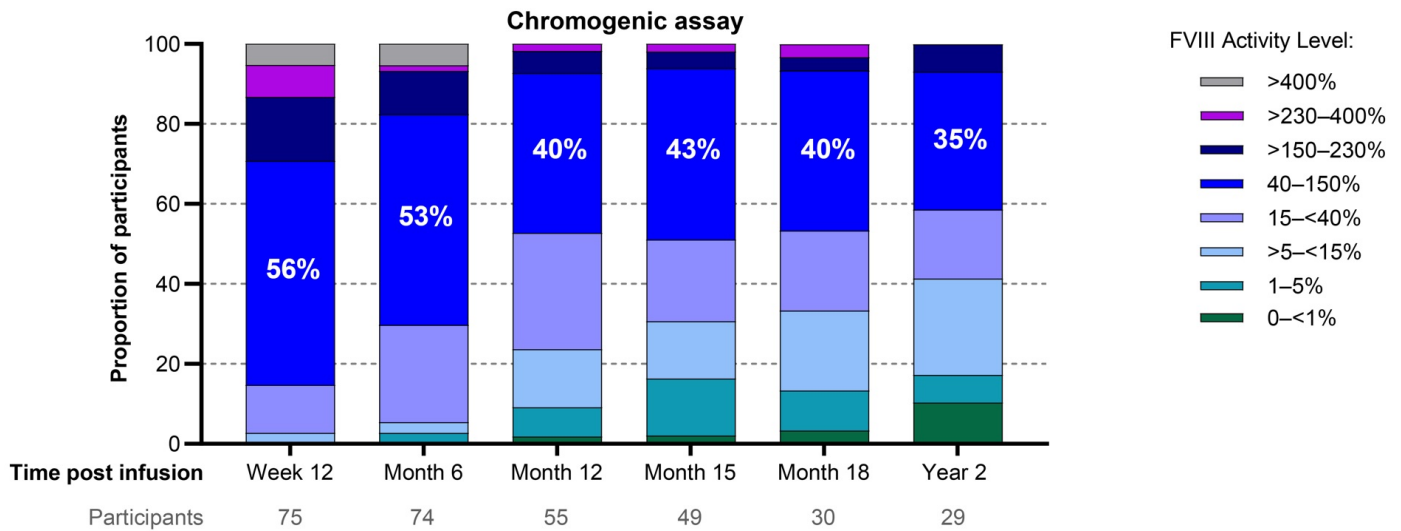


Figure 19: FVIII activity levels post infusion

CA=chromogenic assay; FVIII=factor VIII; LLOQ=lower limit of quantification

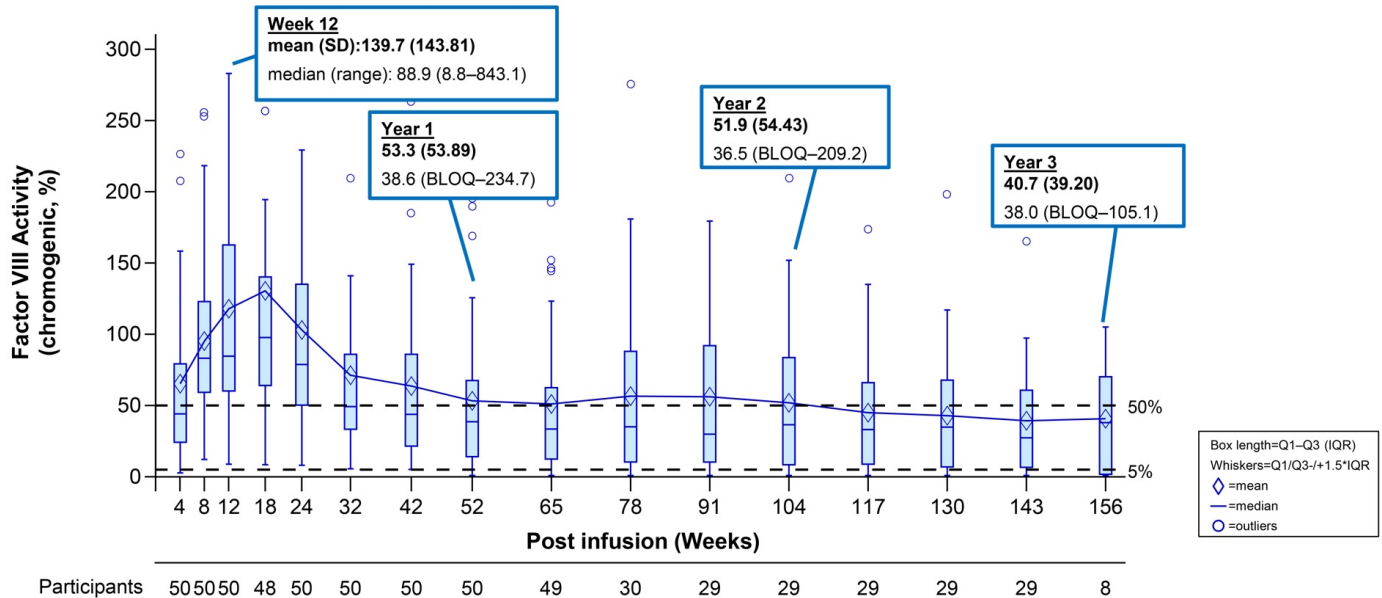


Figure 20: FVIII activity levels through Year Three

Values >300% not shown.

CA=chromogenic assay; BLOQ=below lower limit of quantification; FVIII=factor VIII; IQR=interquartile range; max=maximum; min=minimum; Q=quartile



Participants with AEs, n (%) and number of events (when specified)	Dosed Population N=75
AEs	74 (98.7)
Number of events	740
Discontinued due to AEs	0 (0)
SAEs	15 (20.0)
Number of events	26
Treatment-related AEs	68 (90.7)
Selected treatment-related AESIs	
Hepatotoxicity (transaminase increased)	47 (62.7)
Infusion-related reactions	55 (73.3)
Pyrexia	38 (50.7)
Headache	23 (30.7)
Chills	14 (18.7)
Deep vein thrombosis	1 (1.3)

*Figure 21: Safety overview*

AE=adverse event; AESI=adverse event of special interest; DVT=deep vein thrombosis; FVIII=factor VIII; PE=pulmonary embolism; SAE=serious adverse event



<b>ALT and corticosteroid use</b>	<b>N=75</b>
Treatment-related AEs related to hepatotoxicity (transaminase increased), n (%)	47 (62.7)
SAEs related to transaminase increased, n (%)	2 (2.7)
Participants with ALT increase >ULN, n (%)	46 (61.3)
ALT grades (CTCAE grading) <sup>a</sup> among all dosed participants, n (%)	
Normal	30 (40.0)
Grade 1	40 (53.3)
Grade 2	4 (5.3)
Grade 3	1 (1.3)
Grade 4	0 (0)
Pts with corticosteroid use, n (%)	47 (62.7)
Time to corticosteroid initiation, median (range), days	84 (7–193)
Corticosteroid courses per participant, mean (range), days	2.0 (1–5)
Duration of corticosteroid use, mean (range), days	114.6 (11–296)

*Figure 22: ALT elevations and corticosteroid use*

<sup>a</sup> The highest CTCAE grade among all post baseline assessments from each participant are reported.

AE=adverse event; ALT=alanine aminotransferase; CTCAE=common terminology criteria for adverse events; MMF=mycophenolate mofetil; pts=participants; SAE=serious adverse event; ULN=upper limit of normal



FVIII elevations throughout follow-up	N=75
≥1 FVIII activity level >150% (CA), n (%)	37 (49.3)
Time to first FVIII activity level >150%, mean (range), days	74.7 (15–540)
Days with FVIII >150%, mean (range)	143.8 (4–953)
Received prophylactic DOAC, n (%)	23 (30.7)
Time to DOAC initiation, mean (range), days	86.13 (28–370)
Total duration of DOAC, mean (range), days	166 (7–944)

Figure 23: FVIII activity elevations

CA=chromogenic assay; DOAC=direct oral anticoagulant; DVT=deep vein thrombosis; FVIII=factor VIII; OSA=one-stage assay; PD=protocol deviation; PE=pulmonary embolism

## OUR TECHNOLOGIES

Our strategy is to translate our differentiated and versatile technology platforms to create product candidates with best- or first-in-class clinical potential. We believe that the versatility and flexibility of our technology platforms enable us to design therapeutic approaches to resolve the underlying genetic or cellular causes of disease, using whichever technology is best suited to deliver that treatment.

### ZF Platform: Providing Opportunity to Develop a New Class of Human Therapeutics

We believe that our ZF platform provides a unique and proprietary basis for a potentially broad new class of drugs that have differentiated technical advantages over small-molecule drugs, protein pharmaceuticals, RNA-based therapeutics, conventional gene therapy approaches and other gene and genome editing platforms, potentially enabling us to develop therapies that address a broad range of unmet medical needs. We believe that our ZF genomic medicines have the potential to transform treatment strategies for severe diseases from symptom management to lasting cures.

#### *ZFPs: Naturally Occurring Sequence Specific DNA Binding Proteins in Humans*

ZFPs are naturally-occurring sequence-specific DNA-binding proteins in humans that recognize and bind to a specific DNA sequence within or near a particular gene and causes expression of that gene to be “turned on” (activated) or “turned off” (repressed). ZFPs are the most common class of such naturally occurring proteins in a wide range of organisms from yeast to humans. Functional domains may be added to ZFPs that enable genome editing (with enzymes such as nucleases or integrases) or epigenetic regulation (with activators and repressors) at a specific genomic site determined by the ZFP DNA-binding domain.

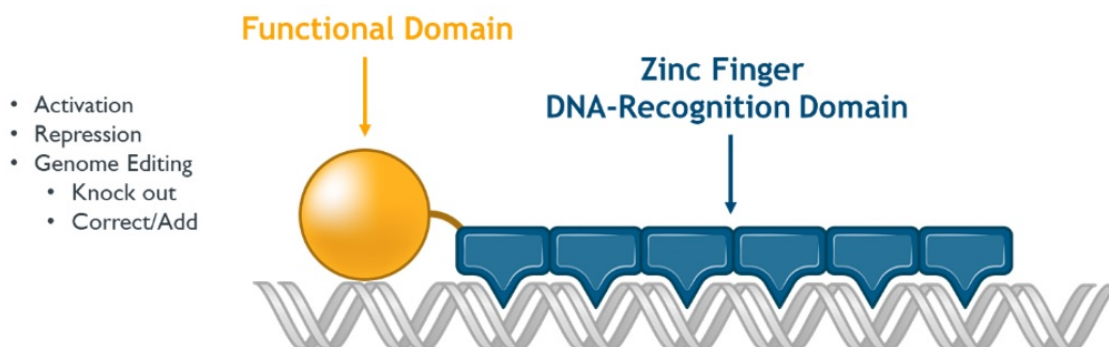


Figure 24: Schematic of the two-domain structure of a zinc finger DNA-binding domain and its functional domain



Consistent with the structure of natural ZFPs, we take a modular approach to the design of the proteins that we engineer. The DNA-recognition part of our engineered proteins is typically composed of four to six zinc fingers. Each individual finger recognizes and binds to a three or four base-pair sequence of DNA and multiple fingers can be linked together to form a zinc finger array that recognizes longer stretches of DNA, thereby improving specificity. By modifying the amino acid sequence of ZFPs, we can engineer novel zinc finger arrays capable of recognizing the unique DNA sequences of a chosen genomic target.



Figure 25: Schematic of a ZFP and a zinc finger array composed of six ZFPs

The engineered DNA-binding zinc finger array is then linked to a functional domain. The DNA-binding zinc finger array brings this functional domain to the target of interest. Our ability to use our highly specific ZFPs to precisely target a DNA sequence to a gene of interest provides us with a range of genome editing and epigenetic regulation functionalities that can be applied to multiple cell types.

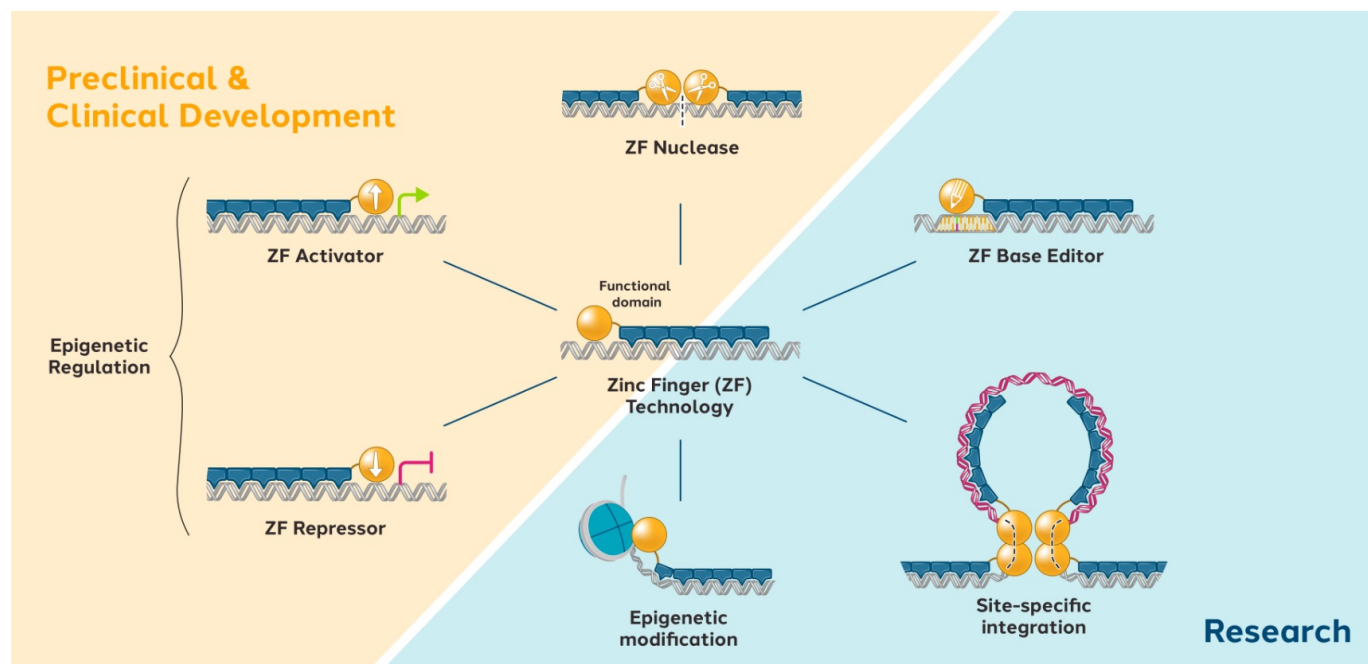


Figure 26: Examples of genome engineering tools that can be offered by our ZF platform

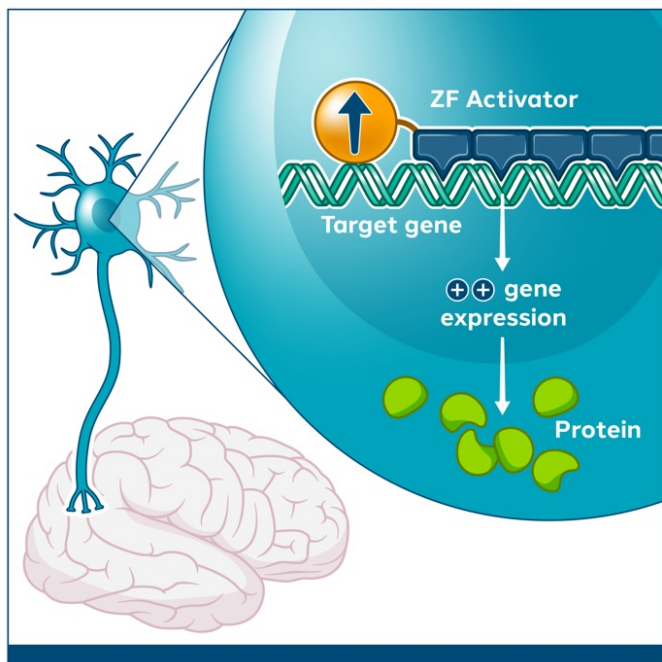
Our engineered zinc fingers can be attached to a cleavage domain of a restriction endonuclease, an enzyme that cuts DNA, creating a ZFN. When a ZFN binds DNA in the correct orientation and spacing, a cut is introduced into the DNA sequence between the ZF binding sites. This break in the DNA triggers a natural process of DNA repair within the cell, effectively “knocking out” the gene function. ZFN-mediated genome editing can be used to disrupt genes that are involved in disease pathology, such as in sickle cell disease. Our ZF platform can also be used to perform base editing, a novel approach that allows for the conversion of a specific target DNA base into another DNA base without the need for double-stranded breaks. Base editing relies on the use of enzymes that can directly change the DNA sequence, such as a deaminase, which changes a specific base in a single strand of DNA. We have developed a compact base editor architecture that can be targeted with high precision and specificity using ZFs, is small enough for packaging into relevant viral vectors, and achieves high



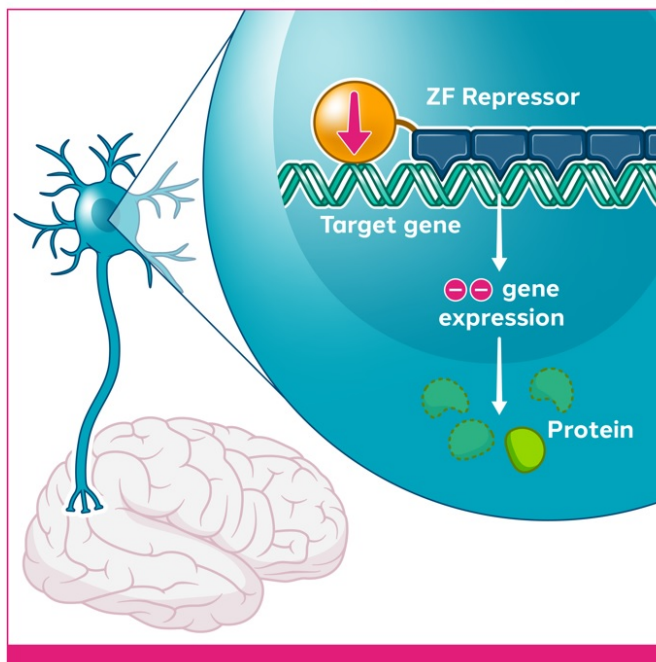
levels of editing that are potentially suitable for therapeutic application. In February 2024, a research paper summarizing the key characteristics of the ZF-base editors was published in Nature Communications.

Our priority focus is to evaluate ZF-transcriptional regulators which have the potential to regulate the expression of a target gene, see Figure 27 below. Zinc finger activators, or ZFAs, are created by attaching a zinc finger array to an activation domain with the aim of increasing the expression of a target gene relative to an untreated cell. Alternatively, ZFRs are created by attaching a zinc finger array to a repression domain in order to down regulate or completely turn off a gene. ZFRs can also be designed to selectively repress expression of a mutant allele while allowing for the expression of the healthy allele. We have several preclinical programs evaluating the potential of ZFRs that have been designed to down-regulate the expression of genes as potential treatments for neurological diseases, including the downregulation of the SCN9A gene that encodes the Nav1.7 sodium channels to treat chronic neuropathic pain, and the repression of prion gene expression to treat prion disease. In addition, we are party to a license agreement with Genentech for tauopathies and one other undisclosed neurologic target.

**ZF Transcriptional Regulator using an activation domain to increase gene expression (ZF Activator)**



**ZF Transcriptional Regulator using a repression domain to decrease gene expression (ZF Repressor)**



*Figure 27: ZFRs have the potential to regulate the expression of a target gene*

Due to their compact size, multiple ZFRs can be combined in a single viral construct to achieve efficient multigene modulation in a single transduction event and without the need for double-strand breaks. As proof of concept for this novel platform, we engineered primary human T cells using multiple ZFRs encoded in a single lentivirus with and without a Chimeric Antigen Receptor, or CAR, to repress expression of several allogeneic engineering targets or checkpoint inhibitors. We demonstrated that ZFRs act with high efficiency and specificity on target genes of choice at both the RNA and the protein level.



## Novel AAV Capsid Delivery Platform: Engineering AAVs to target the CNS

We are evaluating several potential routes of administration for our CNS-targeted investigational therapies, as delivery of genomic medicines to the CNS is a significant obstacle to developing potential therapies treating neurological disorders.

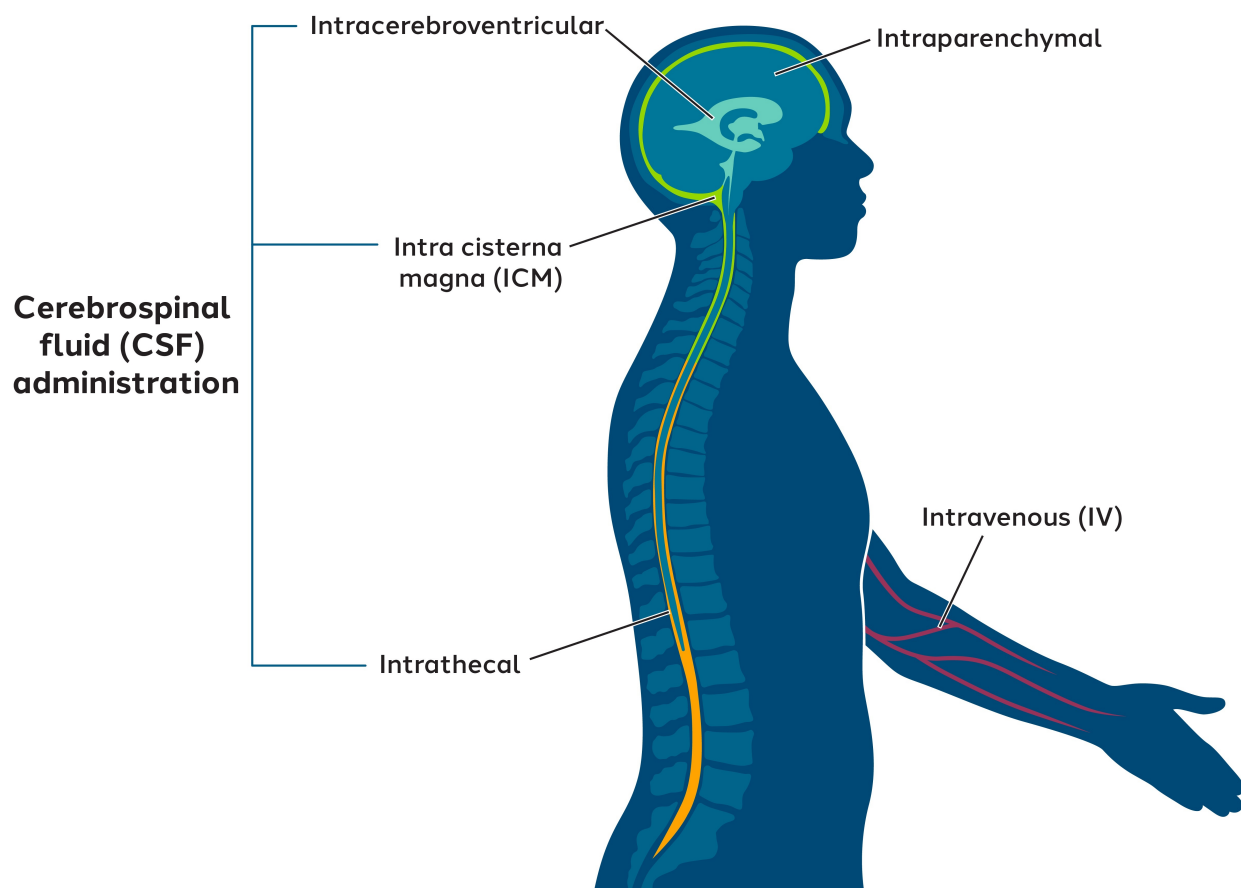


Figure 28: Potential routes of administration to target the central nervous system

Several AAV serotypes, most notably benchmark capsid AAV9, distribute to the brain but require high doses to achieve limited expression. We have developed a proprietary AAV capsid engineering platform, SIFTER, with the aim of engineering capsids with improved CNS transduction. We are applying SIFTER to screen tens of millions of unique capsids in order to identify certain capsids that mediate superior delivery to the CNS. Successive rounds of screening are conducted to find capsids that reproducibly demonstrate a desired therapeutic profile.

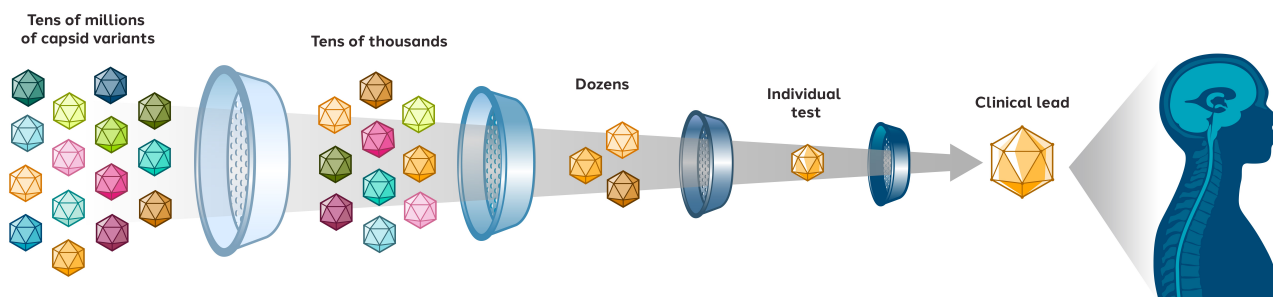


Figure 29: Sangamo's proprietary SIFTER platform to develop novel AAV capsids targeting the CNS

In May 2022, we presented results obtained with the SIFTER platform for CSF administration. This platform notably allowed us to identify new capsids exhibiting improved delivery relative to benchmark capsid AAV9 when delivered intrathecally: STAC-102 and STAC-103 (STAC = Sangamo Therapeutics AAV Capsid).



In March 2024, we announced data for our proprietary AAV capsid variant, STAC-BBB, which demonstrated an ability to cross the BBB in NHPs and mediated robust transduction, transgene expression, and targeted, potent epigenetic repression throughout the brain and spinal cord of NHPs after IV administration. STAC-BBB also demonstrated industry-leading brain tropism and enrichment in NHPs, resulting in 700-fold higher transgene expression than the benchmark capsid AAV9 and outperformed all other known published neurotropic capsid variants evaluated in the preclinical study.

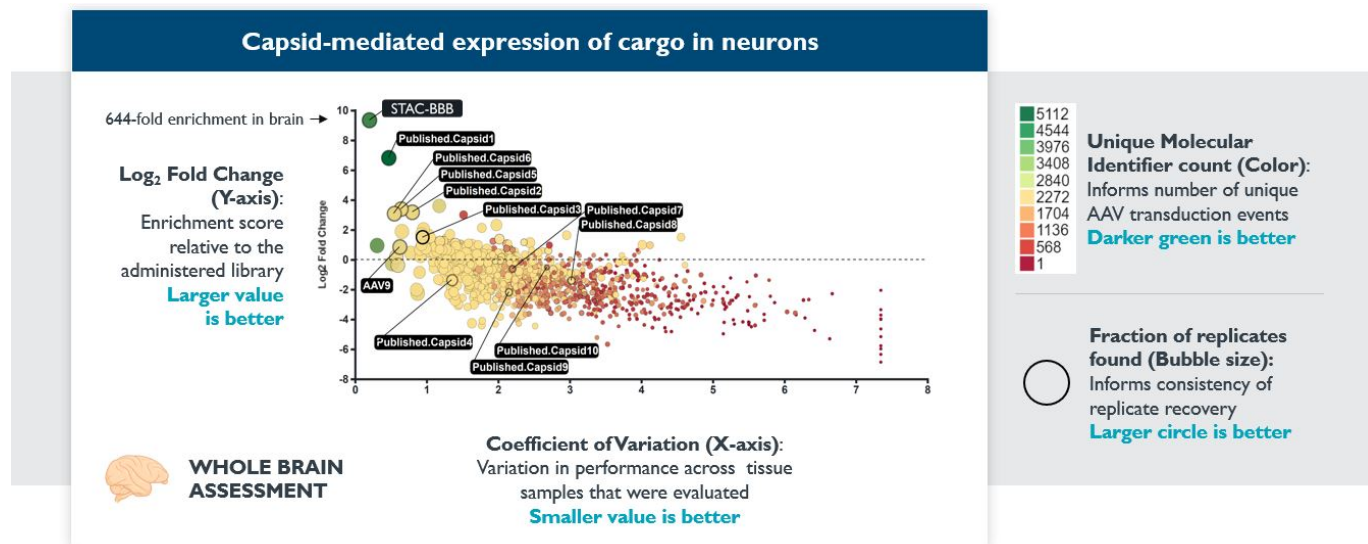


Figure 30: *In vivo* library evaluation in cynomolgus macaques identifies STAC-BBB as the top performing BBB-penetrant capsid for delivery to the brain

STAC-BBB demonstrated robust penetration of the BBB and widespread transgene expression throughout the brain of NHPs, including in key regions integral to neurological disease pathology such as Alzheimer's disease, Parkinson's disease, ALS, Huntington's disease and other neurodegenerative, neurodevelopmental, neuromuscular, and neuropsychiatric diseases with a defined neurogenetic etiology.

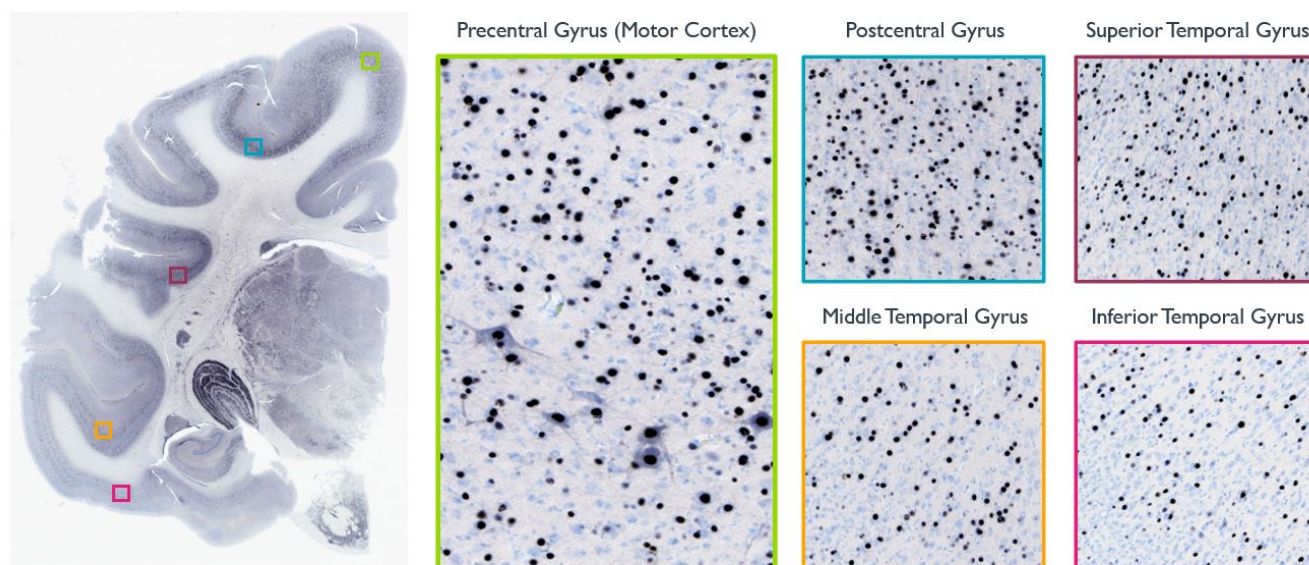


Figure 31: STAC-BBB shows widespread neuronal transduction across all cortical regions

STAC-BBB mediated robust expression of zinc finger cargo in neurons, the key cell type to target for treatment of neurological diseases. Moreover, results were highly consistent across all animal subjects. The capsid-enabled delivery of zinc finger payloads resulted in the repression of prion and tau genes across key brain regions, offering potential for modification of disease progression in prion disease and various tauopathies. STAC-BBB biodistribution was enriched in the CNS and



de-targeted from the liver, dorsal root ganglia, or DRG, and other peripheral organs. We believe this biodistribution profile is optimal for treatment of neurological diseases with AAV-based treatments.

STAC-BBB was generally well tolerated in NHPs, with no notable treatment related pathological findings in brain, spinal cord and peripheral tissues. We believe STAC-BBB is manufacturable at commercial scale using standard cell culture and purification processes, is soluble using known excipients, and can be characterized using available analytics.

We believe that improved AAV capsids with higher delivery efficiency and specificity for target tissues have the potential to facilitate development of safe and effective genomic medicines to treat CNS disorders.

STAC-BBB is the subject of current license agreements with each of Genentech and Astellas and we are actively engaged in advanced contract negotiations with a potential collaborator for a third STAC-BBB license agreement.

### **Modular Integrase Platform**

Building on Sangamo's deep expertise in protein-DNA interactions derived from its zinc finger platform, the MINT platform is a versatile, protein-guided genome editing method designed to integrate large sequences of DNA into the genome to potentially treat – with a single medicine – patients who have unique mutations in the same gene.

The MINT platform utilizes Bxb1, a serine recombinase, to integrate large sequences of DNA into the genome and is intended to avoid double stranded DNA breaks as well as the need for assistance from ancillary genome editing or DNA-repair modulating cargo. This flexible approach is cell-type agnostic, has been engineered to be simpler to manufacture than most other targeted integration technologies and is compatible with AAV, lentiviral and lipid nanoparticle, or LNP delivery. With minimal dependence on cell DNA repair machinery, we believe the MINT platform carries a reduced translocation risk and our preclinical data have demonstrated high levels of on-target integration in certain locations.

Based on these initial findings, we believe the MINT platform could be deployed internally for neurology-focused indications, and could provide potential new collaboration opportunities, both for human disease and in agricultural biotech settings.

### **Current Licenses, Partnerships and Collaborations**

We have entered into strategic collaborations with larger biopharmaceutical companies for certain of our therapeutic programs, licenses to our novel AAV capsid, STAC-BBB, and other partnerships for several non-therapeutic applications of our technology. We will continue to pursue further collaborations when appropriate to fund internal research and development activities and to assist in product development, manufacturing, regulatory approval and commercialization. Decisions to collaborate or not will be based on review of our internal resources, institutional knowledge and commercial considerations.

#### ***Genentech, Inc.***

On August 2, 2024, we entered into a global epigenetic regulation and capsid delivery license agreement, or the Genentech Agreement, with Genentech to develop intravenously administered genomic medicines to treat certain neurodegenerative diseases. Under the Genentech Agreement, we granted an exclusive license to Genentech for our ZFRs that are directed to tau and a second undisclosed neurology target. We also granted an exclusive license to Genentech to STAC-BBB for use with therapies directed to tau or to the second neurology target.

Under the Genentech Agreement, we have received from Genentech \$50.0 million in an upfront license fee and a milestone payment. In addition, we are eligible to earn up to \$1.9 billion in development and commercial milestones spread across multiple potential products under the Genentech Agreement and tiered mid-single digit to sub-teen double digit royalties on the net sales of such products, subject to certain specified reductions.

#### ***Astellas Gene Therapies, Inc.***

On December 18, 2024, we entered into a global epigenetic regulation and capsid delivery license agreement, or the Astellas Agreement, with Astellas to develop intravenously administered genomic medicines to treat certain neurodegenerative diseases. Under the Astellas Agreement, we granted a worldwide exclusive license to Astellas to utilize the STAC-BBB capsid for one target, with the right for Astellas to add up to four additional targets after paying us additional licensed target fees.

Under the Astellas Agreement, we have received from Astellas a \$20.0 million upfront license payment. In addition, we are eligible to earn up to \$1.3 billion in additional licensed target fees and milestone payments across the five potential neurology disease targets under the Astellas Agreement, as well as tiered mid-to-high single digit royalties on the net sales of any resulting products, subject to certain specified reductions and other conditions.



### ***Pfizer Inc. – Giroctocogene Fitelparvovec – Hemophilia A***

In May 2017, we entered into an exclusive, global collaboration and license agreement with Pfizer for the research, development and commercialization of giroctocogene fitelparvovec, also known as SB-525, our gene therapy product candidate for hemophilia A, and closely related products, which we amended in December 2019. In December 2024, Pfizer notified us that it will terminate the Collaboration Agreement effective April 2025 due to its decision not to proceed with the BLA and MAA submissions for, or continue to pursue commercialization of, giroctocogene fitelparvovec, or SB-525.

AFFINE is a global Phase 3, open-label, multicenter, single arm trial evaluating the efficacy and safety of a single infusion of giroctocogene fitelparvovec in more than 60 adult (ages 18-64 years) male patients with moderately severe to severe hemophilia A. The primary endpoint is impact on ABR through 12 months following treatment with giroctocogene fitelparvovec, compared to ABR on FVIII replacement therapy collected in the Phase 3 lead-in study period.

For recent updates on giroctocogene fitelparvovec, see “*Overview—Product Pipeline—Clinical Programs*” above.

### ***Prevail Therapeutics Inc.***

In July 2023, we entered into a research evaluation and option agreement with Prevail Therapeutics Inc., a wholly owned subsidiary of Eli Lilly, or Prevail, which granted Prevail rights to evaluate certain proprietary engineered CSF-administered AAV capsids developed by us. Under the agreement, Prevail has an option to obtain an exclusive license to use the capsids for certain neurological targets. If Prevail exercises its option for a target, Prevail would lead and fund all further research, development, manufacturing and commercialization of Prevail products incorporating the licensed capsids for that target.

### ***nChroma Bio***

In July 2023, we entered into a research evaluation, option and license agreement with Chroma Medicine (now nChroma Bio), or nChroma, to develop epigenetic medicines leveraging our ZFPs for sequence-specific DNA recognition of targets outside of the CNS. If nChroma exercises its option for a target, nChroma would lead and fund all further research, development, manufacturing, and commercialization of nChroma products incorporating the licensed Sangamo ZFPs for that target.

### ***Alexion Pharmaceuticals, Inc. – ALS and Frontotemporal Lobar Degeneration***

We and Pfizer entered into an exclusive, global collaboration and license agreement in December 2017 to develop preclinical genome engineering product candidates that use allele-specific ZF-transcriptional repressors to treat ALS and frontotemporal lobar degeneration, or FTL, linked to mutations in the *C9ORF72* gene. The most frequent genetic cause of ALS is the expansion of hexanucleotide repeats, or G4C2 repeats, in the first intron of the *C9ORF72* gene.

In October 2023, Pfizer notified us that it had assigned to Alexion the collaboration and license agreement between Sangamo and Pfizer for the development and commercialization of potential gene therapy products that use ZF-transcriptional regulators, to treat ALS and FTL linked to mutations of the *C9ORF72* gene.

### ***Takeda – Huntington’s Disease***

In January 2012, we entered into a collaboration and license agreement with Shire International GmbH, a wholly owned subsidiary of Takeda, which we amended and restated in September 2015, to research, develop and commercialize human therapeutics and diagnostics for monogenic diseases based on our ZF technology. We and Takeda developed genome engineering product candidates to treat Huntington’s Disease that use a ZFR designed to differentially down regulate the mutated disease-causing huntingtin gene, or HTT gene, while preserving the expression of the normal version of the gene. Pursuant to the amended and restated agreement, Takeda has an exclusive, worldwide license to ZF therapeutics for treating Huntington’s disease.

### ***Other Partnerships***

In addition to our partnerships for the development of human therapeutic applications, we have also licensed our technology in several other areas, such as plant agriculture and research reagents, including the production of transgenic animals and cell-line engineering. These license partners include Corteva AgriScience, formerly known as Dow AgroSciences LLC, or DAS, Sigma-Aldrich Corporation (now MilliporeSigma in the United States and Merck KGaA outside the United States), and Open Monoclonal Technology, Inc. (now Ligand Pharmaceuticals Inc.).

## **INTELLECTUAL PROPERTY**

Patents, trade secrets, know-how and licensed technologies are important to our business. Our strategy includes filing, obtaining, maintaining, licensing, and when necessary, defending our patents and patent applications to protect technologies,



inventions, and improvements to inventions that we consider important for the research, development, and commercialization of our technologies and our product candidates. We have filed numerous patent applications with the U.S. Patent and Trademark Office, or USPTO, and with patent offices in multiple foreign jurisdictions. Our proprietary intellectual property includes methods relating to the design of ZFPs, Transcription Activator-Like Effector, or TALE, proteins and CRISPR/Cas editing systems, therapeutic applications of genome editing technology, viral vector delivery platforms, enabling technologies related to our platform and the use of genome editing across a variety of applications. We rely on a combination of patents, copyrights, trademarks, proprietary know-how, continuing technological innovations and trade secret protections, as well as confidentiality agreements, materials transfer agreements, research agreements and licensing agreements, to establish and protect our proprietary rights.

### **In-licensed Technology**

We have exclusively licensed in relevant fields certain intellectual property directed to the design, selection, and use of ZFPs and ZFNs for genome editing from academic institutions. Although no individual in-license is material to our overall protection of our ZFP and ZFN platforms, we believe that these in-licenses, in combination with our own know-how, patent applications and patents, may help protect us from unauthorized third parties who might try to copy or use our products or technologies.

### **Our Intellectual Property**

In addition to our in-licensed patent portfolio, we have numerous issued patents and pending patent applications comprising approximately 140 patent families that are directed to the design, compositions and uses of ZFPs, ZFNs, ZF-transcriptional factors, TALE proteins and CRISPR/Cas editing systems, viral vector delivery platforms, and other technologies related to our programs.

Given our over two-decade history with zinc finger technology, some of the earliest zinc finger patents in our portfolio began expiring in 2015. However, we have continued to build on this patent portfolio and have been issued additional patents and have applications pending that provide protection for our ZF technology, therapeutic programs, modular integrase and capsid delivery technologies. Additionally, patents that may be issued from our pending applications will extend the patent exclusivity of our patent estate.

We believe that our in-licensed and our owned patents and patent applications, in combination with our know-how and trade secrets, in the aggregate, will provide us with substantial protection of and exclusivity around the commercial development of our gene therapy and genome engineering programs. In this regard, patents issued to us, applied for by us, or exclusively and non-exclusively licensed to us, cover our commercially relevant technologies, including the following types of inventions, processes and products:

- *ZFP and ZFN design, engineered nucleases, and compositions (multiple patents issued with expected expiration dates ranging from 2029 to 2036), absent any PTA, PTE or disclaimers*: These patents cover inventions including DNA target site selection, zinc finger binding domain design, nuclease domain design, linker design, DNA nickases, ZFP libraries databases and methods of construction, as well as methods to increase zinc finger binding specificity (see, e.g., US9982245, US10066242, US10113207);
- *Nuclease Therapeutics (multiple patents issued with expected expiration dates ranging from 2031 to 2036, absent any PTA, PTE or disclaimers)*: These patents cover inventions including treatments for hemophilia inherited metabolic diseases, genome editing, Parkinson's Disease, regulation of the expression of PD1; Immunomodulatory therapeutics; CNS disease; Modified T cells, including HLA knock out and methods of editing stem cells (see, e.g., US10081661, US10143760); and
- *Capsid Delivery Technologies (one patent issued with expected expiration date in 2040, absent any PTA, PTE or disclaimers)*: This patent covers novel AAV capsid sequences for the delivery of genome engineering molecules and gene therapy molecules and describes related capsid engineering and screening inventions/technologies (see, e.g., US11981967).

The patent positions of biopharmaceutical companies, including our patent position, are uncertain and involve complex legal and factual questions for which important legal tenets are largely unresolved and are subject to administrative, judicial, and regulatory interpretation and refinement. Obtaining, maintaining, and enforcing patent protection in the United States and other countries remains uncertain and depends, in part, upon decisions of the patent offices, courts, administrative bodies and lawmakers in these countries. It is also possible that we may develop proprietary products or technologies in the future that are not patentable. Patent applications may not result in the issuance of patents and the coverage claimed in a patent application may be significantly reduced before a patent is issued. It is possible that, under certain circumstances, patent applications will be rejected and we subsequently abandon them. It is possible that we may decide that an issued patent or pending patent application may provide us with little or no competitive advantage in view of its associated costs, in which case we may



abandon or allow to lapse such patent or patent applications. Although we have filed for patents on some aspects of our technology, we cannot provide assurances that patents will be issued as a result of these pending applications or that any patent that has been or may be issued will be upheld. It is possible that our current patents, or patents which we may later acquire, may be successfully challenged, invalidated in whole or in part, or deemed unenforceable. The laws of some foreign countries may not protect our proprietary rights to the same extent as do the laws of the United States.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

We may not be able to protect our intellectual property rights throughout the world. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in 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. Competitors may use our technologies in jurisdictions where we have not 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. Ultimately, patent protection must be sought on a country-by-country basis, which is an expensive and time-consuming process with uncertain outcomes. Accordingly, we may choose not to seek or maintain patent protection in certain countries, and we will not have the benefit of patent protection in such countries.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against third parties, including government agencies or government contractors. In some countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

In the future, third parties may assert patent, copyright, trademark, and other intellectual property rights to technologies that are important to our business. The outcome following any potential legal assertions of infringement, invalidity and unenforceability is unpredictable. Any claims asserting that our products infringe or may infringe proprietary rights of third parties, if determined adversely to us, could significantly harm our business. See “Risk Factors—*Risks Relating to Our Intellectual Property*”

## **COMPETITION**

We and our biopharmaceutical collaborators are leaders in the research and development of gene therapies, cell therapies and genome engineering therapies using ZF DNA-binding proteins.

We are aware of several other companies focused on other methods for editing genes and regulating gene expression and a limited number of commercial and academic groups pursuing the development of ZF genome engineering technologies. The fields of gene therapy and genome engineering are highly competitive, and we expect competition to persist and intensify in the future from a number of different sources, including other biopharmaceutical companies; academic and research



institutions; and government agencies that will seek to develop ZFs as well as technologies that will compete with our ZF technology platform, such as TALE proteins and the CRISPR-Cas editing system.

Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA approval or commercializing competitive products before we do. If we commence commercial product sales, we may be competing against companies with greater marketing, sales, distribution and manufacturing capabilities, areas in which we have limited or no experience. In addition, any product candidate that we successfully develop may compete with existing products that have long histories of safe and effective use.

Although we are in the clinical development phase of operations and have no current therapeutic product sales, we believe the following companies, products and/or technologies may potentially be competitive with our technology or our product candidates under development:

- Protein pharmaceuticals under development at pharmaceutical and biotechnology companies such as F. Hoffman-LaRoche Ltd., Protalix Biotherapeutics, Inc., Sanofi S.A. and numerous other biopharmaceutical firms.
- Gene therapy companies developing gene-based products in clinical trials such as BioMarin Pharmaceutical, Inc., F. Hoffman-LaRoche Ltd. through their wholly owned subsidiary Spark Therapeutics, Spur Therapeutics Limited, ExeGenesis Bio Co. and 4D Molecular Therapeutics, Inc. and numerous other gene therapy companies.
- Nuclease and base editing technologies under development for therapeutic applications of genome modification including companies such as Caribou Biosciences, Inc., CRISPR Therapeutics AG, Editas Medicine, Inc., Intellia Therapeutics, Inc. and Beam Therapeutics developing the CRISPR/Cas editing system, Cellectis S.A. developing TALE nucleases and meganucleases, bluebird bio, Inc. developing Homing Endonucleases and MegaTALs and Precision BioSciences, Inc. developing meganucleases and numerous other gene editing companies.
- Antisense therapeutics and RNA interference technology, including RNAi and microRNA, which are technologies that may compete with ours in the development of novel therapeutic products acting through the regulation of gene expression. These technologies are being developed by several companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc., Moderna, Inc., Regulus Therapeutics Inc., Voyager Therapeutics, Inc., Wave Life Sciences, Inc. and numerous other companies.
- Small molecules in development by pharmaceutical companies such as Biogen, Inc., Pfizer, Inc., Vertex Pharmaceuticals, Inc. and numerous other companies.
- AAV capsid technologies developed by companies such as 4D Molecular Therapeutics, Affinia Therapeutics Inc., Capsida Biotherapeutics, Dyno Therapeutics, Inc., StrideBio, Inc., Voyager Therapeutics, Inc. and numerous other companies.

We expect to face intense competition from other companies for collaborative arrangements with biopharmaceutical companies, for establishing relationships with academic and research institutions, for licenses to proprietary technology and for subjects in our clinical trials of treatments for rare diseases. These competitors, either alone or with their collaborative partners, may succeed in developing technologies or products that are more effective or less costly than ours.

Our ability to compete successfully will depend, in part, on our ability to:

- develop safe, efficacious and commercially attractive proprietary products;
- obtain access to gene transfer technology on commercially reasonable terms;
- obtain required regulatory approvals;
- obtain reimbursement for our products in approved indications;
- attract and retain qualified scientific and product development personnel;
- enter into collaborative and strategic partnerships with others, including our competitors, to develop our technology and product candidates;
- obtain and enforce patents, licenses or other proprietary protection for our products and technologies;
- formulate, manufacture, market and sell any product that we develop;
- develop and maintain products that reach the market first and are technologically superior to or are of lower cost than other products in the market; and
- recruit patients into our clinical trials in a timely fashion.



## MANUFACTURING

We are substantially reliant on external partners to manufacture preclinical and clinical supply for our neurology portfolio. We operate in-house analytical and process development capabilities.

We rely on contract manufacturing organizations, or CMOs, to produce our preclinical and clinical AAV product candidates in accordance with FDA and EMA regulations, leveraging current Good Manufacturing Practices, or cGMPs. We employ a technical operations staff in the areas of process development, analytical development, quality control, quality assurance, supply chain, project management, and external manufacturing to facilitate appropriate transfer to and oversight of our CMOs, support our regulatory filings, and supply our clinical trials.

We currently leverage a distinct manufacturing platform for AAV vector production for our genome engineering and gene therapy product candidates. We use both a commercial scale insect-based baculovirus manufacturing platform and a clinical scale HEK293 mammalian platform to manufacture our various AAV vectors for genome editing and gene therapy, with each AAV vector packaging a different transgene specific to the target indication or ZFN.

## GOVERNMENT REGULATION

We operate within the heavily regulated biopharmaceutical industry and much of our operations, including nonclinical and clinical trials, development, manufacturing, commercialization, marketing and reimbursement are subject to regulatory approvals. Relevant regulatory authorities include, but are not limited to, the FDA, the EMA, the European Commission, national competent authorities of the European Union, or EU, Member States and the U.K. Medicines and Healthcare Products Regulatory Agency.

### Product Regulation

In the United States, the FDA regulates biologic products including gene therapy and human cellular therapy products under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the Public Health Service Act, or the 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, advertising and other promotional practices involving biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. FDA approval also must be obtained before marketing of biologic products. In the EU, approval from the competent authorities of EU Member States must be obtained before commencing clinical trials. In addition, medicinal products can only be marketed if a marketing authorization, or MA, from the competent regulatory agencies has been obtained.

The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes, regulations and applicable guidance require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

### *U.S. Biologic Products Development Process*

Our product candidates must be approved by the FDA before they may be legally marketed 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, or GLP, 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 FDA objects within 30 days;
- approval by an independent institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's Good Clinical Practice, or GCP, 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 a BLA for marketing approval that includes substantial evidence of safety and efficacy from results of nonclinical testing and clinical trials and payment of user fees, if applicable;
- 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 inspection of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA.

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, 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 GLPs.

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

Human gene transfer protocols are subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level as set forth in National Institutes of Health, or NIH, Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA. However, many companies and other institutions, not otherwise subject to the NIH Guidelines, voluntarily follow them.

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 the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. 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 will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

### ***EU Drug Development Process***

Similar to the United States, the various phases of preclinical and clinical research in the EU are subject to significant regulatory controls. Certain preclinical (also termed "non-clinical") data is required in order to enable clinical trials and later be used in dossier for a MAA. All studies should be conducted in accordance with GLP and all applicable EMA, European Commission and European Pharmacopoeia guidelines related to preclinical studies, including guidance on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells.

The requisite amount of preclinical data enables the design of a clinical trial, from Phase 1 (first-in-human clinical trials) through to Phases 2 and 3, which are quality, safety and efficacy studies. Similar restrictions and requirements apply as in the United States regarding preclinical data to support trials using viral vectors. The preclinical tests should establish parameters such as toxicity, pharmacodynamics and pharmacokinetic properties, as well as the quality of the gene therapy medicinal products. Due to the particular nature of gene therapy medicinal products, it is recognized that it may not always be possible for the non-clinical safety studies to be in conformity with the principles of GLP and a proper justification should be submitted where a pivotal non-clinical safety study has not been conducted under GLP rules.

Clinical studies are crucial to obtaining the required data and the requirements governing the conduct of clinical trials are further analyzed below.

All medicinal products and advanced therapy medicinal products, or ATMPs, must be manufactured in accordance with the guidelines on GMP and in a GMP licensed facility, which can be subject to GMP inspections.



## Human Clinical Trials

Clinical trials involve the administration of the biologic product candidate to patients under the supervision of qualified investigators which 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. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent.

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers 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.

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 human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. Phase 1 clinical trials of gene and cell 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 a study which presents the data that the FDA or other relevant regulatory agency 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 efficacy 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 initial 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. Sometimes approval for a product is conditional upon the completion of post-marketing clinical studies.

During all phases of clinical development, regulatory agencies (such as the FDA, the EMA, national competent authorities of EU Member States and other comparable 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.

Written IND safety reports must be promptly submitted to the FDA and the investigators for: serious and unexpected adverse events; any findings from other trials, *in vivo* laboratory tests or *in vitro* testing that suggest a significant risk for human subjects; or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

The FDA and comparable foreign regulatory authorities or the sponsor or its data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable safety risk. Similarly, an IRB and comparable foreign regulatory authorities can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's or comparable foreign regulatory authority's requirements or if the biologic product candidate has been associated with unexpected serious harm to patients.

The FDA and comparable regulatory authorities in the EU usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for up to a 15-year period.



In the EU, clinical trials are governed by the Clinical Trials Regulation (EU) No 536/2014, or the CTR, which entered into application on January 31, 2022. The CTR is intended to harmonize and streamline CTAs, simplify adverse-event reporting procedures, improve the supervision of clinical trials and increase clinical trial transparency. Specifically, the CTR, which is directly applicable in all EU Member States, introduces a streamlined application procedure through a single-entry point, the Clinical Trials Information System, or CTIS, a single set of documents to be prepared and submitted for the application, as well as simplified reporting procedures for clinical trial sponsors. A harmonized procedure for the assessment of applications for clinical trials has been introduced and is divided into two parts. Part I assessment is led by the competent authorities of a reference Member State selected by the trial sponsor and relates to clinical trial aspects that are considered to be scientifically harmonized across EU Member States. This assessment is then submitted to the competent authorities of all the concerned Member States in which the trial is to be conducted for their review. Part II is assessed separately by the competent authorities and ethics committees in each concerned EU Member State. Individual EU Member States retain the power to authorize the conduct of clinical trials in their territory. All ongoing trials are now subject to the provisions of the CTR.

If the medicinal product is considered to be a genetically modified organism, or GMO, then GMO approval may also be required from the national GMO competent authorities of EU Member States. There is no harmonization between EU Member States regarding the approach to and timelines of GMO approval, which may result in diverging requirements between EU Member States. In addition, the submission of applications for approval of GMOs to national competent authorities of EU Member States is not made in tandem with applications for the approval of clinical trials that must be submitted via CTIS. As a result, sponsors of clinical trials that include GMOs requiring separate approval cannot benefit from submission of a single application dossier for the approval of a clinical trial and the subsequent synchronized response from EU Member States. This may impact study initiation in a given country.

The conduct of clinical trials should follow the approved clinical trial protocol, informed consents requirements, including patient informed consents, procedures and controls designed and approved for such studies, accepted standard medical and scientific research procedures and be conducted in accordance with the relevant principles of GCP and all applicable laws and regulations. Gene therapy medicinal products are in addition subject to the rules of GCP for ATMPs, which outline specific additional safeguards and requirements. Record retention requirements are increased for ATMPs as there are relevant long term follow-up and human safety and traceability requirements.

### ***Compliance with cGMP Requirements***

Manufacturers of biologics must comply with applicable current cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies, as well as foreign authorities including the competent authorities of the EU Member States. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA, as well as foreign authorities including the competent authorities of the EU Member States, upon their initial participation in the manufacturing process. Any material changes to the manufacturing equipment, process or location of the approved manufacturing site must be reported to the relevant agency/authority. Establishments may be subject to periodic, unannounced inspections by government authorities (including regulatory agencies) to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer or holder of an approved BLA or authorization for clinical trial, and may extend to requiring withdrawal of the product from the market, issue warning or similar letters or seeking civil, criminal or administrative sanctions against the company. The FDA and foreign authorities including the competent authorities of the EU Member States will not approve a BLA unless they determine that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specification.

Concurrent with clinical trials, companies develop additional information about the physical and biological characteristics of the product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

For a product candidate that is also a human cellular or tissue product, the FDA also requires compliance with current Good Tissue Practices, or cGTPs. These are FDA and EU regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues and cellular and tissue-based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP



requirements is to ensure that cell and tissue-based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA and EU regulations also require tissue establishments to register and list their HCT/Ps with the FDA or the competent authorities of the EU Member States and, when applicable, to evaluate donors through screening and testing.

### ***U.S. 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 requesting approval to market the product for one or more indications.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA 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 program fee for approved 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 or for a product indication for orphan diseases.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency 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 effective, for its intended use 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, or REMS, 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 IND trial requirements and GCP 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 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.

### ***EU Review and Approval Process***

In the EU, medicinal products can only be commercialized after a related MA has been granted. To obtain an MA for a product in the European Economic Area, or EEA (which is comprised of the 27 Member States of the EU plus Norway, Iceland and Liechtenstein), an applicant must submit a MAA, either under a centralized procedure administered by the EMA or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual recognition procedure). An MA may be granted only to an applicant established in the EU.

The centralized procedure provides for the grant of a single MA by the European Commission that is valid for all EU Member States. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for (i) medicinal products derived from biotechnological processes, (ii) products designated as orphan medicinal products, (iii) ATMPs and (iv) products with a new active substance indicated for the treatment of HIV/AIDS, cancer, neurodegenerative diseases, diabetes, autoimmune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, authorization through the centralized procedure is optional on related approval.

Under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use, or CHMP, conducts the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing MA. The maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated assessment may be granted by the CHMP in exceptional cases. If the CHMP accepts a request for accelerated assessment, the time limit of 210 days will be reduced to 150 days (excluding clock stops). The CHMP can, however, revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

Unlike the centralized authorization procedure, the decentralized MA procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States that, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the Heads of Medicines Agencies' Coordination Group for Mutual Recognition and Decentralised Procedures – Human for review. The subsequent decision of the European Commission is binding on all EU Member States.

The mutual recognition procedure allows companies that have a medicinal product already authorized in one EU Member State to apply for this authorization to be recognized by the competent authorities in other EU Member States. Like the decentralized procedure, the mutual recognition procedure is based on the acceptance by the competent authorities of the EU Member States of the MA of a medicinal product by the competent authorities of other EU Member States. The holder of a national MA may submit an application to the competent authority of an EU Member State requesting that this authority recognize the MA delivered by the competent authority of another EU Member State.

An MA has, in principle, an initial validity of five years. The MA may be renewed after five years on the basis of a reevaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State in which the original MA was granted. To support the application, the MA holder must provide the EMA or the competent authority with a consolidated version of the electronic Common Technical Document providing up-to-date data concerning the quality, safety and efficacy of the product, including all variations introduced since the MA was granted, at least nine months before the MA ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide on justified grounds relating to pharmacovigilance to proceed with one further five-year renewal period for the MA. Once subsequently definitively renewed, the MA shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EU market (for a centralized MA) or on the market of the authorizing EU Member State (for a decentralized MA) within three years after authorization ceases to be valid (the so-called sunset clause).

Innovative products that target an unmet medical need and are expected to be of major public health interest may be eligible for a number of expedited development and review programs, such as PRIME designation. Products eligible for PRIME must target conditions for which there is an unmet medical need and demonstrate the potential to address the unmet medical need by introducing new methods of therapy or improving existing ones. Benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions



on clinical trial designs and other development program elements and potentially accelerated MAA assessment once a dossier has been submitted.

In the EU, a “conditional” MA may be granted in cases where all the required safety and efficacy data are not yet available. The European Commission may grant a conditional MA for a medicinal product if it is demonstrated that all of the following criteria are met: (i) the benefit-risk balance of the medicinal product is positive, (ii) it is likely that the applicant will be able to provide comprehensive data post-authorization, (iii) the medicinal product fulfils an unmet medical need and (iv) the benefit of the immediate availability to patients of the medicinal product is greater than the risk inherent in the fact that additional data are still required. The conditional MA is subject to conditions to be fulfilled for generating the missing data or ensuring increased safety measures. It is valid for one year and must be renewed annually until all related conditions have been fulfilled. Once any pending studies are provided, the conditional MA can be converted into a traditional MA. However, if the conditions are not fulfilled within the timeframe set by the EMA and approved by the European Commission, the MA will cease to be renewed.

An MA may also be granted “under exceptional circumstances” where the applicant can show that it is unable to provide comprehensive data on efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. These circumstances may arise in particular when the intended indications are very rare and, in the state of scientific knowledge at that time, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. Like a conditional MA, an MA granted in exceptional circumstances is reserved to medicinal products intended to be authorized for treatment of rare diseases or unmet medical needs for which the applicant does not hold a complete data set that is required for the grant of a standard MA. However, unlike the conditional MA, an applicant for authorization in exceptional circumstances is not subsequently required to provide the missing data. Although the MA “under exceptional circumstances” is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually, and the MA will be withdrawn if the risk-benefit ratio is no longer favorable.

### ***Manufacturing Regulations in the EU***

Various requirements apply to the manufacturing and placing on the EU market of medicinal products. The manufacturing of medicinal products in the EU requires a manufacturing authorization, and import of medicinal products into the EU requires a manufacturing authorization allowing for import. The manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including EU cGMP standards. Similarly, the distribution of medicinal products within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of EU Member States. Marketing authorization holders and/or manufacturing and import authorization, or MA holders and/or distribution authorization holders may be subject to civil, criminal or administrative sanctions, including suspension of manufacturing authorization, in case of non-compliance with the EU or EU Member States’ requirements applicable to the manufacturing of medicinal products.

### ***Post-approval Requirements***

Rigorous and extensive FDA and EU 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, record-keeping 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. Failure to comply with the FDA’s post-approval regulations can result in withdrawal of product approval and licensure.

A sponsor also must comply with the FDA’s or EU and/or the applicable EU Member States’ laws and requirements governing 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 (or Summary of Product Characteristics in the EU) (known as “off-label use”). 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 approval by the FDA or competent foreign regulatory authority 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 review and approval.

### ***Orphan and RMAT Designation; Accelerated Approval***

Products that are intended for treating rare conditions that affect fewer than 200,000 people in the United States, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug, may qualify for orphan designation. In the EU, a medicinal product can be designated as an orphan medicinal product by the European Commission if the product sponsor can establish that: (i) the product is intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions; (ii) either (a) such conditions affect not more than five in 10,000 persons in the EU when the application is made, or (b) the product without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in developing the medicinal product; and (iii) there exists no satisfactory authorized method of diagnosis, prevention or treatment of the condition that has been authorized in the EU, or even if such method exists, the product will be of significant benefit to those affected by that condition.

Once marketing authorization has been granted in relation to a medicinal product with orphan designation, the product can benefit from a market exclusivity period in respect of the specific orphan indication for which the drug has been approved for a period of seven years in the United States and for up to ten years in the EU. If the manufacturer is no longer able to assert that the product meets the orphan designation criteria or is not able to supply sufficient quantities of the product, it may lose orphan market exclusivity. In the EU, the period of market exclusivity 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 on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, an MA may be granted to a similar medicinal product with the same orphan indication during the ten year period if: (i) if the applicant consents to a second original orphan medicinal product application, (ii) if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) if the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

RMAT designation is intended to expedite review of a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and for which preliminary clinical evidence indicates the potential to address unmet medical needs for such a disease or condition.

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 of the related BLA. However, RMAT designation does not change the FDA's standards for product approval. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

Accelerated Approval may be granted for products that are intended to treat a serious or life-threatening condition and that provide a meaningful therapeutic advantage to patients over existing treatments. A product eligible for Accelerated Approval may be approved on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. The Accelerated Approval pathway may be contingent on a sponsor's agreement to complete ongoing longer term studies or conduct additional post-approval confirmatory studies to verify and describe the product's clinical benefit, and upon submission of such data satisfying the contingency, the BLA is given full approval. Confirmatory data must be provided with due diligence. Failure to confirm a clinical benefit would allow the FDA to withdraw the product from the market on an expedited basis. All promotional materials for products that receive Accelerated Approval are subject to prior review by the FDA.

### ***Clinical Trial Data Disclosure***

Many jurisdictions have mandatory clinical trial information obligations incumbent on sponsors. In the EU, transparency requirements relating to clinical trial information are established in the CTR, which establishes a general principle according to which information contained in CTIS shall be made publicly accessible unless confidentiality is justified on grounds of protecting personal data or commercially confidential information, protecting confidential communications between EU Member States in relation to the preparation of an assessment report or ensuring effective supervision of the conduct of a clinical trial by EU Member States. This confidentiality exception may be overruled if there is an overriding public interest in disclosure. The publication of data and documents in relation to the conduct of a clinical trial will take place in accordance with



specific timelines. The timelines are established by the EMA and are determined based on the documents and the categorization of the clinical trial.

In addition, Regulation No. 1049/2001 on access to documents, or the ATD Regulation, and the related EMA policy 0043 on access to documents provide for a wide right for EU-based interested parties to submit an access to documents request to the EMA to access certain information held by the EMA. Only very limited information is exempted from disclosure (i.e., commercially confidential information, which is construed increasingly narrowly and protected personal data). It is possible for competitors to access and use this data in their own research and development programs anywhere in the world, once these data are in the public domain.

## **Regulation of Our Operations**

Although we currently do not have any products on the market, we may be subject to healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws may include, without limitation:

- the federal healthcare Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, 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 a federal healthcare program such as Medicare and Medicaid;
- federal civil and criminal false claims laws, including the federal False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment or approval that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit, among other things, knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information held by certain healthcare providers, health plans and healthcare clearinghouses, known as covered entities, and individuals and entities that perform services for them that involve individually identifiable health information, known as business associates as well as covered subcontractors;
- the federal Physician Payments Sunshine Act created under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare and Medicaid Services, or CMS, information related to payments and other transfers of value to physicians (currently defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members;
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; some state and foreign laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the government, require drug manufacturers to report information related to payments and other transfers of value to other healthcare providers and healthcare entities, marketing expenditures; or drug pricing; and/or ensure the registration of sales personnel; and
- state and foreign laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to significant penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of our operations, exclusion from participation in federal and



state healthcare programs, imprisonment, suspension or withdrawal of our marketing and commercialization in respect of our commercially approved products, and additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, any of which could adversely affect our ability to operate our business and our financial results. Responding to investigations can be time- and resource-consuming and can divert management's attention from the business. Any such investigation or settlement could increase our costs or otherwise have an adverse effect on our business.

## **Healthcare Reform**

The U.S. and some foreign jurisdictions are considering enacting or have enacted a number of additional legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our product candidates profitably, if approved. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts, which include major legislative initiatives, such as the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, to reduce the cost of care through changes in the healthcare system, including limits on the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

There have been amendments and legal and political challenges to certain aspects of the ACA, as well as efforts to repeal or replace certain aspects of the ACA. For example, on August 16, 2022, the Inflation Reduction Act of 2022, or the IRA, was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost through a newly established manufacturer discount program. It is possible that the ACA will be subject to additional challenges in the future. It is unclear how additional challenges and the healthcare reform measures of the second Trump administration will impact the ACA.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. These changes include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and, due to subsequent legislative amendments to the statute, will remain in effect through 2032 unless additional Congressional action is taken. Additionally, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, effective January 1, 2024. Further, additional health reform measures may be implemented in the future, particularly in light of the recent U.S. Presidential and Congressional elections.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. For example, the IRA, among other things, (1) directs the U.S. Department of Health and Human Services, or HHS to negotiate the price of certain high-expenditure single-source biologics that have been on the market for at least 11 years covered under Medicare, or the Medicare Drug Price Negotiation Program, and (2) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively in fiscal year 2023, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On August 15, 2024, HHS announced the agreed-upon prices of the first ten drugs that were subject to price negotiations, which take effect in January 2026. On January 17, 2025, HHS selected 15 additional products covered under Part D for price negotiation in 2025. Each year thereafter, more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights, which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. It is unclear whether these or similar policy initiatives will be implemented in the future.

The current Trump administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, the Centers for Medicare & Medicaid Services, or CMS, and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions may, for example, include directives to reduce agency workforce, rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation, or CMMI, to consider new payment and healthcare models to limit drug spending and eliminating the Biden administration's executive order that directed HHS to establishing an AI task force and developing a strategic plan. Additionally, in its June 2024 decision in *Loper Bright Enterprises v. Raimondo*, Loper Bright, the U.S. Supreme Court overturned the longstanding *Chevron* doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The *Loper Bright* decision could result in additional legal challenges to current regulations and guidance issued by federal agencies applicable to our operations, including those issued by the FDA. Congress may introduce



and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program created under the IRA.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

In the United States, the EU and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

### **Pricing, Coverage and Reimbursement**

Pricing and reimbursement of a therapeutic product will largely determine the affordability of the product, and whether the product is prescribed and supplied to patients and private insurance companies may take into account government reimbursement methodologies. Due to these proposed and enacted laws, as well as other actions, significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval, particularly for novel products. In both domestic and foreign markets, sales and reimbursement of any approved products will depend, in part, on the extent to which third-party payors, such as government health programs, commercial insurance and managed healthcare organizations provide coverage, and establish adequate reimbursement levels, for such products. Third-party payors are increasingly challenging the prices charged for medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication. Additionally, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. If third-party payors do not consider our products to be cost-effective compared to other therapies, these payors may not cover our products after approved as a benefit under their plans or, if they do, the level of reimbursement may not be sufficient to allow us to sell our products on a profitable basis. Further, coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the EU, pricing and reimbursement schemes vary widely from country to country. The EU provides options for its Member States to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product, or they may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures. Some countries may also require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. The Health Technology Assessment, or HTA, process is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. In December 2021, Regulation No 2021/2282 on Health Technology Assessment (“HTA”) Regulation, was adopted in the EU. The HTA Regulation is intended to boost cooperation among Member States in assessing health technologies, including new medicinal products, and providing the basis for cooperation at level of the EU for joint clinical assessments in these areas. The HTA Regulation has applied from January 12, 2025 although it will enter into force iteratively and initially apply to new active substances to treat cancer and to all advanced therapy medicinal products (ATMPs), it will then be expanded to orphan medicinal products in January 2028, and to all centrally authorized medicinal products as of 2030. Selected high-risk medical devices will also be assessed under the HTA Regulation as of 2026. The HTA Regulation is intended to harmonize the clinical benefit assessment of HTA across the EU. Pricing and reimbursement decisions, based on these assessments, remain the responsibility of individual Member States. See “Risk Factors—*Even if we are able to commercialize any approved products, such products may not receive coverage and adequate reimbursement from third-party payors in the United States and in other countries in which we seek to commercialize them, which could harm our business.*”



## Environmental Regulation

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

## Privacy and Cybersecurity Regulation

We are, or may become, subject to numerous privacy and data security laws and regulations in the United States and in other foreign jurisdictions, including, as applicable, the Federal Trade Commission Act, the EU General Data Protection Regulation, or EU GDPR, the EU GDPR as it forms part of the United Kingdom's law by virtue of Section 3 of the European Union (Withdrawal) Act 2018, as amended, or U.K. GDPR, and the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020, or collectively the CCPA.

The collection, use, disclosure, transfer or other processing of personal data regarding individuals in the EEA and U.K., including personal health data, is subject to the EU GDPR, and U.K. GDPR, or collectively the GDPR, as applicable. The GDPR, which is wide-ranging in scope, imposes several requirements on us relating to, among other things, the control over personal data by individuals to whom the personal data relates, notice we must provide to individuals regarding our processing of their personal data, the documentation we must maintain, the security and confidentiality of the personal data, data breach notification, and the use of third-party processors in connection with the processing of personal data. The GDPR also imposes strict rules on the transfer of personal data to countries that the European Commission does not consider to provide an adequate level of privacy and data security (including the United States). The GDPR authorizes the imposition of large penalties and other corrective actions for noncompliance, including potential fines of up to €20 million (£17.5 million) or 4% of the annual global revenue of the noncompliant company, whichever is greater, temporary or definitive bans on data processing and other corrective actions or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. The GDPR requirements related to international data transfers apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries such as Sangamo France, including employee information. The GDPR has increased our responsibility and potential liability in relation to personal data that we process, particularly in light of our acquisition of Sangamo France, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, which could divert management's attention and increase our cost of doing business.

In the EU, the Network and Information Security Directive, or NIS2, which entered into force in January 2023, aims to improve the resilience and incident response capabilities of entities operating in a number of sectors, including the health sector. As a Directive, NIS2 does not automatically have legal force throughout every Member State, instead NIS2 had to be transposed into the national laws of each Member State by October 17, 2024. Non-compliance with NIS2 may result in administrative fines of a maximum of €10 million or up to 2% of the total worldwide turnover of the preceding financial year.

In the United States, federal, state and local governments have enacted numerous privacy and data security laws, including laws on data breach notification, personal data privacy and consumer protection. For example, the CCPA applies to personal data of consumers, business representatives and employees who are California residents and requires businesses to provide detailed disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights related to their personal data. The CCPA provides for civil penalties of up to \$7,500 per intentional violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA (like other U.S. comprehensive privacy laws) exempts some data processed in the context of clinical trials, the CCPA may increase compliance efforts and costs and potential liability with respect to other personal data we may maintain about California residents. Similar laws have been enacted or proposed by several other states, as well as at the federal and local levels. These laws may further complicate compliance efforts, and may increase legal risk and compliance costs for us and the third parties upon whom we rely.

Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations. Furthermore, the laws are not consistent, and compliance in the event of a widespread data breach is costly. See "Risk Factors—*Our current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse, privacy, data*



*security and other healthcare laws and regulations. If we fail to comply with such regulations, we could face regulatory investigations or actions, litigation (including class claims) and mass arbitration demands, and substantial fines and penalties, and our business, reputation, results of operations, financial condition and prospects could be adversely affected.”*

## **HUMAN CAPITAL MANAGEMENT**

### **Our Mission and Our Employees**

At Sangamo, we are committed to translating ground-breaking science into genomic medicines that transform patients' lives. We are a passionate group of biotechnology professionals with years of experience and technical expertise, committed to developing best-in-class genomic medicines. We embrace collaboration, discipline and efficiency while welcoming fresh ideas and stimulating personal development. We encourage and embrace an inclusive culture and believe it enhances our work towards one common goal: to transform the lives of the patients we aim to serve.

We view our employees as one of our most valuable assets in serving our mission. We compete in the highly competitive biotechnology industry, and attracting, retaining and developing a diverse group of talented employees is crucial to our strategy and our ability to compete effectively. We are committed to the development and retention of our workforce. There continues to be a shortage of skilled individuals with substantial experience discovering, developing and manufacturing genomic medicines, which is likely to continue. As a result, there continues to be competition between biopharmaceutical companies and academic institutions for individuals with these skills.

### **Our Values**

We believe success comes when we align our core values with our mission to deliver genomic medicines that replace today's symptomatic treatments and transform patients' lives. Our core values are:

- Doing what's right for patients:
  - We collaborate with purpose and are driven by results that benefit patients.
  - We strive to put patient safety and quality of care first.
  - Patient needs drive our sense of urgency to deliver medicines.
  - We embrace our responsibility to pioneer the field of genomic medicine bioethically.
  - We take an inclusive approach to guide our development efforts.
- Succeeding through teamwork:
  - We are driven by our shared vision that genomic medicine will transform the lives of patients and the field of healthcare.
  - We are a passionate and dedicated group of individuals who collaborate proactively and openly to execute and progress our business forward.
  - We define our priorities clearly, communicate them, and take collective accountability to deliver results for all stakeholders.
  - We are resilient and determined to succeed together because patients are depending on us.
- Innovating through smart decisions:
  - We courageously, relentlessly, and urgently pursue the journey of innovation to succeed in the field of genomic medicine.
  - We mine scientific possibilities with the goal of unlocking new treatment solutions for serious diseases.
  - We strive to achieve our business goals through agile, inclusive and efficient decision making.
  - We learn and grow from decades of scientific experience to develop therapies at the cutting edge of medicine.
  - We learn from failure, and seek to continuously improve performance, as part of the journey to achieve breakthroughs.
- Fostering belonging:
  - We develop shared goals that create a sense of belonging.
  - We are a company where individuals from all backgrounds can flourish, grow and develop their expertise while bringing their authentic selves to work.



- We feel connected to our local communities, the environment in which we live and the patient communities we serve.
- We come together to understand our scientific learnings and progress the evolution of our business.
- We are committed to nurturing diverse and inclusive environments to advance healthcare equity.

## Our Management of Human Capital

Our human resource function focuses on the attraction and recruitment of candidates, leadership training and development, total rewards packages consisting of compensation and benefits, and employee engagement and retention. As of December 31, 2024, our global human resource function comprised six full time human resource professionals.

As of December 31, 2024, we had 183 full time employees located in the United States, France, Germany and the United Kingdom. Of these employees, 176 were located in the United States, primarily in the San Francisco Bay Area, three were located in Valbonne, France and the remaining four were located in Germany and the United Kingdom. On March 1, 2024, our Board of Directors approved a wind-down of our French research and development activities and a corresponding reduction in workforce, which was completed as of January 3, 2025.

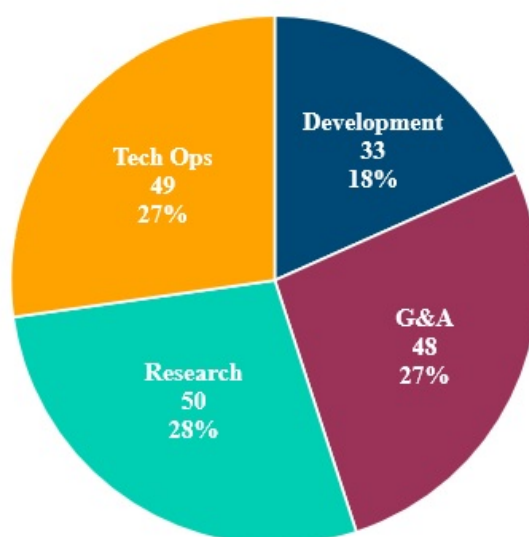
Of the 183 full time employees, 83 were primarily engaged in research and development activities, 49 were primarily engaged in technical operations and manufacturing and 51 were primarily engaged in general and administrative activities. We also engage the services of independent contractors and consultants as needed for special or temporary projects or specific expertise.

To manage our human resources, we track and report internally on key talent metrics including headcount by business unit and country, historical headcount growth, turnover, new hires and terminations, open roles and employee demographics including gender, race and ethnicity. Our senior executives use these metrics to assist with resource planning, recruitment and retention initiatives and the design of our compensation and benefits programs. We share these metrics quarterly with the Compensation Committee of our Board of Directors to assist it in fulfilling its duties to establish our enterprise compensation philosophy, administer our compensation and benefit plans, evaluate the performance of our executive officers and key employees and review and monitor management development and succession plans.

Our leaders work closely with managers and employees to understand and shape our culture and work dynamics to identify areas of focus that will increase overall employee engagement.

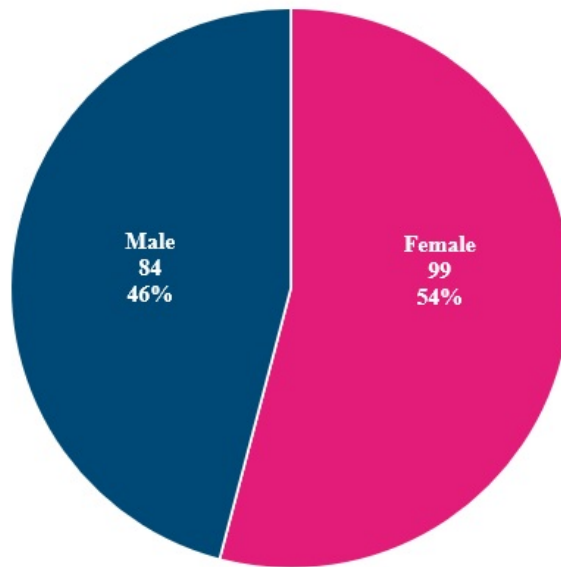
The following graphs represent the composition of employees at Sangamo as of December 31, 2024:

### Org Unit (Global)

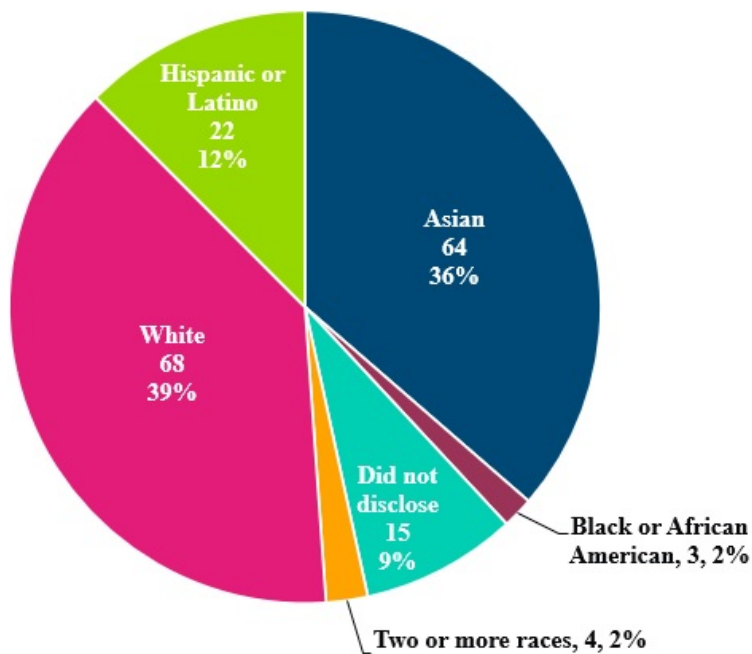




## Gender (Global)



## Ethnicity (US Only)



## Our Compensation and Benefits

Given the highly competitive nature of our industry and the importance of recruitment and retention to our success, we strive to provide our employees with what we believe is a very competitive and comprehensive total rewards package of compensation, benefits and development opportunities. This package includes at or above-market pay; healthcare benefits for employees and family members; a health savings account for eligible U.S. employees with above market employer contributions; generous paid time off benefits; family leave; bereavement leave; flexible work schedules; contributions to retirement and/or pension plans; a supplemental long term disability plan; mental health benefits and onsite gym access. In



addition, we offer a monthly stipend for employees to spend on health and well-being. We also offer every full-time employee globally the benefit of equity ownership in Sangamo through stock option grants and/or restricted stock units. Our U.S. employees are also eligible to participate in an employee stock purchase plan, which offers the opportunity to purchase our common stock at a discount of at least 15%.

## **Properties**

Sangamo is headquartered in Richmond, California, and has an additional facility in Brisbane, California. We closed our facility in Valbonne, France at the end of 2024 and are in the process of closing our facility in Brisbane, California.

## **Trademarks and Tradenames**

BETTER THERAPEUTICS BY DESIGN is a registered trademark in the United States and SANGAMO is a registered trademark in Australia, Canada, the European Union and the United Kingdom. SANGAMO THERAPEUTICS and SANGAMO THERAPEUTICS Design are registered trademarks in Australia, Canada, the European Union, Japan and the United Kingdom. They have been allowed, but are not yet registered, in the United States. The trademarks UNIVERSAL GENE RECOGNITION, UNIVERSAL GENETOOLS and ZFP THERAPEUTIC are registered in Canada. All other trademarks or trade names referred to in this Annual Report on Form 10-K are the property of their respective owners.

## **Company Information**

We were incorporated in June 1995 in the state of Delaware and in January 2017, we changed our name from “Sangamo BioSciences, Inc.” to “Sangamo Therapeutics, Inc.” Our principal executive offices are located at 501 Canal Blvd., Richmond, California 94804. Our telephone number is (510) 970-6000.

## **Available Information**

Our website is located at [www.sangamo.com](http://www.sangamo.com). This Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and our Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available free of charge on our website as soon as reasonably practicable after we electronically file this material with, or furnish it to, the Securities and Exchange Commission, or SEC. Information found on, or accessible through, our website is not a part of, and is not incorporated into, this Annual Report on Form 10-K. In addition, the SEC maintains a website at [www.sec.gov](http://www.sec.gov) that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC.

## **ITEM 1A – RISK FACTORS**

*Our business involves material risks, which are described below. Before making investment decisions regarding our common stock, you should carefully consider these risks, as well as the other information in this Annual Report on Form 10-K, including our financial statements and the related notes and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” The occurrence of any of the events or developments described below could have a material adverse effect on our business, results of operations, financial condition and prospects. In such event, the market price of our common stock could decline, and you could lose all or part of your investment. In addition, there are additional risks not described below that either are not presently known to us or that we currently deem immaterial, and these additional risks could also materially impair our business, operations or market price of our common stock.*

### **Risks Relating to our Finances**

***We have historically incurred significant operating losses since inception and anticipate continued losses for the foreseeable future. We may never become profitable.***

We have a history of recurring net losses, including \$97.9 million and \$257.8 million for the years ended December 31, 2024 and 2023, respectively, and we have otherwise generated operating losses since we began operations in 1995. The extent of our future losses and the timing of profitability are uncertain, and we expect to incur losses for the foreseeable future. We have been engaged in developing our ZF technology since inception, which has and will continue to require significant research and development expenditures. To date, we have generated our funding from issuance of equity securities, revenues derived from collaboration agreements, licenses to our capsid technology, other strategic partnerships in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. We expect to continue to incur additional operating losses for the next several years as we continue, subject to our ability to raise substantial additional capital, to develop our preclinical core neurology therapeutic programs and capsid engineering platform. If the time required to generate significant product revenues and achieve profitability is longer than we



currently anticipate or if we are unable to generate liquidity through equity financing or other sources of funding, we may be forced to further curtail or suspend, or entirely cease, our operations.

***There is substantial doubt about our ability to continue to operate as a going concern. We need substantial additional funding to execute our operating plan and to continue to operate as a going concern. If adequate funds are not available to us on a timely basis, or at all, we will be required to take additional actions to address our liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering our research and development activities, which would have a material adverse effect on our business and prospects, or we may be required to cease operations entirely, liquidate all or a portion of our assets, and/or seek protection under the U.S. Bankruptcy Code, and you may lose all or part of your investment. Future sales and issuances of equity securities would also result in substantial dilution to our stockholders.***

We have incurred significant operating losses and negative operating cash flows since inception and have not achieved profitability. Based on our current operating plan, which includes our estimates that our cash and cash equivalents as of December 31, 2024, together with \$10.1 million generated through our at-the-market offering program in 2025 and the \$5.0 million payment expected from Pfizer by the end of March 2025, we expect to meet our liquidity requirements only into the middle of the second quarter of 2025. Our financial position raises substantial doubt about our ability to continue to operate as a going concern. Our ability to continue to operate as a going concern is dependent upon our ability to raise substantial additional capital to fund our operations and support our research and development endeavors, including to progress our preclinical and clinical programs as described in this Annual Report on Form 10-K. In this regard, we have been seeking, and continue to actively seek substantial additional capital, including through public or private equity or debt financing, royalty financing or other sources, such as strategic collaborations and other direct investments in our programs. Additional capital may not be available on acceptable terms or at all. In particular, the perception of our ability to continue to operate as a going concern may make it more difficult to obtain financing for the continuation of our operations, particularly in light of currently challenging macroeconomic and market conditions. Further, we may be unable to attract new investments as a result of the speculative nature of our newly reprioritized core neurology preclinical programs and the absence of partners to progress our more advanced clinical-stage programs. In particular, if we are not able to secure a commercialization partner for our Fabry disease program in the near term, our ability to raise additional capital needed to support our operations will be substantially impaired. If adequate funds are not available to us on a timely basis, or at all, we will be required to take additional actions to address our liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering our research and development activities, which would have a material adverse effect on our business and prospects, or we may be required to cease operations entirely, liquidate all or a portion of our assets, and/or seek protection under the U.S. Bankruptcy Code, and you may lose all or part of your investment. We have explored, and will continue to explore, whether filing for bankruptcy protection is in the best interest of our company and our stakeholders.

In April 2023, we announced a restructuring of operations and a reduction in force and a significant reduction in our internal manufacturing and allogeneic research footprints in California, or the April 2023 Restructuring, and in November 2023, we announced a further restructuring of operations and reduction in force, or the November 2023 Restructuring, including a strategic transformation to focus resources on our proprietary neurology-focused epigenetic regulation programs and AAV capsid delivery technology and move all U.S. operations, including our headquarters, to our Richmond, California facility. On March 1, 2024, our board of directors approved the wind-down of our research and development activities in France and closure of our facility in Valbonne, France, or the France Restructuring. While the April 2023 Restructuring was completed in the third quarter of 2024 and the France Restructuring was substantially completed in the fourth quarter of 2024, we expect the November 2023 Restructuring to be completed in the near future, we may also incur other cash expenses or charges not currently contemplated or estimable due to events that may occur as a result of, or associated with, the November 2023 restructuring. In addition, we may not achieve the expected benefits of these cost reduction measures and other cost reduction plans on the anticipated timeline, or at all, or we may use our available capital more quickly than we expect, which could otherwise accelerate our liquidity needs and could force us to further curtail or suspend, or entirely cease, our operations.

Moreover, we have historically relied in part on collaboration partners to provide funding for and otherwise advance our preclinical and clinical programs. However, in June 2023, our collaboration agreements with Biogen and Novartis terminated, our collaboration agreement with Kite expired pursuant to its terms in April 2024, and in December 2024, Pfizer notified us of its termination for convenience, effective April 21, 2025, of its collaboration agreement with us. Further, while we may identify new collaboration partners who can progress some of the programs that were the subject of these collaborations, as well as our Fabry disease program, STAC-BBB capsid and modular integrase platform, we have not yet been, and may never be, successful in doing so in a timely manner, or on acceptable terms or at all, and we may otherwise fail to raise sufficient additional capital in order to progress these and our other programs ourselves, in which case, we will not receive any return on our investments in these programs. Although we have received \$50.0 million in upfront license fees and milestone payments and are eligible to earn future development and commercial milestone payments in connection with our license



agreement with Genentech and we also received \$20.0 million in an upfront license fee and are eligible to earn future additional licensed target fees and milestone payments in connection with our license agreement with Astellas, we may never receive any further payments under either agreement. In any event, we need substantial additional funding in order to advance our core neurology programs, including to make planned regulatory submissions and commence planned clinical trials as described in this Annual Report, as well as our Fabry disease and hemophilia A programs, capsid engineering efforts and modular integrase platform, and to otherwise execute on our current operating plan.

If we raise additional capital through public or private equity offerings, including sales pursuant to our at-the-market offering program with Jefferies LLC, the ownership interest of our existing stockholders will be diluted, and such dilution may be substantial given our current stock price decline. In addition, the terms of any new equity securities we may issue may have a preference over, and include rights superior to, our common stock. If we raise additional capital through royalty financings or other collaborations, strategic alliances or licensing arrangements with third parties, we may need to relinquish certain valuable rights to our product candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable. If we raise additional capital through debt financing, we may be subject to specified financial covenants or covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or pursuing certain transactions, any of which could restrict our ability to commercialize our product candidates or operate as a business.

In addition, as we focus our efforts on proprietary human therapeutics, we will need to seek regulatory approvals of our product candidates from the FDA or other comparable foreign regulatory authorities, a process that could cost in excess of hundreds of millions of dollars per product. We may experience difficulties in accessing the capital markets due to external factors beyond our control, such as volatility in the equity markets for emerging biotechnology companies and general economic and market conditions both in the United States and abroad. In particular, our ability to raise the substantial additional capital we need in order to fund our business may be adversely impacted by global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide, such as has been experienced recently, including as a result of the imposition of tariffs and escalating trade tensions. We cannot be certain that we will be able to obtain financing on terms acceptable to us, or at all. Our failure to obtain adequate and timely funding will adversely affect our ability to continue to operate as a going concern and our ability to develop our technology and products candidates, and we may be required to cease operations.

***If we seek to reorganize under the U.S. Bankruptcy Code, our future operations are uncertain, and such reorganization could be unsuccessful and/or result in no recovery for holders of our common stock. If we are unable to successfully reorganize, we may be forced to pursue a liquidation of some or all of our assets.***

Based on our current operating plan we expect to meet our liquidity requirements only into the middle of the second quarter of 2025. We continue to actively seek substantial additional capital, including through public or private equity or debt financing, royalty financing or other sources, such as strategic collaborations and other direct investments in our programs. We have explored, and will continue to explore, whether filing for bankruptcy protection is in the best interest of our company and our stakeholders. In the event we file for relief under the U.S. Bankruptcy Code, our operations, our ability to develop our product candidates and execute on our operating plan, and our ability to continue as a going concern will be subject to the risks and uncertainties associated with bankruptcy proceedings, including, among others: our ability to execute, confirm and consummate a plan of reorganization; the additional, significant costs of bankruptcy proceedings and related fees; our ability to obtain sufficient financing to allow us to emerge from bankruptcy and execute our business plan thereafter, and our ability to comply with terms and conditions of any such financing; our ability to continue our operations in the ordinary course; our ability to maintain our relationships with our collaborators, counterparties, employees and other third parties; our ability to obtain, maintain or renew contracts that are critical to our operations on reasonably acceptable terms and conditions or at all; our ability to attract, motivate and retain key employees; the ability of third parties to use certain provisions of the U.S. Bankruptcy Code to terminate contracts without first seeking Bankruptcy Court approval; the ability of third parties to seek and obtain court approval to terminate or shorten the exclusivity period for us to propose and confirm a plan of reorganization, to appoint a trustee, or to convert a proceeding under Chapter 11 of the U.S. Bankruptcy Code to a proceeding under Chapter 7 of the U.S. Bankruptcy Code; and the actions and decisions of our stakeholders and other third parties who have interests in our bankruptcy proceedings that may be inconsistent with our operational and strategic plans. Any delays in our bankruptcy proceedings would increase the risks that we may not be able to reorganize our business and emerge from bankruptcy proceedings and may increase our costs associated with the bankruptcy process or result in prolonged operational disruption. In addition, we would need the prior approval of the Bankruptcy Court for transactions outside the ordinary course of business during the course of any bankruptcy proceedings, which may limit our ability to respond timely to certain events or take advantage of certain opportunities. Because of the risks and uncertainties associated with any bankruptcy proceedings, we cannot accurately predict or quantify the ultimate impact of events that could occur during any such proceedings. There can be no guarantees that if we seek protection under the U.S. Bankruptcy Code, we will emerge from any such proceedings as a going concern or that holders of our common stock will receive any recovery from any bankruptcy proceedings.



In the event we are unable to pursue protection under Chapter 11 of the U.S. Bankruptcy Code, or, if pursued, successfully emerge from such proceedings, it may be necessary for us to pursue protection under Chapter 7 of the U.S. Bankruptcy Code for all or a part of our businesses. In such event, a Chapter 7 trustee would be appointed or elected to liquidate our assets for distribution in accordance with the priorities established by the U.S. Bankruptcy Code. We believe that liquidation under Chapter 7 would result in significantly smaller distributions being made to our stakeholders than those we might obtain under Chapter 11, or no distribution at all, primarily because of the likelihood that the assets would have to be sold or otherwise disposed of in a distressed fashion over a short period of time rather than in a controlled manner and as a going concern. In such event, you may lose part or all of your investment.

***We have fully impaired our goodwill and indefinite-lived intangible assets, have recorded significant impairment of our long-lived assets, and may be required to record significant additional charges if our long-lived assets become further impaired in the future.***

We evaluate the carrying value of long-lived assets, which include property and equipment, leasehold improvements and right-of-use assets, for impairment whenever events or changes in circumstances indicate that the carrying amounts of the asset may not be fully recoverable. Factors that may indicate potential impairment and trigger an impairment test include, but are not limited to, general macroeconomic conditions, conditions specific to the industry and market, an adverse change in legal factors, business climate or operational performance of the business, and sustained decline in our stock price and market capitalization compared to the net book value. During the years ended December 31, 2024 and 2023, we recognized impairment charges of \$5.5 million and \$155.0 million, respectively. We have fully impaired our goodwill and indefinite-lived intangible assets in 2023 and have significantly impaired our long-lived assets in both 2023 and 2024. We will continue to assess whether our long-lived assets are impaired in future periods. We have completed the wind-down of our France research and development activities and corresponding reduction in force of all France employees, and expect to close our Brisbane, California facility in the near future, and we have recognized related impairments in both 2023 and 2024. It is reasonably possible that additional impairment charges will be recognized, for example, if sublease rates of leased facilities are less than those estimated. For additional information regarding these impairment charges, see Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

It is possible that changes in circumstances, many of which are outside of our control, or in the numerous variables associated with the assumptions and estimates used in assessing the appropriate valuation of our long-lived assets, could in the future result in significant additional impairment charges to our long-lived assets, which could adversely affect our results of operations.

***Our ability to use net operating losses to offset future taxable income may be subject to limitations.***

Although a certain amount of our federal net operating loss carryforwards carry forward indefinitely (but are subject to a percentage limitation), a significant amount of our federal and all of our state net operating loss carryforwards will begin to expire, if not utilized, beginning in 2025 and 2029, respectively. The net operating loss carryforwards subject to expiration could expire unused and be unavailable to offset future income tax liabilities. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50 percentage point change in its equity ownership value over a three-year period, the corporation’s ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We have experienced an ownership change in the past and we may also experience additional ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. In addition, at the state level, there may be periods during which the use of net operating loss carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, California recently enacted legislation that, with certain exceptions, suspends the ability to use California net operating loss carryforwards to offset California income and limits the ability to use California business tax credits to offset California taxes, for taxable years beginning after 2023 and before 2027. As a result, if we earn net taxable income, we may be unable to use all or a material portion of our net operating loss carryforwards and other tax attributes, which could potentially result in increased future tax liability to us and adversely affect our future cash flows.

## **Risks Relating to Research, Development, Regulatory Approval and Commercialization of Our Product Candidates and Technologies**

***We are a biotechnology company with no approved products or product revenues. Our success depends substantially on results of preclinical studies and clinical trials demonstrating safety and efficacy of our product candidates***



***to the satisfaction of applicable regulatory authorities. Obtaining positive clinical trial results and regulatory approvals is expensive, lengthy, challenging and unpredictable and may never occur for any product candidates.***

We are a biotechnology company with no approved products or product revenues. Since our strategic reprioritization in 2023, we have focused substantially all of our efforts on our core preclinical neurology programs. As a result, we may find that we reduce spending and resources on product candidates or indications that later prove to have greater commercial potential than our core preclinical neurology programs. Our spending on current and future research and development programs may not yield any commercially viable products.

Should we be successful in raising additional funds necessary to execute our operating plan and to continue to operate as a going concern, we anticipate initiating clinical trials in the future on our product candidates if our preclinical studies are supportive. We are and will be substantially dependent on the results of our preclinical studies and subsequent clinical trials, and there is no guarantee that final results of clinical trials conducted on our product candidates now or in the future will demonstrate the safety and efficacy of any of our product candidates. In addition, none of our product candidates has obtained regulatory approval. Obtaining positive clinical trial results and regulatory approvals is expensive, lengthy, challenging and unpredictable and may never occur for any of our product candidates. If we fail to obtain positive results from our preclinical studies and subsequent clinical trials and regulatory approvals for our product candidates, our anticipated revenues from our product candidates and our prospects for profitability would be adversely affected, which would likely cause the market price of our common stock to significantly decline.

***Conducting clinical trials and obtaining regulatory approvals is complex and exposes our business to numerous risks, including potential unexpected costs and delays.***

We must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates to the satisfaction of regulatory authorities in order to obtain regulatory approvals necessary for commercialization. We have limited experience in conducting later stage clinical trials and may not possess the necessary resources and expertise to complete such trials. Clinical trials are expensive, lengthy and unpredictable. 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. Events that may delay or prevent successful or timely completion of clinical development and regulatory approval include, among others:

- delays in reaching a consensus with regulatory authorities on clinical trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB ethics committee or national competent authority approval at each clinical trial site;
- delays or interruptions in recruiting, screening and enrolling suitable patients to participate in our clinical trials and dosing enrolled patients;
- the imposition of clinical holds by regulatory authorities on our clinical trials or those of our collaborators;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the Good Clinical Practice and Good Laboratory Practice regulations of the FDA, or applicable comparable foreign regulations in the EU and other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions, or as a result of manufacturing or formulation changes to our product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selections of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrences of serious adverse events or other safety concerns associated with product candidates that are viewed to outweigh their potential benefits, result in approval delays or other regulatory restrictions, or harm our reputation;
- occurrences of serious adverse events or other safety concerns in clinical trials of the same class of agents conducted by other sponsors;
- failures to demonstrate that product candidates are safe and effective for their proposed indication;



- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- unexpected costs and expenses and lack of sufficient funding to develop our product candidates; and
- losses of licenses to critical intellectual properties.

We have not yet reached agreement with regulatory authorities on the complete development pathway for certain product candidates, and such authorities have the ability to change decisions or guidance with respect to approvable endpoints, particularly as the technology continues to develop in these areas. For example, we are aware of another company developing a gene therapy to treat hemophilia A that the FDA recommended complete its Phase 3 study and submit two-year follow-up safety and efficacy data on all study participants notwithstanding the company's contention that it and the FDA had previously agreed on the extent of data necessary to support a BLA. While we and Pfizer announced pivotal data readouts for our Phase 3 AFFINE trial based on full analyses of all study participants, when the first 50 patients were twelve months past reaching a steady-state of FVIII expression, the FDA or other comparable foreign regulatory authorities could determine that we need to follow patients for longer than expected to generate the required data or make other modifications to the trial, or to conduct additional studies, which could negatively impact the ability to complete the trial and seek regulatory approvals for giroctocogene fitelparvovec, which could in turn materially and adversely affect its competitive position and commercial viability and therefore our business, prospects and market price of our stock.

Due to the novelty of certain product candidates and their technologies, the endpoints needed to support regulatory approvals will likely be different from those originally anticipated. Any inability to successfully complete preclinical and clinical development of our product candidates, or complete such trials in the timeframes anticipated, could result in additional costs to us or impair our ability to generate revenues from product sales or achieve regulatory and commercialization milestones and royalties, or shorten any periods during which we may have exclusivity.

Even if a product candidate successfully obtains approval from the FDA or comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. Also, any regulatory approval of our product candidates, once obtained, may be withdrawn, varied or suspended. If we are unable to obtain and maintain regulatory approvals for our product candidates in one or more jurisdictions, or if any approval contains significant limitations, we would not be able to generate anticipated revenues and may struggle to become profitable, which would have an adverse effect on our business operations and financial condition.

***Our core preclinical neurology programs, which are the current focus of our research and development efforts, are in the early stages. We may encounter difficulties in advancing product candidates from research programs to preclinical and clinical development and may fail to capitalize on product candidates with a greater commercial opportunity or for which there is a greater likelihood of success.***

Our core preclinical neurology programs, which are the current focus of our research and development efforts, are in the early stages of development. We intend to advance our core neurology program product candidates from research programs through preclinical development and to submit new INDs, applications for clinical trial approval and equivalent filings in other jurisdictions necessary to conduct human clinical trials evaluating our product candidates. While the FDA cleared our IND application for ST-503 for the treatment of intractable pain due to idiopathic small fiber neuropathy in November 2024 and we expect to commence the Phase 1/2 clinical study in mid-2025, subject to our receipt of substantial additional capital, we have not yet demonstrated our ability to successfully commence any clinical trials of any product candidates from our core preclinical neurology programs. The preparation and submission of applications to conduct clinical trials require us to conduct rigorous and time-consuming preclinical testing and studies and prepare documentation relating to, among other things, the toxicity, safety, manufacturing, chemistry and clinical protocols of our product candidates. We may experience unforeseen difficulties that could delay or otherwise prevent us from executing this strategy successfully. For example, we may encounter problems in the manufacturing of a product candidate and may fail to demonstrate consistency in the formulation of a product candidate. Our preclinical tests may produce negative or inconclusive results, which may lead us to decide, or which may lead regulators to require us, to conduct additional preclinical testing. If we cannot obtain positive results in preclinical testing, we may decide to abandon a product candidate altogether. In addition, our ability to complete and submit such applications to conduct clinical trials may depend on the support of collaborators and the timely performance of their obligations under relevant collaboration agreements. If our collaborators are not able to perform such obligations or if they choose to slow down or delay the development of a product candidate, we may not be able to submit the clinical trial applications on a timely basis or at all. Furthermore, the submission of applications to conduct clinical trials involves significant cost and labor, and we may not have sufficient resources and personnel to complete the filing of all intended applications, which may force us to scale back the number of applications or forego potential applications that we believe are promising. Any delay, suspension or reduction of our efforts to pursue our preclinical and clinical development strategy could have an adverse effect on our business and cause the market price of our common stock to decline.



Furthermore, if our existing product candidates do not receive regulatory approval or are not successfully commercialized, then the success of our business will depend on our ability to continue to expand our product pipeline through discovery, in-licensing or acquisitions. We may be unable to do so. If we do identify potential product candidates for licensing or acquisition, we may be unable to reach acceptable terms with the licensors or sellers. Further, there may be risks and liabilities associated with the product candidates which our due diligence efforts fail to discover, that are not disclosed to us, that we inadequately assess, or that we are unable to manage effectively. Additionally, we may not realize the anticipated benefits of such licenses or acquisitions for a variety of reasons, including the possibility that the product candidates prove not to be safe or effective in clinical trials, that we are unable to successfully integrate the product candidate into our operations, or that the anticipated benefits will not otherwise be realized within the expected timeframe.

***Success in research and preclinical studies or early clinical trial results may not be indicative of results obtained in later trials. Likewise, preliminary, initial or interim data from clinical trials may be materially different from final data.***

Results from research and preclinical studies or early clinical trials are not necessarily predictive of future clinical trial results, and preliminary, initial and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical trials despite demonstrating positive results in preclinical studies or having successfully advanced through initial clinical trials or preliminary stages of clinical trials. For example, there can be no assurance that the effects demonstrated by the STAC-BBB capsid variant in our recent preclinical study in NHPs will translate into similar results in any clinical trial of human subjects. Our inability to demonstrate positive results in clinical trials using the STAC-BBB capsid could result in delays and difficulties in furthering development of our capsid platform and the epigenetic regulation therapies that incorporate or depend on the use of the STAC-BBB capsid or other capsids we may discover, or may require us to cease development of such therapies entirely.

From time to time, we have and may in the future publish or report preliminary, initial or interim data. Preliminary, initial or interim data from our clinical trials and those of our collaborators may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. In this regard, such data may show initial evidence of clinical benefit, but as patients continue to be followed and more patient data becomes available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time, or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim data should be considered carefully and with caution until the final data are available. For example, there can be no assurance that the FVIII levels shown in the updated data announced in December 2024 by Pfizer and us from the Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec will persist in future follow-up or any other data from the Phase 1/2 Alta study or the Phase 3 AFFINE clinical trial. Mean FVIII levels shown in the Phase 3 AFFINE clinical trial, after an initial peak, have tended to fall to lower levels post peak and then stabilize. We cannot anticipate whether and to what extent this trend will continue downward over time. For this reason and potentially other reasons, giroctocogene fitelparvovec may not ultimately demonstrate a durable, safe and effective clinical benefit to the satisfaction of regulatory authorities in the final results of the Phase 1/2 Alta study and the Phase 3 AFFINE clinical trial, as applicable, and even if satisfactory to regulatory authorities, such benefit may not be sufficient to yield a commercially-viable product.

There is no guarantee that any of our clinical trials will be successful. Many of our product candidates currently use our ZF technology platform, including ZFN and ZF-transcriptional regulator-technologies, which has not yet yielded any approved therapeutic products. Moreover, most of our product candidates are still in preclinical development and have never demonstrated any clinical benefit. In addition, our engineered capsids, including STAC-BBB, continue to evolve and have not been used in any approved products. If our product candidates using our ZF technology platform and viral delivery systems are not able to demonstrate safe, effective and durable results, we may be forced to suspend or terminate development of some or all of our product candidates or seek alternative technologies to develop or deliver product candidates.

In addition, there is a high failure rate for product candidates proceeding through clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. Any such setbacks could adversely affect our business, financial condition, results of operations and prospects.

***Our product candidates are subject to a lengthy and unpredictable regulatory approval process in each jurisdiction where approval is sought.***

A regulatory authority such as the FDA, the European Commission or comparable foreign regulatory authorities must approve any human therapeutic product before it can be marketed in the jurisdiction it governs. The process for receiving regulatory approval is lengthy and unpredictable, and a product candidate may not withstand the rigors of testing under the process. Before commencing clinical trials in humans in the United States, we must submit an IND to the FDA. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like



the IND prior to the commencement of human clinical trials. In the EU, for example, an application for the approval of a clinical trial must be submitted for each clinical trial to each national competent authority and relevant ethics committee of EU Member States in which sponsor wishes to conduct the clinical trial. Only after an IND becomes effective and/or the CTA has been obtained may clinical trials begin. See “Business—*Government Regulation*.” for details regarding the regulatory approval processes applicable to our product candidates. While there is some overlap, the regulatory requirements to conduct clinical trials and seek marketing approval vary by jurisdiction. There is no guarantee that the safety studies and other data generated will be sufficient to permit us to conduct clinical trials in all jurisdictions where planned, or once generated, that such clinical trial data will be sufficient to obtain marketing approval in all jurisdictions in which we intend to seek such approval. If we are not able to obtain the necessary regulatory approvals to conduct our clinical trials and commercialize our product candidates, or if such approvals are delayed or suspended, our business, prospects and market price of our common stock would be adversely affected.

***We may not be able to identify, qualify and enroll sufficient patients for our clinical trials or complete our clinical trials in a timely manner, which could delay or prevent us from proceeding with the development of our product candidates.***

Identifying, qualifying and enrolling patients in clinical trials of our product candidates, and completing these clinical trials, is critical to our success. Patient enrollment and trial completion is affected by factors including:

- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of genomic approaches to treatment of diseases;
- availability of competing therapies and clinical trials;
- delays or interruptions related to voluntary pauses of our clinical trials or those of our collaborators and the activation of trial sites;
- the imposition of clinical holds by regulatory authorities on our clinical trials or those of our collaborators, and the potential inability of Sangamo and our collaborators to lift clinical holds imposed by regulatory authorities in a timely manner or on acceptable terms, or at all;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- required and desired characteristics of patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

The timing of our clinical trials depends on our ability to recruit patients to participate as well as completion of required follow-up periods. There are also a number of other product candidates in development by our competitors, who compete for the same limited patient populations. If we are not able to enroll the necessary number of patients in a timely manner, we may not be able to complete our clinical trials on our desired timelines or at all, which could negatively impact the competitive position and commercial viability of our product candidates or delay or reduce the product revenues, milestone payments or royalty payments we expect to earn from our product candidates.

In addition, if fewer patients are willing to participate in our clinical trials because of negative publicity from adverse events related to genomic medicines, competitive clinical trials for similar patient populations or for other reasons, the timelines for conducting clinical trials of our product candidates and presenting clinical data may be delayed. These delays could result in increased costs, limitation or termination of clinical trials, and delays in product development timelines. If we are forced to expand to additional jurisdictions to address these challenges, it could impose additional costs, delays and risks. If we are not successful in conducting our clinical trials as planned, it would have an adverse effect on our business, financial condition, results of operations, prospects and market price of our common stock.



***Special regulatory designations, such as RMAT, orphan drug designations, fast track designation, or PRIME may not be available for our product candidates or may not lead to a faster development or regulatory review or approval process.***

We have received RMAT designation for our product candidates to treat severe hemophilia A and Fabry disease. Additionally, some of our product candidates, including our product candidate to treat Fabry disease, have also been granted Orphan Drug Designation by the FDA and PRIME eligibility by the EMA, and some have also been designated Orphan Medicinal Products by the European Commission. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. In addition, our product candidate to treat Fabry disease was granted FDA Fast Track Designation in May 2023. In October 2024, we announced that the FDA agreed that the ongoing Phase 1/2 STAAR study can serve as the primary basis for seeking Accelerated Approval for our product candidate to treat Fabry disease. For additional information regarding these special regulatory designations, see “Business—Government Regulation.”

If we request such designations for our other current or future product candidates, there can be no assurances that the FDA, the European Commission or comparable foreign regulatory authorities will grant any of our product candidates such designations or that pursuit of Accelerated Approval will ultimately lead to approval faster than seeking full approval directly, or at all. Additionally, such designations do not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, those product candidates, nor does it limit the ability of any regulatory agency to grant such designations to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving marketing approval. Such designations can also be revoked. RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges. Orphan drug exclusivity may be revoked if any regulatory authority determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA’s priority review procedures. Moreover, eligibility for the Accelerated Approval Program does not guarantee FDA approval, and the FDA has authority to withdraw approval of a product or an indication approved under Accelerated Approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product.

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

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions, particularly as many of the diseases we are studying have complex comorbidities. If clinical experience indicates that a product candidate has side effects or causes serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked, which would severely harm our business, prospects, operating results and financial condition.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other trials using other genomic therapies. 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 significantly 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. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration that, while not necessarily adverse to the patient’s health, could substantially limit the effectiveness of the treatment. Possible adverse side effects, including SAEs, could develop in the future, which could delay or halt any further development or potential commercialization of the applicable product candidate.

***Even if our product development efforts are successful and even if the requisite regulatory approvals are obtained, our products may not gain market acceptance among physicians, patients, healthcare payors and the medical community.***

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the approved product may not gain market acceptance among physicians, healthcare payors, patients or the medical community. Market acceptance of approved products depends on a number of factors, including:

- the efficacy and safety of the product as demonstrated in clinical trials;
- the clinical indications and patient populations for which the product is approved;
- acceptance by physicians, treatment centers and patients of the product as a safe and effective treatment;
- the adoption of novel genomic therapies by physicians, hospitals and third-party payors;



- the potential and perceived advantages of the product over alternative treatments;
- the safety of the product seen in a broader patient group, including its use outside the approved indications;
- any restrictions on product use together with other medications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other comparable foreign regulatory authorities;
- the timing of market introduction of the product as well as competitive products;
- the development of manufacturing and distribution processes for the product;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and the willingness of patients to pay out-of-pocket in the absence of coverage or inadequacy of reimbursement by third-party payors and government authorities;
- relative convenience and ease of administration; and
- the effectiveness of our sales and marketing efforts and those of our collaborators.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, healthcare payors or treatment centers, we will not be able to generate significant revenues from the approved product, which would compromise our ability to become profitable.

***Even if we are able to commercialize any approved products, such products may not receive coverage and adequate reimbursement from third-party payors in the United States and in other countries in which we seek to commercialize them, which could harm our business.***

Our ability to commercialize any product successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels, which can affect demand for, or the price of, any approved product. Given the nature of the product candidates that we are developing, some patients may require treatment only one-time (e.g., single dose administration), and there is substantial uncertainty about the pricing structure for such products, and the level of coverage and reimbursement that will be available for a shift to single-dose treatment as compared to chronic therapy over a patient's lifetime. If other companies establish a new pricing structure or business model, including payment based on demonstration of long-term efficacy, our ability to price or obtain reimbursement for our products may be adversely affected. If such pricing structure or business model do not adequately fund the costs of our research and development, manufacturing and commercialization efforts, our business may be adversely affected.

In addition to uncertainty about the potential pricing structure for certain of our product candidates, cost containment is a recurrent trend in the healthcare industry. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for certain medications. We cannot be sure that coverage and adequate reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. If reimbursement is not available or is available only at limited levels, we may be unable to successfully commercialize any product candidate for which we obtain regulatory approval. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have an adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Many EU Member States periodically review their reimbursement procedures for medicinal products, which could have an adverse impact on reimbursement status. We expect that legislators, policymakers and healthcare insurance funds in the EU Member States will continue to propose and implement cost-containing measures, such as lower maximum prices, lower or lack of reimbursement coverage and incentives to use cheaper, usually generic, products as an alternative to branded products, and/or branded products available through parallel import to keep healthcare costs down. These measures could include limitations on the prices we would be able to charge for product candidates that we may successfully develop and for which we may obtain regulatory approval or the level of reimbursement available for these products from governmental authorities or third-party payors. Further, an increasing number of EU and other foreign countries use prices for medicinal products established in other countries as "reference prices" to help determine the price of the product in their own territory. Consequently, a downward trend in prices of medicinal products in some countries could contribute to similar downward trends elsewhere.



Moreover, in order to obtain reimbursement for our products in some European countries, including some EU Member States, we may be required to compile additional data comparing the cost-effectiveness of our products to other available therapies. HTA of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. In December 2021, the EU HTA Regulation was adopted, which will enter into application in 2025 and is intended to harmonize the clinical benefit assessment of HTA across the EU. However, individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social and ethical) aspects of health technologies and making decisions on pricing and reimbursement. If we are unable to maintain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected.

***Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of and commercialize our product candidates and affect the prices we may obtain.***

The regulations that govern, among other things, regulatory approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell any product candidates for which we obtain regulatory approval. Also, there has been heightened governmental scrutiny recently over biopharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent Presidential executive orders, Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for biopharmaceutical products. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, have been designed to encourage importation from other countries and bulk purchasing. For a discussion of health reform activity and the current pricing framework, see “Business—Government Regulation—Healthcare Reform” and “Business—Government Regulation—Pricing, Coverage and Reimbursement.”

The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

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

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

In addition, the policies of the FDA, the competent authorities of the EU Member States, the EMA, the European Commission and other comparable regulatory authorities with respect to clinical trials may change, and additional government regulations may be enacted. For instance, the regulatory landscape related to clinical trials in the EU recently evolved. The CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each EU Member State leading to a single decision for each EU Member State, along with a harmonized procedure, including a joint assessment by all EU Member States concerned, and a separate assessment by each EU Member State with respect to specific requirements related to its own territory, including ethics rules. Compliance with the CTR requirements by us and our third-party service providers, such as CROs, may impact our developments plans. It is currently unclear to what extent the U.K. will seek to align its regulations with the EU in the future. A decision by the U.K. not to closely align its regulations with the new approach that will be adopted in the EU may have an effect on the cost of conducting clinical trials in the U.K. as opposed to other countries and/or make it harder to seek a marketing authorization in the EU for our product candidates on the basis of clinical trials conducted in the U.K.



If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

***Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.***

Even if we obtain regulatory approval in a jurisdiction, the competent regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved BLA is required to comply with FDA rules and is subject to FDA review and periodic inspections, in addition to other potentially applicable federal and state laws, to ensure compliance with cGMP and adherence to commitments made in the BLA.

If we or a regulatory authority discovers previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. Moreover, product labeling, advertising and promotion for any approved product will be subject to regulatory requirements and continuing regulatory review. Failure to comply with such requirements, when and if applicable, could subject us to a number of actions ranging from warning letters to product seizures or significant fines, among other actions.

Any government investigation of alleged violations of laws or regulations 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 product candidates and generate revenues.

Failure to comply with EU and EU Member State laws that apply to the conduct of clinical trials, manufacturing approval, marketing authorization of medicinal products and marketing of such products, both before and after grant of the marketing authorization, or with other applicable regulatory requirements may result in administrative, civil or criminal penalties. These penalties could include delays or refusal to authorize the conduct of clinical trials, or to grant marketing authorization, product withdrawals and recalls, product seizures, suspension, withdrawal or variation of the marketing authorization, total or partial suspension of production, distribution, manufacturing or clinical trials, operating restrictions, injunctions, suspension of licenses, fines and criminal penalties.

See “Business—Government Regulation—Post-approval Requirements” for more information.

***Our employees or contractors may engage in misconduct or other improper activities, including noncompliance with research, development, manufacturing or regulatory standards and requirements, which could cause significant liability for us and harm our reputation.***

We are exposed to the risk of fraud or other misconduct by our employees and contractors, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Misconduct by our employees and contractors could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such 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 governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such 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 and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, fines, disgorgement, personal imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, or comparable foreign programs, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

***We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on other programs or product candidates that may be more profitable or for which there is a greater likelihood of success.***

We have substantially limited resources and may forego or delay pursuit of certain research programs or product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to



capitalize on viable commercial products or profitable market opportunities or pursue collaborations rather than retain sole responsibility for development. Our current and future research and development programs for core neurology program product candidates may not yield any commercially viable products. The evaluation of the commercial potential or target market for a particular product candidate is forward-looking and based upon assumptions involving, for example and not limited to, market evolution, advances in disease standard of care, competition and reimbursement. This reliance on assumptions means that, if our assumptions prove to be inaccurate or incomplete, we may pursue opportunities that end up having a number of competitors that are more advanced than our product candidates, or we may relinquish valuable rights to a product candidate through strategic collaboration, licensing or other royalty arrangements in cases where it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We have implemented several strategic decisions to reallocate resources among our various clinical and preclinical development programs, including the decision to defer new investments in our Fabry disease gene therapy program unless and until we are able to successfully secure a commercialization partner or external investment in this program. There can be no assurance that such efforts will be successful in a timely manner, or at all, in which case, we will not receive any return on our investments in this program. If we are not able to secure a commercialization partner for our Fabry disease program in the near term, our ability to raise additional capital needed to support our operations will be substantially impaired. As part of our restructurings and the related strategic reprioritization, we have determined to focus substantially all of our efforts on our core preclinical neurology programs. Investment in preclinical programs is highly speculative, as it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect and/or an acceptable safety profile. In addition, we are developing, and may in the future develop, certain product candidates designed to treat neurological diseases using our novel capsid delivery technology. If our capsid development efforts are not successful, we may be required to defer, or cease entirely, development of such product candidates. For additional details on our collaboration terminations, see “Risk Factors—Risks Relating to our Industry.” As a result of these strategic decisions, we could miss valuable opportunities to capitalize on the potential of our discontinued and halted programs. We may also allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaboration or that does not prove to have viable commercial opportunities. Any failure to use our financial and human resources efficiently could harm our business and operations.

***ZF technology is novel and has never been used to develop any approved, commercially viable therapeutic products.***

Our ZF technology is a novel technology which to date has not yielded any approved commercially viable therapeutic products, and there can be no guarantee that our product development efforts using ZF technology will be fruitful. We have invested heavily in development of this technology, and our failure to develop approved, commercially viable products using ZF technology would significantly limit our business and prospects and would adversely impact the market value of our common stock.

## **Risks Relating to Manufacturing**

***The transition of our manufacturing processes to third parties is a complex process, and there can be no assurance that third parties will be able to continue to manufacture our product candidates as intended and without delays.***

In connection with our restructurings and the closure of our Valbonne, France facility and the anticipated closure of our Brisbane, California facility, we expect to rely solely on CMOs to manufacture clinical supply. Transferring manufacturing processes and know-how is complex and involves review and incorporation of both documented and undocumented processes that may have evolved over time. In addition, transferring production to different facilities may require utilization of new or different processes and equipment to meet the specific requirements of a given facility. Additional studies may also need to be conducted to support the transfer of certain manufacturing processes and process improvements. We cannot be certain that all relevant know-how and data have been adequately incorporated into the manufacturing process until the completion of studies and evaluations intended to demonstrate the comparability of material previously produced by our facilities with that generated by our CMOs.

***The manufacture, storage and transport of our product candidates is complex, expensive, highly regulated and risky, which could hamper their commercial viability.***

There are significant risks associated with manufacturing, storing and transporting our product candidates including, among others, cGMP compliance, cost overruns, technical problems with process scale-up, specialized facilities and equipment, process reproducibility, stability issues, lot consistency, yields and timely availability of highly specific raw materials. Even though product batches released for use in clinical trials undergo sample testing, some defects may only be identified following release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products not complying with stability requirements or specifications. Also, our product candidates must be stored and transported at temperatures within a certain range. If these environmental conditions deviate, our product candidates' remaining shelf-lives could be impaired or their efficacy and safety could be adversely affected, making them no longer suitable for use.



Moreover, product candidates that are biologics involve complex processes, including the development of cell lines or cell systems to produce the biologic, with the challenge of significant variability. There are difficulties in growing large quantities of such cells, consistently and sufficiently isolating certain types of cells and harvesting and purifying the biologic produced by them. The cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process can be difficult to reproduce.

Moreover, manufacturing, storing and transporting our product candidates is subject to strict regulatory standards, which adds additional production risk. Even if efficacy and safety data from our clinical trials would otherwise support regulatory approval of a product candidate, there is no assurance that we or our CMOs will be able to manufacture our product candidates to specifications at levels necessary to support or maintain regulatory approval by the FDA or other comparable foreign regulatory authorities.

Thus, there is no guarantee we will be successful in establishing a larger-scale commercial manufacturing process for our product candidates or obtaining the needed manufacturing capacity. Due to these manufacturing challenges, there is risk that some of our product candidates could be subject to inventory outages, reputational damage and product liability risks, and result in additional expense and delays to clinical trials and commercialization. Supply interruptions or shortages could result in potential negative impacts to our business, prospects and market price of our common stock.

***If we use chemical, biological or hazardous materials in a manner that causes injury or violates laws, we may be liable for damages.***

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials and chemicals typically employed in the study of molecular and cellular biology. We also routinely use cells in culture and gene delivery vectors. Although we maintain up-to-date licensing and training programs, we cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling, or disposal of these materials. In the event of contamination or injury, we could be held liable for damages that result, and any liability could exceed our resources. We currently carry insurance covering certain claims arising from our use of these materials. However, if we are unable to maintain our insurance coverage at a reasonable cost and with adequate coverage, our insurance may not cover any liability that may arise. We are subject to federal, state, and local laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Failure to comply with these laws and regulations could result in fines, penalties and additional liabilities and restrictions on our operations.

***We are reliant on third parties for the manufacture of our product candidates for preclinical and clinical development. If one of our third-party manufacturers fails to perform adequately or fulfill our needs, we may be required to incur significant costs and devote significant efforts to find new suppliers or manufacturers.***

In connection with our restructurings and the anticipated closure of our Brisbane, California facility in the near future, we now rely solely on CMOs to manufacture preclinical and clinical supply. We intend to continue to rely on third parties for the manufacture of product candidates for later stage clinical trials, and for commercial-scale manufacturing for any approved product. The manufacture of biopharmaceutical products in compliance with the FDA's cGMP regulations and guidance, or comparable foreign GMP regulations, requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of biopharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality control, including stability of the product candidate and quality control testing, shortages of qualified personnel, as well as compliance with strictly enforced cGMP requirements, other federal and state regulatory requirements and foreign regulations. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to conduct clinical trials could be jeopardized. Any delay or interruption in the supply of clinical trial materials could delay the completion of our clinical trials, increase the costs associated with developing our product candidates and, depending upon the period of delay, require us to commence new clinical trials at significant additional expense or terminate the clinical trials completely.

We and our CMOs must comply with cGMP requirements enforced by the FDA through its facilities inspection program and comparable foreign regulatory authorities. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. We and our CMOs may be unable to comply with these cGMP requirements and with other FDA, state and comparable foreign regulatory requirements. The FDA or similar foreign regulatory agencies may also implement new standards at any time or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. We have limited control over our manufacturers' compliance with these regulations and standards. Failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension, variation or delay in product approval, product seizure or recall or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturers' failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products and we may be held



liable for any injuries sustained as a result. Any of these factors could cause a delay of clinical trials, regulatory submissions, approvals or commercialization of our product candidates, entail higher costs or impair our reputation.

Our current agreements with our CMOs do not provide for the entire supply of the drug product necessary for all anticipated clinical trials or for full scale commercialization. If we and our CMOs cannot agree to the terms and conditions for them to provide the drug product necessary for our clinical and commercial supply needs, we may not be able to manufacture the product candidate until a qualified alternative manufacturer is identified, which could also delay the development of, and impair our ability to commercialize our product candidates.

The number of third-party CMOs with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative CMOs, which could have an adverse effect on our business. New manufacturers of any product candidate would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing the product candidate. Obtaining the necessary approvals or other qualifications under applicable regulatory requirements and ensuring non-infringement of third-party intellectual property rights could result in a significant interruption of supply and could require the new manufacturer to bear significant additional costs which may be passed on to us.

***We and third parties on which we rely may be adversely affected by natural disasters and catastrophic or other events outside of our control, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster or event.***

Natural disasters could severely disrupt our operations and our facilities and the manufacturing facilities of our CMOs, and any disruption would likely have a negative impact on our business, financial condition, results of operations and prospects. If a natural disaster, pandemic or epidemic, geopolitical crisis, including the ongoing conflict between Russia and Ukraine and conflicts in the Middle East, power outage or any other event that is out of our control occurred that prevented us or third parties on which we rely from using all or a significant portion of our or their facilities, that damaged critical infrastructure or that otherwise disrupted our or their operations, it may be difficult or, in certain cases, impossible for us to continue our business and operations for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have an adverse effect on our business, financial condition, results of operations and prospects. Such disasters or events occurring at facilities of third parties on which we rely could also negatively impact our business and operations.

## **Risks Relating to our Industry**

***Our product candidates are based on novel genomic medicine technologies, which makes it difficult to predict the timing and costs of development and of subsequently obtaining regulatory approval.***

We have concentrated our research and development efforts on genomic medicine, consisting of gene therapy and genome engineering. The regulatory approval process for novel product candidates such as ours is unclear and may be lengthier and more expensive than the process for other, better-known or more extensively studied product candidates.

Regulatory review committees and advisory groups, and any new guidelines they promulgate, may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our current or future product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. 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 potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be harmed. Even if our product candidates are approved, we expect that the FDA, or comparable foreign regulatory authorities, will require us to submit follow-up data regarding our clinical trial patients for a number of years after any approval. If this follow-up data shows negative long-term safety or efficacy outcomes for these patients, the FDA, or comparable foreign regulatory authorities, may revoke their approval or change the label of our products in a manner that could have an adverse impact on our business.

In addition, adverse developments in clinical trials of genomic medicines conducted by others may cause the FDA or other comparable foreign regulatory authorities to change the requirements for approval of our product candidates. The FDA and European Commission have only very recent and limited experience in the approval of *in vivo* gene therapy products. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates.



***Biotechnology and genomic medicine are highly competitive businesses. Our competitors may develop rival technologies and products that are superior to or are commercialized more quickly than our technologies and product candidates.***

The biopharmaceutical industry is highly competitive and subject to significant and rapid technological change. We are aware of several companies focused on other methods for editing cells, editing genes and regulating gene expression and a growing number of commercial and academic groups pursuing the development of genome engineering technology. The field of genomic medicine is highly competitive, and we expect competition to persist and intensify in the future from a number of different sources, including biopharmaceutical companies, academic and research institutions, and government agencies that will seek to develop competing products as well as technologies that will compete with our ZF technology platform. For example, in genome engineering and gene therapy products, competing proprietary technologies with our product development focus include but are not limited to, recombinant proteins, other gene therapy/cDNAs, nuclease and base editing technologies, antisense therapeutics and RNA interference technologies, siRNA, RNAi and microRNA approaches, exon skipping, small molecule drugs, monoclonal antibodies, CRISPR/Cas technology and TALE proteins, meganucleases, and MegaTALs. See “Business—Competition” for more information on the competition we may face.

Any products that we or our collaborators or strategic partners develop will enter into highly competitive markets. Even if we are able to generate products that are safe and effective for their intended use, competing technologies may prove to be more effective or less expensive, which, to the extent these competing technologies achieve market acceptance, will limit our revenue opportunities. In some cases, competing technologies have proven to be effective and less expensive. Competing technologies may include other methods of regulating gene expression or modifying genes. ZFNs and ZF-transcriptional regulators have broad application in the life sciences industry and compete with a broad array of new technologies and approaches being applied to genetic research by many companies.

In addition to possessing competing technologies, our competitors include biopharmaceutical companies with:

- substantially greater capital resources than ours;
- larger research and development staffs and facilities than ours; and
- greater experience in product development and in obtaining regulatory approvals and patent protection.

These organizations also compete with us to attract qualified personnel, attract parties for acquisitions, joint ventures or other collaborations and license the proprietary technologies of academic and research institutions that are competitive with our technology, which may preclude us from pursuing similar opportunities. Accordingly, our competitors may succeed in obtaining patent protection or commercializing products before we do. Even if our product candidate is more effective, it may be disadvantaged if it is not first to market. In addition, any products that we develop may compete with existing products or services that are well established in the marketplace. Further, some of our product candidates in development are designed for use once. Any success in developing one-time use therapeutics could cause us to lose potential recurring revenues from therapeutics that are designed to be taken over a patient’s lifetime.

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

Genetically modified products are currently subject to public debate and heightened regulatory scrutiny. Gene therapy remains a novel technology, with only two *in vivo* gene therapy products approved for a genetic disease to date in the United States and only a few *in vivo* gene therapy products for genetic diseases approved to date in the EU. 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. For example, reports of serious adverse events in a retroviral gene transfer trial for infants with X-linked severe combined immunodeficiency, or X-linked SCID, in France and subsequent FDA actions putting related trials on hold in the United States had a significant negative impact on the public perception and stock price of certain companies involved in gene therapy, whether or not the specific company was involved with retroviral gene transfer, or whether the specific company’s clinical trials were placed on hold in connection with these events. Other adverse events could occur in the field of genomic medicine that could result in increased regulatory scrutiny, potential regulatory delays or negative impact on public perception genomic medicines, which could cause our stock price to decline.

In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

Even if the regulatory approval for genetically modified products developed using our technology is obtained, our success will also depend on public acceptance of the use of genetically modified products including medicines, plants and plant products. Claims that genetically modified products are unsafe for consumption or pose a danger to the environment may



influence public attitudes. Our genetically modified products may not gain public acceptance. 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 development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' 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 current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse, privacy, data security and other healthcare laws and regulations. If we fail to comply with such regulations, we could face regulatory investigations or actions, litigation (including class claims) and mass arbitration demands, and substantial fines and penalties, and our business, reputation, results of operations, financial condition and prospects could be adversely affected.***

Healthcare providers, including physicians, and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain regulatory approval. Arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we would market, sell and distribute our products. As a biotechnology company, even though we will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse, transparency, health privacy and security and patients' rights and comparable foreign legislation are and will be applicable to our business. Outside the United States, interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of EU Member States, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. If we fail to comply with these, or to comply with these adequately or appropriately, we could be subject to significant penalties.

For details regarding the restrictions under applicable federal and state healthcare laws and regulations that may affect our ability to operate, see "Business—Government Regulation—Additional Regulation"

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Scrutiny has also increased, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Responding to investigations can be time- and resource-consuming and can divert management's attention from the business. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If our operations or if any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws or applicable regulations, we and they could be subjected to significant civil, criminal and administrative enforcement actions, see "Business—Government Regulation—Additional Regulation"

Further, we are required to comply with domestic and international privacy and data security laws, such as the EU GDPR and the CCPA, which apply to the collection, use, disclosure, transfer, or other processing of personal data, including data we collect about trial participants in connection with clinical trials. Numerous U.S. states have enacted comprehensive privacy and data security laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance.

Certain jurisdictions have enacted data localization and cross-border data transfer laws, which could make it more difficult to transfer information across jurisdictions. In particular, the EEA and the U.K. have significantly restricted the transfer of personal data to the United States and other countries whose privacy and data security laws they believe to be inadequate. Other jurisdictions may adopt or have already adopted similarly stringent interpretations of data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and United Kingdom to the United States in compliance with law, such as the EEA standard contractual clauses, the U.K.'s International Data Transfer Agreement / Addendum and the EU-U.S. Data Privacy Framework and the U.K. extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to



lawfully transfer personal data to the United States. If we are unable to implement a legal mechanism to ensure that our transfers of personal data from the EEA or the U.K. are lawful, we could face adverse consequences, including increased exposure to regulatory actions, substantial fines and penalties and injunctions against processing or transferring personal data, and could be required to increase our data processing capabilities in the EEA, the U.K. or elsewhere at significant expense. Restrictions on our ability to transfer personal data from the EEA, the U.K. or elsewhere could impact our clinical trial activities in the EEA or the U.K. and limit our ability to collaborate with CROs and other third parties. Additionally, companies that transfer personal data out of the EEA and U.K. to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. For more information regarding these regulations, see “Business—Government Regulation—Privacy Regulation.”

We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. For example, certain privacy laws, such as the GDPR and the CCPA, require our customers to impose specific contractual restrictions on their service providers. We publish privacy policies, marketing materials and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, concerning data privacy and security. Regulators in the United States are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences. In addition, privacy advocates and industry groups have proposed, and may propose, standards with which we are legally or contractually bound to comply, or may become subject to in the future.

Our obligations related to privacy and data security (and consumers’ expectations regarding them) are quickly changing and becoming increasingly stringent, creating uncertainty. These obligations may be subject to differing applications and interpretations, which may be inconsistent or in conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources. These obligations may also necessitate changes to our information technologies, systems and practices and those of any third parties that process personal data on our behalf. We may at times fail, or be perceived to have failed, in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties upon which we rely may fail to comply with such obligations, which could negatively impact our business operations and compliance posture.

Any failure or alleged failure (including as a result of deficiencies in our policies, procedures or measures relating to privacy, data security, marketing or communications) by us or our third-party partners to comply with laws, regulations, policies, legal or contractual obligations, industry standards or regulatory guidance relating to privacy or data security, may result in significant consequences. These consequences may include, but are not limited to, governmental enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar), litigation (including class action claims) and mass arbitration demands, additional reporting requirements and/or oversight, bans or restrictions on processing personal data, orders to destroy or not use personal data, civil and criminal liability and imprisonment of company officials. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business or financial condition, including but not limited to interruptions or stoppages in business operations (including clinical trials), inability to process personal data or to operate in certain jurisdictions, limited ability to develop or commercialize our products, expenditure of time and resources to defend any claim or inquiry or revision or restructuring of our operations.

Additionally, our employees and personnel may use generative artificial intelligence, or AI, technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology may result in additional compliance costs, regulatory investigations and actions and lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages. We use AI to assist us in making certain decisions, which is regulated by certain privacy laws. Due to inaccuracies or flaws in the inputs, outputs or logic of the AI, the model could be biased and could lead us to make decisions that could bias certain individuals or classes of individuals, and adversely impact their rights, employment and ability to obtain certain pricing, products, services or benefits.

***Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.***

We face inherent risks of product liability exposure related to the testing of our product candidates in human clinical trials and will face even greater product liability risks if we commercially sell any approved products. Product liability claims may be brought against us by patients enrolled in our clinical trials, patients, healthcare providers or others using, administering



or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- termination of clinical trial sites or entire trial programs;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to clinical trial patients;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize any products that we may develop.

We currently hold product liability insurance coverage at a level that we believe is customary for similarly situated companies and adequate to provide us with insurance coverage for foreseeable risks, but which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain regulatory approval for our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products that receive regulatory approval. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

***Unfavorable global economic conditions could have a negative impact on our operations, which could materially and adversely affect our ability to continue to operate as a going concern and otherwise have a material adverse effect on our business, financial condition, results of operations, prospects and market price of our common stock.***

Financial instability and a general decline in economic conditions in the United States and other countries caused by political instability and conflict, including the ongoing conflict between Russia and Ukraine and conflicts in the Middle East, geopolitical challenges arising from the imposition of tariffs and escalating trade tensions, and economic or financial challenges caused by recent and potential future bank failures or by general health crises, have led to market disruptions, including significant volatility in commodity prices, credit and capital markets instability, including disruptions in access to bank deposits and lending commitments, supply chain interruptions, rising interest rates and global inflationary pressures. These macroeconomic factors could materially and adversely affect our ability to continue to operate as a going concern and could otherwise have a material adverse effect on our business, operations, operating results and financial condition as well as the price of our common stock. The failure of any bank in which we deposit our funds could reduce the amount of cash we have available for our operations or delay our ability to access such funds. Any such failure may increase the possibility of a sustained deterioration of financial market liquidity, or illiquidity at clearing, cash management and/or custodial financial institutions. In the event we have a commercial relationship with a bank that has failed or is otherwise distressed, we may experience delays or other issues in meeting our financial obligations. If other banks and financial institutions fail or become insolvent in the future in response to financial conditions affecting the banking system and financial markets, our ability to access our cash and cash equivalents may be threatened and our ability to raise additional capital when needed could be substantially impaired, which could have a material adverse effect on our business, operations, operating results and financial condition as well as the price of our common stock. In particular, failure to secure any necessary financing in a timely manner and on favorable terms could require us to delay or abandon clinical development plans or we may be forced to further curtail or suspend, or entirely cease, our operations. In addition, any or all of these factors could disrupt our and our collaborators' supply chains and adversely affect our and our collaborators' ability to conduct ongoing and future clinical trials of our product candidates.

### **Risks Relating to our Reliance on Third Parties**

***If conflicts arise with our contractors, collaborators or other business partners, these conflicts may limit our ability to implement our strategies and may harm our business and prospects.***

If conflicts arise with our contractors, collaborators or other business partners, the other party will likely act in its self-interest, which may limit our ability to implement our strategies. For example, some of our collaborators are conducting multiple product development efforts within each area that is the subject of their collaboration with us. Our collaborators may



develop, either alone or with others, product candidates in related fields that are competitive with the product candidates that are the subject of their collaborations with us. Competing products, either developed by the collaborators or to which the collaborators or have rights, may result in the withdrawal of their support for our product candidates.

Some of our collaborators could also become our competitors in the future. Our collaborators could develop or invest in competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate or breach their agreements with us unexpectedly or prematurely, or fail to devote sufficient resources to the development and commercialization of product candidates covered by the collaboration.

In addition, conflicts could arise between us and our collaborators resulting from disputes regarding our or our collaborators' or strategic partners' performance under the applicable agreement, including disputes arising from alleged breaches of our agreements with our collaborators.

Any of these conflicts could harm our product development efforts and otherwise adversely affect our business and prospects.

***Our collaborators control certain aspects of our product development efforts, including clinical trials and regulatory submissions, which could result in unanticipated delays and other obstacles in the commercialization of our product candidates.***

Our lack of control over aspects of product development in our collaborations could cause delays or other difficulties in the development and commercialization of our product candidates, which may prevent us from receiving any milestone, royalty payments and other benefits under the agreement. In addition, under their respective agreements, our third-party collaborators have certain rights to terminate the agreements by providing us with advance notices, therefore, the actual milestone payments that we may receive under these agreements may be substantially lower than the full amounts provided for under these agreements. For example, in December 2024, Pfizer notified us of its termination of its collaboration agreement with us, or the Pfizer Agreement, for convenience, effective April 21, 2025, as a result of Pfizer's decision to not submit a BLA or MAA for, or pursue commercialization of, giroctocogene fitelparvovec. We had previously depended on Pfizer to diligently seek to lift the clinical hold on the Phase 3 AFFINE clinical trial and resume the trial, as well as to make submissions for regulatory approval.

Further, in June 2023, our collaborations with Biogen and Novartis terminated, and in April 2024, our collaboration agreement with Kite expired. As a result, we are no longer entitled to any milestone payments or royalties from Biogen, Novartis, Kite or Pfizer, and such counterparties have no further obligations to develop or to reimburse the costs of any of the programs under the applicable agreement.

***Our collaborators licensing our ZF technologies or AAV capsid technologies may decide to adopt alternative technologies or products or may be unable or unwilling to develop commercially viable products with our ZF technologies or AAV capsid technologies, which would negatively impact our revenues and our strategy to develop product candidates using ZF technologies or AAV capsid technologies.***

Some of our ongoing collaborations leverage our ZF technology and AAV capsid technology platform. These collaborators may elect to adopt alternative technologies in the future, which could decrease the value of either or both of our ZF technology platform and AAV capsid technology platform and impede the development of product candidates using these platforms. Additionally, because our collaborators are likely to be working on more than one development project, they could choose to shift their resources to projects other than those they are working on with us. If they do so, this would delay our ability to test and develop our ZF technology platform and AAV capsid technology platform and would delay or terminate the development of our product candidates using such platform. Further, our collaborators may elect not to develop product candidates arising out of our collaborations or not to devote sufficient resources to the development, manufacturing, marketing or sale of these product candidates. If they terminate the collaborations with us or allow them to expire, such as the terminations for convenience of our collaboration agreements with Biogen, Novartis and Pfizer and the expiration of our collaboration agreement with Kite, and we wish to continue developing the product candidates, we will be required to seek the support of other collaborators or develop the products ourselves. Particularly as a result of our restructurings, we do not expect to have sufficient resources and expertise internally to allow us to continue the development of these product candidates and we may not be able to identify a suitable partner or negotiate a favorable collaboration agreement to allow us to continue the development of these product candidates.



***Our ability to continue funding our operations, advance development of our product candidates and ultimately commercialize our technologies depends on our ability to secure collaboration partners for our programs. If we are not able to find collaborators, or if our collaborators do not diligently pursue product development efforts, we may not be able to secure sufficient capital to continue to operate as a going concern or to develop our technologies or product candidates.***

We do not have financial resources ourselves to fully develop, obtain regulatory approval for and commercialize our product candidates. We have relied, and expect to continue to rely, on collaborations with other biopharmaceutical companies to provide funding for our research and development efforts, including preclinical studies and clinical trials, and expect to rely significantly on such collaborations to provide funding for the lengthy regulatory approval processes required to commercialize our product candidates.

In 2024, we entered into the Genentech Agreement and the Astellas Agreement, each for the development of intravenously administered genomic medicines to treat certain neurodegenerative diseases. Under the terms of Genentech Agreement, we were responsible for completing a technology transfer and certain preclinical activities, and Genentech is solely responsible for all clinical development, regulatory interactions, manufacturing and global commercialization of resulting products. Under the Astellas Agreement, we granted a worldwide exclusive license to Astellas to utilize the STAC-BBB capsid for one target, with the right to add up to four additional targets after paying additional licensed target fees.

We were party to collaboration agreements with Novartis and Biogen to develop product candidates to treat certain neurological diseases. In June 2023, our collaboration agreements with Novartis and Biogen terminated. We were also party to a collaboration agreement with Kite to develop engineered cell therapies for cancer, which expired by its terms in April 2024. Additionally, we are currently party to a collaboration agreement with Pfizer relating to the research, development and commercialization of giroctocogene fitelparvovec, our gene therapy product candidate for hemophilia A, which Pfizer has elected to terminate effective April 21, 2025. As a result of these terminations and expirations, we are no longer entitled to any milestone payments or royalties from Novartis, Biogen, Kite or Pfizer, and such counterparties have no further obligations to develop or to reimburse the costs of any of the programs under the applicable agreement. We cannot guarantee that we will be able to successfully secure new collaborations in the future, whether for the programs that were previously the subject of terminated collaborations or otherwise.

If we are unable to secure additional collaborations or if our collaborators are unable or unwilling to diligently advance the development, regulatory approval and commercialization of our product candidates, whether due to internal portfolio decisions or otherwise, our growth may slow and adversely affect our ability to generate funding for development of our technologies and product candidates as well as our ability to continue to operate as a going concern, and we may be required to cease operations. Although we are actively seeking partners for our Fabry disease program, there can be no assurance our efforts to secure a collaboration will be successful in a timely manner, or at all, in which case, we may not receive any return on our investments in these programs and our ability to operate as a going concern may be materially and adversely affected. Moreover, if we are unable to secure a commercialization partner for our Fabry disease program in the near term, our ability to raise additional capital necessary to fund our operations will be substantially impaired. While we are also seeking additional partnerships for our capsid delivery technology, there can be no assurance that we will be successful in doing so, and such partnerships, including the Genentech Agreement and the Astellas Agreement, do not generally provide for upfront license and near-term milestone payments in amounts sufficient to fully fund our ongoing and planned operations. In addition, our ongoing collaborators may sublicense or abandon development programs with little advance notice, or we may have disagreements or disputes with our collaborators, which would cause associated product development to slow or cease. In addition, the business or operations of our collaborators may change significantly through restructurings, acquisitions, other strategic transactions that may negatively impact their ability to advance our programs.

Under typical collaborations, we expect to receive revenue for the research and development of our product candidates based on achievement of specific milestones, as well as royalties based on a percentage of sales of any commercialized products. Achieving these milestones will depend, in part, on the efforts and internal decision making of our collaborators, which we have no control over, as well as our own efforts. In addition, business combinations, changes in a collaborator's business strategy and financial difficulties or other factors could result in that collaborator abandoning or delaying development of any product candidates covered by our collaboration agreement with that collaborator. For example, Novartis's, Biogen's and Pfizer's decisions to terminate their respective collaboration agreements with us each related to strategic reviews. Further, if we fail or any collaboration partner fails to meet specific milestones, then the collaboration agreement may be terminated, which would preclude our ability to earn any additional milestone payments under that collaboration agreement and would reduce our revenues. In addition, even if a collaboration product candidate is successfully developed and approved for marketing by relevant regulatory authorities, if sales of the commercialized product fail to meet expectations, we could receive lower royalties than expected. In any event, the milestone and royalty payment opportunities associated with our collaborations involve a substantial degree of risk to achieve and may never be received. Accordingly, investors should not assume that we will receive all of the potential milestone payments provided for under our ongoing collaborations, and it is possible that we may never receive any further significant milestone payments or any royalty payments under our collaborations.



## Risks Relating to our Intellectual Property

***Because it is difficult, time consuming and costly to obtain, maintain and enforce patent protections for our technologies and product candidates, and because third parties may have made inventions that are similar to ours, we may not be able to secure optimal patent protections of our technologies and product candidates.***

Our commercial success may depend in part on obtaining, maintaining and enforcing patent protection for our technologies and product candidates and successfully defending any of our patents that may be challenged. Obtaining, maintaining and enforcing biopharmaceutical patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications in all desired jurisdictions, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner or at all. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent positions of biopharmaceutical companies can be highly uncertain and can involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date. In addition, future patent laws, regulations, rules, and court decisions may affect the scope, validity, enforceability, and associated remedies of our current and future patent claims. Accordingly, we cannot predict the breadth of claims that may issue from any patent applications that we own or license, nor are we able to predict whether any third-party patents might issue with claims that are relevant to our product candidates or technologies. Even if patents do successfully issue and even if such patents cover our technologies and product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated or deemed unenforceable. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, the existence of which could invalidate a patent or prevent a patent from issuing from a pending patent application. Furthermore, if third parties have made similar inventions, there are multiple ways they could impact the coverage of our own applications.

We are a party to various license agreements that grant us rights under specified patents and patent applications. We are also party to various license agreements by which we grant third parties rights under specified patents and patent applications. Our current licenses contain performance obligations. If we fail to meet those obligations, the licenses could be terminated. If we are unable to continue to license these technologies on commercially reasonable terms, or at all, we may be forced to delay or terminate aspects of our product development and research activities.

We are unable to exercise the same degree of control over intellectual property that we license from third parties as we exercise over our internally developed intellectual property. We do not control the prosecution of certain of the patent applications that we license from third parties; therefore, the patent applications may not be prosecuted as we desire or in a timely manner.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to conceive and/or reduce to practice the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- the patents of others will not have an adverse effect on our ability to do business;
- others will not independently develop similar or alternative technologies or reverse engineer any of our products, processes or technologies;
- any of our pending patent applications will result in issued patents;
- any patents issued or licensed to us, our collaborators or strategic partners will provide a basis for commercially viable products or will provide us with any competitive advantages;
- any patents issued or licensed to us will not be challenged and invalidated by third parties;
- the laws, regulations, rules, or court decisions in the United States and foreign countries will not change or be interpreted in a way that modifies our patent rights or impacts our ability to enforce or maintain our patent rights; or
- we will develop additional products, processes or technologies that are patentable.

Others have filed and in the future are likely to file patent applications that are similar to ours. We are aware that there are academic groups and other companies that are attempting to develop technology that is based on the use of zinc finger, TALE, CRISPR/Cas and other DNA-binding proteins, engineered integrase and AAV capsid delivery technologies, and that these groups and companies have filed patent applications. Several patents with claims directed to these technologies have issued, although we have no current plans to use the claimed inventions. If these or other patent applications issue as patents, it is possible that the holder of any patent or patents granted on these applications may bring an infringement action against us,



our collaborators, or strategic partners claiming damages and seeking to enjoin research, development or commercial activities relating to the affected products and processes. The costs of litigating the claim could be substantial regardless of outcome. Moreover, we cannot predict whether we, our collaborators, or strategic partners would prevail in any actions. In addition, if the relevant patent claims were upheld as valid and enforceable and our products or processes were found to infringe a patent or patents, we or our collaborators may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, and we may be prevented from making, using, selling, offering to sell, or importing into the United States the relevant product or process unless we or our collaborators could obtain a license or were able to design around the patent claims. We can give no assurance that such a license would be available to us or our collaborators on commercially reasonable terms, or at all, or that we would be able to successfully design around the relevant patent claims. There may be significant litigation in the genomics or cell therapy industry regarding patent and other intellectual property rights, which could subject us to costly, lengthy and distracting litigation with unpredictable results.

We rely on trade secrets to protect technology where we believe patent protection is not appropriate or obtainable. Trade secrets, however, are difficult to protect. While we require employees, academic collaborators and consultants to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary information or enforce these confidentiality agreements.

Our collaborators, strategic partners, and scientific advisors have rights to publish data and information in which we may have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations and strategic partnerships, then we may not be able to receive patent protection or protect our proprietary information.

***Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time and may vary based on jurisdiction.***

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date or from the filing date of the corresponding international application. Various means to extend this expected expiration date may be available. Regardless, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Further, recent judicial decisions in the United States have raised questions regarding the award of patent term adjustment, or PTA, for patents in families where related patents have issued without PTA. Therefore, we cannot be certain how PTA will be viewed in the future and whether our patent expiration dates may be impacted.

***If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be adversely affected, and our business would be harmed.***

We rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, partners and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures have been and may in the future be breached, and we may not have adequate remedies for any breach. See also the risk factor titled "*If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions, litigation, fines and penalties, disruptions of our business operations, reputational harm and other adverse consequences.*" In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, collaborators, partners and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have an adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may



have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could adversely affect our business, results of operations and financial condition.

***We may not be successful in obtaining or maintaining necessary rights to product components, platforms and processes for our development pipeline through acquisitions and in-licenses.***

Presently, we believe we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene therapy and zinc-finger product candidates. Because our programs may involve additional product candidates, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify on commercially reasonable terms, if at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights, including from other companies and academic institutions, that we may consider attractive. Other companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. Once an intellectual property right that we desire is licensed to another company, we may be precluded from obtaining our own license to such rights.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

***If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

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

In many cases, patent prosecution of our in-licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have an adverse effect on our business, financial condition, results of operations and prospects. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on



acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have an adverse effect on our business, financial condition, results of operations and prospects.

***We may be involved in patent or intellectual property lawsuits or similar disputes involving patents under our control or patents of third parties claiming infringement, which lawsuits could be expensive, time-consuming and impair or prevent development and commercialization activities.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, declaratory judgment lawsuits, invalidity proceedings, interferences, oppositions, ex parte or inter partes reexaminations, post-grant reviews and inter partes review proceedings before the U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization, and such parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. For example, we are aware of certain patents held by third parties related to certain vector and vector manufacturing methods that are related to certain of our product candidates. We have not yet finalized the commercial scale manufacturing process for any of our product candidates. If our commercial scale manufacturing process utilizes these vector manufacturing methods, and if these third-party patents are valid and in force at the time of commercialization, we may need to challenge these patents, use or develop non-infringing alternatives or seek a license to these patents. In any event, if any third-party patents were held by a court of competent jurisdiction to cover our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block or hinder our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations or processes for manufacture or methods of use, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license, or until such patents expires. Moreover, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe.

In some instances, third parties may allege that we are infringing their patents or other proprietary rights even if they are not competitors or have an associated business. Such litigants would bring such infringement actions or threats of action with the goal of obtaining settlement money from us instead of engaging in costly and time-consuming litigation.

Defense of these claims, regardless of their merit, would involve substantial litigation expense, could expose proprietary information and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

Competitors may also infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable, in whole or in part, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly and could put our patent applications at risk of not issuing. Moreover, if we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidate. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have an adverse impact on our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the U.S. PTO may be necessary to determine the priority of inventions or other matters of inventorship with respect to our patents or patent



applications or those of our licensors. An unfavorable outcome could expose us to significant monetary damages, result in the loss of valuable intellectual property, require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation, interference, derivation or other proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***We may be unable to license gene transfer technologies that we may need to commercialize our ZF technology and potential products, if approved.***

In order to regulate or modify a gene in a cell, the ZFP must be efficiently delivered to the cell. We have developed our own gene transfer technologies, but may rely on other license agreements to provide us with rights to the necessary gene transfer technology. Our approach has been to license appropriate technology as required. For example, we are aware of certain patents held by third parties related to certain vector manufacturing methods that may be of interest to us. We have not yet finalized the commercial scale manufacturing process for any of our product candidates. If our commercial scale manufacturing process utilizes these vector manufacturing methods, and if these third-party patents are in force at the time of commercialization, we may need to use or develop a non-infringing manufacturing method or seek a license to these patents. However, we may not be able to license the gene transfer technologies on reasonable terms, if at all, required to develop and commercialize our product candidates. The inability to obtain a license to use gene transfer technologies with entities that own such technology on reasonable commercial terms, if at all, could delay or prevent the preclinical evaluation, drug development collaborations, clinical testing and/or commercialization of our therapeutic product candidates.

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

Filing, prosecuting and defending all current and future patents and patent applications in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive or more difficult to enforce than those in 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, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use, make, sell, or import our technologies in or into jurisdictions where we have not 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 our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protections, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights. For example, some foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, some countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit. Further, the complexity and uncertainty of European patent laws have increased in recent years. In Europe, the new unitary patent system that came into effect in June 2023 would significantly impact European patents, including those granted before the introduction of such a system. Under the unitary patent system, European applications will have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the newly formed Unified Patent Court, or UPC. As the UPC is a new court system, there is no precedent or established body of case law on which the court can base its



decisions, thus increasing the uncertainty of any litigation before the UPC. Patents granted before the implementation of the UPC will have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

***We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.***

We, our licensors and collaborators may be subject to claims that former employees, consultants, independent contractors, collaborators or other third parties have an interest in our patents or other intellectual property as an owner, co-owner, inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable or invalid. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co-ownership of potential joint inventions. In addition, while it is our policy to require our employees, consultants, advisors, contractors and other third parties who may be involved in the conception or development of intellectual property rights to execute agreements assigning such intellectual property rights to us, we, our licensors and collaborators may be unsuccessful in executing or perfecting such agreements with each party who, in fact, conceives or develops intellectual property rights that we regard as our own. The assignment of intellectual property rights may not be self-executing or sufficient in scope, or the assignment agreements may be breached. Furthermore, individuals executing agreements with us may have competing obligations to a third party, such as an academic institution, and thus an agreement with us or our licensors may be ineffective in perfecting ownership of inventions developed by that individual. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use or enforce against third parties, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

## **Risks Relating to our Business Operations**

***If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions, litigation, fines and penalties, disruptions of our business operations, reputational harm and other adverse consequences.***

We are increasingly dependent on information technology systems and infrastructure to operate our business, which are large and complex. In the ordinary course of our business, we and the third parties upon which we rely, may collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, share and transmit large amounts of proprietary, confidential and sensitive information, including intellectual property, trade secrets and personal data (such as health-related information). It is critical that we do so in a secure manner to maintain the confidentiality, integrity and availability of such sensitive information. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, we manage a number of third-party vendors who may have access to our computer networks or our confidential information. Many of those third parties in turn subcontract or outsource some of their responsibilities to other third parties. Our ability to monitor third parties' information security practices is limited, and these third parties may not have adequate information security measures in place.

While all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the size, complexity, accessibility and distributed nature of our information technology systems, and the large amounts of sensitive information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks on our technology environment. Attacks of this nature are increasing in their frequency, levels of persistence, sophistication and intensity. Threats to information systems and data are increasingly difficult to detect and come from a variety of sources, including traditional computer "hackers," threat actors, "hacktivists," organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation-states and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including nation-state actors



for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties on which we rely may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems, operations and supply chain. We and the third parties upon which we rely may be subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks (such as credential stuffing), credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, natural disasters (such as earthquakes, fires, floods), war, terrorism, attacks enhanced or facilitated by artificial intelligence, or AI, and other similar threats. Ransomware attacks are becoming increasingly prevalent and severe and can lead to significant interruptions in our operations, loss of data and income, reputational harm and diversions of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. In addition, our updated work from home policies have intensified our dependence on information technology systems and could increase our cybersecurity risk as many of our critical business activities are currently being conducted remotely utilizing network connections, computers and devices outside our premises or network and our increased reliance on personnel working from home, while in transit and in public locations.

While we have implemented security measures designed to protect against data security incidents, there can be no assurance that these measures will be effective. We have not always been able in the past and may be unable in the future to detect vulnerabilities in our information technology systems. We take steps designed to detect, mitigate and remediate vulnerabilities in our information security systems (such as our hardware and/or software, including that of third parties upon which we rely), but we may not be able to detect, mitigate and remediate all such vulnerabilities on a timely basis. For example, in April 2018, we announced a data security incident involving the compromise of a senior executive's company email account. Our investigation of the incident did not reveal any evidence that our systems were otherwise compromised in connection with the incident or that personal data about patients or other individuals besides the executive were accessed or disclosed. However, proprietary, confidential and other sensitive information of ours and that of other entities was accessed and may have been compromised as a result of the incident. Unforeseen developments related to this incident could occur, which could have a further adverse impact on us. Any litigation or regulatory review or investigation arising from this incident could result in significant legal exposure to us. A security incident or other interruption could also result in a material disruption of our development programs and our business operations. 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.

While we have no reason to believe we have experienced any recent security incidents, attackers have become very sophisticated in the way they conceal access to systems, and many companies that have been attacked are not aware that they have been attacked. Any delay in the discovery of an attack may result in increased expense and may harm our reputation. Any security incident or interruption that we, or a third-party upon which we rely, experience could lead to material adverse consequences, including government enforcement actions (for example, investigations, fines, penalties, audits and inspections), additional reporting requirements and/or oversight, restrictions on processing sensitive data (including personal data), litigation (including class claims), indemnification obligations, harm to our reputation, monetary fund diversions, diversion of management attention, interruptions in our operations (including availability of data) and financial loss. Applicable data privacy and security obligations may require us to notify relevant stakeholders, including affected individuals, customers, regulators and investors, of security incidents. Such disclosures are costly, and the disclosures or the failure to comply with such requirements could lead to adverse consequences. In addition, failure to maintain effective internal accounting controls related to security breaches and cybersecurity in general could impact our ability to produce timely and accurate financial statements and subject us to regulatory scrutiny. We may expend significant resources or modify our business activities in an effort to protect against security incidents or other interruptions. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages or claims related to our data privacy and security obligations. While we may be entitled to damages if our third-party partners fail to satisfy their privacy or data security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. Additionally, we cannot be sure that our insurance coverage, if any, will be adequate or sufficient to protect us from or mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms, or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect or infer sensitive information about us from public sources, data brokers or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, our sensitive information could be leaked,



disclosed or revealed as a result of or in connection with our employees', personnel's or vendors' use of generative AI technologies.

***We have business operations in the United Kingdom, which exposes us to additional costs and risks.***

Our business operations in the United Kingdom subject us to certain additional costs and risks associated with doing business outside the United States, including:

- the increased complexity and costs inherent in managing international operations in geographically disparate locations;
- challenges of complying with diverse regulatory, financial and legal requirements, which are subject to change at any time;
- potentially adverse tax consequences, including changes in applicable tax laws and regulations;
- potentially costly trade laws, tariffs, export quotas, custom duties or other trade restrictions, and any changes to them, including escalating trade tensions as a result of actions of the U.S. government;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- liabilities for activities of, or related to, our international operations;
- challenges inherent in efficiently managing employees in diverse geographies, including the need to adapt systems, policies, benefits and compliance programs to differing labor and other regulations;
- natural disasters, political and economic instability, including wars, terrorism and political unrest, including the conflicts between Russia and Ukraine and in the Middle East, outbreak of health epidemics and the resulting global economic and social impacts;
- workforce uncertainty in countries where labor unrest is more common than in the United States; and
- differing laws and regulations relating to data security and the unauthorized use of, or access to, commercial and personal information.

In addition, our international operations in the United Kingdom expose us to fluctuations in currency exchange rates between the Pound Sterling and the U.S. dollar. Given the volatility of currency exchange rates, there is no assurance that we will be able to effectively manage currency transaction and/or conversion risks. To date, we have not entered into derivative instruments to offset the impact of foreign exchange fluctuations, which fluctuations could have an adverse effect on our financial condition and results of operations. In any event, difficulties resulting from these and other risks related to our operations outside of the United States could expose us to increased expenses, impair our development efforts, adversely affect our financial condition and results of operations and harm our competitive position.

***We have experienced and may continue to experience difficulties in hiring, integrating and retaining qualified skilled employees.***

The stability and potential growth of our organization is critical to our ability to successfully achieve our strategic objectives. We may not be able to hire, integrate and retain a sufficient number of qualified employees with the appropriate levels of experience and skills to accomplish our growth objectives.

There currently is a shortage of skilled individuals with substantial experience discovering, developing and manufacturing genomic medicines, which is likely to continue. As a result, competition for these individuals is intense and the turnover rate can be high. We have experienced, and may continue to experience, difficulty hiring, integrating and retaining employees with these skills on acceptable terms given the uncertainty regarding our ability to obtain sufficient additional funding and to continue to operate as a going concern as well as the competition among numerous biopharmaceutical companies and academic institutions for individuals with these skills. In this regard, as a result of the April 2023 and November 2023 Restructurings, approximately 272 roles at our company were eliminated, and as a result of the France Restructuring, 93 roles were eliminated. Accordingly, we have been and are operating with a shortage of resources and may not be able to effectively conduct our operations with our substantially reduced number of employees. In addition, our history of implementing significant workforce reductions, along with the potential for future workforce reductions, may negatively affect our ability to retain or attract talented employees. Moreover, any negative or unexpected results in our preclinical studies or clinical trials or applications for marketing approval would make it more challenging to hire and retain qualified skilled employees. If we do not obtain sufficient additional funding in the near term so that we can continue to operate as a going concern and to potentially achieve our growth objectives, the progress of our research, development, manufacturing and regulatory efforts will slow down or halt altogether, which would materially and adversely affect our business, financial condition, results of operations and prospects, and we may be required to cease operations.



We are dependent on certain key members of our executive team and certain of our scientific, clinical development and manufacturing personnel, the loss of whose services may impede the progress of our research, development and regulatory efforts. For example, in 2024, our former Senior Vice President and Chief People Officer, our former Chief Medical Officer and our former Vice President, Head of Research resigned from their positions. In connection with our restructurings, the employment of each of our former Executive Vice President, Technical Operations, Executive Vice President, Chief Operating Officer, and Senior Vice President, Chief Scientific Officer, was terminated. We could experience resignations of other executives and employees in the future given the uncertainty regarding our ability to obtain sufficient additional funding and to continue to operate as a going concern as well as the intensity of the competition for talent in the biotechnology industry, particularly in the San Francisco Bay Area. Additional resignations or workforce reductions could result in more significant disruptions and threats to our stability and potential growth. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We do not have “key person” insurance on any of our employees.

## **Risks Relating to our Common Stock and Corporate Organization**

***Our stock price has been volatile and will likely continue to be volatile, which could result in substantial losses for investors and potentially class action securities litigation against us, and could be influenced by public perception of genomic medicines and the biotechnology sector.***

Our stock price has been volatile and may continue to be volatile, which could cause stockholders to incur substantial losses. An active public market for our common stock may not be sustained, and the market price of our common stock may continue to be volatile. The market price of our common stock has fluctuated significantly in response to various factors, some of which are beyond our control, including but not limited to the following:

- announcements by us or collaborators providing updates on the progress or development status of product candidates or data from clinical trials;
- initiation or termination of clinical trials;
- changes in market valuations of similar companies;
- overall market and economic conditions, including the equity markets for emerging biotechnology companies;
- deviations in our results of operations from the guidance given by us;
- announcements by us or our competitors of new or enhanced products or technologies or significant contracts, acquisitions, strategic relationships, joint ventures or capital commitments;
- announcement of changes in business and operations by our collaborators, or changes to or terminations of our collaboration agreements;
- changes in public opinions of genomic medicines;
- regulatory developments, including increased regulatory scrutiny of genomic medicines;
- changes by one or more of our securities analysts in recommendations, ratings or coverage of our stock;
- additions or departures of key personnel; and
- sales of our common stock or other securities by us, officers or directors, liquidation of institutional funds that comprised large holdings of our stock and decreases in our cash balances.

In addition, emerging biotechnology stocks have recently experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many biotechnology companies, which has resulted in decreased stock prices for many emerging biotechnology companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. These fluctuations have often been unrelated or disproportionate to the operating performance of those biotechnology companies. Broad market and industry factors, including worsening macroeconomic conditions and other adverse effects or developments relating to current political, geopolitical, regulatory and other market conditions, may negatively affect the market price of shares of our common stock, regardless of our actual operating performance.

Additionally, holders of our stock may seek to bring class action securities litigation claims against us as a result of the volatility in our stock price. If any of our stockholders were to bring a lawsuit of this type against us, even if the lawsuit is without merit, we could incur substantial costs defending the lawsuit and the time and attention of our management could be diverted from other business concerns, either of which could seriously harm our business.



***We have in the past, and may in the future, be unable to comply with the listing standards of the Nasdaq Stock Market LLC, or Nasdaq. If we fail to comply with listing standards in the future, our common stock may be delisted. Delisting could adversely affect the liquidity of our common stock and the market price of our common stock could decrease, and our ability to obtain sufficient additional capital to fund our operations and to continue to operate as a going concern would be substantially impaired.***

Our common stock is currently listed on the Nasdaq Capital Market, which has minimum requirements that a company must meet in order to remain listed. These requirements include maintaining a minimum closing bid price of \$1.00 per share, which closing bid price cannot fall below \$1.00 per share for a period of more than 30 consecutive trading days, or the Bid Price Requirement. We have previously received deficiency notices from the Listing Qualifications Staff, or the Staff, of Nasdaq, notifying us that we have failed to satisfy the Bid Price Requirement. We have in the past been able to regain compliance with the Bid Price Requirement, however closing prices of our common stock on Nasdaq have fluctuated to below \$1.00 more recently and, accordingly, there can be no assurance that we will be able to maintain compliance with the Bid Price Requirement. In the event we fail to comply with listing standards in the future, and we do not regain compliance with the Bid Price Requirement prior to the expiration of the compliance period, unless Nasdaq exercises its discretion to extend this period, our common stock may be subject to a delisting action by Nasdaq.

If we again fail to satisfy the Bid Price Requirement, a reverse stock split may allow us to meet the Bid Price Requirement, but we cannot assure you that a reverse stock split will be approved by our stockholders or that any reverse stock split, if implemented, will be sufficient to enable us to maintain our Nasdaq listing. Additionally, if a reverse stock split is implemented, there can be no assurance that the market price per new share of our common stock following the reverse stock split will remain unchanged or will increase in proportion to the reduction in the number of shares of our common stock outstanding before the reverse stock split. The liquidity of the shares of our common stock may be affected adversely by any reverse stock split given the reduced number of shares of our common stock that will be outstanding following such reverse stock split. Furthermore, following any reverse stock split, the resulting market price of our common stock may not attract new investors and may not satisfy the investing requirements of those investors.

In the event that our common stock is delisted from Nasdaq as a result of our failure to comply with the Bid Price Requirement, as a result of Nasdaq not granting us an extension or the panel not granting us a favorable decision or due to our failure to continue to comply with any other requirement for continued listing on Nasdaq, trading of our common stock could be conducted in the over-the-counter market or on an electronic bulletin board established for unlisted securities such as the Pink Sheets or the OTC Bulletin Board, but there can be no assurance that our common stock will be eligible for trading on such alternative exchange or market.

Additionally, if our common stock is delisted from Nasdaq, the liquidity of our common stock would be adversely affected, the market price of our common stock could decrease, our ability to obtain sufficient additional capital to fund our operations and to continue to operate as a going concern would be substantially impaired and transactions in our common stock could lose federal preemption of state securities laws. Furthermore, there could also be a further reduction in our coverage by securities analysts and the news media and broker-dealers may be deterred from making a market in or otherwise seeking or generating interest in our common stock, which could cause the price of our common stock to decline further. Moreover, delisting may also negatively affect our collaborators', vendors', suppliers' and employees' confidence in us and employee morale.

***Actual or potential sales of significant amounts of shares of our common stock into the market could cause the market price of our common stock to fall or prevent it from increasing for numerous reasons.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Our outstanding shares of common stock generally may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended, or the Securities Act, or to the extent the issuance of such shares has already been registered under the Securities Act and are held by non-affiliates of ours. We have also filed registration statements registering the shares of common stock that we may issue under our equity compensation plans. Such shares can be freely sold in the public market upon issuance, subject to volume limitations and black-out periods applicable to affiliates. Additionally, we are party to a sales agreement with Jefferies LLC which permits us from time to time at our discretion to sell up to \$325.0 million of shares of our common stock in the public markets at prevailing market prices. Approximately \$186.9 million remained available under the sales agreement as of December 31, 2024.

In addition, in accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and our policies regarding stock transactions, certain of our employees, executive officers and directors have adopted, and may continue to adopt, stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public disclosure. Our employees, executive officers, directors and affiliated stockholders also may buy or sell



additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information. Actual or potential sales of our common stock by such persons could be viewed negatively by other investors and could cause the price of our common stock to fall or prevent it from increasing.

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

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

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

The market price of our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. In the event securities or industry analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

***Anti-takeover provisions in our certificate of incorporation, Delaware law and our bylaws could make an acquisition of our company more difficult and could prevent attempts by our stockholders to remove or replace current management.***

Anti-takeover provisions of Delaware law and in our certificate of incorporation and our bylaws may discourage, delay or prevent a change in control of our company, even if a change in control would be beneficial to our stockholders. In addition, 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. In particular, under our certificate of incorporation our board of directors may issue up to 5,000,000 shares of preferred stock with rights and privileges that might be senior to our common stock, without the consent of the holders of the common stock. Moreover, without any further vote or action on the part of the stockholders, the board of directors would have the authority to determine the price, rights, preferences, privileges, and restrictions of the preferred stock. This preferred stock, if it is ever issued, may have preference over, and harm the rights of, the holders of common stock. Although the issuance of this preferred stock would provide us with flexibility in connection with possible acquisitions and other corporate purposes, this issuance may make it more difficult for a third party to acquire a majority of our outstanding voting stock.

Similarly, our authorized but unissued common stock is available for future issuance without stockholder approval. Our certificate of incorporation further provides that stockholders may not take action by written consent.

In addition, our amended and restated bylaws:

- establish advance notice requirements for nominations for election to the board of directors or proposing matters that can be acted upon at stockholders' meetings; and
- prohibit stockholders from calling a special meeting of stockholders.

We are also subject to Section 203 of the General Corporation Law of the State of Delaware, which provides, subject to certain exceptions, that if a person acquires 15% of our voting stock, the person is an "interested stockholder" and may not engage in "business combinations" with us for a period of three years from the time the person acquired 15% or more of our voting stock. The application of Section 203 may, in some circumstances, deter or prevent a change in control of our company even when such change may be beneficial to our stockholders.

***Our amended and restated bylaws designate exclusive forums for the adjudication of certain disputes, which could limit our stockholders' ability to bring claims in a judicial forum it finds favorable for disputes with us or our directors, officers, or employees.***

Our amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware or, if such court does not have subject matter jurisdiction, the federal district court of the State of Delaware, will be the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee or stockholder of Sangamo to us or our stockholders;



- any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware, our charter or our bylaws, as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware; and
- any action asserting a claim governed by the internal affairs doctrine.

Our amended and restated bylaws further provide that a federal district court of the United State is the sole and exclusive forum for any complaint asserting a cause of action arising under the Securities Act of 1933, as amended. These provisions further provide that any person or entity that acquires any interest in shares of our capital stock will be deemed to have notice of and consented to these provisions.

These provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find any of these provisions to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

## **ITEM 1B – UNRESOLVED STAFF COMMENTS**

None.

## **ITEM 1C – CYBERSECURITY**

### **Risk Management and Strategy**

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third-party hosted services, communications systems, hardware and software, and our critical data, including intellectual property, confidential information that is proprietary, strategic or competitive in nature, or Information Systems and Data.

Our cybersecurity risk management program includes a risk assessment methodology designed to escalate cybersecurity risks to the appropriate channels within our organization in order to help identify material cybersecurity risks to our critical systems, information, products, services and our broader enterprise IT environment. The information technology and legal departments help identify, assess and manage Sangamo's cybersecurity threats and risks. The information technology department, in coordination with the legal department, identifies and assesses risks from cybersecurity threats by monitoring and evaluating our threat environment and Sangamo's risk profile using various methods including, for example, evaluating threats reported to us, conducting audits, performing threat assessments, and conducting vulnerability assessments to identify vulnerabilities.

Depending on the environment, we implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data, including, for example: an incident response plan that includes procedures for responding to cybersecurity incidents and escalating cybersecurity incidents to cross-functional teams, management and our board of directors, business continuity plans, encryption of data, network security controls, systems monitoring, employee training, and cybersecurity insurance.

Our assessment and management of material risks from cybersecurity threats are integrated into our risk management protocols. Our cybersecurity risk management program shares common methodologies, reporting channels and governance processes that apply across the enterprise risk management program to other legal, compliance, strategic, operational and financial risk areas, including the involvement of cross-functional teams and, depending on the nature and severity of an incident, an escalation path to notify our executive and senior management teams and our board of directors. For example, the information technology department works with management to prioritize our risk management processes and mitigate cybersecurity threats that are more likely to lead to a material impact on our business.

We use third-party service providers to assist us to identify, assess, and manage material risks from cybersecurity threats, including for example: professional service firms, including legal counsel, cybersecurity software providers and managed cybersecurity service providers.

We use third-party service providers to perform a variety of functions throughout our business, such as application providers, hosting companies, contract research organizations, contract manufacturing organizations and supply chain resources. Depending on the nature of the services provided, the sensitivity of the Information Systems and Data at issue, and the identity of the provider, our vendor management process may involve different levels of assessment designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on the provider.



For a description of the risks from cybersecurity threats that may materially affect us and how they may do so, see our risk factors included in Part I, Item 1A. “Risk Factors” of this Annual Report on Form 10-K, including “Risk Factors—*If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions, litigation, fines and penalties, disruptions of our business operations, reputational harm and other adverse consequences.*”

## **Governance**

Our board of directors addresses Sangamo’s cybersecurity risk management as part of its general oversight function. The board of directors’ audit committee is responsible for overseeing Company’s cybersecurity risk management processes, including oversight of mitigation of risks from cybersecurity threats.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain Company management, including our Associate Director of IT, who has over 25 years of technology experience which includes extensive cybersecurity implementation and oversight.

Our Vice President of Finance & Corporate Controller is responsible on an interim basis for hiring appropriate personnel, helping to integrate cybersecurity risk considerations into our overall risk management strategy, and communicating key priorities to relevant personnel. Our Associate Director of IT is responsible for helping prepare for cybersecurity incidents, approving cybersecurity processes, and reviewing security assessments and other security-related reports. Our Chief Financial Officer is responsible for approving budgets related to the foregoing.

Our cybersecurity incident response and vulnerability management protocol are designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including the executive leadership team. The executive leadership team works with the incident response team to help us mitigate and remediate cybersecurity incidents of which they are notified. In addition, our incident response and vulnerability management protocol include reporting to the Audit Committee of the Board of Directors for certain cybersecurity incidents.

The Audit Committee of our Board of Directors receives periodic reports from the Associate Director of IT concerning Sangamo’s significant cybersecurity threats and risk and the processes we have implemented to address them. The Board of Directors also has access to various reports, summaries or presentations related to cybersecurity threats, risk and mitigation.

## **ITEM 2 – PROPERTIES**

Our corporate headquarters occupies approximately 59,485 square feet of research and office space, pursuant to a lease that expires in August 2031, and we occupy approximately 7,700 of office space, pursuant to a lease that expires in August 2026, in Richmond, California. We also lease approximately 103,089 square feet of office space and research and development laboratory facility in Brisbane, California, pursuant to a lease that expires in May 2029. We believe that our facilities are currently adequate to meet our needs. We expect to close our facility in Brisbane, California in the near future.

## **ITEM 3 – LEGAL PROCEEDINGS**

We are not a party to any material pending legal proceeding. From time to time, we may be involved in legal proceedings arising in the ordinary course of business.

## **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 Capital Market under the symbol “SGMO.”

#### **Holders**

As of March 13, 2025, there were 50 holders of record of our common stock. This number does not include “street name,” or beneficial holders, whose shares are held of record by banks, brokers, financial institutions and other nominees.

#### **Dividends**

We have not paid dividends on our common stock, and currently do not plan to pay any cash dividends in the foreseeable future.

#### **Unregistered Sales of Equity Securities**

None.

#### **Purchases of Equity Securities by the Issuer and Affiliated Purchasers**

None.

### **ITEM 6 – [RESERVED]**

Data responsive to Item 6 have not been presented in accordance with amendments to Item 301 of Regulation S-K contained in SEC Release No. 33-10890.

### **ITEM 7 – MANAGEMENT’S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS**

The discussion in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” contains trend analysis, estimates and other forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act. These forward-looking statements include, without limitation, statements containing the words “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “seeks,” “should,” “will,” and other words of similar import or the negative of those terms or expressions. Such forward-looking statements are subject to known and unknown risks, uncertainties, estimates and other factors that may cause our actual results, performance or achievements, or industry results, to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Actual results could differ materially from those set forth in such forward-looking statements as a result of, but not limited to, the “Risk Factors” described in Part I, Item 1A of this Annual Report on Form 10-K. You should read the following discussion and analysis along with the Consolidated Financial Statements and notes attached to those statements included elsewhere in this report.

In addition, the “Results of Operations” section of this “Management’s Discussion and Analysis of Financial Condition and Results of Operations” generally discusses 2024 and 2023 items and year-to-year comparisons between 2024 and 2023.

#### **Overview**

We are a genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases. We believe our zinc finger epigenetic regulators are ideally suited to potentially address devastating neurology disorders and our capsid engineering platform has demonstrated the ability to expand delivery beyond currently available intrathecal delivery capsids, including in the central nervous system, or CNS, in preclinical studies. For additional information regarding our business, see “Business” in Part I, Item 1 of this Annual Report on Form 10-K.



## Recent Business Highlights

### Corporate Updates

#### *Financial Position – Going Concern*

Based on our current operating plan, which includes our estimates that our cash and cash equivalents as of December 31, 2024, together with \$10.1 million generated through our at-the-market offering program in 2025 and the \$5.0 million payment expected from Pfizer by the end of March 2025, we expect to meet our liquidity requirements only into the middle of the second quarter of 2025. Our history of significant losses, negative cash flows from operations, limited liquidity resources currently on hand and dependence on our ability to obtain additional financing to fund our operations have resulted in management's assessment that there is substantial doubt about our ability to continue as a going concern for at least the next 12 months from the date the financial statements included in this Annual Report are issued. Our ability to continue to operate as a going concern is dependent upon our ability to raise substantial additional capital to fund our operations and support our research and development endeavors, including to progress our preclinical and clinical programs as described in this Annual Report. We need substantial additional capital in order to continue to operate as a going concern and fund our operations. We have been actively seeking, and continue to actively seek, substantial additional capital, including through additional strategic collaborations and other direct investments in our programs, public or private equity or debt financing, royalty financing and other sources. Additional capital may not be available on acceptable terms or at all. In particular, the perception of our ability to continue to operate as a going concern may make it more difficult to obtain financing for the continuation of our operations, particularly in light of currently challenging macroeconomic and market conditions. We may be unable to attract new investments as a result of the speculative nature of our newly reprioritized core neurology preclinical programs and the absence of partners to progress our more advanced clinical programs. In particular, if we are not able to secure a commercialization partner for our Fabry disease program in the near term, our ability to raise additional capital needed to support our operations will be substantially impaired. If adequate funds are not available to us on a timely basis, or at all, we will be required to take additional actions to address our liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering our research and development activities, which would have a material adverse effect on our business and prospects, or we may be required to cease operations entirely, liquidate all or a portion of our assets, and/or seek protection under the U.S. Bankruptcy Code, and you may lose all or part of your investment. We have explored, and will continue to explore, whether filing for bankruptcy protection is in the best interest of our Company and our stakeholders.

### Core Preclinical Neurology Programs and Technology

#### *Chronic Neuropathic Pain – ST-503*

- Since our last update in November 2024, the U.S. Food and Drug Administration, or FDA, cleared the investigational new drug application, or IND, for the ST-503 program, an investigational epigenetic regulator for the treatment of intractable pain due to idiopathic small fiber neuropathy, or iSFN, a type of chronic neuropathic pain.
- We are preparing for the Phase 1/2 clinical study to assess the safety, tolerability and preliminary efficacy of a one-time dose of ST-503, administered intrathecally to patients with intractable pain due to iSFN, and expect to commence the enrollment and dosing of patients in mid-2025, subject to our ability to secure adequate additional funding.
- Preliminary proof of efficacy data from the Phase 1/2 study is expected in the fourth quarter of 2026.

#### *Prion Disease*

- Clinical trial authorisation, or CTA, enabling activities continue to advance for our epigenetic regulation product candidate to treat prion disease, leveraging our novel proprietary neurotropic adeno-associated virus, or AAV, capsid variant, known as STAC-BBB, which demonstrated industry-leading blood-brain barrier, or BBB, penetration in nonhuman primates, or NHPs, following a single intravenous administration.
- Published a manuscript in *bioRxiv* titled, “Zinc Finger Repressors mediate widespread prion depletion from the nonhuman primate brain and profoundly extend survival in prion disease mice” demonstrating nonclinical proof of concept for this approach. A single infusion of Sangamo's zinc finger repressors, or ZFR, significantly reduced expression of prion mRNA and protein in the mouse brain, extended mouse survival and improved an array of molecular, histological, biomarker and behavior readouts – even when administered post-symptomatically to mice with prion disease. In addition, a single intravenous administration of the prion ZFR, delivered via STAC-BBB to NHPs, resulted in potent and widespread repression of prion expression in transduced neurons throughout the brain.
- A CTA submission for the prion program is expected in the first quarter of 2026, subject to our ability to secure adequate additional funding. We plan to begin clinical trial enrollment and dosing in mid-2026, with preliminary clinical data anticipated in the fourth quarter of 2026.



## *Novel AAV Capsid Delivery Technology*

- In December 2024, we and Astellas Gene Therapies, Inc., or Astellas, entered into a license agreement, or the Astellas Agreement, allowing Astellas to leverage our novel AAV capsid, STAC-BBB. The Astellas Agreement grants Astellas a worldwide exclusive license to utilize the STAC-BBB capsid for one target, with the right for Astellas to add up to four additional targets after paying us additional licensed target fees to deliver their intravenously administered genomic medicines to treat certain neurological diseases. We received a \$20.0 million upfront license payment from Astellas and are eligible to earn up to \$1.3 billion in additional licensed target fees and milestone payments across all five potential neurology disease targets, as well as tiered mid-to-high single digit royalties on net sales of any resulting products, subject to certain specified reductions and other conditions.
- We are actively engaged in advanced contract negotiations with a potential collaborator for a third STAC-BBB license agreement for use in delivering intravenously administered genomic medicines for certain specified neurological diseases.

## **Clinical Programs**

### *Fabry Disease*

- On February 6, 2025, we announced updated preliminary clinical data from our Phase 1/2 STAAR study evaluating 33 Fabry patients dosed with isaralgagene civaparvovec, also known as ST-920, via a platform presentation at the 21<sup>st</sup> Annual *WORLD Symposium*. The data showed that as of the September 12, 2024 data cutoff date, sustained benefit was demonstrated with elevated expression of alpha-galactosidase A, or  $\alpha$ -Gal A, activity maintained for nearly four years for the longest treated patient. All 18 patients who began the study on enzyme replacement therapy, or ERT, have been withdrawn from, and remain off, ERT as of March 17, 2025. In the 23 patients followed for 12-months or more after treatment, a positive, statistically significant, mean annualized estimated glomerular filtration rate, or eGFR, slope of 3.061 mL/min/1.73m<sup>2</sup>/year (95% confidence interval: 0.863, 5.258) was observed, indicating notable improvements in renal function. Of the ten patients who had measurable titers of total antibodies, or Abs, or neutralizing antibodies, or Nabs, against  $\alpha$ -Gal A associated with ERT at baseline, total Ab or Nab titers decreased markedly in nine patients and became undetectable in seven, following treatment. Important improvements in disease severity were reported in the Fabry Outcome survey adaptation of the Mainz Severity Score, or FOS-MSSI, and significant improvements were observed in the general health, physical component, bodily pain, physical, vitality, social function, and emotional short-form 36, or SF-36 scores. ST-920 continued to be generally well-tolerated, with no liver function test elevations post-dosing requiring steroids.
- Enrollment and dosing are complete in the Phase 1/2 STAAR study.
- In October 2024, we announced that the FDA had provided a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec using data from the ongoing Phase 1/2 STAAR study, avoiding the requirement for an additional registrational study and accelerating estimated time to potential approval by approximately three years. The FDA agreed in a Type B interaction that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using eGFR slope at 52 weeks across all patients as an intermediate clinical endpoint.
- The 52-week eGFR slope data from all enrolled patients in the Phase 1/2 STAAR study will be available in the first half of 2025 and a potential Biologics License Application, or BLA, submission is anticipated in the second half of 2025.
- We are executing BLA readiness activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

## **Partnered Program**

### *Hemophilia A*

- On December 9, 2024, Pfizer Inc., or Pfizer, presented detailed data from the Phase 3 AFFINE trial of giroctocogene fitelparvovec, an investigational gene therapy that we have been developing with Pfizer for patients with moderately severe to severe hemophilia A, in an oral presentation at the 66<sup>th</sup> American Society for Hematology Annual Meeting and Exposition. The data showed that as of the June 2024 data cut-off date, the AFFINE trial achieved its primary objective of non-inferiority, as well as superiority, of total annualized bleeding rate, or ABR, from Week 12 through at least 15 months of follow up post-infusion compared with routine FVIII replacement prophylaxis treatment. Following a single 3e13 vg/kg dose, giroctocogene fitelparvovec demonstrated a statistically significant reduction in mean total ABR compared to the pre-infusion period. Key secondary endpoints as defined by the trial protocol were met and also



demonstrated superiority compared to prophylaxis. In the AFFINE trial, giroctocogene fitelparvovec was generally well tolerated.

- On December 30, 2024, we reported that we are scheduled to regain development and commercialization rights to giroctocogene fitelparvovec following a decision by Pfizer to terminate the global collaboration and license agreement, or Pfizer Collaboration Agreement, between the parties, effective April 21, 2025, or the Termination Date. Pfizer has indicated to us that this termination reflects its decision not to proceed with the BLA and Marketing Authorization Application, or MAA, submissions for, or to pursue commercialization of, giroctocogene fitelparvovec.
- Under the Pfizer Collaboration Agreement, we were eligible to earn from Pfizer up to \$220.0 million in milestone payments upon the achievement of certain regulatory and commercial milestones for giroctocogene fitelparvovec and product royalties of 14% - 20% if giroctocogene fitelparvovec was approved and commercialized, subject to certain reductions. As of the Termination Date, the Pfizer Collaboration Agreement will be terminated in its entirety, and we will be entitled to receive an exclusive, worldwide, royalty-bearing, sublicensable license from Pfizer to use Pfizer's relevant intellectual property to continue developing, manufacturing and commercializing giroctocogene fitelparvovec. In return, Pfizer would be eligible to receive low single digit royalties on net sales of giroctocogene fitelparvovec. In addition, at our request and expense following the Termination Date, Pfizer is required to transition the SB-525 program back to us and provide certain transition-related services to us. All trial participants will continue to be monitored as planned during the transition period. We continue to explore how to maximize the value of the SB-525 program, including a search for a potential new collaboration partner.

### **Certain Components of Results of Operations**

Our revenues have consisted primarily of revenues from collaboration agreements, including upfront license fees, reimbursements for research services, and milestone achievements, and research grant funding. In 2023, our collaboration agreements with Biogen MA, Inc. and Biogen International GmbH, which we refer to together as Biogen, and Novartis Institutes for BioMedical Research, Inc., or Novartis, were terminated. In April 2024, the collaboration agreement with Kite Pharma, Inc., a Gilead Sciences, Inc. subsidiary, or Kite, expired pursuant to its terms, and in December 2024, Pfizer notified us of its termination for convenience of the Pfizer Collaboration Agreement effective April 21, 2025. In 2024, we entered into revenue-generating partnerships for STAC-BBB with Genentech Inc., a member of the Roche Group, or Genentech, and Astellas. In 2024, we entered into revenue-generating partnerships for STAC-BBB with Genentech and Astellas. We expect revenues to continue to fluctuate from period to period and there can be no assurance that our collaborations or partner reimbursements will continue beyond their initial terms or that we are able to meet the milestones specified in these agreements, or that we will be able to secure additional collaborations. For additional information concerning the terms of our ongoing collaboration agreements, see Note 4 – *Major Customers, Partnerships and Strategic Alliances* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

We have historically incurred net losses since inception and expect to incur losses for at least the next several years as we continue our research and development activities. To date, we have funded our operations primarily through the issuance of equity securities and revenues from collaborations and research grants.

We expect research and development expenses to increase in the near-term due to Fabry disease program BLA readiness activities and we expect to continue to devote substantial resources to research and development in the future and expect research and development expenses to increase in the next several years if we are successful in raising substantial additional capital and advancing our product candidates from research stage through clinical trials.

General and administrative expenses consist primarily of salaries and personnel related expenses for executive, finance and administrative personnel, stock-based compensation expense, professional fees, allocated facilities and information technology expenses, patent prosecution expenses and other general corporate expenses. Although we expect general and administrative expenses to remain consistent in the near term, we expect the growth of our business to require increased general and administrative expenses if we are successful in raising substantial additional capital and advancing our product candidates from research stage through clinical trials.

### **Critical Accounting Policies and Estimates**

Our Consolidated Financial Statements and the related disclosures have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these Consolidated Financial Statements requires us to make estimates, assumptions and judgments that affect the reported amounts in our Consolidated Financial Statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under



different assumptions or conditions. We believe the following policies to be the most critical to an understanding of our financial condition and results of operations because they require us to make estimates, assumptions and judgments about matters that are inherently uncertain.

We believe our critical accounting policies and estimates relating to revenue recognition and valuation of long-lived assets are the most significant estimates and assumptions used in the preparation of our Consolidated Financial Statements.

For a complete description of our significant accounting policies, see Note 1 – *Organization, Basis of Presentation and Summary of Significant Accounting Policies* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

### ***Revenue Recognition***

Our revenues are primarily derived from collaboration agreements, including licensing arrangements and research services. Research and license agreements typically include nonrefundable upfront signing or license fees, payments at negotiated rates for time incurred by our researchers, third-party cost reimbursements, additional target selection fees, sublicense fees, milestone payments tied to ongoing development and product commercialization, and royalties on future licensees’ product sales. All funds received from our collaboration partners are generally not refundable. Non-refundable upfront fees are fixed at the commencement of the contract. All other fees represent variable consideration in contracts. For contracts that contain a provision where we reimburse our customer for certain costs they incur and where we do not acquire any distinct goods or services in exchange for such payments, we account for it as a reduction to the contract transaction price. Deferred revenue primarily represents the portion of nonrefundable upfront fees received but not earned.

For a further description of our revenue recognition, see Note 1 – *Organization, Basis of Presentation and Summary of Significant Accounting Policies* and Note 4 – *Major Customers, Partnerships and Strategic Alliances* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

### ***Valuation of Long-lived Assets***

We evaluate the carrying value of long-lived assets, which include property and equipment, leasehold improvements and right-of-use assets, for impairment whenever events or changes in circumstances indicate that the carrying amounts of the asset may not be fully recoverable. If a change in circumstance occurs that indicates long-lived assets may be impaired, we perform a test of recoverability by comparing the carrying value of the asset or asset group to its undiscounted expected future cash flows. The long-lived asset evaluation is performed at the asset group level, i.e., the lowest level for which identifiable cash flows are largely independent of the cash flows of other assets and liabilities. We reassess the composition of our asset groups whenever there are changes in its operations that affect whether the cash flows associated with assets included in asset groups are largely independent. If the impairment review indicates that the carrying amount of an asset group is not recoverable, an impairment loss is measured as the amount by which the carrying amount of an asset group exceeds its fair value. Any impairment loss is allocated to the long-lived assets of the group on a pro rata basis using the relative carrying amounts of those assets, except that the carrying amount of an individual asset shall not be reduced below its fair value.

Factors that may indicate potential impairment and trigger an impairment test include, but are not limited to, general macroeconomic conditions, conditions specific to the industry and market, an adverse change in legal factors, business climate or operational performance of the business, and sustained decline in the stock price and market capitalization compared to the net book value.

Calculating the fair value of a reporting unit, an asset group and an individual asset involves significant estimates and assumptions. These estimates and assumptions include, among others, projected future cash flows, risk-adjusted discount rates, future economic and market conditions, and the determination of appropriate market comparables. Changes in these factors and assumptions used can materially affect the amount of impairment loss recognized in the period the asset was considered impaired.

For a further description of our valuation of long-lived assets, see Note 1 – *Organization, Basis of Presentation and Summary of Significant Accounting Policies* and Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

As of December 31, 2023, we had fully impaired our goodwill and our indefinite-lived intangible asset. For a further description of the impairment, see Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.



## Recent Accounting Pronouncements

For a summary of recent accounting pronouncements and the anticipated effects on our Consolidated Financial Statements, see Note 1 – *Organization, Basis of Presentation and Summary of Significant Accounting Policies* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

## Results of Operations

### Years Ended December 31, 2024 and 2023

#### Revenues

	Year Ended December 31,			
	(in thousands, except percentage values)			
	2024	2023	Change	%
Revenues	\$ 57,800	\$ 176,232	\$ (118,432)	(67)%

Revenues in 2024 primarily consisted of revenues from the collaboration agreements with Genentech and Astellas, and royalties from our license agreements with Sigma-Aldrich Corporation, or Sigma, and Open Monoclonal Technology, Inc. (now Ligand Pharmaceuticals Inc.), or Ligand. We anticipate revenues in the future will be derived primarily from our license agreements. The terminations of our collaboration agreements with Biogen and Novartis became effective in June 2023, following which we are not entitled to any further milestone payments or royalties from either Biogen or Novartis, nor does either Biogen or Novartis have any further obligations to develop or to reimburse us the costs of any of the programs previously subject to the Biogen and Novartis collaborations. Further, our collaboration agreement with Kite expired pursuant to its terms in April 2024. Moreover, in December 2024, Pfizer notified us of its termination for convenience of the collaboration agreement effective April 21, 2025, and we are not entitled to receive any further milestone payments or royalties from Pfizer.

The decrease of \$118.4 million in revenues in 2024 compared to 2023 was primarily attributed to decreases of \$134.8 million and \$12.2 million in revenues relating to our collaboration agreements with Biogen and Novartis, respectively, due to the termination of such agreements, a decrease of \$20.5 million in revenue relating to our collaboration agreement with Kite due to its expiration, a decrease of \$4.7 million in revenue relating to our license agreements with Sigma and Ligand, and a decrease of \$2.7 million in revenue relating to our other license agreements. These decreases were offset by \$50.0 million in revenue relating to our collaboration agreement with Genentech and \$6.5 million in revenue relating to our collaboration agreement with Astellas.

#### Operating Expenses

	Year Ended December 31,			
	(in thousands, except percentage values)			
	2024	2023	Change	%
Operating expenses:				
Research and development	\$ 111,521	\$ 234,057	\$ (122,536)	(52)%
General and administrative	44,727	61,167	(16,440)	(27)%
Impairment of long-lived assets	5,521	89,485	(83,964)	(94)%
Impairment of goodwill and indefinite-lived intangible assets	—	65,528	(65,528)	(100)%
Total operating expenses	\$ 161,769	\$ 450,237	\$ (288,468)	(64)%

#### Research and Development Expenses

Research and development expenses consisted primarily of compensation related expenses, including restructuring charges and stock-based compensation, laboratory supplies, preclinical and clinical studies, manufacturing clinical supply, contracted research and development, and allocated facilities and information technology expenses.

The decrease of \$122.5 million in research and development expenses in 2024 compared to 2023 was primarily attributable to lower preclinical, clinical and manufacturing expenses of \$52.6 million primarily related to the termination of collaboration agreements with Biogen and Novartis and deferral and reprioritization of certain programs, lower compensation and other personnel costs of \$43.1 million due to lower headcount as a result of restructurings of operations and corresponding reductions in workforce announced during 2023 and restructuring charges, lower allocated overhead costs of \$14.5 million due to changes in the pool of allocable costs as a result of restructuring of operations, and lower facilities and infrastructure related



expenses of \$12.1 million, including depreciation. Stock-based compensation expense included in research and development expenses was \$5.7 million and \$15.2 million for the years ended December 31, 2024 and 2023, respectively.

The table below shows research and development expenses related to our clinical, preclinical and other research and development programs. As shown in the table below, the Fabry clinical program contributed \$19.2 million of the decrease in our research and development expenses in 2024 as compared to 2023, primarily driven by favorable outcome of our interaction with the FDA, which resulted in the decreased spend on the program. Preclinical and research programs decreased by \$47.3 million and other research and development programs decreased by \$56.0 million primarily driven by termination of collaboration agreements with Biogen and Novartis and deferral and reprioritization of certain programs.

Programs	Year Ended December 31,	
	(in thousands)	
	2024	2023
Fabry clinical program	\$ 27,725	\$ 46,938
Wholly owned preclinical programs and early research activities	68,255	115,577
Other research and development programs	15,541	71,542
Total research and development expenses	<u>\$ 111,521</u>	<u>\$ 234,057</u>

We expect research and development expenses to increase in the near-term due to Fabry disease program BLA readiness activities. We expect to continue to devote substantial resources to research and development in the future and expect research and development expenses to increase in the next several years if we are successful in raising substantial additional capital and advancing our product candidates from research stage through clinical trials.

The length of time required to complete our development programs and our development costs for those programs may be impacted by the results of preclinical testing, scope and timing of enrollment in clinical trials for our product candidates, our decisions to pursue development programs in other therapeutic areas, whether we pursue development of our product candidates with a partner or collaborator or independently and our ability to secure the necessary funding to progress the development of our programs. In addition, we are actively seeking commercialization and collaboration partners or a direct external investment, as applicable, to progress our Fabry disease and hemophilia A programs, STAC-BBB capsid and modular integrase platforms. Furthermore, the scope and number of clinical trials required to obtain regulatory approval for each pursued therapeutic area is subject to the input of the applicable regulatory authorities, and we have not yet sought such input for all potential therapeutic areas that we may elect to pursue, and even after having given such input, applicable regulatory authorities may subsequently require additional clinical studies prior to granting regulatory approval based on new data generated by us or other companies, or for other reasons outside of our control. As a condition to any regulatory approval, we may also be subject to post-marketing development commitments, including additional clinical trial requirements. As a result of the uncertainties discussed above, we are unable to determine the duration of or complete costs associated with our development programs.

Our potential therapeutic products are subject to a lengthy and uncertain regulatory process that may not result in our receipt of any necessary regulatory approvals. Failure to receive the necessary regulatory approvals would prevent us from commercializing the product candidates affected. In addition, clinical trials of our product candidates may fail to demonstrate safety and efficacy, which could prevent or significantly delay regulatory approval. A discussion of the risks and uncertainties with respect to our research and development activities, including completing the development of our product candidates, and the consequences to our business, financial position and growth prospects can be found in “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K.

#### *General and Administrative Expenses*

General and administrative expenses consist primarily of compensation related expenses including restructuring charges and stock-based compensation for executive, legal, finance and administrative personnel, professional fees, allocated facilities and information technology expenses, and other general corporate expenses.

The decrease of \$16.4 million in general and administrative expenses in 2024 compared to 2023 was primarily attributable to lower compensation and other personnel costs of \$12.1 million due to lower headcount as a result of restructurings of operations and corresponding reductions in workforce announced during 2023 and restructuring charges, lower facilities and infrastructure related costs of \$8.1 million, lower external professional services expenses of \$8.0 million, and Biogen contract cost asset amortization of \$2.6 million recorded in 2023 due to the termination of the collaboration agreement. These decreases were partially offset by higher allocated overhead costs of \$14.5 million due to changes in the pool of allocable costs as a result of restructuring of operations. Stock-based compensation expense included in general and administrative expenses was \$6.7 million and \$12.2 million for the years ended December 31, 2024 and 2023, respectively.



### *Restructuring Charges*

In 2023, we executed a series of restructurings of operations and corresponding reductions in workforce announced in April and November 2023. In 2024, we executed a wind-down of our French research and development activities and a corresponding workforce reduction. These restructurings were designed to reduce overall costs and advance our strategic transformation into a neurology focused genomic medicine company focused on epigenetic regulation programs addressing serious neurological diseases and novel AAV capsid delivery technology. No expenses related to the April 2023 Restructuring were incurred during the year ended December 31, 2024. In connection with the November 2023 Restructuring and France Restructuring, the expenses incurred and adjustments recorded during the year ended December 31, 2024 related to employee severance and notice period payments, benefits, and other employee-related costs were not material.

For more information see Note 10 – *Restructuring Charges* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

### *Impairment*

During the year ended December 31, 2024, we recognized impairment charges of \$5.5 million. During the year, our Board of Directors approved the France Restructuring, we initiated several actions aimed at reducing costs, including activities related to the closure of our facility in Brisbane, California, and we faced a decline in our stock price and related market capitalization. There was also a decline in the market rates for facility subleases, indicating the carrying values of right of use and leasehold improvement assets could be impaired. As a result of these factors, we concluded certain long-lived assets, primarily comprising right-of-use assets, related leasehold improvements, and certain manufacturing and laboratory equipment, were impaired.

For more information see Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

### *Interest and other income, net*

Interest and other income, net was \$5.9 million and \$11.1 million for the years ended December 31, 2024 and 2023, respectively. The decrease of \$5.2 million in 2024 compared to 2023 was primarily driven by a decrease of \$5.2 million in interest income due to a decrease in marketable securities and a decrease of \$3.4 million in research tax credits, partially offset by \$2.9 million related to fluctuations in foreign currency exchange rates and \$0.6 million from gain on sale of investments.

### *Income tax benefit*

The income tax benefit was \$0.2 million and \$5.1 million for the years ended December 31, 2024 and 2023, respectively. The income tax benefit for the year ended December 31, 2024 was due to the reversal of the Company’s United Kingdom subsidiary’s long-term payable as a result of the statute of limitations lapsing. The income tax benefit for the year ended December 31, 2023 was primarily driven by the reduction of the foreign deferred tax liability due to the impairment of the indefinite-lived intangible asset and was offset by the setup of the valuation allowance for the Company’s United Kingdom subsidiary.

Beginning in 2022, the 2017 Tax Cuts and Jobs Act amended Section 174 to eliminate current-year deductibility of research and experimentation, or R&E, expenditures and software development costs, collectively, R&E expenditures, and instead require taxpayers to charge their R&E expenditures to a capital account amortized over five years (15 years for expenditures attributable R&E activity performed outside the United States). We generated a deferred tax asset for capitalized R&E expenditures for the years ended December 31, 2024 and 2023, which is fully offset with a valuation allowance.

As of December 31, 2024, we had net operating loss carryforwards for federal and state income tax purposes of approximately \$818.5 million and \$349.8 million, respectively. The federal net operating loss generated before 2018 will begin to expire in 2025 and will keep expiring through 2037, if not utilized. Federal net operating losses generated from 2018 will carry forward indefinitely. If not utilized, the state net operating loss carryforwards will begin to expire in 2029. The Company’s French net operating loss carryforward balance is \$120.4 million, which carries over indefinitely. We also have federal and state research tax credit carryforwards of \$50.0 million and \$33.2 million, respectively. The federal research credits will begin to expire in 2025, while the state research credits have no expiration date. Utilization of our net operating loss carryforwards and research tax credit carryforwards may be subject to substantial annual limitations due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. The annual limitation could result in the expiration of the net operating loss carryforwards and research tax credit carryforwards before use. Due to the carryforwards related to the net operating losses and research and development tax credits, we do not expect to pay any U.S. federal taxes related to income in the near future.



## Liquidity and Capital Resources

### *Liquidity*

Since inception, we have incurred significant net losses, and we have funded our operations primarily through the issuance of equity securities, payments from corporate collaborators and strategic partners and research grants.

As of December 31, 2024, we had cash and cash equivalents of \$41.9 million compared to cash, cash equivalents and marketable securities of \$81.0 million as of December 31, 2023. Our most significant use of capital during the year was for external research and development expenses, such as manufacturing, clinical trials and preclinical activity related to our therapeutic programs, and employee compensation. Cash in excess of immediate requirements is invested in accordance with our investment policy with a view toward capital preservation and liquidity.

In August 2020, we entered into an Open Market Sale Agreement<sup>SM</sup>, or the sales agreement, with Jefferies LLC, providing for the sale of up to \$150.0 million of our common stock from time to time in “at-the-market” offerings under an existing shelf registration statement. In December 2022, we entered into Amendment No. 2 to the Open Market Sale Agreement<sup>SM</sup>, which increased the aggregate offering price under the sales agreement by an additional \$175.0 million. Approximately \$186.9 million remained available under the sales agreement as of December 31, 2024. We sold 3,638,740 shares of our common stock for net proceeds of approximately \$7.1 million during the year ended December 31, 2024 and 8,249,261 shares of our common stock for net proceeds of approximately \$15.1 million during the year ended December 31, 2023. Additionally, in March 2024, we issued 24,761,905 shares of common stock, pre-funded warrants to purchase 3,809,523 shares of common stock and accompanying warrants to purchase an aggregate of 28,571,428 shares of common stock at a price per share of common stock (or pre-funded warrant in lieu thereof) and accompanying warrant of \$0.84 per share, for total net proceeds of approximately \$21.9 million, after deducting placement agent fees and offering expenses.

Under Accounting Standard Codification Topic 205-40, *Presentation of Financial Statements—Going Concern*, or ASC Topic 205-40, we have the responsibility to evaluate whether conditions and/or events raise substantial doubt about our ability to meet our future financial obligations as they become due within one year after the date that the Consolidated Financial Statements included in this Annual Report on Form 10-K are issued. As required under ASC Topic 205-40, management’s evaluation should initially not take into consideration the potential mitigating effects of management’s plans that have not been fully implemented as of the date the Consolidated Financial Statements are issued. When substantial doubt exists, management evaluates whether the mitigating effects of its plans sufficiently alleviate the substantial doubt about the company’s ability to continue as a going concern. The mitigating effects of management’s plans, however, are only considered if both (i) it is probable that the plans will be effectively implemented within one year after the date that the financial statements are issued, and (ii) it is probable that the plans, when implemented, will mitigate the relevant conditions or events that raise substantial doubt about the entity’s ability to continue as a going concern within one year after the date that the financial statements are issued. Generally, to be considered probable of being effectively implemented, the plans must have been approved by the company’s board of directors before the date that the financial statements are issued.

Based on our current operating plan, which includes our estimates that our cash and cash equivalents as of December 31, 2024, together with \$10.1 million generated through our at-the-market offering program in 2025 and the \$5.0 million payment expected from Pfizer by the end of March 2025, we expect to meet our liquidity requirements only into the middle of the second quarter of 2025. Our history of significant losses, negative cash flows from operations, limited liquidity resources currently on hand and dependence on our ability to obtain additional financing to fund our operations have resulted in management’s assessment that there is substantial doubt about our ability to continue as a going concern for at least the next 12 months from the date the financial statements included in this Annual Report are issued. Our ability to continue to operate as a going concern is dependent upon our ability to raise substantial additional capital to fund our operations and support our research and development endeavors, including to progress our preclinical and clinical programs as described in this Annual Report. We need substantial additional capital in order to continue to operate as a going concern and fund our operations. We have been actively seeking, and continue to actively seek, substantial additional capital, including through additional strategic collaborations and other direct investments in our programs, public or private equity or debt financing, royalty financing and other sources. Additional capital may not be available on acceptable terms or at all. In particular, the perception of our ability to continue to operate as a going concern may make it more difficult to obtain financing for the continuation of our operations, particularly in light of currently challenging macroeconomic and market conditions. Further, we may be unable to attract new investments as a result of the speculative nature of our newly reprioritized core neurology preclinical programs and the absence of partners to progress our more advanced clinical programs. In particular, if we are not able to secure a commercialization partner for our Fabry disease program in the near term, our ability to raise additional capital needed to support our operations will be substantially impaired. If adequate funds are not available to us on a timely basis, or at all, we will be required to take additional actions to address our liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering our research and development activities, which would have a material adverse effect on our business and prospects, or we may be required to



cease operations entirely, liquidate all or a portion of our assets, and/or seek protection under the U.S. Bankruptcy Code, and you may lose all or part of your investment. We have explored, and will continue to explore, whether filing for bankruptcy protection is in the best interest of our Company and our stakeholders.

Moreover, we rely in part on our collaboration partners to provide funding for and otherwise advance our preclinical and clinical programs. While we continue to advance ongoing business development discussions with potential commercialization and collaboration partners, we may not be successful in doing so in a timely manner, on acceptable terms or at all, and we may otherwise fail to raise sufficient additional capital to advance our programs, in which case, we may not receive the expected return on our investments in these programs, platforms and technologies. In any event, we need substantial additional funding in order to execute on our current operating plan, and our ability to raise such funding will be substantially impaired if we are not able to secure a commercialization partner for our Fabry disease program in the near term. If we raise additional capital through public or private equity offerings, including sales pursuant to our at-the-market offering program with Jefferies LLC, the ownership interest of our existing stockholders will be diluted, and such dilution may be substantial given our current stock price decline, and the terms of any new equity securities may have a preference over, and include rights superior to, our common stock. If we raise additional capital through royalty financings or other collaborations, strategic alliances or licensing arrangements with third parties, we may need to relinquish certain valuable rights to our product candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable. If we raise additional capital through debt financing, we may be subject to specified financial covenants or covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or pursuing certain transactions, any of which could restrict our ability to commercialize our product candidates or operate as a business.

In addition, as we focus our efforts on proprietary human therapeutics, we will need to seek regulatory approvals of our product candidates from the FDA or other comparable foreign regulatory authorities, a process that could cost in excess of hundreds of millions of dollars per product. We may experience difficulties in accessing the capital markets due to external factors beyond our control, such as volatility in the equity markets for emerging biotechnology companies and general economic and market conditions both in the United States and abroad. In particular, our ability to raise the substantial additional capital we need in order to fund our business may be adversely impacted by global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide, such as has been experienced recently due in part to, among other things, the ongoing conflict between Russia and Ukraine, conflicts in the Middle East, and geopolitical challenges arising from the imposition of tariffs and escalating trade tensions. We cannot be certain that we will be able to obtain financing on terms acceptable to us, or at all.

## **Cash Flows**

### *Operating activities*

Net cash used in operating activities was \$67.1 million in 2024, primarily due to:

- a net loss of \$97.9 million, adjusted for non-cash goodwill, indefinite-lived intangible assets, and long-lived asset impairment charges of \$5.5 million, other non-cash expenses related to stock-based compensation of \$12.4 million, depreciation and amortization of \$5.1 million, amortization of operating lease right-of-use assets of \$4.3 million, and other non-cash adjustments of \$0.3 million, offset by gain on sale of assets classified as held for sale of \$1.0 million, and accretion of discounts and impairment of marketable securities of \$0.3 million; and
- a decrease in accounts payable and other accrued liabilities of \$14.2 million, a decrease in lease liabilities of \$5.6 million, and a decrease in other non-current liabilities of \$0.3 million. These were partially offset by an increase in deferred revenues of \$13.4 million, an increase in accrued compensation and employee benefits of \$5.7 million, a decrease in prepaid expenses and other assets of \$4.4 million, a decrease in refundable research income tax credits of \$0.6 million, and a decrease in accounts receivable of \$0.4 million.

Net cash used in operating activities was \$224.8 million in 2023, primarily due to:

- a net loss of \$257.8 million, adjusted for non-cash goodwill, indefinite-lived intangible assets, and long-lived asset impairment charges of \$155.0 million, other non-cash expenses related to stock-based compensation of \$27.4 million, depreciation and amortization of \$15.1 million, amortization of operating lease right-of-use assets of \$7.1 million, and other non-cash adjustments of \$1.1 million, offset by income tax benefit of \$6.3 million related to reversal of the deferred tax liability as a result of impairment of the associated indefinite-lived intangible assets, accretion of discounts and impairment of marketable securities of \$2.3 million; and
- a decrease in deferred revenues of \$161.2 million, mainly attributed to the impact of the termination and related contract modification of our collaboration agreement with Biogen and changes in estimate for our collaboration agreement with Kite, a decrease in accrued compensation and employee benefits of \$12.7 million, a decrease in lease liabilities of \$5.0 million, and an increase in refundable research income tax credits of \$3.7 million. These



were partially offset by a decrease in prepaid expenses and other assets of \$10.3 million, an increase in accounts payable and other accrued liabilities of \$5.6 million, and a decrease in accounts receivable of \$2.8 million.

#### *Investing activities*

Net cash provided by investing activities was \$37.5 million in 2024, primarily related to sales of marketable securities of \$34.7 million, proceeds from sale of assets classified as held for sale of \$2.0 million, and maturities of marketable securities of \$1.1 million, partially offset by purchases of property and equipment of \$0.3 million.

Net cash provided by investing activities was \$153.5 million in 2023, primarily related to maturities of marketable securities of \$214.5 million and sales of marketable securities of \$19.7 million, partially offset by purchases of marketable securities of \$59.6 million and purchases of property and equipment of \$21.2 million.

#### *Financing activities*

Net cash provided by financing activities was \$28.4 million in 2024, primarily related to \$21.9 million of proceeds from issuance of common stock, net of offering expenses of \$2.1 million, \$7.1 million of proceeds from the at-the-market offering, net of offering expenses of \$0.4 million, and proceeds from purchases of common stock under employee stock purchase plan of \$0.3 million, partially offset by taxes paid related to net share settlement of equity awards of \$0.9 million.

Net cash provided by financing activities was \$14.6 million in 2023, primarily related to \$15.1 million of proceeds from the at-the-market offering, net of offering expenses of \$0.4 million, and proceeds from purchases of common stock under the employee stock purchase plan of \$0.9 million, offset by a decrease of \$1.5 million for taxes paid related to net share settlement of equity awards.

#### ***Operating Capital and Capital Expenditure Requirements***

We anticipate continuing to incur operating losses for at least the next several years and need to raise substantial additional capital. The effects of the current macroeconomic environment, including the effects of war in Ukraine and conflicts in the Middle East, geopolitical challenges resulting from the imposition of tariffs and escalating trade tensions, inflation, climate change, fluctuations in interest rates and other economic uncertainty and volatility, has resulted and may continue to result in significant disruption of global financial markets, which could impair our ability to access capital on terms that are acceptable or at all, and in turn could negatively affect our liquidity and our ability to continue to operate as a going concern. Future capital requirements beyond the period into which we expect our existing cash and cash equivalents will be sufficient to fund our planned operations, will be substantial, and we need to raise substantial additional capital to continue to operate as a going concern and to fund the development, manufacturing and potential commercialization of our product candidates (see “*Financial Position—Going Concern*” and “*Liquidity and Capital Resources—Liquidity*” above).

As we focus our efforts on proprietary human therapeutics, we will need to seek FDA approvals of our product candidates, a process that could cost in excess of hundreds of millions of dollars per product. Our future capital requirements will depend on many forward-looking factors, including the following:

- the results of preclinical testing of our early-stage core neurology program product candidates;
- the initiation, progress, timing and completion of clinical trials for our product candidates and potential product candidates;
- the outcome, timing and cost of regulatory approvals;
- the success of our existing collaboration agreements and our ability to secure additional collaborations;
- delays that may be caused by changing regulatory requirements;
- the number of product candidates that we pursue;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the timing and terms of future in-licensing and out-licensing transactions;
- the cost and timing of establishing sales, marketing, manufacturing and distribution capabilities;
- the cost of procuring clinical and commercial supplies of our product candidates;
- the extent to which we acquire or invest in businesses, products or technologies, including the costs associated with such acquisitions and investments; and
- the costs of potential disputes and litigation.



### ***Contractual Obligations***

Our contractual obligations as of December 31, 2024 relate primarily to (i) operating leases consisting of base rents for facilities we occupy in Brisbane, California and Richmond, California, (ii) purchase obligations related to manufacturing, facilities, and equipment, and (iii) license obligations for ongoing license maintenance fees associated with cancellable in-licensed patent agreements. These agreements are enforceable and legally binding and specify all significant terms, including fixed or minimum services to be used, fixed, minimum or variable price, and the approximate timing of the actions under the contracts. For more information regarding our contractual obligations and commitments as of December 31, 2024, see Note 7 – *Commitments and Contingencies* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K.

### **ITEM 7A – QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information otherwise required under this item.



**ITEM 8 – FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA**

**SANGAMO THERAPEUTICS, INC.**

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## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Sangamo Therapeutics, Inc.

### Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Sangamo Therapeutics, Inc. (the Company) as of December 31, 2024 and 2023, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles.

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, 2024, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated March 17, 2025 expressed an unqualified opinion thereon.

### The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has suffered recurring losses from operations and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

### Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

### Critical Audit Matters

The critical audit matters communicated below are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of critical audit matters 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 matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.



### ***Impairment of long-lived assets and assets held for sale***

#### ***Description of the Matter***

As discussed in Note 1 of the consolidated financial statements, the Company reviews its long-lived assets, including right-of-use assets (“RoU”), property and equipment and assets held for sale, for impairment, whenever events or changes in circumstances indicate that the carrying amounts of the assets may not be fully recoverable. The Company compares expected future cash flows of each asset group to the carrying value of those assets. If the expected future cash flows (undiscounted) are less than the carrying amount of such assets, the Company recognizes an impairment loss for the difference between the carrying value of the asset group and its estimated fair value, which is allocated to assets of the group on a pro rata basis using the relative carrying amounts of those assets, with the carrying amount of an individual asset not being reduced below its fair value. During 2024, the Company determined its long-lived assets comprised two asset groups for its recoverability test. During the year ended December 31, 2024, the Company recorded impairment of \$5.5 million, consisting of \$2.9 million related to right-of-use assets, \$0.6 million related to leasehold improvements and \$2.0 million related to assets held for sale.

Auditing the Company’s impairment of long-lived assets and assets held for sale was complex and required a high degree of auditor judgment when performing procedures due to the significant estimation uncertainty in determining the fair value of long-lived assets and assets held for sale. Significant judgments made by management related to valuation of right-of-use assets included determining the discount rate, the length of time to enter into a sublease and market rental rates. Management also made significant judgments in estimating market-based pricing for machinery and equipment held for sale.

#### ***How We Addressed the Matter in Our Audit***

We obtained an understanding, evaluated the design and tested the operating effectiveness of controls over management’s evaluation of impairment of long-lived assets and assets held for sale. For example, we tested management’s controls over the determination of the discount rate, the length of time to enter into a sublease, market rental rates and other key assumptions used in the determination of the fair value of long-lived assets and assets held for sale.

Our audit procedures included, among others, evaluating the methodology and valuation models used and testing the key inputs and significant assumptions discussed above. We evaluated the significant assumptions described above comparing them to external market data for right-of-use assets and comparable executed transactions for machinery and equipment held for sale. Our procedures included evaluating the data sources used by management in determining its significant assumptions and included an evaluation of available information that either corroborated or contradicted management’s conclusions. We involved our valuation professionals to assess the methodology and valuation of long-lived assets including right-of-use assets, property and equipment and assets held for sale, including evaluating the reasonableness of certain significant assumptions.



### *Accounting for revenue from collaboration agreements*

*Description of  
the Matter*

The Company recorded \$56.5 million in revenue from collaboration agreements for the year ended December 31, 2024. As discussed in Note 1 of the consolidated financial statements, terms of the Company's collaboration agreements include a license for the Company's technology or programs and research and development services. Amounts received under such arrangements include nonrefundable upfront payments, milestone and other contingent payments for the achievement of defined collaboration objectives and certain preclinical, clinical, regulatory and sales-based events, as well as royalties on sales of any commercialized products. Revenues are recognized at a point in time, in the case of the license, upon satisfying the relevant performance obligations, or over time, and in the case of research and development services, by measuring progress towards satisfaction of the relevant performance obligation, using an input method (i.e., cumulative actual costs incurred relative to total estimated costs).

Auditing the Company's accounting for revenue from the collaboration agreements was complex and required significant judgments primarily in identifying which elements represent distinct performance obligations, determining the standalone selling price of the identified performance obligations, measurement and allocation of arrangement consideration and evaluating estimates of the total expected inputs under the input method for revenue recognized over time.

*How We  
Addressed the  
Matter in Our  
Audit*

We obtained an understanding, evaluated the design and tested the operating effectiveness of controls over management's evaluation of revenue recognition process. For example, we tested management's controls over review of new revenue agreements, the determination of the standalone selling price of the identified performance obligations as well as the Company's estimates of the total costs required to complete each performance obligation and its periodic reassessment of the estimates of total costs expected to complete the performance obligations.

Our audit procedures included, among others, evaluating whether the identified performance obligations were properly determined, and the transaction price was properly measured and allocated to the identified performance obligations. To test the measurement of efforts toward satisfying the performance obligation, our audit procedures included, among others, testing of cash receipts, reviewing management's analysis for accuracy and completeness by agreeing data to the underlying contract, inspecting research or steering committee minutes, testing the application of the input method for the recognition of revenue, including testing the estimated total inputs and actual inputs incurred.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 1997.

San Mateo, California

March 17, 2025



**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED BALANCE SHEETS**  
(in thousands, except share and per share amounts)

	December 31,	
	2024	2023
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 41,918	\$ 45,204
Marketable securities	—	35,798
Accounts receivable	526	923
Refundable research income tax credits	4,072	3,144
Prepaid expenses and other current assets	5,175	9,259
Total current assets	51,691	94,328
Property and equipment, net	17,887	26,874
Operating lease right-of-use assets	16,869	25,991
Refundable research income tax credits, non-current	12,809	15,362
Other non-current assets	879	1,265
Restricted cash	1,500	1,500
Total assets	<u>\$ 101,635</u>	<u>\$ 165,320</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 15,485	\$ 15,259
Accrued compensation and employee benefits	14,569	8,918
Other accrued liabilities	8,195	23,554
Deferred revenues	7,556	—
Total current liabilities	45,805	47,731
Deferred revenues, non-current	5,874	—
Long-term portion of lease liabilities	26,253	33,515
Other non-current liabilities	933	1,187
Total liabilities	78,865	82,433
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.01 par value, 5,000,000 shares authorized, and no shares issued or outstanding	—	—
Common stock, \$0.01 par value; 960,000,000 shares authorized; 212,837,679 and 178,133,548 shares issued and outstanding at December 31, 2024 and 2023, respectively	2,128	1,781
Additional paid-in capital	1,532,489	1,492,077
Accumulated deficit	(1,504,317)	(1,406,376)
Accumulated other comprehensive loss	(7,530)	(4,595)
Total stockholders' equity	22,770	82,887
Total liabilities and stockholders' equity	<u>\$ 101,635</u>	<u>\$ 165,320</u>

See accompanying Notes to Consolidated Financial Statements.



**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS**  
(in thousands, except per share amounts)

	<b>Year Ended December 31,</b>	
	<b>2024</b>	<b>2023</b>
Revenues	\$ 57,800	\$ 176,232
Operating expenses:		
Research and development	111,521	234,057
General and administrative	44,727	61,167
Impairment of long-lived assets	5,521	65,528
Impairment of goodwill and indefinite-lived intangible assets	—	89,485
Total operating expenses	161,769	450,237
Loss from operations	(103,969)	(274,005)
Interest and other income, net	5,861	11,102
Loss before income taxes	(98,108)	(262,903)
Income tax benefit	(167)	(5,072)
Net loss	\$ (97,941)	\$ (257,831)
Basic and diluted net loss per share	\$ (0.49)	\$ (1.48)
Shares used in computing basic and diluted net loss per share	201,699	174,444

See accompanying Notes to Consolidated Financial Statements.



**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**  
(in thousands)

	Year Ended December 31,	
	2024	2023
Net loss	\$ (97,941)	\$ (257,831)
Foreign currency translation adjustment	(2,943)	2,848
Net pension gain (loss)	241	(163)
Unrealized (loss) gain on marketable securities, net of tax	(233)	1,124
Comprehensive loss	<u>\$ (100,876)</u>	<u>\$ (254,022)</u>

See accompanying Notes to Consolidated Financial Statements.



**SANGAMO THERAPEUTICS, INC.**

**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**

(in thousands, except share amounts)

	Common Stock		Additional	Accumulated	Accumulated	Total
	Shares	Amount	Paid-in	Deficit	Other Comprehensive Loss	Stockholders' Equity
Balances at December 31, 2022	166,793,320	\$ 1,668	\$ 1,450,239	\$ (1,148,545)	\$ (8,404)	\$ 294,958
Issuance of common stock in at-the-market offering, net of offering expenses	8,249,261	82	15,024	—	—	15,106
Issuance of common stock upon exercise of stock options and vesting of restricted stock units, net of tax	1,744,118	18	(1,471)	—	—	(1,453)
Issuance of common stock under employee stock purchase plan	1,346,849	13	922	—	—	935
Stock-based compensation	—	—	27,363	—	—	27,363
Foreign currency translation adjustment	—	—	—	—	2,848	2,848
Net pension losses	—	—	—	—	(163)	(163)
Net unrealized gain on marketable securities, net of tax	—	—	—	—	1,124	1,124
Net loss	—	—	—	(257,831)	—	(257,831)
Balances at December 31, 2023	178,133,548	1,781	1,492,077	(1,406,376)	(4,595)	82,887
Issuance of common stock, net of offering expenses	24,761,905	248	21,540	—	—	21,788
Issuance of common stock upon exercise of pre-funded warrants	3,809,523	38	33	—	—	71
Issuance of common stock in at-the-market offering, net of offering expenses	3,638,740	36	7,096	—	—	7,132
Issuance of common stock upon exercise of stock options and vesting of restricted stock units, net of tax	1,746,271	17	(921)	—	—	(904)
Issuance of common stock under employee stock purchase plan	747,692	8	282	—	—	290
Stock-based compensation	—	—	12,382	—	—	12,382
Foreign currency translation adjustment	—	—	—	—	(2,943)	(2,943)
Net pension gains	—	—	—	—	241	241
Net unrealized loss on marketable securities, net of tax	—	—	—	—	(233)	(233)
Net loss	—	—	—	(97,941)	—	(97,941)
Balances at December 31, 2024	<u>212,837,679</u>	<u>\$ 2,128</u>	<u>\$ 1,532,489</u>	<u>\$ (1,504,317)</u>	<u>\$ (7,530)</u>	<u>\$ 22,770</u>

See accompanying Notes to Consolidated Financial Statements.



**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
(in thousands)

	<b>Year Ended December 31,</b>	
	<b>2024</b>	<b>2023</b>
<b>Operating Activities:</b>		
Net loss	\$ (97,941)	\$ (257,831)
Adjustments to reconcile net loss to net cash used in operating activities:		
Impairment of long-lived assets	5,521	65,528
Depreciation and amortization	5,107	15,065
Accretion of discount and impairment on marketable securities	(273)	(2,329)
Amortization in operating lease right-of-use assets	4,254	7,127
Stock-based compensation	12,382	27,363
Gain on sale of assets classified as held for sale	(1,026)	—
Other	330	1,119
Impairment of goodwill and indefinite-lived intangible assets	—	89,485
Deferred income tax benefit	—	(6,335)
Net changes in operating assets and liabilities:		
Accounts receivable	397	2,755
Refundable research income tax credits	556	(3,731)
Prepaid expenses and other assets	4,384	10,256
Accounts payable and other accrued liabilities	(14,169)	5,589
Accrued compensation and employee benefits	5,743	(12,690)
Deferred revenues	13,430	(161,156)
Lease liabilities	(5,579)	(5,037)
Other non-current liabilities	(255)	(20)
Net cash used in operating activities	(67,139)	(224,842)
<b>Investing Activities:</b>		
Purchases of marketable securities	—	(59,551)
Maturities of marketable securities	1,110	214,500
Proceeds from sale of marketable securities	34,730	19,737
Proceeds from sale of assets classified as held for sale	1,951	—
Purchases of property and equipment	(267)	(21,155)
Net cash provided by investing activities	37,524	153,531
<b>Financing Activities:</b>		
Proceeds from at-the-market offering, net of offering expenses	7,132	15,106
Proceeds from issuance of common stock, net of offering expenses	21,859	—
Taxes paid related to net share settlement of equity awards	(904)	(1,453)
Proceeds from issuance of common stock under employee stock purchase plan	290	935
Net cash provided by financing activities	28,377	14,588
Effect of exchange rate changes on cash and cash equivalents and restricted cash	(2,048)	1,483
Net decrease in cash, cash equivalents and restricted cash	(3,286)	(55,240)
Cash, cash equivalents and restricted cash, beginning of period	46,704	101,944
<b>Cash, cash equivalents and restricted cash, end of period</b>	<b>\$ 43,418</b>	<b>\$ 46,704</b>
<b>Supplemental cash flow disclosures:</b>		
Property and equipment included in unpaid liabilities	\$ 236	\$ 447

See accompanying Notes to Consolidated Financial Statements



## SANGAMO THERAPEUTICS, INC.

### NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

#### NOTE 1 – ORGANIZATION, BASIS OF PRESENTATION AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

##### Organization and Description of Business

Sangamo Therapeutics, Inc. (“Sangamo” or “the Company”) was incorporated in the State of Delaware in June 1995 and changed its name from Sangamo Biosciences, Inc. in January 2017. Sangamo is a genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases. The Company believes its zinc finger (“ZF”) epigenetic regulators are ideally suited to potentially address devastating neurology disorders and its capsid engineering platform has demonstrated the ability to expand delivery beyond currently available intrathecal delivery capsids, including in the central nervous system (“CNS”), in preclinical studies.

In 2023, the Company announced its strategic transformation into a neurology-focused genomic medicine company focused on developing epigenetic regulation therapies designed to address serious neurological diseases and novel adeno-associated virus (“AAV”) capsid delivery technology.

##### Basis of Presentation

The accompanying Consolidated Financial Statements include the accounts of the Company and its subsidiaries and have been prepared in conformity with generally accepted accounting principles in the United States of America (“U.S. GAAP”) and pursuant to the rules and regulations of the United States Securities and Exchange Commission (“SEC”). All intercompany balances and transactions have been eliminated in the Consolidated Financial Statements.

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

Sangamo is currently working on a number of long-term development projects that involve experimental technologies. The projects will require several years and substantial expenditures to complete and ultimately may be unsuccessful. In recent years, the Company’s operations have been funded primarily through collaborations and strategic partnerships, research grants and from the issuance of equity securities. As of December 31, 2024, the Company had capital resources of \$41.9 million consisting of cash and cash equivalents.

Under Accounting Standard Codification (“ASC”) Topic 205-40, *Presentation of Financial Statements—Going Concern* (“ASC Topic 205-40”), the Company has the responsibility to evaluate whether conditions and/or events raise substantial doubt about its ability to meet its future financial obligations as they become due within one year after the date that the Consolidated Financial Statements are issued. As required under ASC Topic 205-40, management’s evaluation should initially not take into consideration the potential mitigating effects of management’s plans that have not been fully implemented as of the date the Consolidated Financial Statements are issued. When substantial doubt exists, management evaluates whether the mitigating effects of its plans sufficiently alleviates the substantial doubt about the Company’s ability to continue as a going concern. The mitigating effects of management’s plans, however, are only considered if both (i) it is probable that the plans will be effectively implemented within one year after the date that the financial statements are issued, and (ii) it is probable that the plans, when implemented, will mitigate the relevant conditions or events that raise substantial doubt about the entity’s ability to continue as a going concern within one year after the date that the financial statements are issued. Generally, to be considered probable of being effectively implemented, the plans must have been approved by the Company’s board of directors before the date that the financial statements are issued.

The Company’s history of significant losses, its negative cash flows from operations, its limited liquidity resources currently on hand, and its dependence on additional financing to fund its operations after the current resources are exhausted raise substantial doubt about its ability to continue to operate as a going concern within one year after the date that the Consolidated Financial Statements are issued. Based on the Company’s current operating plan, which includes the Company’s estimates that its cash and cash equivalents as of December 31, 2024, together with \$10.1 million generated through the at-the-market offering program in 2025 and the \$5.0 million payment expected from Pfizer by the end of March 2025, the Company expects to meet its liquidity requirements only into the middle of the second quarter of 2025, which is less than one year following the date these Consolidated Financial Statements are issued.

Successful completion of the Company’s development programs and, ultimately, the attainment of profitable operations are dependent upon future events, including obtaining adequate financing to support the Company’s cost structure



and operating plan. Management's plans include, among other things, pursuing one or more of the following steps to raise additional capital, none of which can be guaranteed or are entirely within the Company's control:

- raise funding through the sale of the Company's common stock, including sales under the at-the-market offering program with Jefferies LLC;
- raise funding through debt or royalty financing; and
- establish collaborations with potential partners to advance the Company's product pipeline.

If the Company is unable to raise capital on acceptable terms, or at all, or if it is unable to procure collaboration arrangements or external direct investments to advance its programs, the Company would be required to discontinue some or all of its operations or develop and implement a plan to further extend payables, reduce overhead or scale back its current operating plan until sufficient additional capital is raised to support further operations. There can be no assurance that such a plan would be successful. Additional capital may not be available to the Company on a timely basis, on terms that are acceptable or at all. In particular, the perception of the Company's ability to continue to operate as a going concern may make it more difficult to obtain financing for the continuation of its operations, particularly in light of currently challenging macroeconomic and market conditions. Further, the Company may be unable to attract new investments as a result of the speculative nature of its newly reprioritized core neurology preclinical programs and the absence of partners to progress its more advanced clinical programs. In particular, if the Company is not able to secure a commercialization partner for its Fabry disease program in the near term, the Company's ability to raise additional capital needed to support its operations will be substantially impaired. If adequate funds are not available to the Company on a timely basis, or at all, the Company will be required to take additional actions to address its liquidity needs, including additional cost reduction measures such as further reducing operating expenses and delaying, reducing the scope of, discontinuing or altering its research and development activities, which would have a material adverse effect on its business and prospects, or the Company may be required to cease operations entirely, liquidate all or a portion of its assets, and/or seek protection under the U.S. Bankruptcy Code.

The accompanying Consolidated Financial Statements have been prepared assuming the Company will continue to operate as a going concern, which contemplates the realization of assets and the settlement of liabilities in the normal course of business. The Consolidated Financial Statements do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts of liabilities that may result from uncertainty related to the Company's ability to continue as a going concern.

## **Summary of Significant Accounting Policies**

### ***Use of Estimates***

The preparation of the Consolidated Financial Statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the Consolidated Financial Statements and the accompanying notes. On an ongoing basis, management evaluates its estimates including critical accounting policies or estimates related to revenue recognition, fair value of assets and liabilities, useful lives and impairment of long-lived assets, and stock-based compensation. Estimates are based on historical experience and on various other market specific and other relevant assumptions that the Company believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results could differ from those estimates.

During the year ended December 31, 2023, the Company recorded additional revenue related to changes in estimates in connection with the collaboration agreement with Kite Pharma, Inc., a Gilead Sciences, Inc. subsidiary ("Kite"). These changes in estimates were driven by reductions in the estimated future level of the Company's research and development services in March and September 2023, and as a result, future project costs. These changes resulted in an increase in proportional cumulative performance on this collaboration and increased revenue by \$13.9 million, decreased net loss by \$13.9 million, and decreased the Company's basic and diluted net loss per share by \$0.08 for the year ended December 31, 2023.

### ***Revenue Recognition***

The Company accounts for its revenues pursuant to the provisions of ASC Topic 606, *Revenue from Contracts with Customers* ("ASC Topic 606"). The Company's contract revenues are derived from collaboration agreements including licensing arrangements and research services. Research and license agreements typically include nonrefundable upfront signing or license fees, payments at negotiated rates for time incurred by Company researchers, third-party cost reimbursements, additional target selection fees, sublicense fees, milestone payments tied to ongoing development and product commercialization, and royalties on future licensees' product sales. All funds received from the Company's collaboration partners are generally not refundable. Non-refundable upfront fees are fixed at the commencement of the contract. All other



fees represent variable consideration in contracts. For contracts that contain a provision where the Company reimburses its customer for certain costs they incur and where the Company does not acquire any distinct goods or services in exchange for such payments, the Company accounts for it as a reduction to the contract transaction price. Deferred revenue primarily represents the portion of nonrefundable upfront fees received but not earned.

In determining the appropriate amount of revenue to be recognized as the Company fulfills its obligations under its agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The Company's performance obligations in its collaboration agreements frequently represent distinct bundles of licenses of intellectual property and research and development services, with these components being individually non-distinct as the customer cannot benefit from the licenses independently from the research and development activities. In some instances, the Company has determined that the customer can benefit from the licensed intellectual property separately from the research and development activities, and the licenses of intellectual property and research and development services are individual distinct performance obligations. Options to license the Company's intellectual property and/or acquire research and development services also represent performance obligations when they grant customers a material right, e.g. a right to a discount the customer would not have received if they did not purchase the Company's services under the existing contract.

Revenues from grants of licenses to intellectual property that are distinct and therefore separate performance obligations are recognized at the point in time when the license is effective and the Company has completed the transfer of a copy of the licensed intellectual property to the customer. Revenues from distinct research and development services as well as from distinct bundles of licenses of intellectual property and research and development services, are recognized over time using a proportional performance method. Under this method, revenue is recognized by measuring progress towards satisfaction of the relevant performance obligation using a measure that best depicts the progress towards satisfaction of the relevant performance obligation. For most of the Company's agreements the measure of progress is an input measure based on a level of effort incurred, which includes the value of actual time by Company researchers plus third-party cost reimbursements.

Consideration allocated to options that include material rights is deferred until the options are exercised or expire. The exercise of such options is accounted for as contract continuation, with target selection fees and estimated variable consideration included in the transaction price at that time and allocated specifically to the respective target's performance obligations.

Significant management judgment is required to determine the level of effort required under an arrangement, and the period over which the Company expects to complete its performance obligations under the arrangement. Changes in these estimates can have a material effect on revenue recognized. If the Company cannot reasonably estimate when its performance obligations either are completed or become inconsequential, then revenue recognition is deferred until the Company can reasonably make such estimates. For variable consideration, the amount included in the transaction price is constrained to the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint and, if necessary, adjusts its estimate of the overall transaction price. A cumulative catch-up is then recorded in the current period to reflect the updated transaction price and the updated measure of progress. The estimated period of performance and level of effort, including the value of Company researchers' time and third-party costs, are reviewed quarterly and adjusted, as needed, to reflect the Company's current expectations.

As part of the accounting for these arrangements, the Company must develop assumptions that require judgment to determine the standalone selling price of each performance obligation identified in the contract. The Company uses key assumptions to determine the standalone selling price, which may include forecasted revenues, development timelines, discount rates and probabilities of exercise of technical and regulatory success, and the expected level of effort for research and development services.

Contract modifications occur when the price and/or scope of an arrangement changes. If the modification consists of adding new distinct goods or services in exchange for consideration that reflects standalone selling prices of these goods and services, the modification is accounted for as a separate contract with the customer. Otherwise, if the remaining goods and services are distinct from those previously provided, the existing contract is considered terminated, and the remaining consideration is allocated to the remaining goods and services as if this was a newly signed contract. If the remaining goods and services are not distinct from those previously provided, the effects of the modification are accounted for in a manner similar to the effect of a change in the estimated measure of progress, with cumulative catch-up in revenue recorded at the time of the modification. If some of the remaining goods and services are distinct from those previously provided and others are not, to



account for the effects of the modification the Company applies principles consistent with the objectives of the modification accounting.

Revenues from collaboration and license agreements as a percentage of total revenues were as follows:

	Year Ended December 31,	
	2024	2023
Genentech, Inc.	87 %	— %
Astellas Gene Therapies, Inc.	11 %	— %
Other licensing agreements	2 %	11 %
Biogen MA, Inc.	— %	77 %
Kite Pharma, Inc.	— %	12 %

### ***Accounts Receivable***

Accounts receivable consists of amounts billed to the Company's collaboration partners for cost reimbursements for research services, sublicensing revenue, and royalty payments. Receivables from collaborations are typically unsecured and are concentrated in the biopharmaceutical industry. Accordingly, the Company may be exposed to credit risk generally associated with biopharmaceutical companies or specific to its collaboration agreements. The Company records trade receivables net of allowances for credit losses. The Company applies an aging method to estimate credit losses and considers its historical loss information, adjusted to account for current conditions, and reasonable and supportable forecasts of future economic conditions affecting its customers. Accounts receivable as of December 31, 2024 and 2023 were \$0.5 million and \$0.9 million, respectively, and the Company had not incurred any losses related to accounts receivable. As of December 31, 2024 and 2023, the percentage of accounts receivable by collaboration partners who individually accounted for 10% or more of accounts receivable were as follows:

	As of December 31,	
	2024	2023
Sigma-Aldrich Corporation	70 %	78 %
Ligand Pharmaceuticals Inc.	20 %	5 %
Kite Pharma, Inc.	— %	18 %

### ***Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets***

During a portion of the year ended December 31, 2023, the Company had goodwill and indefinite-lived in-process research and development intangible assets ("IPR&D"). Goodwill represented the excess of consideration transferred over the fair values of assets acquired and liabilities assumed in a business combination. The IPR&D intangible asset was also acquired in a business combination. Goodwill is considered to be indefinite-lived and is not amortized. IPR&D intangible assets are also considered indefinite-lived until the completion or abandonment of the associated research and development efforts.

Goodwill and indefinite-lived intangible assets are assessed for impairment on an annual basis and whenever events and circumstances indicate that these assets may be impaired. Goodwill is impaired if the carrying value of its reporting unit, including goodwill, exceeds the reporting unit's fair value. IPR&D intangible assets are impaired if the carrying value of the assets exceeds their estimated fair value. The Company's goodwill and IPR&D intangible assets were written off in full during the year ended December 31, 2023 as the Company recognized impairment losses, see Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale*.

The Company evaluates the carrying value of long-lived assets, which include property and equipment, leasehold improvements and right-of-use assets, for impairment whenever events or changes in circumstances indicate that the carrying amounts of the asset may not be fully recoverable. Recoverability is tested by comparing the carrying value of the asset or asset group to its undiscounted expected future cash flows. The long-lived asset evaluation is performed at the asset group level, i.e., the lowest level for which identifiable cash flows are largely independent of the cash flows of other assets and liabilities. The Company reassesses the composition of its asset groups whenever there are changes in its operations that affect whether the cash flows associated with assets included in asset groups are largely independent. If the impairment review indicates that the carrying amount of an asset group is not recoverable, an impairment loss is measured as the amount by which the carrying amount of an asset group exceeds its fair value. Any impairment loss is allocated to the long-lived assets of the group on a pro rata basis using the relative carrying amounts of those assets, except that the carrying amount of an individual asset cannot be reduced below its fair value.

Factors that may indicate potential impairment and trigger an impairment test include, but are not limited to, general macroeconomic conditions, conditions specific to the industry and market, an adverse change in legal factors, impairment of



indefinite-lived intangible assets, business climate or operational performance of the business, and sustained decline in the Company's stock price and market capitalization compared to the net book value.

Calculating the fair value of a reporting unit, an asset group and an individual asset involves significant estimates and assumptions. These estimates and assumptions include, among others, projected future cash flows, risk-adjusted discount rates, future economic and market conditions, and the determination of appropriate market comparables. Changes in these factors and assumptions used can materially affect the amount of impairment loss recognized in the period the asset was considered impaired.

### ***Fair Value Measurements***

The carrying amounts for financial instruments consisting of cash and cash equivalents, accounts receivable, accounts payable and other accrued liabilities approximate fair value due to their short-term maturities. Marketable securities are stated at their estimated fair values.

### ***Cash, Cash Equivalents and Restricted Cash***

Sangamo considers all highly liquid investments purchased with original maturities of three months or less at the purchase date to be cash equivalents. Cash and cash equivalents consist of cash, deposits in money market accounts and U.S. government-sponsored entity debt securities. Restricted cash consists of a letter of credit for \$1.5 million, representing a deposit for the lease of office and research and development laboratory facility in Brisbane, California.

A reconciliation of cash, cash equivalents and restricted cash reported within the accompanying Consolidated Balance Sheets to the amounts reported within the accompanying Consolidated Statements of Cash Flows is as follows (in thousands):

	As of December 31,	
	2024	2023
Cash and cash equivalents	\$ 41,918	\$ 45,204
Non-current restricted cash	1,500	1,500
Cash, cash equivalents and restricted cash as reported within the Consolidated Statements of Cash Flows	<u>\$ 43,418</u>	<u>\$ 46,704</u>

### ***Marketable Securities***

Sangamo classifies its marketable securities as available-for-sale and records its investments at estimated fair value based on quoted market prices or observable market inputs of almost identical assets, with the unrealized holding gains and losses included in accumulated other comprehensive loss. The Company classifies those investments that are not required for use in current operations and that mature in more than 12 months as non-current marketable securities in its Consolidated Balance Sheets.

The Company's investments are subject to a periodic impairment review. The Company considers various factors in determining whether to recognize an impairment charge, including the length of time and extent to which the fair value has been less than the cost basis, the Company's financial condition and its intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value. Realized gains and losses on marketable securities are included in interest and other income, net, which are determined using the specific identification method. Credit losses related to marketable securities are recorded in interest and other income, net in the Consolidated Statements of Operations through an allowance for credit losses rather than as a reduction in the amortized cost basis of the securities.

If the Company intends to sell, or if it is more likely than not that the Company will be required to sell, a security before recovery of its amortized cost basis, the allowance for credit losses is written off, and the amortized cost of the security is written down to its fair value, with any incremental impairment charge recognized in earnings. This also results in a reversal of any unrealized gains and losses for this security that were previously included in accumulated other comprehensive loss. Impairment charges are included in interest and other income, net in the Consolidated Statements of Operations.

### ***Concentrations of Credit Risk and Other Risks***

Cash, cash equivalents and marketable securities consist of financial instruments that potentially subject the Company to a concentration of credit risk to the extent of the fair value recorded in the Consolidated Balance Sheets. The Company invests cash that is not required for immediate operating needs primarily in highly liquid instruments that bear minimal risk. The Company has established policies relating to the quality, diversification, and maturities of securities to enable the Company to manage its credit risk. The Company is exposed to credit risk in the event of a default by the financial institutions or issuers of investments holding its cash, cash equivalents and marketable securities to the extent recorded on the Consolidated Balance Sheets.



Certain materials and key components that the Company utilizes in its operations are obtained through single suppliers. Since the suppliers of key components and materials must be named in an investigational new drug application filed with the U.S. Food and Drug Administration for a product, significant delays can occur if the qualification of a new supplier is required. If delivery of material from the Company's suppliers were interrupted for any reason, the Company may be unable to supply any of its product candidates for clinical trials.

### ***Property and Equipment***

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation is calculated using the straight-line method based on the estimated useful lives of the related assets which is generally three to five years. For leasehold improvements, amortization is calculated using the straight-line method based on the shorter of the useful life or the lease term. The Company reviews its property and equipment for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable.

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

Research and development expenses consist primarily of compensation related expenses, including restructuring charges and stock-based compensation, laboratory supplies, preclinical and clinical studies, manufacturing clinical supply, contracted research and development, and allocated facilities and information technology expenses. Research and development costs are expensed as incurred.

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

General and administrative expenses consist primarily of compensation related expenses including restructuring charges and stock-based compensation for executive, legal, finance and administrative personnel, professional fees, allocated facilities and information technology expenses, and other general corporate expenses.

### ***Stock-based Compensation***

The Company measures and recognizes compensation expense for all stock-based payment awards made to Sangamo employees and directors, including employee share options, restricted stock units ("RSUs") and employee stock purchases related to the Employee Stock Purchase Plan ("ESPP") based on estimated fair values at the award grant date. The fair value of stock-based awards is amortized over the vesting period of the award using a straight-line method.

To estimate the fair value of an award, the Company uses the Black-Scholes option pricing model. This model requires inputs such as expected life, expected volatility, expected dividend yield of stock and risk-free interest rate. These inputs are subjective and generally require significant analysis and judgment to develop. While estimates of expected life and volatility are derived primarily from the Company's historical data, the risk-free rate is based on the U.S. Treasury yield curve in effect at the time of grant commensurate with the expected life assumption. The Company accounts for forfeitures in the period they occur.

### ***Warrants to Purchase Shares of Company Stock***

The Company determines the accounting classification of warrants to purchase shares of its stock as either liability or equity by first assessing whether the warrants meet liability classification criteria in accordance with ASC Topic 480, *Distinguishing Liabilities from Equity* ("ASC Topic 480"). Under ASC Topic 480, a financial instrument other than an outstanding share that embodies an obligation to repurchase the entity's shares or is indexed to such an obligation, and that requires or may require the entity to settle it by transferring assets, is classified as a liability. In addition, a financial instrument that embodies an unconditional obligation, or a financial instrument other than an outstanding share that embodies a conditional obligation, that the issuer must or may settle by issuing a variable number of its equity shares must be classified as a liability (or an asset in some circumstances) if, at inception, the monetary value of the obligation is based solely or predominantly on any one of the following: (a) a fixed monetary amount known at inception, (b) variations in something other than the fair value of the issuer's equity shares, or (c) variations inversely related to changes in the fair value of the issuer's equity shares.

If financial instruments, such as warrants, are not required to be classified as liabilities under ASC Topic 480, the Company assesses whether such instruments are indexed to the Company's own stock under ASC Topic 815-40, *Derivatives and Hedging*. In order for an instrument to be considered indexed to an entity's own stock, its settlement amount must always equal the difference between the following: (a) the fair value of a fixed number of the Company's equity shares, and (b) a fixed monetary amount or a fixed amount of a debt instrument issued by the Company. Certain adjustments to this amount are allowed, if they are based on non-levered inputs into the fair value of a fixed price/fixed consideration-option.

Warrants are also required to meet equity classification criteria to be classified in stockholders' equity. Under these criteria, warrants have to provide for settlement in shares, or cash or shares at the entity's option. With limited exceptions, a possibility of net cash settlement under any circumstances will result in the warrants being classified as liabilities.



Warrants classified as equity are generally measured using the Black-Scholes valuation model on the date of issuance. Warrants classified as liabilities are remeasured at any reporting date using valuation models consistent with their terms, with changes recognized in earnings.

### ***Income Taxes***

Income tax expense has been calculated using the liability method. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities as measured by the enacted tax rates that will be in effect when these differences reverse. The Company provides a valuation allowance against net deferred tax assets if, based upon the available evidence, it is not more likely than not that the deferred tax assets will be realized.

The Company recognizes a tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the taxing authorities, based on the technical merits of the position. The tax benefits recognized in the Company's Consolidated Financial Statements from such positions are measured based on the largest benefit that has a greater than 50% likelihood of being realized. The Company recognizes interest and penalties associated with tax matters as part of the income tax provision and includes accrued interest and penalties with the related income tax liability within other accrued liabilities on its Consolidated Balance Sheets. The Company evaluates uncertain tax positions on a regular basis and makes adjustments to these accruals when facts and circumstances change, such as the closing of a tax audit or the refinement of an estimate.

### ***Leases***

The Company determines if an arrangement is or contains a lease at inception by assessing whether the arrangement contains an identified asset and whether it has the right to control the identified asset. Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Lease liabilities are recognized at the lease commencement date based on the present value of future lease payments over the lease term. Right-of-use assets are based on the measurement of the lease liability and also include any lease payments made prior to or on lease commencement and exclude lease incentives and initial direct costs incurred, as applicable.

As the implicit rate in the Company's leases is generally unknown, the Company uses its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of remaining lease payments. The incremental borrowing rate represents an estimate of the interest rate the Company would incur at lease commencement to borrow an amount equal to the lease payments on a collateralized basis over the term of a lease in a similar economic environment. The Company considers its credit risk, term of the lease, and total lease payments and adjusts for the impacts of collateral, as necessary, when calculating its incremental borrowing rates. The lease terms may include options to extend or terminate the lease when it is reasonably certain the Company will exercise any such options. Rent expense for the Company's operating leases, calculated as the sum of the amortization of the right of use asset and accretion of the lease liability, is recognized on a straight-line basis over the lease term, unless the right of use asset was previously written down due to impairment. The Company evaluates the lease arrangement for impairment whenever events or changes in circumstances indicate that the carrying amounts of the right-of-use asset may not be fully recoverable. To the extent an impairment of the right-of-use asset is identified, the Company will recognize the impairment expense and subsequently amortize the remaining right of use asset into rent expense on a straight-line basis (unless another systematic basis is more representative of the pattern in which the Company expects to consume the future economic benefits from the asset) from the date of impairment to the earlier of the end of the right-of-use asset's useful life or the end of the lease term.

If there is a change to the terms and conditions of a contract that results in a change in the scope of or the consideration for a lease, the Company determines if the lease modification results in a separate contract or a change in the accounting for the existing lease and not a separate contract. For lease modifications that result in a separate contract, the Company accounts for the new contract in the same manner as other new leases. For lease modifications that do not result in a separate contract, the Company reassesses the classification of the lease at the effective date of the modification, remeasures and reallocates the remaining consideration in the contract, and remeasures the lease liability using the discount rate determined at the effective date of the modification.

The Company has elected not to separate lease and non-lease components for its real estate and copier leases and, as a result, accounts for any lease and non-lease components as a single lease component. The Company has also elected not to apply the recognition requirement to any leases with a term of 12 months or less and does not include an option to purchase the underlying asset that the Company is reasonably certain to exercise.

### ***Foreign Currency Translation***

The functional currency of the Company's foreign subsidiaries is primarily the Euro. Assets and liabilities denominated in foreign currencies are translated to U.S. dollars using the exchange rates at the balance sheet date. Foreign currency translation adjustments are recorded as a component of accumulated other comprehensive loss within stockholders'



equity. Revenues and expenses from the Company's foreign subsidiaries are translated using the monthly average exchange rates in effect during the period in which the transactions occur. Foreign currency transaction gains and losses are recorded in interest and other income, net, on the Company's Consolidated Statements of Operations.

### ***Net Loss Per Share***

Basic net loss per share has been computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is calculated by dividing net loss by the weighted-average number of shares of common stock plus potentially dilutive securities outstanding during the period. Potential shares of common stock exercisable for little or no consideration are included in both basic and diluted weighted-average number of shares of common stock outstanding.

The computation of diluted net loss per share for the year ended December 31, 2024 excluded 50.5 million shares subject to stock options outstanding, RSUs outstanding, warrants to purchase common stock, and the ESPP shares reserved for issuance because their inclusion would have had an anti-dilutive effect on diluted net loss per share. The computation of diluted net loss per share for the year ended December 31, 2023 excluded 21.3 million shares subject to stock options outstanding, RSUs outstanding, and the ESPP shares reserved for issuance because their inclusion would have had an anti-dilutive effect on diluted net loss per share.

### ***Segments***

The Company operates in one segment. Management uses a single measure of net loss for its single reportable segment and does not segregate its business for internal reporting. As of December 31, 2024 and 2023, the majority of the Company's property and equipment was located in the United States. For the years ended December 31, 2024 and 2023, all of the Company's revenues were generated and earned in the United States.

### ***Restructuring***

The Company records employee severance costs based on whether the termination benefits are provided under an on-going benefit arrangement or under a one-time benefit arrangement. The Company accounts for on-going termination benefit arrangements, such as those arising from employment agreements, applicable regulations or past practices, in accordance with ASC Topic 712, *Compensation—Nonretirement Postemployment Benefits* ("ASC Topic 712"). Under ASC Topic 712, liabilities for post-employment benefits related to past services and that vest or are accumulated over time are recorded at the time the obligations are probable of being incurred and can be reasonably estimated. The Company accounts for one-time employment benefit arrangements in accordance with ASC Topic 420, *Exit or Disposal Cost Obligations* ("ASC Topic 420"). One-time termination benefits are expensed at the date the entity notifies the employee, unless the employee must provide future service over a period extending past the minimum notification period, in which case the benefits are expensed ratably over the future service period. Other associated costs are recognized in the period in which the liability is incurred.

Costs incurred to terminate contracts are recognized upon their termination, e.g., when notice of termination is provided to the counterparty. Costs related to contracts without future benefit are recognized at the cease-use date. Other exit-related costs are recognized as incurred.

### ***Recent Accounting Pronouncements***

#### **Recently Adopted**

In November 2023, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* ("ASU 2023-07"). The standard requires disclosures to include significant segment expenses that are regularly provided to the chief operating decision maker ("CODM"), a description of other segment items by reportable segment, and any additional measures of a segment's profit or loss used by the CODM when deciding how to allocate resources. The ASU also requires all annual disclosures currently required by Topic 280 to be included in interim periods. The Company adopted the standard for its annual reporting for the year ended December 31, 2024 and will adopt it for its interim reporting starting with the quarter ending March 31, 2025. The Company has applied this standard retrospectively. See Note 13 – *Segment Information*, for the additional required disclosures with retrospective presentation to all prior periods presented in the Consolidated Financial Statements.

#### **Not Yet Adopted**

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* ("ASU 2023-09"), which requires public entities, on an annual basis, to provide disclosure of specific categories in the rate reconciliation, as well as disclosure of income taxes paid disaggregated by jurisdiction. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company is currently evaluating the impact of adopting ASU 2023-09.



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* (“ASU 2024-03”), which requires disaggregated disclosure of certain costs and expenses on an interim and annual basis. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The disclosure updates are required to be applied prospectively with the option for retrospective application. The Company is currently evaluating the impact of adopting ASU 2024-03.

## NOTE 2 – FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including cash equivalents and marketable securities. Fair value is determined based on a three-tier hierarchy under the authoritative guidance for fair value measurements and disclosures that prioritizes the inputs used in measuring fair value as follows:

Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

Level 2: Quoted prices in markets that are not active or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability; and

Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurements and unobservable (*i.e.*, supported by little or no market activity).

The Company had no marketable securities as of December 31, 2024. The fair value measurements of the Company’s cash equivalents as of December 31, 2024 are identified at the following levels within the fair value hierarchy (in thousands):

	December 31, 2024			
	Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents:				
Money market funds	\$ 4,138	\$ 4,138	\$ —	\$ —
Total cash equivalents	<u>\$ 4,138</u>	<u>\$ 4,138</u>	<u>\$ —</u>	<u>\$ —</u>

The fair value measurements of the Company’s cash equivalents and marketable securities as of December 31, 2023 are identified at the following levels within the fair value hierarchy (in thousands):

	December 31, 2023			
	Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents:				
Money market funds	\$ 2,508	\$ 2,508	\$ —	\$ —
Total	<u>2,508</u>	<u>2,508</u>	<u>—</u>	<u>—</u>
Marketable securities:				
U.S. government-sponsored entity debt securities	22,566	—	22,566	—
Commercial paper securities	2,826	—	2,826	—
Corporate debt securities	1,405	—	1,405	—
Asset-backed securities	2,377	—	2,377	—
U.S. treasury bills	5,593	—	5,593	—
Certificates of deposit	1,031	—	1,031	—
Total	<u>35,798</u>	<u>—</u>	<u>35,798</u>	<u>—</u>
Total cash equivalents and marketable securities	<u>\$ 38,306</u>	<u>\$ 2,508</u>	<u>\$ 35,798</u>	<u>\$ —</u>

### *Cash Equivalents and Marketable Securities*

The Company generally classifies its marketable securities as Level 2. Instruments are classified as Level 2 when observable market prices for identical securities that are traded in less active markets are used. When observable market prices



for identical securities are not available, such instruments are priced using benchmark curves, benchmarking of like securities, sector groupings, matrix pricing and valuation models. These valuation models are proprietary to the pricing providers or brokers and incorporate a number of inputs, including in approximate order of priority: benchmark yields, reported trades, broker/dealer quotes, issuer spreads, two-sided markets, benchmark securities, bids, offers and reference data including market research publications. For certain security types, additional inputs may be used, or some of the standard inputs may not be applicable. Evaluators may prioritize inputs differently on any given day for any security based on market conditions, and not all inputs listed are available for use in the evaluation process for each security evaluation on any given day.

### NOTE 3 – CASH EQUIVALENTS AND MARKETABLE SECURITIES

The Company had no marketable securities as of December 31, 2024. The table below summarizes the Company's cash equivalents as of December 31, 2024 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
<b>December 31, 2024</b>				
Assets				
Cash equivalents:				
Money market funds	\$ 4,138	\$ —	\$ —	\$ 4,138
Total cash equivalents	<u>\$ 4,138</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 4,138</u>

The table below summarizes the Company's cash equivalents and marketable securities as of December 31, 2023 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
<b>December 31, 2023</b>				
Assets				
Cash equivalents:				
Money market funds	\$ 2,508	\$ —	\$ —	\$ 2,508
Total	<u>2,508</u>	<u>—</u>	<u>—</u>	<u>2,508</u>
Marketable securities:				
U.S. government-sponsored entity debt securities	22,347	219	—	22,566
Commercial paper securities	2,825	2	(1)	2,826
Corporate debt securities	1,399	6	—	1,405
Asset-backed securities	2,368	9	—	2,377
U.S. treasury bills	5,599	—	(6)	5,593
Certificates of deposit	1,026	5	—	1,031
Total	<u>35,564</u>	<u>241</u>	<u>(7)</u>	<u>35,798</u>
Total cash equivalents and marketable securities	<u>\$ 38,072</u>	<u>\$ 241</u>	<u>\$ (7)</u>	<u>\$ 38,306</u>

The fair value of marketable securities by contractual maturity were as follows (in thousands):

	December 31, 2023
Maturing in one year or less	\$ 10,855
Maturing after one year through five years	24,943
Total	<u>\$ 35,798</u>

Realized gains and losses on the sales of investments were not material during the years ended December 31, 2024 and 2023. Total unrealized gains for securities with net gains in accumulated other comprehensive loss were not material for the year ended December 31, 2024.



The Company manages credit risk associated with its investment portfolio through its investment policy, which limits purchases to high-quality issuers and also limits the amount of its portfolio that can be invested in a single issuer. The Company did not record an allowance for credit losses related to its marketable securities for the years ended December 31, 2024 and 2023.

The Company had no unrealized losses related to its marketable securities for the year ended December 31, 2024. The Company had no material unrealized losses related to its marketable securities for the years ended December 31, 2023. The Company had no material unrealized losses, individually and in the aggregate, for marketable securities that were in a continuous unrealized loss position for greater than 12 months as of December 31, 2024 and 2023. These unrealized losses were not attributed to credit risk and were associated with changes in market conditions. The Company periodically reviews its marketable securities for indications of credit losses. No significant facts or circumstances had arisen to indicate that there had been any significant deterioration in the creditworthiness of the issuers of the securities held by the Company. Based on the Company's review of these securities, the Company determined that no allowance for credit losses related to its marketable securities was required at December 31, 2023.

For the periods the Company had investment in debt securities, the Company also considered whether it was more likely than not that the Company will be required to sell the debt securities before recovery of their amortized cost basis. No impairment charges were recorded during the year ended December 31, 2024. The Company recorded impairment charges of \$0.4 million related to certain marketable securities during the year ended December 31, 2023.

#### **NOTE 4 – MAJOR CUSTOMERS, PARTNERSHIPS AND STRATEGIC ALLIANCES**

##### ***Genentech, Inc.***

In August 2024, the Company entered into a global epigenetic regulation and capsid delivery license agreement with Genentech, Inc., a member of the Roche Group ("Genentech") to develop intravenously administered genomic medicines to treat certain neurodegenerative diseases. Under the terms of the agreement, the Company granted an exclusive license to Genentech for the Company's proprietary zinc finger repressors ("ZFRs") that are directed to tau and a second undisclosed neurology target. The Company also granted an exclusive license to Genentech to the Company's proprietary, neurotropic adeno-associated virus capsid, STAC-BBB, for use with therapies directed to tau and to the second neurology target. The Company is prohibited from exploiting (for itself or with or for a third party) products directed to tau and to the second neurology target during the applicable exclusivity periods set forth in the agreement. The Company was responsible for completing the technology transfer and certain preclinical activities, and Genentech is solely responsible for all clinical development, regulatory interactions, manufacturing and global commercialization of resulting products.

In August 2024, the Company received a \$40.0 million upfront license payment from Genentech. In October 2024, the Company received a \$10.0 million milestone payment related to the technology transfer. Under the terms of the agreement, the Company is also eligible to earn up to \$1.9 billion in development and commercial milestones spread across multiple potential products. In addition, the Company is also entitled to receive escalating, tiered mid-single digit to sub-teen double digit royalty payments on the net sales of such products, subject to adjustments for patent expiration, entry of competitive products to the market and payments made under certain licenses for third-party intellectual property.

The agreement will continue, on a product-by-product and country-by-country basis, until the date when there is no remaining royalty payment obligation in such country with respect to such product, at which time the agreement will expire with respect to such product in such country. Royalty obligations cease upon the later of expiry of the last valid patent claim covering the product in the country or ten years from the date of the first commercial sale of the product in such country. Genentech has the right to terminate the agreement for convenience. Each party has the right to terminate the agreement on account of the other party's uncured material breach.

The Company assessed the agreement with Genentech in accordance with ASC Topic 606 and concluded that Genentech is a customer. The initial transaction price of \$50.0 million includes the upfront license fee of \$40.0 million and the \$10.0 million technology transfer milestone payment. None of the development milestones have been included in the transaction price, as all such amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price as uncertain events are resolved or other changes in circumstances occur. Potential sales-based milestones and royalty payments are not estimated as they meet the sales-or usage-based royalty exception under ASC Topic 606 and are recognized in the period they are earned, provided the related performance obligations have been completed.

The Company has identified two performance obligations within the Genentech Agreement. All licenses were accounted for as a performance obligation to provide functional intellectual property that is satisfied at a point in time that was satisfied upon completion of the technology transfer in September 2024. The preclinical activities represent research and



development services and are satisfied over time as the Company conducts and Genentech benefits from the associated activities. Revenue related to the preclinical activities is recognized using an input method of cumulative actual costs incurred relative to total estimated costs.

The Company allocated the initial transaction price to the performance obligations based on the relative standalone selling price of each performance obligation. In the absence of an observable standalone selling price, the Company used a methodology that maximized the use of observable inputs. This included a cost plus margin approach for the preclinical activities, which required the estimation of total costs and an expected margin. The standalone selling price of the licenses was determined based on the analysis of the probability-adjusted discounted cash flows and potential sales of licensed products. Significant estimates and assumptions were used that include but are not limited to, expected market opportunity and pricing, timelines, and likelihood of success of clinical, regulatory and commercialization activities. The Company expects to allocate variable consideration payable upon achievement of future milestones and royalty payments to the specific performance obligation to which they relate, i.e. the license performance obligation, as such allocation would meet the allocation objective in ASC Topic 606.

As of December 31, 2024, the Company had no receivable, no deferred revenue, and no amounts included in transaction price remaining to be recognized related to the agreement.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Revenue related to Genentech agreement:		
Recognition of license revenue	\$ 48,679	\$ —
Research services	1,321	—
Total	<u>\$ 50,000</u>	<u>\$ —</u>

#### ***Astellas Gene Therapies, Inc.***

In December 2024, the Company entered into a global capsid delivery license agreement with Astellas Gene Therapies, Inc. (“Astellas”), or the Astellas Agreement. Under the terms of the Astellas Agreement, the Company granted an exclusive license to Astellas to the Company’s proprietary, neurotropic adeno-associated virus capsid, STAC-BBB for use with therapies directed to an initial neurodevelopmental target and up to four additional targets and for up to three indications per target. In addition, Astellas has a potential right to exchange its license to the STAC-BBB capsid for a license to another capsid. This substitution right may be exercised twice during the initial three-year period of the Astellas Agreement and is subject to the availability of a substitute capsid at the time the request is made. The Company is prohibited from exploiting (for itself or with or for a third party) products directed to the initial target, any reserved targets, and any additional licensed targets under the Astellas Agreement for licensed or reserved indications during the applicable exclusivity periods set forth in the Astellas Agreement.

The Company completed the technology transfer with respect to the initial target and indication in December 2024, and Astellas is solely responsible for all preclinical and clinical development, regulatory interactions, manufacturing and global commercialization of resulting products.

In December 2024, the Company received a \$20.0 million upfront license payment from Astellas under the Astellas Agreement. Under the terms of the Astellas Agreement, the Company is also eligible to earn up to \$1.3 billion in license fees and research, development and commercial milestones across up to five potential targets, including a license fee for each additional licensed target. In addition, the Company is also entitled to receive escalating, tiered mid-single digit to high-single digit royalty payments on the net sales of products sold under these licenses, subject to adjustments for patent expiration, entry of biosimilar or interchangeable products to the market and payments made under certain licenses for third-party intellectual property.

The Astellas Agreement will continue, on a product-by-product and country-by-country basis, until the date when there is no remaining royalty payment obligation in such country with respect to such product, at which time the Astellas Agreement will expire with respect to such product in such country. Royalty obligations cease upon the latest of expiration of regulatory exclusivity for such product in such country, the last expiration of certain valid patent claims covering such product in such country or ten years from the date of the first commercial sale of such product in such country. Astellas has the right to terminate the Astellas Agreement for convenience. Each party has the right to terminate the Astellas Agreement for other party’s uncured material breach and for specified bankruptcy events. The Company also has the right to terminate the Astellas Agreement if Astellas challenges any of the Company’s licensed patents under the Astellas Agreement.



The Company assessed the agreement with Astellas in accordance with ASC Topic 606 and concluded that Astellas is a customer. The initial transaction price includes the upfront license fee of \$20.0 million. None of the research or development milestones have been included in the transaction price, as all such amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon successful continuation of research and development activities in future periods. The Company will re-evaluate the transaction price at each reporting date, as certain events are resolved or other changes in circumstances occur. Potential sales-based milestones and royalty payments are not estimated as they meet the sales-or usage-based royalty exception under ASC Topic 606 and are recognized in the period they are earned, provided the related performance obligations have been completed.

The Company has determined that Astellas' option to add additional targets and indications would result in the grant of separate licenses from the license to the initial target and indication. Rights to these optional licenses can be acquired by Astellas at a discount from their standalone selling price, and accordingly, represent material rights granted to Astellas. Both the initial and any optional licenses are distinct and license Astellas to use functional intellectual property. Accordingly, they would be recognized at a point in time when granted, provided Astellas has received a copy of the associated intellectual property. Optional licenses will not be recognized until exercise of the underlying option or until expiration of the option.

The Company allocated the initial transaction price to the performance obligations based on the relative standalone selling price of each performance obligation. In the absence of observable prices, the Company used a methodology that maximized the use of observable inputs. The Company took into consideration the total amounts paid and potentially payable by Astellas and potential market for each license. In addition, included in the estimates of the standalone selling prices of the options with material rights were the implied level of discount and the probability of the option exercise. Of the transaction price of \$20.0 million, \$6.5 million was allocated to the initial license, and \$13.5 million to the options for additional licensed targets.

The initial license was transferred upon completion of the technology transfer in December 2024, and the associated amount of \$6.5 million recognized in revenue at that time. As of December 31, 2024, the Company had deferred revenue of \$13.5 million related to the options with material rights, of which \$7.6 million is classified as current based on the contractually required timing of exercise or expiration of the underlying options within the next four years. The remaining \$5.9 million is classified as non-current.

#### ***Pfizer Inc.***

In May 2017, the Company entered into an exclusive global collaboration and license agreement with Pfizer Inc. ("Pfizer"), pursuant to which it established a collaboration for the research, development and commercialization of giroctocogene fitelparvovec, its gene therapy product candidate for hemophilia A, and closely related products.

In December 2024, Pfizer notified the Company of its termination for convenience, effective April 21, 2025 (the "Pfizer Termination Date"), of the collaboration agreement. Pfizer had indicated to Sangamo that the termination relates to its decision not to submit a Biologics License Application or Marketing Authorization Application for, or pursue commercialization of, giroctocogene fitelparvovec. As of the Pfizer Termination Date, the collaboration agreement will be terminated in its entirety, and all licenses and other rights granted by the Company to Pfizer will terminate. Sangamo is entitled to receive from Pfizer an exclusive, worldwide, royalty-bearing, sublicensable license from Pfizer to use Pfizer's relevant intellectual property to continue developing, manufacturing and commercializing giroctocogene fitelparvovec; in return, Pfizer would be eligible to receive low single digit royalties on net sales of giroctocogene fitelparvovec and would be released from certain liabilities to the extent they exist.

Under this agreement, the Company was responsible for conducting the Phase 1/2 clinical trial and for certain manufacturing activities for giroctocogene fitelparvovec, while Pfizer was responsible for subsequent worldwide development, manufacturing, marketing and commercialization of giroctocogene fitelparvovec.

Subject to the terms of the agreement, the Company granted Pfizer an exclusive worldwide royalty-bearing license, with the right to grant sublicenses, to use certain technology controlled by the Company for the purpose of developing, manufacturing and commercializing giroctocogene fitelparvovec and related products. Pfizer granted the Company a non-exclusive, worldwide, royalty-free, fully paid license, with the right to grant sublicenses, to use certain manufacturing technology developed under the agreement and controlled by Pfizer to manufacture the Company's products that utilize the AAV delivery system.

The agreement had a term that continued on a per product and per country basis until the later of (i) the expiration of patent claims that cover the product in a country, (ii) the expiration of regulatory exclusivity for a product in a country, and (iii) 15 years after the first commercial sale of a product in a country. Pfizer had the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement could also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. Upon termination for any reason, the license



granted by the Company to Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec and related products automatically terminates. Upon termination by the Company for cause or by Pfizer in any country or countries, Pfizer will automatically grant the Company an exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec in the terminated country or countries.

Upon execution of the agreement, the Company received an upfront fee of \$70.0 million and was eligible to receive up to \$208.5 million in payments upon the achievement of specified clinical development, intellectual property and regulatory milestones and up to \$266.5 million in payments upon first commercial sale milestones for giroctocogene fitelparvovec and potentially other products. To date, two milestones of \$55.0 million in aggregate had been achieved and paid. In addition, Pfizer had agreed to pay the Company royalties for each potential licensed product developed under the agreement that are 14% - 20% of the annual worldwide net sales of such product and are subject to reduction due to patent expiration, entry of biosimilar products to the market and payment made under certain licenses for third-party intellectual property.

In connection with the termination of the agreement by Pfizer, the Company will be entitled to receive \$5.0 million in additional consideration in 2025. Following the termination notice, this amount is payable upon 60 days after the effective date of the termination, unless the Company transfers a specified sublicense to Pfizer, in which case it will be payable 30 days after such transfer. Sangamo transferred to Pfizer the requisite sublicense in February 2025. Pfizer will not be obligated to pay the Company the remaining milestone payments and royalties.

The Company assessed the agreement with Pfizer in accordance with ASC Topic 606 and concluded that Pfizer was a customer. The Company completed its performance obligations and recognized the amounts included in the transaction price of \$134.0 million during the periods through December 31, 2020. No revenue was recognized during the years ended December 31, 2024 and 2023. The remaining development, intellectual property and regulatory milestone amounts were not included in the transaction price and were not recognized as their achievement was dependent on the progress and outcomes of Pfizer's development activities and was therefore uncertain.

The Company accounted for the notice of termination of the agreement by Pfizer as a modification in accordance with ASC Topic 606. The \$5.0 million payment will be recognized in revenue upon transfer of the sublicense, during the first quarter of 2025.

#### ***Alexion Pharmaceuticals, Inc., AstraZeneca Rare Disease***

In December 2017, the Company entered into an exclusive, global collaboration and license agreement with Pfizer, subsequently assigned to Alexion, AstraZeneca Rare Disease ("Alexion") in September 2023, for the development and commercialization of potential gene therapy products that use zinc finger transcriptional regulators ("ZF-transcriptional regulators") to treat amyotrophic lateral sclerosis and frontotemporal lobar degeneration linked to mutations of the *C9ORF72* gene. Pursuant to this agreement, the Company agreed to work with Pfizer on a research program to identify, characterize and preclinically develop ZF-transcriptional regulators that bind to and specifically reduce expression of the mutant form of the *C9ORF72* gene.

Subject to the terms of this agreement, the Company granted Pfizer (now Alexion) an exclusive, royalty-bearing, worldwide license under the Company's relevant patents and know-how to develop, manufacture and commercialize gene therapy products that use resulting ZF-transcriptional regulators that satisfy pre-agreed criteria. During a specified period, neither the Company nor Alexion will be permitted to research, develop, manufacture or commercialize outside of the collaboration any zinc finger proteins ("ZFPs") that specifically bind to the *C9ORF72* gene.

Unless earlier terminated, the agreement has a term that continues on a per licensed product and per country basis until the later of (i) the expiration of patent claims that cover the licensed product in a country, (ii) the expiration of regulatory exclusivity for a licensed product in a country, and (iii) 15 years after the first commercial sale of a licensed product in a major market country. Alexion also has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. Upon termination for any reason, the license granted by the Company to Alexion to develop, manufacture and commercialize licensed products under the agreement would automatically terminate. Upon termination by the Company for cause or by Alexion without cause for any licensed product or licensed products in any country or countries, the Company would have the right to negotiate with Alexion to obtain a non-exclusive, royalty-bearing license under certain technology controlled by Alexion to develop, manufacture and commercialize the licensed product or licensed products in the terminated country or countries.

Following any termination by the Company for Alexion's material breach, Alexion would not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the *C9ORF72* gene for a period of time. Following any termination by Alexion for the Company's material breach, the Company would not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the *C9ORF72* gene for a period of time.



The Company received a \$12.0 million upfront payment from Pfizer and is eligible to receive up to \$60.0 million in development milestone payments from Alexion contingent on the achievement of specified preclinical development, clinical development and first commercial sale milestones, and up to \$90.0 million in commercial milestone payments if annual worldwide net sales of the licensed products reach specified levels. In addition, Alexion will pay the Company royalties of 14% - 20% of the annual worldwide net sales of the licensed products. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property. Each party is responsible for the cost of its performance of the research program. Alexion is operationally and financially responsible for subsequent development, manufacturing and commercialization of the licensed products. To date, a milestone of \$5.0 million has been earned and paid, however no products have been approved and therefore no royalty fees have been earned under the *C9ORF72* agreement.

The Company assessed the agreement with Alexion in accordance with ASC Topic 606 and concluded that Alexion is a customer. The Company completed its performance obligations and recognized the amounts included in the transaction price of \$17.0 million during the periods through December 31, 2020. No revenue was recognized during the years ended December 31, 2024 and 2023. The remaining development milestone amounts have not been included in the transaction price and have not been recognized as their achievement is dependent on the progress and outcomes of Alexion's development activities and is therefore uncertain. If and when these milestones become probable of being achieved, they would be recognized in full at that time. Sales related milestones and royalties are not recognized until triggered based on the contractual terms.

In October 2023, Pfizer notified the Company of Pfizer's assignment of the collaboration and license agreement to Alexion, AstraZeneca Rare Disease, pursuant to a definitive purchase and license agreement for preclinical gene therapy assets and enabling technologies that closed on September 20, 2023.

#### ***Kite Pharma, Inc.***

In February 2018, the Company entered into a global collaboration and license agreement with Kite which became effective on April 5, 2018 ("Effective Date"), and was amended and restated in September 2019, for the research, development, and commercialization of potential engineered cell therapies for cancer. The collaboration and license agreement related to the design of zinc finger nucleases ("ZFNs") and viral vectors to disrupt and insert certain genes in T-cells and natural killer cells ("NK-cells") including the insertion of genes that encode chimeric antigen receptors ("CARs"), T-cell receptors ("TCRs"), and NK-cell receptors ("NKR") directed to mutually agreed targets. Under the agreement, Kite was responsible for all clinical development, manufacturing and commercialization of any resulting products. The agreement expired pursuant to its terms in April 2024.

Subject to the terms of this agreement, the Company granted Kite an exclusive, royalty-bearing, worldwide sublicensable license under the Company's relevant patents and know-how to develop, manufacture and commercialize, for the purpose of treating cancer, specific cell therapy products that may result from the research program and that are engineered ex vivo using selected ZFNs and viral vectors developed under the research program to express CARs, TCRs or NKR directed to candidate targets.

Following the Effective Date, the Company received a \$150.0 million upfront payment from Kite. In addition, Kite reimbursed the Company's direct costs to conduct the joint research program. Sangamo was also eligible to receive contingent development- and sales-based milestone payments that could total up to \$3.0 billion if all of the specified milestones set forth in this agreement are achieved. Of this amount, approximately \$1.3 billion related to the achievement of specified research, clinical development, regulatory and first commercial sale milestones, and approximately \$1.8 billion relates to the achievement of specified sales-based milestones if annual worldwide net sales of licensed products reach specified levels. Each development- and sales-based milestone payment was payable (i) only once for each licensed product, regardless of the number of times that the associated milestone event is achieved by such licensed product, and (ii) only for the first ten times that the associated milestone event is achieved regardless of the number of licensed products that may achieve such milestone event. In addition, the Company was entitled to receive escalating, tiered royalty payments with a percentage in the single digits based on future annual worldwide net sales of licensed products. These royalty payments were subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

The initial research term in the agreement was six years from the Effective Date and expired in April 2024. Kite had an option to extend the research term for up to two additional one-year periods for a separate upfront fee of \$10.0 million per year, which was not exercised and expired in October 2023. All contingent payments under the agreement, when earned, were non-refundable and non-creditable. Through the amendment and restatement of the agreement in September 2019, the Company and Kite agreed to expand the scope of the collaboration program to incorporate the use of lentiviral or retroviral vectors provided by Kite. Kite had the right to terminate this agreement in its entirety or on a per licensed product or per candidate target basis



for any reason after a specified notice period. Each party had the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach.

The Company assessed the agreement with Kite in accordance with ASC Topic 606 and concluded that Kite is a customer. The transaction price includes the upfront license fee of \$150.0 million and estimated reimbursable service costs for the research projects over the estimated performance period. None of the clinical or regulatory milestones were included in the transaction price, as none of the milestones had yet been achieved, and all amounts were fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time was uncertain and contingent upon future periods when the uncertainty related to the variable consideration was resolved.

The transaction price also included actual and estimated payments by Kite for the work by Company researchers and reimbursement of the Company's costs incurred with third parties. The Company used the expected value method to estimate payments related to the Company's researchers' work, taking into account the impact of constraint. Variable consideration was included in the transaction price only to the extent it is probable a significant reversal of cumulative revenues recognized would not occur.

The Company had identified four performance obligations within the Kite agreement as follows: (1) a license to the technology combined with the obligation to perform research and development services to apply the Company's technology to Kite-selected targets; (2) production of research materials; and (3-4) two material rights, each for an extension of the research period for an additional one-year term. Such extensions contained material rights because their exercise did not require payment of a fee that is commensurate with the value of the incremental research term. The license to the Company's intellectual property was not distinct from the related research and development activities as the licensed technology was not shared with and could not be utilized by Kite without the research services performed by the Company.

The Company allocated variable consideration (payments by Kite for the work performed by the Company's researchers and third-party costs, as well as any future milestones and royalties) to the specific performance obligations to which they relate, as such allocation meets the allocation objective in ASC Topic 606. The Company allocated the fixed consideration of \$150.0 million to the performance obligations based on their relative standalone selling prices. Standalone selling prices of optional research years were similar to those of the initial year, but additionally take into account the intrinsic value of the discount upon exercise and the likelihood of exercise.

Fees allocated to options with material rights were deferred until the options were exercised or expired. The exercise of options was accounted for as contract continuation, with target selection fees and estimated variable consideration included in the transaction price at that time and allocated specifically to the respective target's performance obligation.

Revenue for the combined license and research services performance obligations was recognized over time, as Kite consumed the benefit of such services as they were being performed by the Company. For the license combined with research and development services performance obligation, the Company recognized revenue based on proportional performance of the ongoing research services over the period during which the Company performed the services. The estimation of progress towards the satisfaction of this performance obligation and project costs were reviewed quarterly and adjusted, as needed, to reflect the Company's assumptions regarding the estimated volume of required activities. The production of research materials performance obligation was accounted for under the right to invoice practical expedient, as the Company had the right to invoice Kite for these services in an amount that corresponds directly with the value of the services.

As of December 31, 2024 and 2023 the Company had a receivable of zero and \$0.2 million, respectively, related to this agreement. As of December 31, 2024 and 2023, the Company had no deferred revenue, and no amounts included in transaction price remaining to be recognized related to the agreement.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Revenue related to Kite agreement:		
Recognition of license fee fixed consideration	\$ —	\$ 19,423
Research services variable consideration	—	1,097
Total	\$ —	\$ 20,520

During the year ended December 31, 2023, the Company recorded additional revenue related to changes in estimates in connection with the collaboration agreement with Kite. These changes in estimates were driven by reductions in the estimated future level of the Company's research and development services in March and September 2023, and as a result, future project costs. These changes resulted in an increase in proportional cumulative performance on this collaboration and



increased revenue by \$13.9 million, decreased net loss by \$13.9 million, and decreased the Company's basic and diluted net loss per share by \$0.08 for the year ended December 31, 2023.

***Novartis Institutes for BioMedical Research, Inc.***

On July 27, 2020, the Company entered into a collaboration and license agreement with Novartis Institutes for BioMedical Research, Inc. ("Novartis") for the research, development and commercialization of gene regulation therapies to treat three neurodevelopmental disorders. Under the agreement, which was effective upon execution, the Company granted Novartis an exclusive, royalty bearing and worldwide license, under its relevant patents and know-how, to develop, manufacture and commercialize certain of its ZF-transcriptional regulators targeted to three undisclosed genes that are associated with certain neurodevelopmental disorders, including autism spectrum disorder and intellectual disability. The Company was performing early research activities over the collaboration period for each gene target and manufacture the ZF-transcriptional regulators required for such research, costs of which were funded by Novartis. Novartis was responsible for additional research activities, studies enabling investigational new drug ("IND") applications, clinical development, regulatory approvals, manufacturing of preclinical, clinical and approved products, and global commercialization. Subject to certain exceptions set forth in the agreement, the Company was prohibited from developing, manufacturing or commercializing any therapeutic product targeting any of the three genes that are the subject of the collaboration. Novartis also had the option to license certain of the Company's proprietary AAVs for the sole purpose of developing, manufacturing and commercializing licensed products arising from the collaboration.

In March 2023, Novartis notified the Company of its termination for convenience, effective June 11, 2023 (the "Novartis Termination Date"), of the collaboration agreement. Novartis had indicated to the Company that the termination relates to a recent strategic review. As of the Novartis Termination Date, the collaboration agreement was terminated in its entirety and following the Novartis Termination Date the Company is not entitled to receive any further milestone payments or royalties from Novartis. As of the Novartis Termination Date, the parties have no further obligations to develop or to fund the development of any collaboration research programs under the collaboration agreement.

Upon entering the agreement, Novartis paid the Company a \$75.0 million upfront license fee. Novartis was also obligated to pay the Company for the use of its resources and reimburse third-party costs incurred in the Company's conduct of early research activities. The Company was also eligible to earn from Novartis development and commercial milestones and royalties on potential commercial sales of licensed products arising from the collaboration, none of which were triggered or earned. The agreement was going to continue, on a product-by-product and country-by-country basis, until the expiration of the applicable royalty term.

All payments received under the agreement were non-refundable and non-creditable. The transaction price of \$95.1 million included the upfront license fee of \$75.0 million and research costs of \$20.1 million. All clinical or regulatory milestone amounts were considered fully constrained throughout the term of the agreement.

The Company assessed the agreement with Novartis in accordance with ASC Topic 606 and concluded that Novartis was a customer. The Company had identified a single performance obligation within this arrangement as a license to the technology and ongoing research services. The Company concluded that the license was not discrete as it did not have standalone value to Novartis apart from the research services to be performed pursuant to the agreement. As a result, the Company recognized revenue from the upfront payment based on proportional performance of the ongoing research services through the estimated research period. The estimation of progress towards the satisfaction of performance obligation and project cost was reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its performance obligation.

The notice of termination was accounted for as a modification of the contract, as it changed both the scope of the Company's remaining services and the consideration to which the Company was entitled. The effect of the modification was not material, as the Company was nearing the completion of its assigned early research activities, and consequently, of its sole performance obligation.

As of December 31, 2024 and 2023, the Company had no receivable, no deferred revenue, and no amounts included in transaction price remaining to be recognized related to the agreement.



Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Revenue related to Novartis agreement:		
Recognition of upfront license fee	\$ —	\$ 9,568
Research services	—	2,611
Total	\$ —	\$ 12,179

The Company paid \$1.5 million for financial advisory fees during the year ended December 31, 2020, equal to 2% of \$75.0 million received for the upfront license fee related to the collaboration and license agreement with Novartis. The Company recognized \$1.5 million as a contract asset as such amount represents a cost of obtaining the agreement. This balance was amortized and included in general and administrative expenses on a systematic basis consistent with the transfer of the services to Novartis in accordance with ASC Topic 340, *Other Assets and Deferred Costs* (“ASC Topic 340”). The Company amortized zero and \$0.2 million during the years ended December 31, 2024 and 2023, respectively.

### ***Biogen MA, Inc.***

In February 2020, the Company entered into a collaboration and license agreement with Biogen MA, Inc. (“BIMA”) and Biogen International GmbH (together with BIMA, “Biogen”) for the research, development and commercialization of gene regulation therapies for the treatment of neurological diseases. The companies planned to leverage the Company’s proprietary ZF technology delivered via AAV to modulate expression of key genes involved in neurological diseases. Concurrently with the execution of the collaboration agreement, the Company entered into a stock purchase agreement with BIMA, pursuant to which BIMA agreed to purchase 24,420,157 shares of the Company’s common stock (the “Biogen Shares”), at a price per share of \$9.2137, for an aggregate purchase price of approximately \$225.0 million. The collaboration agreement became effective in April 2020.

In March 2023, Biogen notified the Company of its termination for convenience, effective June 15, 2023 (the “Biogen Termination Date”), of the collaboration agreement. Biogen had indicated to the Company that the termination relates to a recent strategic review. As of the Biogen Termination Date, the collaboration agreement was terminated in its entirety and following the Biogen Termination Date the Company is not entitled to receive any further milestone payments or royalties from Biogen. As of the Biogen Termination Date, the parties have no further obligations to develop or to fund the development of any collaboration research programs under the collaboration agreement.

Under the collaboration agreement, Biogen paid the Company an upfront license fee of \$125.0 million in May 2020. The Company was also eligible to receive target selection, research, development, regulatory and commercial milestone payments and royalties on potential net commercial sales of licensed products arising from the collaboration, none of which were triggered or earned.

Under the collaboration agreement, the Company granted to Biogen an exclusive, royalty bearing and worldwide license, under its relevant patents and know-how, to develop, manufacture and commercialize ZF and/or AAV-based products directed to certain neurological disease gene targets selected by Biogen. Biogen had selected four targets over the course of the collaboration and had exclusive rights to nominate up to seven additional targets. These rights expired upon the Biogen Termination Date. For each gene target selected by Biogen, the Company performed early research activities, costs of which were shared by the companies, aimed at the development of the combination of proprietary central nervous system delivery vectors and ZF-transcriptional regulators (or potential other ZF products) targeting therapeutically relevant genes.

The Company assessed the collaboration agreement with Biogen in accordance with ASC Topic 606 and concluded that Biogen was a customer. The transaction price included the upfront license fee of \$125.0 million and the excess consideration from the stock purchase of \$79.6 million, which represented the difference between the \$225.0 million received for the purchase of the Biogen Shares and the \$145.4 million estimated fair value of the equity issued. The equity issued to Biogen was valued using an option pricing model to reflect certain holding period restrictions. None of the clinical or regulatory milestones were included in the transaction price, as all such amounts were fully constrained throughout the term of the collaboration agreement. The transaction price also included actual and estimated cost-sharing payments by Biogen for the work by Company researchers and reimbursement of the Company’s costs incurred with third parties. The amounts paid and expected to be paid to Biogen for the use of Biogen’s resources and its expenses were consideration paid to a customer. Since the Company did not acquire distinct goods or services in exchange for these payments, they reduced the transaction price and were recorded as a reduction in revenue. The Company used the expected value method to estimate cost sharing payments, taking into account the impact of the constraint. Variable consideration was included in the transaction price only to the extent it was probable a significant reversal of cumulative revenues recognized would not occur. The Company re-evaluated the transaction price as uncertain events were resolved or other changes in circumstances occurred.



The Company concluded that the licenses to its intellectual property for each target were not distinct from the related research and development activities, as the licensed technology was not shared with and could not be utilized by Biogen without the research services to be performed by the Company pursuant to the agreement. On the other hand, each combination of a license to the Company's intellectual property as applied to a specific target and the related research and development activities are a discrete research project that is distinct from any other target's project. The targets Biogen could select were options that provided Biogen with material rights, as the exercise of the options did not require payment of a fee commensurate with the value of the incremental license rights. As a result, such options also represented performance obligations.

At contract inception, the Company allocated fixed consideration of \$204.6 million included in the initial transaction price to the existing targets' license and research services performance obligations and those performance obligations for options that include material rights, based on their relative standalone selling prices. Through June 30, 2023, all such material rights expired.

The notice of termination was accounted for as a modification of the contract, as it changed both the scope of the Company's remaining services and the consideration to which the Company was entitled. The remaining research and development activities to be undertaken by the Company after the notice of termination were not distinct from the related activities performed prior to the modification on the same targets but were distinct from the activities on other targets. The remaining material rights were also distinct from the prior research and development activities. To account for the effects of the modification, the Company updated its estimate of the transaction price and allocated the remaining transaction consideration based on the relative standalone selling prices of the remaining distinct goods and services. Progress for each ongoing performance obligation was then remeasured using an updated estimate of the total level of effort required for each performance obligation and the total revised transaction price and a cumulative catch-up in revenue was recorded. The modification resulted in a decrease to the transaction price of \$17.3 million and an increase in revenue of \$127.1 million.

As of December 31, 2024 and 2023, the Company had no receivable, no deferred revenue, and no amounts included in transaction price remaining to be recognized related to the agreement.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Revenue related to Biogen agreement:		
Recognition of license and other fixed consideration	\$ —	\$ 132,165
Cost-sharing payments for research services, net variable consideration	—	2,684
Total	\$ —	\$ 134,849

The Company paid \$7.0 million for financial advisory fees during the year ended December 31, 2020, equal to 2% of \$225.0 million received for the sale of shares and 2% of \$125.0 million received for the upfront fee. The fees incurred related to both the collaboration agreement with Biogen and to the stock purchase agreement for the sale of shares. The Company believes that the allocation of fees on a relative fair value basis between the two agreements was reasonable. The Company recognized \$4.1 million, which represents 2% of the initial transaction price of \$204.6 million, as a contract cost asset. This balance was released into general and administrative expenses on a systematic basis consistent with the transfer of the services to Biogen in accordance with ASC Topic 340. In March 2023, as a result of the notice of termination and resulting modification of the contract, the progress for each performance obligation was remeasured and the Company recognized \$2.5 million as a cumulative catch-up to expense. The Company recognized as expense zero and \$2.6 million during the years ended December 31, 2024 and 2023, respectively.

### ***Agreement with Sigma-Aldrich Corporation***

In 2007, Sangamo entered into a license agreement with Sigma-Aldrich Corporation ("Sigma") to provide Sigma with access to Sangamo's proprietary ZF technology and the exclusive right to use the technology to develop and commercialize research reagent products and services in the research field, excluding certain agricultural research uses that Sangamo previously licensed to Dow AgroSciences LLC ("DAS"), a wholly owned subsidiary of Dow Chemical Company. Sangamo developed laboratory research reagents using its ZF technology over a three-year research services period. Sangamo has since transferred the ZF manufacturing technology to Sigma.

In October 2009, Sangamo expanded its license agreement with Sigma. In addition to the original terms of the license agreement, Sigma received exclusive rights to develop and distribute ZF-modified cell lines for commercial production of protein pharmaceuticals and certain ZF-engineered transgenic animals for commercial applications. Under the terms of the agreement, Sigma made an upfront cash payment of \$20.0 million consisting of a \$4.9 million purchase of 636,133 shares of Sangamo common stock, valued at \$4.9 million, and a \$15.1 million upfront license fee. Sangamo is also eligible to receive commercial license fees of \$5.0 million based upon a percentage of net sales and sublicensing revenue and thereafter a reduced



royalty rate of 10.5% of net sales and sublicensing revenue. In addition, upon the achievement of certain cumulative commercial milestones, Sigma will make milestone payments to Sangamo up to an aggregate of \$42.0 million. Sangamo does not have additional ongoing performance obligations under the agreement.

Revenues recognized under the agreement with Sigma for the years ended December 31, 2024 and 2023 were \$0.9 million and \$4.7 million, respectively.

## **NOTE 5 – IMPAIRMENT OF GOODWILL, INDEFINITE-LIVED INTANGIBLE ASSETS AND LONG-LIVED ASSETS AND WRITE-DOWN OF ASSETS HELD FOR SALE**

### ***Year ended December 31, 2024***

During the three months ended March 31, 2024, the Company's Board of Directors approved the wind-down of research and development activities in France and corresponding reduction in workforce, including closure of the Company's cell therapy manufacturing facility and research labs in Valbonne, France (the "France Restructuring"). The Company concluded its equipment, furniture and fixtures located in France met the held for sale criteria as of March 31, 2024. The Company wrote down the carrying value of these assets to their estimated fair value of \$1.0 million, net of the estimated costs to sell, recognizing a loss of \$1.8 million. An additional loss of \$0.1 million was recorded during the three months ended June 30, 2024. The fair value measurements represent Level 3 nonrecurring fair value measurements. The loss is included in impairment of long-lived assets in the accompanying Consolidated Statements of Operations. The Company sold assets held for sale during the three months ended December 31, 2024, and recognized a gain of \$1.0 million included in general and administrative expenses. See Note 10 – *Restructuring Charges*, for the additional effects of the France Restructuring.

During the three months ended March 31, 2024, the Company also initiated actions to commence the closure of its facility in Brisbane, California. The Company expects to close this facility in the near future.

In connection with the changes in the manner in which the right-of-use assets and leasehold improvements related to the Company's Brisbane, California and Valbonne, France facilities are used, costs incurred to cease use of these assets, the France Restructuring, and the Company's activities to market these facilities for sublease, the Company concluded the identifiable operations and cash flows of these assets were now largely independent of the operations and the cash flows of each other, as well as of the remainder of the Company. Accordingly, the Company assessed impairment for each of these asset groups separately and concluded that the carrying values of Brisbane, California and Valbonne, France facilities asset groups were not recoverable. The Company proceeded to determine their fair values using a discounted cash flow method, which represents a Level 3 nonrecurring fair value measurement. As a result, the Company recognized pre-tax long-lived asset impairment charges of \$2.0 million on the right-of-use assets and \$0.5 million on the related leasehold improvements during the three months ended March 31, 2024. Additional impairment charges of \$0.9 million on the right-of-use assets and \$0.2 million on the related leasehold improvements were recognized during the three months ended June 30, 2024, triggered by the ongoing wind-down of the France research and development activities and a decline in the market rates for facility subleases in Brisbane, California.

The Company will continue to assess whether its long-lived assets are impaired in future periods. As the Company finalizes the closure of its Brisbane, California facility, it is reasonably possible that additional impairment charges will be recognized, for example, if sublease rates of leased facilities are less than those estimated.

### ***Year ended December 31, 2023***

During the three months ended March 31, 2023, as a result of the sustained decline in the Company's stock price and related market capitalization, termination of the collaboration agreements with Biogen and Novartis, and restructuring of operations the Company performed an impairment assessment of goodwill, indefinite-lived intangible assets, and other long-lived assets.

The Company operated as a single reporting unit based on its business and reporting structure. For goodwill, a quantitative impairment assessment was performed using a market approach, whereby the Company's fair value of equity was compared to its carrying value. The fair value of equity was derived using both the market capitalization of the Company and an estimate of a reasonable range of values of a control premium applied to the Company's implied business enterprise value. The control premium was estimated based upon control premiums observed in comparable market transactions. This represented a Level 2 nonrecurring fair value measurement. Based on this analysis, the Company recognized a pre-tax goodwill impairment charge of \$38.1 million during the year ended December 31, 2023. As a result, the goodwill was fully impaired as of December 31, 2023.

Throughout the year ended December 31, 2023, the Company determined all of its long-lived assets represented one asset group for purposes of long-lived asset impairment assessment. At each of the periods ended March 31, June 30, September 30 and December 31, 2023, the Company concluded that the carrying value of the asset group was not recoverable as it exceeded the future undiscounted cash flows the assets were expected to generate from the use and eventual disposition. To



allocate and recognize the impairment loss, the Company determined individual fair values of its long-lived assets. The Company applied a discounted cash flow method to estimate fair values of its leasehold improvements and right-of-use assets, including leasehold improvements in the process of construction and a cost replacement method and market approach to estimate the fair value of its furniture, fixtures and laboratory and manufacturing equipment. These represented Level 3 nonrecurring fair value measurements.

Based on these analyses, the Company recognized pre-tax long-lived asset impairment charges of \$28.9 million on the right-of-use assets, \$18.7 million on the related leasehold improvements and \$4.4 million on construction-in-progress, and \$13.5 million on furniture, fixtures, and laboratory and manufacturing equipment during the year ended December 31, 2023.

The Company reassessed its indefinite-lived and long-lived assets for impairment as of June 30, 2023. Given the actions contemplated above, the Company determined that it was more likely than not that its indefinite-lived intangible assets were impaired. Accordingly, the Company developed an estimate of the fair value of its indefinite-lived intangible assets using the multi-period excess earnings model (income approach) and concluded the carrying value of its indefinite-lived intangible assets were fully impaired. This represents a Level 3 nonrecurring fair value measurement. As a result, an indefinite-lived intangible assets impairment charge of \$51.3 million, as well as the related income tax benefit of \$6.3 million due to the reversal of a deferred tax liability associated with the indefinite-lived intangible assets was recognized during the year ended December 31, 2023. The impairment charge was primarily driven by a higher discount rate applied to future cash flows based on market participants' view of increased risk related to the asset.

## NOTE 6 – OTHER BALANCE SHEET DETAILS

### *Property and Equipment, Net*

Property and equipment, net consist of the following (in thousands):

	December 31,	
	2024	2023
Laboratory equipment	\$ 21,391	\$ 34,630
Leasehold improvements	24,923	29,809
Furniture and fixtures	3,546	4,610
Manufacturing equipment	7,845	7,784
Construction in progress	—	1,087
	57,705	77,920
Less: accumulated depreciation and amortization	(39,818)	(51,046)
Property and equipment, net	\$ 17,887	\$ 26,874

Depreciation and amortization expense was \$5.1 million and \$15.1 million during the years ended December 31, 2024 and 2023, respectively.

During the year ended December 31, 2024, the Company recorded impairment losses of \$1.9 million for its laboratory equipment and \$0.7 million for its leasehold improvements. During the year ended December 31, 2023, the Company recorded impairment losses of \$23.1 million for its leasehold improvements, \$8.0 million for its laboratory equipment, \$2.3 million for its manufacturing equipment, \$1.6 million for its furniture and fixtures, and \$1.6 million for its construction in progress.

### *Intangible Assets*

The changes in intangible assets were as follows (in thousands):

	December 31,	
	2024	2023
Balance at beginning of year	\$ —	\$ 50,729
Foreign currency translation adjustment	—	618
Impairment of indefinite-lived intangible asset	—	(51,347)
Balance at end of year	\$ —	\$ —



### **Goodwill**

The changes in goodwill were as follows (in thousands):

	December 31,	
	2024	2023
Balance at beginning of year	\$ —	\$ 37,552
Foreign currency translation adjustment	—	586
Impairment of goodwill	—	(38,138)
Balance at end of year	<u>\$ —</u>	<u>\$ —</u>

### **Other Accrued Liabilities**

Other accrued liabilities consist of the following (in thousands):

	December 31,	
	2024	2023
Operating lease liabilities – current	\$ 4,313	\$ 4,589
Accrued professional fees	1,368	1,505
Accrued restructuring charges	896	11,733
Accrued research and development expenses	213	3,763
Other	1,405	1,964
Total other accrued liabilities	<u>\$ 8,195</u>	<u>\$ 23,554</u>

## **NOTE 7 – COMMITMENTS AND CONTINGENCIES**

### **Leases**

Sangamo's corporate headquarters occupies approximately 59,485 square feet of research and office space, pursuant to a lease that expires in August 2031, and approximately 7,700 of office space, pursuant to a lease that expires in August 2026, in Richmond, California. Sangamo also occupies approximately 103,089 square feet of office and research and development laboratory facility in Brisbane, California pursuant to a lease that expires in May 2029. In December 2024, in connection with the France Restructuring, the Company terminated its leases for office and research and development space in Valbonne, France. See Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* for more information on impairment charges related to the associated right-of-use and leasehold improvement assets.

On February 5, 2024, the Company entered into an amendment to the operating lease of office and research and development laboratory facility in Brisbane, California. The amendment established early termination rights for the landlord upon thirty days' notice to the Company. Additionally, the amendment authorized the landlord to draw on the existing letter of credit to satisfy the majority of the Company's February 2024 through April 2024 rent payments and obligated the Company to provide a cash security deposit or replenish the letter of credit back to \$1.5 million by June 1, 2024. On July 3, 2024, the Company entered into another amendment to extend the deadline for replenishing the letter of credit, which was replenished as of September 30, 2024.

The Company concluded that the amendment represented a lease modification to be accounted for as a single contract with the existing lease under ASC Topic 842, *Leases*, and remeasured its lease liability using the current incremental borrowing rate of 9.6%, and recorded an adjustment to reduce both the lease liability and the corresponding right-of-use asset by \$1.9 million as of the lease modification date.

Certain of these leases include renewal options at the election of the Company to renew or extend the lease for an additional five to ten years. These optional periods have not been considered in the determination of the right-of-use assets or lease liabilities associated with these leases as the Company did not consider it reasonably certain it would exercise the options.



The Company performed evaluations of its contracts and determined each of its identified leases are operating leases. Components of operating leases were as follows (in thousands):

	December 31,	
	2024	2023
Operating lease cost	\$ 6,375	\$ 9,423
Variable lease cost	3,544	3,126
Total	<u>\$ 9,919</u>	<u>\$ 12,549</u>

Variable lease expenses were not included in the measurement of the Company's operating right-of-use assets and lease liabilities. This variable expense consists primarily of the Company's proportionate share of operating expenses, property taxes and insurance and is classified as lease expense, due to the Company's election to not separate lease and non-lease components.

Cash paid for amounts included in the measurement of operating lease liabilities for the year ended December 31, 2024 and 2023 was \$7.5 million and \$7.3 million, respectively, and was included in net cash used in operating activities in the Company's Consolidated Statements of Cash Flows.

Future minimum payments under lease obligations at December 31, 2024 consist of the following (in thousands):

	Total
2025	\$ 6,708
2026	7,372
2027	7,319
2028	7,496
2029	4,418
Thereafter	3,701
Total lease payments	<u>37,014</u>
Less:	
Imputed interest	(6,205)
Tenant improvement allowance included in contra-lease liability	(243)
Total	<u>\$ 30,566</u>

Reported as of December 31, 2024:

Short-term portion of lease liabilities (included in other accrued liabilities on the Consolidated Balance Sheet)	\$ 4,313
Long-term portion of lease liabilities	26,253
Total	<u>\$ 30,566</u>

As of December 31, 2024, the weighted-average remaining lease term is 5.2 years and the weighted-average incremental borrowing rate used to determine the operating lease liability was 7.7% for the Company's operating leases.

During the years ended December 31, 2024 and 2023, the Company recorded impairment losses of \$2.9 million and \$28.9 million, respectively, related to its right-of-use assets. See Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* for more information.

### ***Contractual Commitments***

The Company's non-cancelable material contractual commitments under manufacturing-related supplier arrangements as of December 31, 2024 related to Thermo Fisher Scientific Inc. amounting to \$2.9 million and expire in December 2025. The Company also had \$0.5 million of license obligations related to its intellectual property as of December 31, 2024.

### ***Contingencies***

The Company is not party to any material pending legal proceeding. From time to time, the Company is, and may become, involved in litigation and regulatory compliance matters incidental to the Company's business, including employment and wage and hour claims, antitrust, tax, product liability, environmental, health and safety, commercial disputes, intellectual property, contracts and other matters arising out of the normal conduct of the Company's business. Since litigation is inherently unpredictable and unfavorable resolutions can occur, assessing contingencies is highly subjective and requires judgments about future events. Sangamo regularly reviews and accrues for contingencies related to litigation and regulatory compliance matters, if it is probable that a liability has been incurred and the amount of the loss can be reasonably estimated. Based on current



information, in the opinion of the Company, the ultimate resolution of these matters, individually or in aggregate, will not have a material adverse effect on the Company's financial condition, results of operations or cash flows.

## **NOTE 8 – STOCKHOLDERS' EQUITY**

### ***Preferred Stock***

The Company's Certificate of Incorporation authorizes the Company to issue up to 5,000,000 shares of preferred stock, which may be issued at the discretion of the Company's Board of Directors. As of December 31, 2024, no shares of the Company's preferred stock have been issued or are outstanding.

### ***Common Stock***

In June 2023, the Company's stockholders approved an amendment to the Company's Certificate of Incorporation to increase the total number of shares of the Company's common stock authorized for issuance from 320,000,000 shares to 640,000,000 shares. In June 2024, the Company's stockholders approved an amendment to the Company's Certificate of Incorporation to increase the total number of shares of the Company's common stock authorized for issuance from 640,000,000 shares to 960,000,000 shares.

As of December 31, 2024, 212,837,679 shares of the Company's common stock are outstanding.

### ***At-the-Market Offering Agreement***

In August 2020, the Company entered into an Open Market Sale Agreement<sup>SM</sup> with Jefferies LLC ("Jefferies") with respect to an at-the-market offering program under which the Company may offer and sell, from time to time at its sole discretion, shares of the Company's common stock having an aggregate offering price of up to \$150.0 million through Jefferies as the Company's sales agent or principal. In December 2022, the Company entered into Amendment No. 2 to the Open Market Sale Agreement<sup>SM</sup> which increased the aggregate offering price under the at-the-market offering program by an additional \$175.0 million. Approximately \$186.9 million remained available under the sales agreement as of December 31, 2024. The Company is not obligated to sell any shares under the sales agreement. During the years ended December 31, 2024 and 2023, the Company sold 3,638,740 and 8,249,261 shares of its common stock, respectively, for net proceeds of approximately \$7.1 million and \$15.1 million, respectively.

### ***Issuance and Sale of Common Stock and Warrants***

On March 21, 2024, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with certain institutional investors (collectively, the "Investors"). On March 26, 2024 the Company issued and sold in a registered direct offering (the "Registered Direct Offering") an aggregate of 24,761,905 shares of common stock of the Company, par value \$0.01 per share, and pre-funded warrants to purchase up to an aggregate of 3,809,523 shares of common stock, together with accompanying warrants ("Common Warrants") to purchase up to an aggregate of 28,571,428 shares of common stock. The combined offering price of a unit consisting of one share of common stock and the accompanying Common Warrant to purchase one share of common stock was \$0.84. The combined offering price of a unit consisting of a pre-funded warrant to purchase one share of common stock and the accompanying Common Warrant to purchase one share of common stock was \$0.83. The pre-funded warrants are immediately exercisable at any time, until exercised in full, at a price of \$0.01 per share of common stock. The Common Warrants are exercisable six months from issuance, expire five and a half years from the issuance date and have an exercise price of \$1.00 per share. Both pre-funded warrants and Common Warrants can be exercised net in limited circumstances and entitle holders to dividends if and when paid by the Company.

Barclays Capital Inc. and Cantor Fitzgerald & Co. (the "Placement Agents") acted as the placement agents for the offering, pursuant to a Placement Agency Agreement, dated March 21, 2024 (the "Placement Agreement"). Pursuant to the Placement Agreement, the Company paid the Placement Agents a cash placement fee equal to 6.0% of the aggregate gross proceeds raised in the Registered Direct Offering.

The Company received aggregate net proceeds from the Registered Direct Offering of \$21.9 million, net of the Placement Agents' fees of \$1.4 million and other offering costs of \$0.7 million.

Common Warrants and pre-funded warrants were determined to be equity-classified and proceeds received from their issuance were recorded as a component of stockholders' equity within additional paid-in capital. The Company determined that the warrants should be equity classified because they are freestanding financial instruments, do not embody an obligation for the Company to repurchase its shares, do not contain exercise contingencies tied to observable markets or indices, permit the holders to receive a fixed number of shares of common stock upon exercise in exchange for a fixed amount of consideration, subject only to adjustments that are inputs to the fair value of a fixed price/fixed consideration-option, and meet the equity classification criteria. The pre-funded warrants were exercised in full on April 8, 2024 and the Company issued an aggregate of



3,809,523 shares of common stock at an exercise price of \$0.01. The Common Warrants had not been exercised and remained outstanding as of December 31, 2024.

### **2018 Equity Incentive Plan**

In June 2023, the Company's stockholders approved an amendment and restatement of the 2018 Equity Incentive Plan (the "2018 Plan") to, among other things, increase the aggregate number of shares of the Company's common stock reserved for issuance under the 2018 Plan by 10,000,000 shares. In June 2024, the Company's stockholders approved an amendment and restatement of the 2018 Plan to, among other things, increase the aggregate number of shares of the Company's common stock reserved for issuance under the 2018 Plan by 11,000,000 shares. The aggregate number of shares of the Company's common stock reserved for issuance under the 2018 Plan are 38,800,000 shares as of December 31, 2024.

The exercise price of a stock option granted under the 2018 Plan may not be less than 100% of the fair market value of the Company's common stock subject to the stock option on the date of grant, and the option term will not exceed ten years. If the person to whom the stock option is granted is a 10% stockholder of the Company, and the stock option granted qualifies as an incentive stock option, then the exercise price per share will not be less than 110% of the fair market value of the Company's common stock on the date of grant, and the option term will not exceed five years. Generally, stock options granted under the 2018 Plan vest over three or four years and expire ten years after the date of grant, or earlier upon termination of employment or services to the Company.

The number of shares of common stock reserved for issuance under the 2018 Plan will be reduced: (i) on a 1-for-1 basis for each share of common stock subject to a stock option or stock appreciation right granted under the plan, (ii) by a fixed ratio of 1.33 shares of common stock for each share of common stock issued pursuant to a full-value award granted under the plan.

Shares subject to any outstanding stock options or other awards under the 2018 Plan that expire or otherwise terminate prior to the issuance of the shares subject to those stock options or awards will be available for subsequent issuance under the 2018 Plan. Any unvested shares issued under the 2018 Plan that the Company subsequently purchases, pursuant to repurchase rights under the 2018 Plan, will be added back to the number of shares reserved for issuance under the 2018 Plan on a 1-for-1 basis or a 1.33-for-1 basis (depending on the ratio at which the share reserve was debited for the original award) and will accordingly be available for subsequent issuance in accordance with the terms of the 2018 Plan.

As of December 31, 2024, there were 18,826,554 shares of the Company's common stock reserved for future awards under the Company's 2018 Plan.

### **2020 Employee Stock Purchase Plan**

In May 2021, the Company's stockholders approved the Company's 2020 Employee Stock Purchase Plan ("the ESPP"). The ESPP provides for a total of 5.0 million shares of common stock reserved for issuance thereunder. Eligible employees may purchase common stock at 85% of the lesser of the fair market value of the Company's common stock on the first day of the applicable two-year offering period or the last day of the applicable six-month purchase period. As of December 31, 2024, there were 2,110,961 shares of the Company's common stock reserved for future issuance under the ESPP.

### **Stock Option Activity**

A summary of the Company's stock option activity is as follows:

	Number of Shares	Weighted- Average Exercise per Share Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Options outstanding at December 31, 2023	14,862,004	\$ 6.95		
Options granted	400,000	\$ 0.57		
Options exercised	—	\$ —		
Options forfeited and expired	(4,566,063)	\$ 7.02		
Options outstanding at December 31, 2024	<u>10,695,941</u>	\$ 6.68	5.73	\$ 229,853
Options exercisable at December 31, 2024	<u>9,039,081</u>	\$ 7.38	5.28	\$ 104,740

The intrinsic value of options exercised was zero during the years ended December 31, 2024 and 2023.



### ***Restricted Stock Units***

During the years ended December 31, 2024 and 2023, the Company awarded 11,783,300 and 4,811,834 RSUs, respectively. The RSUs awarded in the years ended December 31, 2024 and 2023 had an average grant date fair value per award of \$0.47 and \$2.29, respectively. These awards generally vest over two or three years. The aggregate fair value of RSUs vested during the years ended December 31, 2024 and 2023 was \$10.5 million and \$17.8 million, respectively.

A summary of the Company's RSU activity is as follows:

	Number of Shares	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
RSUs outstanding at December 31, 2023	6,052,923		
RSUs awarded	11,783,300		
RSUs released	(2,607,780)		
RSUs forfeited	(4,205,558)		
RSUs outstanding at December 31, 2024	<u>11,022,885</u>	0.43	\$ 11,243

RSUs that vested in the years ended December 31, 2024 and 2023 were net-share settled such that the Company withheld shares with value equivalent to the employees' minimum statutory obligation for the applicable income and other employment taxes and remitted the cash to the appropriate taxing authorities. The total shares withheld were approximately 861,509 and 788,236 for the years ended December 31, 2024 and 2023, respectively, and were based on the value of the RSUs on their respective issuance dates as determined by the Company's closing stock price. Total payments for the employees' tax obligations to taxing authorities were \$0.9 million and \$1.5 million in the years ended December 31, 2024 and 2023, respectively and are reflected as a financing activity within the accompanying Consolidated Statements of Cash Flows. These net-share settlements had the effect of share repurchases by the Company as they reduced and retired the number of shares that would have otherwise been issued as a result of the vesting and did not represent an expense to the Company.

### **NOTE 9 – STOCK-BASED COMPENSATION**

The following table shows total stock-based compensation expense recognized in the accompanying Consolidated Statements of Operations (in thousands):

	Year Ended December 31,	
	2024	2023
Research and development	\$ 5,698	\$ 15,215
General and administrative	6,684	12,148
Total stock-based compensation expense	<u>\$ 12,382</u>	<u>\$ 27,363</u>

As of December 31, 2024, total stock-based compensation expense to be recognized in future periods related to unvested stock options was \$2.4 million, which is expected to be expensed over a weighted-average period of 1.1 years. As of December 31, 2024, total stock-based compensation expense to be recognized in future periods related to unvested RSUs was \$5.0 million, which is expected to be expensed over a weighted-average period of 1.1 years. There was no capitalized stock-based employee compensation expense as of December 31, 2024 and 2023.

### ***Valuation Assumptions***

Employee stock-based compensation expense was determined using the Black-Scholes option valuation model for stock options and employee share purchases under the ESPP. Option valuation models require the input of subjective assumptions and these assumptions can vary over time. The fair value of RSUs was based on the closing price of the underlying common stock on the date of grant.

The Company bases its determination of expected volatility through its assessment of the historical volatility of its common stock. The Company relied on its historical exercise and post-vested termination activity for estimating its expected term for use in determining the fair value of these options.

The weighted-average estimated fair value per share of options granted during the years ended December 31, 2024 and 2023 was \$0.40 and \$1.53, respectively, based upon the assumptions used in the Black-Scholes valuation model. The



assumptions used for estimating the fair value of the employee stock options were as follows:

	Year Ended December 31,	
	2024	2023
Risk-free interest rate	4.35%	3.44-4.00%
Expected term (in years)	5.36	5.47-5.56
Expected dividend yield of stock	—	—
Expected volatility	82.98%	72.18-76.05%

Employees purchased 747,692 and 1,346,849 shares of common stock through the ESPP at a weighted-average exercise price of \$0.39 and \$0.69 per share during the years ended December 31, 2024 and 2023, respectively. The weighted-average estimated fair values of shares purchased under the Company's ESPP during the years ended December 31, 2024 and 2023 were \$0.21 and \$0.91, respectively, based upon the assumptions used in the Black-Scholes valuation model.

The assumptions used for estimating the fair value of the ESPP purchase rights are as follows:

	Year Ended December 31,	
	2024	2023
Risk-free interest rate	4.13-5.32%	4.28-5.37%
Expected term (in years)	0.5-2.0	0.5-2.0
Expected dividend yield of stock	—	—
Expected volatility	99.27-153.06%	65.71-100.60%

## NOTE 10—RESTRUCTURING CHARGES

### *France Restructuring*

In November 2023, the Company initiated an information and consultation procedure with the Works Council for its Valbonne, France workforce regarding a planned wind-down of Sangamo's French research and development activities and a corresponding reduction in workforce, including planned closure of the Company's cell therapy manufacturing facility and research labs in Valbonne, France. The information and consultation procedure with the Works Council resulted in the definition of an acceptable set of termination provisions including payouts to departing employees and were a required step before the Company could eliminate positions at Sangamo France. The information and consultation procedure of the Works Council was completed in the first quarter of 2024. On March 1, 2024, the Company's Board of Directors approved the France Restructuring which resulted in the elimination of all 93 roles in France, or approximately 24% of the total global workforce at that time. As a result, the Company terminated its research and development activities in France and has substantially completed making severance payments to its French employees as required by French law and the terms of the applicable collective bargaining agreements, and incurring other employee-related costs. The Company will continue the long-term follow up for its clinical studies for previously dosed patients as required by regulations.

The Company had estimated it would incur \$4.8 million in expenses related to employee severance and notice period payments, benefits, contract termination costs, and other related restructuring charges for the France Restructuring. The Company incurred \$4.7 million of expenses during the year ended December 31, 2023, of which \$3.6 million is included in research and development expense and \$1.1 million is included in general and administrative expense in the accompanying Consolidated Statements of Operations. The Company incurred \$0.1 million of expenses during the year ended December 31, 2024, which is included in general and administrative expense in the accompanying Consolidated Statements of Operations. See Note 5 – *Impairment of Goodwill, Indefinite-lived Intangible Assets and Long-lived Assets and Write-Down of Assets Held For Sale* for impairment considerations related to the France Restructuring.

As of December 31, 2024, the Company's France operation no longer has a facility, and its remaining employees departed in January 2025. The Company expects its restructuring-related payments to be complete during the first quarter of 2025.

### *April 2023 Restructuring*

On April 26, 2023, the Company executed a restructuring of operations and a corresponding reduction in workforce (the "April 2023 Restructuring"), designed to reduce costs and increase focus on certain strategic priorities. The April 2023 Restructuring resulted in the elimination of approximately 110 roles in the United States, or approximately 23% of the total United States workforce at that time. The April 2023 Restructuring resulted in the incurrence of severance payments and other employee-related costs, including additional vesting of service-based stock compensation awards.



No expenses related to the April 2023 Restructuring were incurred during the year ended December 31, 2024. The Company incurred approximately \$5.0 million of expenses related to the April 2023 Restructuring during the year ended December 31, 2023, of which \$3.8 million is included in research and development expense and \$1.2 million is included in general and administrative expense in the accompanying Consolidated Statements of Operations. The April 2023 Restructuring and the cash payments related thereto are complete as of December 31, 2024.

### ***November 2023 Restructuring***

On November 1, 2023, the Company executed a restructuring of operations and a corresponding reduction in workforce (the “November 2023 Restructuring”), designed to reduce costs and advance its strategic transformation into a neurology-focused genomic medicine company. The November 2023 Restructuring resulted in the elimination of approximately 162 roles, including 108 full-time employees and 54 contracted employees and eliminated open positions, in the United States, or approximately 40% of the total United States workforce at that time, and included one-time severance payments and other employee-related costs, including additional vesting of service-based stock compensation awards.

The total restructuring charges are estimated to be approximately \$7.8 million to \$8.8 million, related to employee severance and notice period payments, benefits, Brisbane, California facility close-out costs, and other related restructuring charges for the November 2023 Restructuring. The Company incurred \$0.2 million of expenses in the year ended December 31, 2024, which is included in research and development expense in the accompanying Consolidated Statements of Operations. The Company incurred \$6.7 million of expenses in the year ended December 31, 2023, of which \$5.0 million is included in research and development expense and \$1.7 million is included in general and administrative expense in the accompanying Consolidated Statements of Operations. The cash payments relating to employee severance and notice period payments, benefits, and other employee-related costs for the November 2023 Restructuring are complete as of December 31, 2024. The Company expects to incur estimated costs of \$0.9 million to \$1.9 million on Brisbane, California facility close-out costs to be complete in the near future.

The following table is a summary of accrued April 2023, November 2023, and France Restructuring charges included within other accrued liabilities on the Company’s Consolidated Balance Sheet as of December 31, 2024 and 2023 (in thousands):

	December 31,	
	2024	2023
Balance at beginning of year	\$ 11,733	\$ —
Restructuring charges, net	342	16,015
Cash payments	(11,179)	(4,282)
Balance at end of year	<u>\$ 896</u>	<u>\$ 11,733</u>

Sangamo may also incur other cash expenses or charges not currently contemplated or estimable due to events that may occur as a result of, or associated with, the November 2023 Restructuring.

### **NOTE 11 – EMPLOYEE BENEFIT PLAN**

The Company sponsors a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code covering all full-time employees (“Sangamo 401(k) Plan”). The Sangamo 401(k) Plan is intended to qualify under Section 401 of the Internal Revenue Code.

The Company matched employee contributions equal to 100% in 2024 and 2023, up to a limit of \$5,000. Matching funds are fully vested when contributed. Contributions to the Sangamo 401(k) Plan by the Company were \$1.2 million and \$1.8 million for the years ended December 31, 2024 and 2023, respectively.



**NOTE 12 – INCOME TAXES**

The domestic and foreign components of loss before income taxes were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Domestic	\$ (103,729)	\$ (173,375)
Foreign	5,621	(89,528)
Loss before income taxes	<u>\$ (98,108)</u>	<u>\$ (262,903)</u>

Income tax benefit for the years ended December 31, 2024 and 2023 consisted of the following (in thousands):

	Year Ended December 31,	
	2024	2023
Income tax expense:		
Current:		
Federal	\$ —	\$ —
State	—	—
Foreign	(167)	186
Subtotal	<u>(167)</u>	<u>186</u>
Deferred:		
Federal	—	—
State	—	—
Foreign	—	(5,258)
Subtotal	<u>—</u>	<u>(5,258)</u>
Income tax expense	<u>\$ (167)</u>	<u>\$ (5,072)</u>

The difference between the income tax benefit for the years ended December 31, 2024 and 2023 and the amount computed by applying the federal statutory income tax rate to loss before income taxes is explained as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Tax at federal statutory rate	\$ (20,603)	\$ (55,210)
State taxes, net	3,483	(1,372)
Foreign rate differential	263	(4,273)
Global Intangible Low-taxed Income	617	791
Non-deductible stock-based compensation	2,656	4,770
Research credits	(3,201)	(7,020)
Change in valuation allowance	16,172	49,016
Goodwill impairment	—	9,764
Other	446	(1,538)
Income tax expense	<u>\$ (167)</u>	<u>\$ (5,072)</u>



Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's deferred tax assets and liabilities are as follows (in thousands):

	December 31,	
	2024	2023
<b>Assets:</b>		
Deferred tax assets:		
Net operating loss carryforwards	\$ 211,585	\$ 196,719
Research and development tax credit carryforwards	58,409	54,336
Stock-based compensation	3,998	5,983
Deferred revenue	—	—
Capitalized research	74,354	71,675
Fixed assets	15,648	17,152
Intangible assets	31	101
Lease liability	6,666	8,559
Accruals and reserves	331	2,409
Other	235	259
Total deferred tax asset	371,257	357,193
Valuation allowance	367,578	351,430
Deferred tax assets	3,679	5,763
<b>Liabilities:</b>		
Intangible assets	—	—
Operating lease right-of-use assets	(3,679)	(5,763)
Deferred tax liabilities	(3,679)	(5,763)
Total net deferred tax liabilities	\$ —	\$ —

The income tax benefit for the year ended December 31, 2024 was due to reversal of its United Kingdom's ("U.K.") long-term payable as a result of the statute of limitations lapsing. The income tax benefit for the year ended December 31, 2023 was primarily driven by the reduction of the foreign deferred tax liability due to the impairment of the indefinite-lived intangible asset and was offset by the setup of the Sangamo U.K.'s valuation allowance.

A valuation allowance is recorded when it is more likely than not that all or some portion of the deferred income tax assets will not be realized. The Company regularly assesses the need for a valuation allowance against its deferred income tax assets by considering both positive and negative evidence related to whether it is more likely than not that the Company's deferred income tax assets will be realized. In evaluating the Company's ability to recover its deferred income tax assets within the jurisdiction from which they arise, the Company considers all available positive and negative evidence, including scheduled reversals of deferred income tax liabilities, projected future taxable income, tax-planning strategies, and results of recent operations.

The Company continues to maintain a full valuation allowance on its U.S. federal and state, Sangamo France and Sangamo U.K. net deferred tax assets, as the Company believes it is not more likely than not that these benefits will be realized. The valuation allowance increased by \$16.1 million and \$49.6 million for the years ended December 31, 2024 and 2023, respectively.

As of December 31, 2024, Sangamo had net operating loss carryforwards for federal and state income tax purposes of approximately \$818.5 million and \$349.8 million, respectively. The federal net operating loss generated before 2018 will begin to expire in 2025 and will keep expiring through 2037, if not utilized. Federal net operating loss generated from 2018 will carry forward indefinitely. If not utilized, the state net operating loss carryforwards will begin to expire in 2029, respectively. The Company's French net operating loss carryforward balance is \$120.4 million, which carries over indefinitely. The Company also has federal and state research tax credit carryforwards of \$50.0 million and \$33.2 million, respectively. The federal research credits will begin to expire in 2025 and will keep expiring through 2043, while the state research credits have no expiration date. Utilization of the Company's net operating loss carryforwards and research tax credit carryforwards may be subject to substantial annual limitations due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. The annual limitation could result in the expiration of the net operating loss carryforwards and research tax credit carryforwards before utilization.



The Company's policy is to reinvest the earnings of its non-U.S. subsidiaries in those operations. The Company does not provide for U.S. taxes on the earnings of foreign subsidiaries because the Company intends to reinvest such earnings offshore indefinitely. However, if these funds were repatriated, the Company would be required to accrue and pay applicable U.S. taxes and withholding taxes. Due to the cumulative losses generated in foreign countries there are no earnings to repatriate.

Government incentives in the form of refundable research tax credits are recognized when there is reasonable assurance that the incentive will be received and the Company will comply with the conditions specified in the agreement or statutory requirements. The Company is eligible to receive these incentives because it engages in qualifying research and development activities in a foreign jurisdiction as defined by the government entity. As of December 31, 2024 and 2023, the Company had refundable research tax credits of \$4.1 million and \$3.1 million, respectively, in current assets and \$12.8 million and \$15.4 million, respectively, in non-current assets on the Consolidated Balance Sheets.

The Company files federal and state income tax returns with varying statutes of limitations. The tax years from 2004 forward remain open to examination due to the carryover of net operating losses or tax credits. The Company also files the U.K. and French income tax returns, and the tax years from 2008 and thereafter remain open in the U.K., and the tax years 2020 and thereafter in France are still subject to examination.

The Company's practice is to recognize interest and/or penalties related to income tax matters in income tax expense. The Company had \$0.2 million and \$0.3 million accrued interest and/or penalties as of December 31, 2024 and 2023, respectively. Unrecognized tax benefits are not expected to change materially over the next 12 months. The amount of unrecognized tax benefits that, if recognized, would impact the effective tax rate is \$0.9 million and \$1.2 million as of December 31, 2024 and 2023, respectively.

The following table summarizes the activity related to the Company's unrecognized tax benefits (in thousands):

	December 31,	
	2024	2023
Beginning balance	\$ 18,320	\$ 18,179
Additions based on tax positions related to the current year	4,274	2,805
Additions for tax positions of prior years	26	29
Reductions for tax positions of prior years	(224)	(2,693)
Statute lapse	(259)	—
Ending balance	<u>\$ 22,137</u>	<u>\$ 18,320</u>

#### NOTE 13 – SEGMENT INFORMATION

The Company has identified its Chief Executive Officer as the CODM. Management uses one measure of profitability and does not segregate the Company's business for internal reporting. Operating results and assets are reviewed by the CODM primarily at the consolidated entity level for purposes of making resource allocation decisions and for evaluating financial performance. Accordingly, the Company has a single operating and reportable segment comprising all of the Company's operations.

The key measure of segment profit and loss that the CODM uses to allocate resources and assess performance is the Company's net loss. The CODM uses net loss to assess the Company's ongoing financial needs in relation to current resources in assessing performance and allocating resources.



The table below details the Company's revenues, significant expenses, and other segment items and reconciles those amounts to the Company's consolidated net loss as computed under U.S. GAAP in the Consolidated Statements of Operations:

	Year Ended December 31,	
	2024	2023
Revenues	\$ 57,800	\$ 176,232
Less:		
Research and development	70,289	108,497
General and administrative	37,827	45,322
Clinical manufacturing operations	35,345	97,697
Impairment of long-lived assets	5,521	65,528
Impairment of goodwill and indefinite-lived intangible assets	—	89,485
Stock-based compensation	12,382	27,363
Other segment items <sup>(*)</sup>	(5,623)	171
Net loss	<u>\$ (97,941)</u>	<u>\$ (257,831)</u>

<sup>(\*)</sup> Other segment items include restructuring charges, interest and other income, net, and income tax benefit.



## **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**

We maintain disclosure controls and procedures that are designed to provide reasonable assurance that information required to be disclosed in our Exchange Act reports 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 principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Under the supervision of our principal executive officer and principal financial 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, 2024. Based on that evaluation, as of December 31, 2024, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

### **Inherent Limitations on Controls and Procedures**

Our management, including the principal executive officer and principal financial officer, does not expect that our disclosure controls and procedures and our internal control over financial reporting 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, for our company have been or will be detected. As these inherent limitations are known features of the disclosure and financial reporting processes, it is possible to design into the processes 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 also 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 and our internal control over financial reporting 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.

### **Management's Report on Internal Control over Financial Reporting**

Our management is responsible for establishing and maintaining an adequate internal control over financial reporting (as such term is defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) for our company. Our management, including our principal executive officer and principal financial officer, conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework set forth in the "Internal Control—Integrated Framework" issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on an evaluation under that framework, our management concluded that our internal control over financial reporting was effective at the reasonable assurance level as of December 31, 2024.

The effectiveness of our internal control over financial reporting as of December 31, 2024 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report, which is included herein.

### **Changes in Internal Control over Financial Reporting**

There have been no changes in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended December 31, 2024 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 the Board of Directors of Sangamo Therapeutics, Inc.

### **Opinion on Internal Control over Financial Reporting**

We have audited Sangamo Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2024, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Sangamo Therapeutics, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the 2024 consolidated financial statements of the Company and our report dated March 17, 2025 expressed an unqualified opinion thereon.

### **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 included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and 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/ Ernst & Young LLP

San Mateo, California  
March 17, 2025



## **ITEM 9B – OTHER INFORMATION**

None.

## **ITEM 9C – DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS**

None.

## **PART III**

## **ITEM 10 – DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE**

The information required by this item is to be included in our 2025 Proxy Statement as follows:

- The information relating to our directors and nominees for director is to be included in the section entitled “Election of Directors;”
- The information relating to our executive officers is to be included in the section entitled “Executive Officers;”
- The information relating to our audit committee and audit committee financial expert is to be included in the section entitled “Election of Directors – Board Committees and Meetings – Audit Committee;”
- The information relating to the procedures by which stockholders may recommend nominees to our Board of Directors is to be included in the section entitled “Questions and Answers About These Proxy Materials and Voting;”
- The information relating to insider trading policies and procedures; and
- If required, the information regarding compliance with Section 16(a) of the Exchange Act is to be included in the section entitled “Delinquent Section 16(a) Reports.”

Such information is incorporated herein by reference to our 2025 Proxy Statement, provided that if the 2025 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

### **Code of Conduct**

We maintain a Code of Conduct approved by our board of directors, which is applicable to all employees, including our executive officers, and our directors. A copy of our Code of Conduct is available on our website at <https://investor.sangamo.com/corporate-governance/governance-overview> in the Investors & Media Section under Corporate Governance. In the event that we make any future amendments to or grant any waivers of a provision of the Code of Conduct that requires disclosure under applicable SEC rules, we intend to disclose such amendment or waiver and the reasons therefor on our website.

Certain information required by Part III is omitted from this Report on Form 10-K because we intend to file our definitive Proxy Statement for our next Annual Meeting of Stockholders, pursuant to Regulation 14A of the Securities Exchange Act of 1934, as amended, or the 2025 Proxy Statement, no later than 120 days following the end of the fiscal year covered by this Annual Report on Form 10-K, and certain information to be included in the 2025 Proxy Statement is incorporated herein by reference.

## **ITEM 11 – EXECUTIVE COMPENSATION**

The information required by this item is to be included in our 2025 Proxy Statement under the sections entitled “Executive Compensation” (excluding the information presented under the subheading “Pay Versus Performance”) and “Election of Directors – Director Compensation,” and is incorporated herein by reference, provided that if the 2025 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

## **ITEM 12 – SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS**

The information required by this item with respect to equity compensation plans is to be included in our 2025 Proxy Statement under the section entitled “Equity Compensation Plan Information” and the information required by this item with respect to security ownership of certain beneficial owners and management is to be included in our 2025 Proxy Statement under



the section entitled “Security Ownership of Certain Beneficial Owners and Management” and in each case is incorporated herein by reference, provided that if the 2025 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

#### **ITEM 13 – CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE**

The information required by this item is to be included in our 2025 Proxy Statement under the sections entitled “Certain Relationships and Related Party Transactions” and “Election of Directors—Board Independence” and is incorporated herein by reference, provided that if the 2025 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

#### **ITEM 14 – PRINCIPAL ACCOUNTING FEES AND SERVICES**

The information required by this item is to be included in our 2025 Proxy Statement under the section entitled “Ratification of Independent Registered Public Accounting Firm” and is incorporated herein by reference, provided that if the 2025 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.



## PART IV

### ITEM 15 – EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) The following documents are included as part of this Annual Report on Form 10-K:

1. Financial Statements—See Index to Consolidated Financial Statements in Item 8.
2. Financial Statement Schedules—Not Applicable.
3. Exhibits

<u>Exhibit Number</u>	<u>Description of Document</u>
3.1	Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.3 to the Company’s Current Report on Form 8-K filed June 2, 2023).
3.2	Fifth Amended and Restated Bylaws (incorporated by reference to Exhibit 3.1 to the Company’s Current Report on Form 8-K filed December 19, 2022).
4.1	Description of Capital Stock
4.2	Form of Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company’s Current Report on Form 8-K filed January 6, 2017).
10.1(+)	Amended and Restated 2013 Stock Incentive Plan (the “2013 Plan”) (incorporated by reference to Exhibit 10.2 to the Company’s Quarterly Report on Form 10-Q filed May 10, 2018).
10.2(+)	Amended and Restated 2018 Equity Incentive Plan (the “2018 Plan”) (incorporated by reference to Exhibit 10.1 to the Company’s Current Report on Form 8-K filed June 2, 2023).
10.3(+)	2018 Equity Incentive Plan French Stock-Options Sub-Plan (the “French Options Sub-Plan”) (incorporated by reference to Exhibit 10.3 to the Company’s Annual Report on Form 10-K filed March 1, 2019).
10.4(+)	2018 Equity Incentive Plan French Restricted Stock Unit Award Sub-Plan (the “French RSU Sub-Plan”) (incorporated by reference to Exhibit 10.4 to the Company’s Annual Report on Form 10-K filed March 1, 2019).
10.5(+)	2020 Employee Stock Purchase Plan (incorporated by reference to Exhibit 99.1 to the Company’s Registration Statement on Form S-8 filed October 15, 2020).
10.6(+)	Form of Restricted Stock Unit Award Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.2 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.7(+)	Form of Notice of Grant of Stock Option under the 2013 Plan (incorporated by reference to Exhibit 10.3 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.8(+)	Form of Stock Option Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.4 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.9(+)	Form of Notice of Grant of Stock Option – Director Initial Grant under the 2013 Plan (incorporated by reference to Exhibit 10.5 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.10(+)	Form of Notice of Grant of Stock Option – Director Annual Grant under the 2013 Plan (incorporated by reference to Exhibit 10.6 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.11(+)	Form of Automatic Stock Option Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.7 to the Company’s Current Report on Form 8-K filed June 14, 2013).
10.12(+)	Form of Stock Option Grant Notice and Form of Option Agreement (U.S. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.2 to the Company’s Current Report on Form 8-K filed June 15, 2018).
10.13(+)	Form of Stock Option Grant Notice and Form of Option Agreement (non-employee directors) under the 2018 Plan (incorporated by reference to Exhibit 99.3 to the Company’s Current Report on Form 8-K filed June 15, 2018).
10.14(+)	Form of Stock Option Grant Notice and Form of Option Agreement (U.K. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.4 to the Company’s Current Report on Form 8-K filed June 15, 2018).
10.15(+)	Form of Stock Option Grant Notice (French employees) under the 2018 Plan and the French Options Sub-Plan (incorporated by reference to Exhibit 10.14 to the Company’s Annual Report on Form 10-K filed March 1, 2019).
10.16(+)	Form of Stock Option Agreement (French Employees) under the 2018 Plan and the French Options Sub-Plan (incorporated by reference to Exhibit 10.15 to the Company’s Annual Report on Form 10-K filed March 1, 2019).



<b><u>Exhibit Number</u></b>	<b><u>Description of Document</u></b>
10.17(+)	Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (U.S. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.5 to the Company's Current Report on Form 8-K filed June 15, 2018).
10.18(+)	Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (non-employee directors) under the 2018 Plan (incorporated by reference to Exhibit 99.6 to the Company's Current Report on Form 8-K filed June 15, 2018).
10.19(+)	Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (U.K. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.7 to the Company's Current Report on Form 8-K filed June 15, 2018).
10.20(+)	Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (French employees) under the 2018 Plan and the French RSU Sub-Plan. (incorporated by reference to Exhibit 10.19 to the Company's Annual Report on Form 10-K filed March 1, 2019).
10.21(+)	Amended and Restated Executive Severance Plan of Sangamo Therapeutics, Inc. (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 1, 2023).
10.22(+)	Amended and Restated Incentive Compensation Plan (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed May 10, 2018).
10.23(+)	Form of Indemnity Agreement (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed May 11, 2020).
10.24(+)	Employment Agreement between the Company and Alexander (Sandy) Macrae, dated May 17, 2016 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 4, 2016).
10.25(+)	Letter Agreement between the Company and Prathyusha Duraibabu dated as of May 21, 2021 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 5, 2021).
10.26(+)	Letter Agreement between the Company and Scott Willoughby dated as of August 2, 2021 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 4, 2021).
10.27(+)	Letter Agreement between the Company and Nathalie Dubois-Stringfellow dated as of September 28, 2022 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 3, 2022).
10.28(+)	Letter Agreement Regarding Alexander (Sandy) Macrae Cash Retention Award
10.29(+)	Letter Agreement Regarding Prathyusha Duraibabu Cash Retention Award
10.30	Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated May 23, 1997 (incorporated by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 filed February 24, 2000).
10.31	First Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated March 12, 2004 (incorporated by reference to Exhibit 10.20 to the Company's Annual Report on Form 10-K filed February 23, 2005).
10.32	Second Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated March 15, 2007 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 4, 2013).
10.33	Third Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated August 1, 2013 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed November 4, 2013).
10.34	Fourth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated June 10, 2016 (incorporated by reference to Exhibit 10.33 to the Company's Annual Report on Form 10-K filed March 1, 2019).
10.35	Fifth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated July 10, 2017 (incorporated by reference to Exhibit 10.34 to the Company's Annual Report on Form 10-K filed March 1, 2019).
10.36	Sixth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated May 11, 2018 (incorporated by reference to Exhibit 10.9 to the Company's Quarterly Report on Form 10-Q filed August 8, 2018).
10.37	Seventh Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated May 20, 2020 (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).



<b><u>Exhibit Number</u></b>	<b><u>Description of Document</u></b>
10.38	Eighth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated May 29, 2020 (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).
10.39	Ninth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated January 4, 2021 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed May 4, 2021).
10.40	Amended and Restated Office and Laboratory Lease between the Company and Point Richmond R&D Associates II, LLC, dated October 18, 2021 (incorporated by reference to Exhibit 10.34 to the Company's Annual Report on Form 10-K filed February 24, 2022).
10.41	Lease Agreement between the Company and Marina Boulevard Property, LLC dated November 3, 2017 (incorporated by reference to Exhibit 10.21 to the Company's Annual Report on Form 10-K filed March 1, 2018).
10.42	First Amendment to Lease Agreement between the Company and Marina Boulevard Property, LLC dated January 1, 2019 (incorporated by reference to Exhibit 10.37 to the Company's Annual Report on Form 10-K filed March 1, 2019).
10.43	Second Amendment to Lease Agreement between the Company and PPF OFF 7000 Marina Boulevard LP dated February 5, 2024 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed May 9, 2024)
10.44	Third Amendment to Lease Agreement between the Company and PPF OFF 7000 Marina Boulevard LP dated July 3, 2024 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed November 12, 2024).
10.45	Open Market Sale Agreement between the Company and Jefferies LLC, dated August 5, 2020 (incorporated by reference to Exhibit 1.1 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).
10.46	Amendment No. 1 to Open Market Sale Agreement between the Company and Jefferies LLC, dated May 5, 2021 (incorporated by reference to Exhibit 1.3 to the Company's Registration Statement on Form S-3 filed May 5, 2021).
10.47	Amendment No. 2 to Open Market Sale Agreement between the Company and Jefferies LLC, dated December 23, 2022 (incorporated by reference to Exhibit 1.1 to the Company's Current Report on Form 8-K filed December 23, 2022).
10.48	Amendment No. 3 to Open Market Sale Agreement between the Company and Jefferies LLC, dated March 7, 2023 (incorporated by reference to Exhibit 1.1 to the Company's Quarterly Report on Form 10-Q filed May 8, 2023).
10.49	Amendment No. 4 to the Open Market Sale Agreement between the Company and Jefferies LLC, dated November 12, 2024 (incorporated by reference to Exhibit 1.2 to the Company's Registration Statement on Form S-3 filed November 12, 2024).
10.50†	Amended and Restated Collaboration and License Agreement between the Company and Shire International GmbH, dated September 1, 2015 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed October 30, 2015).
10.51*	Collaboration and License Agreement between the Company and Pfizer Inc., dated May 10, 2017 (incorporated by reference to Exhibit 10.53 to the Company's Annual Report on Form 10-K filed February 23, 2023).
10.52*	Letter Amendment, dated December 17, 2019, to the Collaboration and License Agreement between the Company and Pfizer Inc., dated May 10, 2017 (incorporated by reference to Exhibit 10.45 to the Company's Annual Report on Form 10-K filed February 28, 2020).
10.53†	Research Collaboration and License Agreement between the Company and Pfizer Inc., dated December 28, 2017 (incorporated by reference to Exhibit 10.40 to the Company's Annual Report on Form 10-K filed March 1, 2018).
10.54†	Amendment No. 1 to Research Collaboration and License Agreement between the Company and Pfizer Inc., dated March 21, 2019 (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed May 8, 2019).
10.55*	Amendment No. 2 to Research Collaboration and License Agreement between the Company and Pfizer Inc., dated July 31, 2020 (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed November 4, 2020).
10.56	Form of Securities Purchase Agreement, dated March 21, 2024 by and among the Company and the purchasers party thereto (incorporated by reference to Exhibit 10.1 of the Company's Current Report on Form 8-K filed on March 25, 2024).



<u>Exhibit Number</u>	<u>Description of Document</u>
10.57*	License Agreement between the Company and Genentech, Inc., dated August 2, 2024 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 12, 2024).
19.1	Insider Trading Policy.
21.1	Subsidiaries of the Company.
23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney (included on signature page).
31.1	Rule 13a-14(a) Certification of Principal Executive Officer.
31.2	Rule 13a-14(a) Certification of Principal Financial Officer.
32.1*	Certification Pursuant to 18 U.S.C. Section 1350.
97	Incentive Compensation Recoupment Policy
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page from Sangamo's Annual Report on Form 10-K for the year ended December 31, 2024, is formatted in Inline XBRL and it is contained in Exhibit 101

† *Confidential treatment has been granted for certain information contained in this document pursuant to an order of the SEC. Such information has been omitted and filed separately with the SEC.*

\* Certain portions of this exhibit (indicated by "[\*]") have been omitted in accordance with 17 CFR § 229.601(b).

(+) Indicates management contract or compensatory plan or arrangement.

\* *The certifications attached as Exhibit 32.1 accompany this Annual Report on Form 10-K pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Company for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.*

The agreements and other documents filed as exhibits to this Annual Report on Form 10-K are not intended to provide factual information or other disclosure other than with respect to the terms of the agreements or other documents themselves, and you should not rely on them for that purpose. In particular, any representations and warranties made by us in these agreements or other documents were made solely within the specific context of the relevant agreement or document and may not describe the actual state of affairs as of the date they were made or at any other time.

## ITEM 16 – FORM 10-K SUMMARY

None.



## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized, on March 17, 2025.

Date: March 17, 2025

SANGAMO THERAPEUTICS, INC.

By: / S / ALEXANDER D. MACRAE

**Alexander D. Macrae**  
*President and Chief Executive Officer*

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Alexander D. Macrae and Scott Willoughby, and each of them, as his or her true and lawful attorneys-in-fact and agents, each 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 (including post-effective 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, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their, 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:

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/ S / ALEXANDER D. MACRAE</u> <b>Alexander D. Macrae, M.B., Ch.B., Ph.D.</b>	President, Chief Executive Officer (Principal Executive Officer) and Director	March 17, 2025
<u>/ S / PRATHYUSHA DURAIBABU</u> <b>Prathyusha Duraibabu</b>	Senior Vice President and Chief Financial Officer (Principal Financial and Accounting Officer)	March 17, 2025
<u>/ S / H. STEWART PARKER</u> <b>H. Stewart Parker</b>	Director and Chair of the Board	March 17, 2025
<u>/ S / COURTNEY BEERS</u> <b>Courtney Beers, Ph.D.</b>	Director	March 17, 2025
<u>/ S / ROBERT F. CAREY</u> <b>Robert F. Carey</b>	Director	March 17, 2025
<u>/ S / KENNETH J. HILLAN</u> <b>Kenneth J. Hillan, M.B., Ch.B.</b>	Director	March 17, 2025
<u>/ s / MARGARET A. HORN</u> <b>Margaret A. Horn, J.D.</b>	Director	March 17, 2025
<u>/ S / JOHN H. MARKELS</u> <b>John H. Markels, Ph.D.</b>	Director	March 17, 2025
<u>/ S / JAMES R. MEYERS</u> <b>James R. Meyers</b>	Director	March 17, 2025
<u>/ s / KAREN L. SMITH</u> <b>Karen L. Smith, M.D., Ph.D., M.B.A., L.L.M.</b>	Director	March 17, 2025











