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# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION
**WASHINGTON, DC 20549**

# FORM 10-Q

**(Mark One)**

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

**For the quarterly period ended June 30, 2026**

**OR**

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

**For the transition period from to**

**Commission File Number: 001-43409**

# SCRIBE THERAPEUTICS INC.

**(Exact Name of Registrant as Specified in its Charter)**

| **Delaware** | **82-2157847** |
|---|---|
| **(State or other jurisdiction of incorporation or organization)** | **(I.R.S. Employer Identification No.)** |
| **1150 Marina Village Parkway Alameda, California** | **94501** |
| **(Address of principal executive offices)** | **(Zip Code)** |

**Registrant's telephone number, including area code: (510) 626-8587**

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

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common stock, par value $0.001 per share | SCTX | The Nasdaq Stock Market LLC |

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

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

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

| | | | |
|---|---|---|---|
| Large accelerated filer | ☐ | Accelerated filer | ☐ |
| Non-accelerated filer | ☒ | Smaller reporting company | ☒ |
| Emerging growth company | ☒ | | |

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

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

As of August 31, 2026, the registrant had 18,931,680 shares of common stock, $0.001 par value per share, outstanding.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended. In some cases, you can identify forward-looking statements by terms such as "believe," "may," "will," "potentially," "estimate," "continue," "anticipate," "intend," "could," "would," "project," "plan," "expect" and similar expressions that convey uncertainty of future events or outcomes, although not all forward-looking statements contain these words. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the section titled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. Moreover, we operate in a competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

The forward-looking statements in this prospectus include, among other things, statements about:

- our ability to advance our lead product candidate, STX-1150, and our developmental programs STX-1200 and STX-1400 to address the key drivers of ASCVD;
- our plans to engineer, develop and commercialize potential genetic medicines with our proprietary CRISPR-based technologies, ELXR and XE;
- our ability to leverage ELXR and XE to efficiently produce product candidates at a rapid pace and continuously advance our genetic modification platforms;
- our expectations regarding collaboration and licensing arrangements with third parties, including our ability to reach development milestones under such agreements;
- estimates of the addressable market for our potential genetic medicines and any future product candidates and the therapeutic impact of our potential genetic medicines;
- our expectations regarding demand for, and market acceptance of, our potential genetic medicines and platforms;
- our ability to market or commercialize any product candidates we may develop and to compete effectively with existing competitors and new market entrants;
- the potential effects of extensive government regulations relating to the genetic medicine industry;
- our ability to obtain, maintain, protect and enforce intellectual property and proprietary rights;
- our ability to operate our business without infringing, misappropriating or otherwise violating the intellectual property rights and proprietary technology of third parties;
- our ability to attract and retain key management and technical personnel;
- general economic, industry and market conditions, including fluctuating interest rates and inflation, cybersecurity incidents, significant political, trade or regulatory developments, including tariffs or shifting priorities within the FDA, and global and regional conflicts on our operations and the receipt and timing of potential regulatory designations;
- our expectations regarding expenses, future revenue, capital requirements and our needs for additional financing; and
- our expected use of the net proceeds from our initial public offering, the concurrent private placement, and our existing cash, cash equivalents, and investments.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Quarterly Report on Form 10-Q.

The forward-looking statements made in this Quarterly Report on Form 10-Q relate only to events or information as of the date on which the statements are made in this Quarterly Report on Form 10-Q. You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update

publicly any forward-looking statements for any reason after the date of this Quarterly Report on Form 10-Q to conform these statements to actual results or to changes in our expectations, except as required by law.

You should read this Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed with the Securities and Exchange Commission, or SEC, as exhibits to the registration statement of which this Quarterly Report on Form 10-Q is a part with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and you are cautioned not to unduly rely upon these statements.

## PART I—FINANCIAL INFORMATION

**Item 1. Financial Statements.**

**SCRIBE THERAPEUTICS INC.**
**CONDENSED BALANCE SHEETS**
**(In thousands, except share and per share amounts)**
**(Unaudited)**

| | June 30, 2026 | December 31, 2025 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 35,586 | $ 12,796 |
| Short-term investments | 7,423 | 45,167 |
| Receivable from collaboration partners | 1,107 | 7,092 |
| Prepaid expenses and other current assets | 989 | 1,879 |
| Total current assets | 45,105 | 66,934 |
| Operating lease right-of-use asset | 2,782 | 3,064 |
| Property and equipment, net | 5,755 | 7,008 |
| Restricted cash | 675 | 675 |
| Other long-term assets | 3,610 | 538 |
| Total assets | $ 57,927 | $ 78,219 |
| Liabilities, redeemable convertible preferred stock, and stockholders' deficit | | |
| Current liabilities: | | |
| Accounts payable | $ 2,771 | $ 4,566 |
| Accrued expenses and other current liabilities | 7,183 | 6,405 |
| Accrued license fees | 2,100 | 375 |
| Deferred revenue, current | 27,475 | 21,087 |
| Convertible Note | 37,799 | 35,339 |
| Operating lease liabilities, current portion | 1,369 | 1,285 |
| Total current liabilities | 78,697 | 69,057 |
| Operating lease liabilities, net of current portion | 4,102 | 4,804 |
| Other non-current liabilities | 7,920 | 7,485 |
| Deferred revenue, non-current | 11,698 | 19,936 |
| Total liabilities | 102,417 | 101,282 |
| Redeemable convertible preferred stock, $0.001 par value; 29,182,118 shares authorized, and 4,915,299 shares issued and outstanding as of June 30, 2026 and December 31, 2025, respectively; liquidation preference of $120,705 as of June 30, 2026 and December 31, 2025 | 120,356 | 120,356 |
| Common stock, $0.001 par value, 52,000,000 shares authorized as of June 30, 2026 and December 31, 2025; 2,485,234 and 2,451,167 shares issued and outstanding as of June 30, 2026 and December 31, 2025 | 14 | 14 |
| Additional paid-in capital | 16,673 | 14,239 |
| Accumulated deficit | (181,531) | (157,707) |
| Accumulated other comprehensive income | (2) | 35 |
| Total stockholders' deficit | (164,846) | (143,419) |
| Total liabilities, preferred stock, and stockholders' deficit | $ 57,927 | $ 78,219 |

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

**SCRIBE THERAPEUTICS INC.**
**CONDENSED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**
**(In thousands, except share and per share amounts)**
**(Unaudited)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2026 | 2025 | 2026 | 2025 |
| Collaboration revenue | $ 1,918 | $ 4,898 | $ 4,151 | $ 22,023 |
| Operating expenses: | | | | |
| Research and development | 8,823 | 13,884 | 20,128 | 29,876 |
| General and administrative | 2,490 | 2,641 | 5,844 | 9,332 |
| Total operating expenses | 11,313 | 16,525 | 25,972 | 39,208 |
| Loss from operations | (9,395) | (11,627) | (21,821) | (17,185) |
| Interest income and other income (expense), net | 383 | 880 | 898 | 1,944 |
| Interest expense | (276) | (598) | (875) | (1,197) |
| Change in fair value of Convertible Note | 3,023 | 730 | (1,585) | 2,083 |
| Net loss before provision for income taxes | (6,265) | (10,615) | (23,383) | (14,355) |
| (Provision for) / benefit from income tax | (212) | 712 | (441) | 1,027 |
| Net loss | $ (6,477) | $ (9,903) | $ (23,824) | $ (13,328) |
| Other comprehensive loss: | | | | |
| Net unrealized loss on available-for-sale investments | (2) | (36) | (37) | (93) |
| Net comprehensive loss | $ (6,479) | $ (9,939) | $ (23,861) | $ (13,421) |
| Net loss per share attributable to common stockholders, basic and diluted | $ (2.62) | $ (4.08) | $ (9.65) | $ (5.50) |
| Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted | 2,475,898 | 2,429,130 | 2,468,343 | 2,423,315 |

*The accompanying notes are an integral part of these condensed financial statements*

**SCRIBE THERAPEUTICS INC.**
**CONDENSED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT**
**(In thousands, except share amounts)**
**(Unaudited)**

| | Redeemable Convertible Preferred Stock | | Common Stock | | Additional Paid-In | Accumulated Other Comprehensive Income (Loss) | Accumulated Deficit | Total Stockholders' Deficit |
|---|---|---|---|---|---|---|---|---|
| | Shares | Amount | Shares | Amount | Capital | | | |
| **Balance at December 31, 2025** | 4,915,299 | $ 120,356 | 2,451,167 | $ 14 | $ 14,239 | $ 35 | $ (157,707) | $ (143,419) |
| Issuance of common stock upon exercise of stock options | — | — | 17,026 | — | 93 | — | — | $ 93 |
| Stock-based compensation expense | — | — | — | — | 1,970 | — | — | $ 1,970 |
| Net loss | — | — | — | — | — | — | (17,347) | $ (17,347) |
| Net unrealized loss on available-for-sale investments | — | — | — | — | — | (35) | — | $ (35) |
| **Balance at March 31, 2026** | 4,915,299 | $ 120,356 | 2,468,193 | $ 14 | $ 16,302 | $ — | $ (175,054) | $ (158,738) |
| Issuance of common stock upon exercise of stock options | — | — | 17,041 | — | 144 | — | — | $ 144 |
| Stock-based compensation expense | — | — | — | — | 227 | — | — | $ 227 |
| Net loss | — | — | — | — | — | — | (6,477) | $ (6,477) |
| Net unrealized loss on available-for-sale investments | — | — | — | — | — | (2) | — | $ (2) |
| **Balance at June 30, 2026** | 4,915,299 | $ 120,356 | 2,485,234 | $ 14 | $ 16,673 | $ (2) | $ (181,531) | $ (164,846) |

| | Redeemable Convertible Preferred Stock | | Common Stock | | Additional Paid-In | Accumulated Other Comprehensive Income (Loss) | Accumulated | Total Stockholders' |
|---|---|---|---|---|---|---|---|---|
| | Shares | Amount | Shares | Amount | Capital | | Deficit | Deficit |
| **Balance at December 31, 2024** | 4,915,299 | $ 120,356 | 2,417,435 | $ 14 | $ 9,954 | $ 72 | $ (135,899) | $ (125,859) |
| Issuance of common stock upon exercise of stock options | — | — | — | — | — | — | — | $ — |
| Stock-based compensation expense | — | — | — | — | 671 | — | — | $ 671 |
| Net loss | — | — | — | — | — | — | (3,425) | $ (3,425) |
| Net unrealized loss on available-for-sale investments | — | — | — | — | — | (57) | — | $ (57) |
| **Balance at March 31, 2025** | 4,915,299 | $ 120,356 | 2,417,435 | $ 14 | $ 10,625 | $ 15 | $ (139,324) | $ (128,670) |
| Issuance of common stock upon exercise of stock options | — | — | 13,521 | — | 51 | — | — | $ 51 |
| Stock-based compensation expense | — | — | — | — | 676 | — | — | $ 676 |
| Net loss | — | — | — | — | — | — | (9,903) | $ (9,903) |
| Net unrealized loss on available-for-sale investments | — | — | — | — | — | (36) | — | $ (36) |
| **Balance at June 30, 2025** | 4,915,299 | $ 120,356 | 2,430,956 | $ 14 | $ 11,352 | $ (21) | $ (149,227) | $ (137,882) |

*The accompanying notes are an integral part of these condensed financial statements*

**SCRIBE THERAPEUTICS INC.**
**CONDENSED STATEMENTS OF CASH FLOWS**
**(In thousands)**
**(Unaudited)**

| | Six Months Ended June 30, | |
|---|---|---|
| | 2026 | 2025 |
| **Cash flows from operating activities** | | |
| Net loss | $ (23,824) | $ (13,328) |
| Adjustments to reconcile net loss to net cash used in operating activities | | |
| Depreciation expense | 1,277 | 1,468 |
| Loss on disposal of equipment | 4 | 5 |
| Stock-based compensation expense | 2,197 | 1,347 |
| Interest expense | 875 | 1,197 |
| Change in fair value of convertible note | 1,585 | (2,083) |
| Net amortization/accretion on investment securities | 210 | 474 |
| Amortization of right-of-use asset | 281 | 230 |
| Expensed deferred offering costs | — | 3,583 |
| Cash received from government grants | 3,830 | — |
| Changes in operating assets and liabilities: | | |
| Receivables from collaboration partners | 5,985 | 292 |
| Prepaid expenses and other current assets | 890 | 445 |
| Other long-term assets | (520) | (209) |
| Accounts payable | (2,139) | 1,193 |
| Deferred revenue | (1,850) | (11,880) |
| Accrued license fees | 1,725 | (750) |
| Accruals and other current liabilities | (3,236) | (479) |
| Operating lease liabilities | (616) | (531) |
| Other non-current liabilities | 434 | (1,226) |
| Net cash used in operating activities | (12,892) | (20,252) |
| **Cash flows from investing activities** | | |
| Purchases of property and equipment | (27) | (930) |
| Maturities of investments | 42,339 | 56,231 |
| Purchases of investments | (4,842) | (31,722) |
| Net cash provided by investing activities | 37,470 | 23,579 |
| **Cash flows from financing activities** | | |
| Proceeds from exercise of stock options | 238 | 51 |
| Payments of deferred offering costs | (2,026) | — |
| Net cash (used in) / provided by financing activities | (1,788) | 51 |
| Net change in cash, cash equivalents and restricted cash | 22,790 | 3,378 |
| Cash, cash equivalents and restricted cash, at the beginning of the period | 13,471 | 13,277 |
| Cash, cash equivalents and restricted cash, at the end of the period | 36,261 | 16,655 |
| **Supplemental disclosure of non-cash investing and financing activities:** | | |
| Unpaid fixed asset acquisitions | $ — | $ 306 |
| Deferred offering costs related to initial public offering included in accounts payable and accrued expenses and other current liabilities | $ 975 | $ — |

*The accompanying notes are an integral part of these condensed financial statements*

**SCRIBE THERAPEUTICS INC.**
**NOTES TO UNAUDITED CONDENSED FINANCIAL STATEMENTS**

## 1. Business Organization and Liquidity

Scribe Therapeutics Inc. (the "Company") is a clinical-stage biotechnology company engineering purpose-built *in vivo* CRISPR technologies designed to extend healthy lifespan through disease prevention and durable therapeutic intervention. The Company was incorporated in the state of Delaware in June 2017. Its principal offices are in Alameda, California.

### *Liquidity and Going Concern*

The accompanying unaudited condensed financial statements have been prepared under the assumption that the Company will continue as a going concern.

Since inception, the Company has devoted substantially all of its efforts to research and development activities, establishing and protecting its intellectual property, recruiting personnel, raising capital and conducting business planning. The Company has funded its operations primarily through proceeds received from collaboration and license agreements and the issuance of redeemable convertible preferred stock.

As of June 30, 2026, the Company had cash, cash equivalents and available-for-sale investments of $43.0 million. The Company has incurred recurring losses and negative cash flows from operations since inception. Net losses were $23.8 million and $13.3 million for the six months ended June 30, 2026 and 2025, respectively. As of June 30, 2026, the Company had an accumulated deficit of $181.5 million.

On July 27, 2026, the Company completed its initial public offering ("IPO") of 9,867,000 shares of common stock, which included the full exercise by the underwriters of their option to purchase an additional 1,287,000 shares, at a public offering price of $15.00 per share. Gross proceeds from the IPO were approximately $148.0 million, before underwriting discounts, commissions and offering expenses. Concurrently with the IPO, the Company completed a private placement of 500,000 shares of common stock to an affiliate of Sanofi at a price of $15.00 per share, resulting in gross proceeds of $7.5 million. In aggregate, the IPO and concurrent private placement generated approximately $155.5 million of gross proceeds.

After deducting underwriting discounts, commissions and other offering costs, the Company received net proceeds of approximately $140.7 million from the IPO and concurrent private placement. The Company believes that its existing cash, cash equivalents and available-for-sale investments, together with the net proceeds from the IPO and the concurrent private placement, will be sufficient to fund its planned operating expenses and capital expenditure requirements for at least the next twelve months from the issuance date of these financial statements.

Accordingly, management concluded that the substantial doubt about the Company's ability to continue as a going concern that existed as of June 30, 2026 was alleviated prior to the issuance of these financial statements as a result of the completion of the IPO and concurrent private placement.

## 2. Summary of significant accounting policies

### *Basis of Preparation*

The accompanying condensed financial statements have been prepared in accordance with United States generally accepted accounting principles ("GAAP") and applicable rules and regulations of the Securities and Exchange Commission ("SEC") regarding interim financial reporting. The condensed financial statements were also prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the ordinary course of business. Since inception, the Company has not generated any product revenue, and its operations have primarily consisted of establishing facilities, recruiting personnel, conducting research and development, and raising capital. Certain information and footnote disclosures normally included in the condensed financial statements prepared in accordance with U.S. GAAP have been condensed or omitted in accordance with such rules and regulations.

The significant accounting policies and estimates used in the preparation of these financial statements are consistent with those described in the Company's audited financial statements for the year ended December 31, 2025. During the current interim period, there have been no material changes to these policies.

### *Reclassifications*

Certain prior period amounts have been reclassified to conform with the current period presentation. The reclassifications have no impact on the Company's total assets, total liabilities, stockholders' equity, net loss, comprehensive

income (loss) or cash flows.

***Reverse Stock Split***

On July 17, 2026, the Company amended its amended and restated certificate of incorporation and effected a 5.9218-for-1 reverse stock split of its issued and outstanding common stock (the "Reverse Stock Split"). Accordingly, every 5.9218 shares of common stock were combined into one share of common stock. Fractional shares resulting from the Reverse Stock Split were rounded in accordance with the terms of the amendment to the Company's certificate of incorporation.

All common stock share amounts, stock option and warrant shares, conversion ratios, shares reserved for future issuance, net loss per share, and other per share information presented in the accompanying financial statements and notes thereto have been retroactively adjusted to reflect the Reverse Stock Split for all periods presented. The par value of the Company's common stock was not affected by the Reverse Stock Split.

***Use of Estimates***

The preparation of condensed financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed financial statements, and the reported amounts of expenses during the reporting period. On an ongoing basis, the Company evaluates estimates and assumptions, including but not limited to those related to revenue recognition, lease liabilities, fair value of redeemable convertible preferred stock and common stock, stock-based compensation expense, accruals for research and development costs, the valuations of deferred tax assets, the fair value of the convertible note and uncertain income tax positions. Management bases its estimates on historical experience and on various other assumptions that are believed 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 materially from those estimates.

***Government Grants***

The Company accounts for government grants received by business entities in accordance with ASC 832, Government Grants. Government grants are recognized when it is probable that the Company will comply with the conditions attached to the grant and that the grant will be received. Grants related to income are recognized on a systematic and rational basis over the periods in which the Company recognizes the related costs as expenses.

The Company has determined that its grants awarded by the California Institute for Regenerative Medicine ("CIRM") are grants related to income because the grants are intended to reimburse qualifying research and development expenditures and are not conditioned upon the acquisition or construction of long-lived assets. The Company has elected to present amounts recognized in earnings as a reduction of research and development expense in the condensed statements of operations.

Cash received in advance of incurring qualifying expenditures is recorded as a government grant liability within accrued expenses and other current liabilities. Grant receivables are recorded when qualifying expenditures have been incurred and collection is probable. Grant amounts are recognized only when the Company concludes it is probable that all applicable grant conditions will be satisfied and the grant proceeds will be received.

***Recently Adopted Accounting Pronouncements***

In September 2025, the FASB issued ASU 2025-07, Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606): Derivatives Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract. The amendments refine the scope of derivative accounting by excluding contracts or embedded features whose settlement amounts are based on the operations or activities specific to one of the parties to the contract and clarify the accounting for share-based noncash consideration received from a customer in a revenue contract. The Company early adopted ASU 2025-07 effective January 1, 2026. The adoption did not have a material impact on the Company's condensed financial statements.

In December 2025, the FASB issued ASU 2025-10, Government Grants (Topic 832): Accounting for Government Grants Received by Business Entities, which establishes recognition, measurement, presentation and disclosure requirements for government grants received by business entities. Under the guidance, a government grant is recognized when it is probable that the entity will comply with the conditions attached to the grant and that the grant will be received. The Company early adopted ASU 2025-10 effective January 1, 2026 using the modified prospective transition method. The Company adopted the guidance in connection with grants awarded by the California Institute for Regenerative Medicine ("CIRM") and accounts for such grants as income-related government grants under ASC 832. The Company elected to present grant amounts recognized in earnings as a reduction of research and development expense. The adoption of the standard did not have a material impact on the Company's condensed financial statements other than the establishment of accounting policies and related disclosures for government grants.

***Recently Issued Accounting Pronouncements Not Yet Adopted***

In November 2024, the FASB issued ASU 2024-03, Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40), which introduces new disclosure requirements to disaggregate certain natural expenses underlying income statement captions. ASU 2024-03 is effective for annual periods in fiscal years beginning after December 15, 2026, and for interim periods thereafter. Early adoption is permitted. The application of ASU 2024-03 is prospective for periods beginning after the effective date, although retrospective application to prior periods is allowed. The Company is currently assessing the impact that ASU 2024-03 will have on its condensed financial statements and disclosures.

***Concentration of Credit Risk***

The Company recognized revenue from collaboration partners in the three and six months ended June 30, 2026 and 2025, with all revenue generated within the United States. The percentages of collaboration revenue and accounts receivable from each of the Company's customers that individually accounted for 10% or more of its total collaboration revenue and accounts receivable were as follows:

| | Accounts Receivable, net | | Revenue | | Revenue | |
|---|---|---|---|---|---|---|
| | As of June 30, 2026 | As of December 31, 2025 | Three Months Ended June 30, 2026 | Three Months Ended June 30, 2025 | For the Six Months Ended June 30, 2026 | For the Six Months Ended June 30, 2025 |
| Prevail | 96% | 98% | 100% | 98% | 100% | 98% |

As of June 30, 2026 and December 31, 2025, the Company reported no contract assets. The Company routinely assesses its accounts receivable and contract assets for potential impairment and credit losses. As of June 30, 2026 and December 31, 2025, no impairment or credit loss allowance was recorded, respectively.

***Unaudited Interim Financial Information***

The accompanying condensed financial statements as of June 30, 2026, and for the three and six months ended June 30, 2026 and 2025, are unaudited. The condensed balance sheet as of December 31, 2025 was derived from the Company's audited financial statements included in the Company's final prospectus filed with the Securities and Exchange Commission on July 24, 2026 pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended. The unaudited interim condensed financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, consisting of only normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of June 30, 2026, and the results of its operations and its cash flows for the three and six months ended June 30, 2026 and 2025. The results for the three and six months ended June 30, 2026 are not necessarily indicative of the results to be expected for the full year ending December 31, 2026, or for any other future period. These unaudited interim condensed financial statements should be read in conjunction with the audited financial statements and related notes for the year ended December 31, 2025, included in the Company's final prospectus filed with the Securities and Exchange Commission on July 24, 2026 pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended.

**3. Fair Value Measurements**

The Company's financial assets and financial liabilities recognized at fair value consisted of the following (in thousands):

| | June 30, 2026 | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Fair Value |
| **Financial Assets:** | | | | |
| Money market funds | $ 35,508 | $ — | $ — | $ 35,508 |
| U.S. treasury securities | — | 7,423 | — | $ 7,423 |
| Total financial Assets | $ 35,508 | $ 7,423 | $ — | $ 42,931 |
| **Financial Liabilities:** | | | | |
| Convertible note | $ — | $ — | $ 37,799 | $ 37,799 |
| Total financial liabilities | $ — | $ — | $ 37,799 | $ 37,799 |

| | December 31, 2025 | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Fair Value |
| **Financial Assets:** | | | | |
| Money market funds | $ 12,695 | $ — | $ — | $ 12,695 |
| U.S. treasury securities | — | 45,167 | — | 45,167 |
| Total financial Assets | $ 12,695 | $ 45,167 | $ — | $ 57,862 |
| **Financial Liabilities:** | | | | |
| Convertible note | $ — | $ — | $ 35,339 | $ 35,339 |
| Total financial liabilities | $ — | $ — | $ 35,339 | $ 35,339 |

The Company's securities are valued using third-party pricing services or other observable market data. The pricing services utilize industry standard valuation models and observable market inputs to determine value. There have been no transfers within the hierarchies during the six months ended June 30, 2026.

The Company elected to measure the convertible note (Note 8) at fair value with changes in fair value reported in earnings as they occur. The convertible note fair value was determined using the discounted cash flows methodology based on probability weighted scenarios of the convertible note's conversion using Level 3 inputs not observable in the market. A significant increase (decrease) in these inputs would result in a lower (higher) fair value measurement. At issuance on May 11, 2023, the time to a conversion event ranged from 0.22 to 0.39 years and the discount rate applied was 29.1%. On December 31, 2025, the time to event ranged from 0.25 to 0.36 years, and the discount rate applied was 27.55%. The convertible note matured on May 11, 2026.

The following table provides a reconciliation of the beginning and ending balances of the convertible note fair value for the three and six months ended June 30, 2026 (in thousands):

| | Three months ended June 30, 2026 | Six Months Ended June 30, 2026 |
|---|---|---|
| | (in thousands) | (in thousands) |
| Fair value at the beginning of the period | $ 40,546 | $ 35,339 |
| Accrued stated interest | 276 | 875 |
| Change in fair value | (3,023) | 1,585 |
| Fair value at end of period | $ 37,799 | $ 37,799 |

**4. Marketable Securities**

The fair value and amortized cost of cash equivalents and available-for-sale investments, by major security type as of June 30, 2026 and December 31, 2025 are presented in the following tables (in thousands):

| | June 30, 2026 | | | |
|---|---|---|---|---|
| | Amortized Cost | Unrealized Gains | Unrealized Losses | Fair Value |
| **Financial assets:** | | | | |
| Money market funds | $ 35,508 | $ — | $ — | $ 35,508 |
| U.S. treasury securities | 7,424 | 1 | (2) | $ 7,423 |
| Total cash equivalents and investments | $ 42,932 | $ 1 | $ (2) | $ 42,931 |
| **Classified as:** | | | | |
| Cash equivalents | | | | 35,508 |
| Short-term investments | | | | 7,423 |
| Total cash equivalents and investments | | | | $ 42,931 |

As of June 30, 2026, total cash, cash equivalents and available-for-sale investments of $43.0 million includes cash equivalents and marketable securities of $42.9 million plus cash of $0.1 million.

| | December 31, 2025 | | | |
|---|---|---|---|---|
| | Amortized Cost | Unrealized Gains | Unrealized Losses | Fair Value |
| **Financial assets:** | | | | |
| Money market funds | $ 12,695 | $ — | $ — | $ 12,695 |
| U.S. treasury securities | 45,132 | 35 | — | 45,167 |
| Total cash equivalents and investments | $ 57,827 | $ 35 | $ — | $ 57,862 |
| **Classified as:** | | | | |
| Cash equivalents | | | | 12,695 |
| Short-term investments | | | | 45,167 |
| Total cash equivalents and investments | | | | $ 57,862 |

As of December 31, 2025, total cash, cash equivalents and available-for-sale investments of $58.0 million includes cash equivalents and marketable securities of $57.9 million plus cash of $0.1 million.

The fair values of available-for-sale securities by contractual maturity as of June 30, 2026 and December 31, 2025 were as follows:

| | June 30, 2026 | December 31, 2025 |
|---|---|---|
| | (in thousands) | |
| Due in 1 year or less | 7,423 | 45,167 |
| Due in 1 - 2 years | — | — |
| Due in 3 years | — | — |
| Instruments not due at a single maturity date | 35,508 | 12,695 |
| Total available-for-sale securities | $ 42,931 | $ 57,862 |

As of June 30, 2026 and December 31, 2025, the Company recognized $0.1 million and $0.2 million, respectively, of accrued interest receivable from available-for-sale securities within prepaid expenses and other current assets on the balance sheet. As of June 30, 2026 and December 31, 2025, the remaining contractual maturities of available-for-sale securities were less than three years. There have been no realized losses on available-for-sale securities for the three and six months ended June 30, 2026 and 2025, respectively. The Company believes there are no other-than-temporary impairments on these securities as of June 30, 2026 and December 31, 2025, respectively, because the Company does not intend to sell these securities, nor does it believe that it will be required to sell these securities before the recovery of their amortized cost basis. None of the Company's available-for-sale investments have been in a continuous unrealized loss position for 12 months or longer as of June 30, 2026 and December 31, 2025, respectively.

## 5. Balance Sheet Components

### *Prepaid Expenses and Other Current Assets*

Prepaid expenses and other current assets consisted of the following (in thousands):

| | As of June 30, 2026 | As of December 31, 2025 |
|---|---|---|
| Prepaid expenses | $ 865 | $ 1,621 |
| Accrued interest | 104 | 240 |
| Other receivables | 20 | 18 |
| Total | $ 989 | $ 1,879 |

***Property and Equipment, net***

Property and equipment consisted of the following (in thousands):

| | As of June 30, 2026 | As of December 31, 2025 |
|---|---|---|
| Laboratory equipment | $ 11,773 | $ 11,768 |
| Leasehold improvements | 4,942 | 4,939 |
| Computer equipment and software | 906 | 903 |
| Furniture and office equipment | 325 | 325 |
| Total | 17,946 | 17,935 |
| Less: Accumulated depreciation and amortization | (12,191) | (10,927) |
| Property and equipment, net | $ 5,755 | $ 7,008 |

Depreciation expense was $0.6 million and $1.3 million for the three and six months ended June 30, 2026, and $0.7 million and $1.5 million for the three and six months ended June 30, 2025, respectively.

***Accrued Expenses and Other Current Liabilities***

Accrued expenses and other current liabilities consisted of the following (in thousands):

| | As of June 30, 2026 | As of December 31, 2025 |
|---|---|---|
| Accrued compensation | $ 2,890 | $ 4,365 |
| Accrued expenses | 2,414 | 1,974 |
| Government grant liability | 1,792 | — |
| Accrued taxes | 87 | — |
| Other current liabilities | — | 66 |
| Total | $ 7,183 | $ 6,405 |

**6. Collaboration and License Agreements**

***Regents Exclusive License Agreements***

Under the exclusive license agreement with the Regents of the University of California, ("UCB Exclusive License Agreement"), the Company is required to pay annual license maintenance fees as well as the following payments: future development and regulatory milestone payments totaling up to $27.7 million; future commercial milestone payments totaling up to $3.6 million; and royalties on future sales at percentage rates ranging from the low to mid-single digits. The Company accounts for license fees, sublicensee fees and license maintenance fees as research and development expenses when fees are due and payable and accounts for future milestones and royalty payments when the contingency is resolved and consideration is issued or becomes issuable. For the three and six months ended June 30, 2026, the Company recorded less than $0.1 million and $0.4 million in total expenses under this agreement. For the three and six months ended June 30, 2025, the Company recorded $0.4 million and $0.4 million in total expenses under this agreement, respectively. As of June 30, 2026, $0.1 million of milestones were included in accrued and other current liabilities. No milestone payments or royalties were considered probable or estimable as of June 30, 2026 or December 31, 2025.

***Acuitas Agreements***

The Company has a development and option agreement and a non-exclusive license agreement with Acuitas to develop products combining the Company’s gene editing technology and Acuitas’s LNP technology. Under the non-exclusive license agreement, the Company will be required to pay Acuitas up to $20.0 million in future development and regulatory milestone payments and up to $40.0 million in future commercial milestone payments per licensed product. The Company will also be obligated to pay Acuitas royalties at percentage rates in the low single digits on future net sales of licensed products by the Company and its sublicensees, subject to reduction under certain customary conditions. For the three and six months ended June 30, 2026, the Company recognized $2.0 million and $2.0 million in research and development expenses related to these agreements, respectively. For the three and six months ended June 30, 2025, the Company

recognized $0.1 million and $0.5 million in research and development expenses related to these agreements, respectively. As of June 30, 2026, $2.1 million of milestones were included in accrued and other current liabilities. No other milestones have been met or were probable as of June 30, 2026 and December 31, 2025. The Company remains obligated to pay future development, regulatory, and commercial milestones, as well as royalties on net sales, upon the successful achievement of such events.

***Collaboration Revenue***

The following table summarizes the revenue recognized from collaboration partners (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2026 | 2025 | 2026 | 2025 |
| Prevail | $ 1,918 | $ 4,778 | $ 4,151 | $ 21,568 |
| Sanofi | — | 120 | — | 455 |
| Total Revenue | $ 1,918 | $ 4,898 | $ 4,151 | $ 22,023 |

***Sanofi Agreements***

*Sanofi 2022 License Agreement*

The Company recognized zero revenue under the Sanofi 2022 License Agreement for the three and six months ended June 30, 2026 and 2025. All performance obligations under this agreement were satisfied in prior periods. As of June 30, 2026 and December 31, 2025, no development, regulatory, or commercial milestones were achieved or considered probable.

*Sanofi 2023 License Agreement*

The Company recognized zero in collaboration revenue under the Sanofi 2023 License Agreement for the three and six months ended June 30, 2026 and $0.1 million and $0.5 million for the three and six months ended June 30, 2025, respectively. As of June 30, 2026, $16.8 million was classified as current deferred revenue, and $11.7 million was classified as non-current deferred revenue. No milestones were met or were deemed probable in the three and six months ended June 30, 2026 and 2025.

***Prevail License Agreement***

Under the Prevail License Agreement, the Company is eligible to receive up to $110.0 million in research and development milestones and up to $1.1 billion in commercial milestones. The Company recognized $1.9 million and $4.2 million of collaboration revenue for the three and six months ended June 30, 2026 and $4.8 million and $21.6 million for the three and six months ended June 30, 2025, respectively. As of June 30, 2026 and December 31, 2025, the total estimated variable consideration for research and development services was $31.0 million. No additional milestones have been achieved or are considered probable as of June 30, 2026 and December 31, 2025. As of June 30, 2026, the Company had $10.6 million classified as current deferred revenue related to this agreement, which is expected to be recognized over the remaining term of the performance obligations.

*Contract Liabilities*

The following table presents changes in the Company's total contract liabilities for the six months ended June 30, 2026 and 2025(in thousands):

| | Balance at December 31, 2025 | Additions | Revenue Recognized | Balance at June 30, 2026 |
|---|---|---|---|---|
| Contract Liabilities; | | | | |
| Deferred revenue | $ 41,023 | $ - | $ (1,850) | $ 39,173 |

| | Balance at December 31, 2024 | Additions | Revenue Recognized | Balance at June 30, 2025 |
|---|---|---|---|---|
| Contract Liabilities; | | | | |
| Deferred revenue | $ 72,419 | $ - | $ (11,880) | $ 60,539 |

**7. Government Grants**

In May and June 2026, the Company entered into Notices of Award with the California Institute for Regenerative Medicine, or CIRM, for preclinical development funding for the Company's STX-1400 and STX-1200 programs, respectively. The awards provide for aggregate CIRM funding commitments of approximately $25.7 million, consisting of approximately $13.0 million for STX-1400 and approximately $12.7 million for STX-1200, subject to the achievement of specified operational milestones, compliance with reporting requirements, availability of CIRM funds and other conditions. The awards have estimated project periods extending from April 2026 through September 2029 for the STX-1400 program and from April 2026 through March 2029 for the STX-1200 program. The Company is also required to provide aggregate co-funding of approximately $7.1 million from non-CIRM sources.

The CIRM awards include 5% holdback payments that are payable only upon CIRM's receipt and approval of specified final reporting requirements. The awards also include reporting, operational milestone, intellectual property, access, affordability and revenue sharing provisions applicable to CIRM-funded technology. Under the terms of the awards, the Company may be required to make future revenue sharing payments and royalties on commercial sales of products developed using CIRM-funded technology, generally at low single-digit percentage rates. Certain intellectual property, access, affordability and revenue sharing obligations may continue beyond the project periods specified in the award agreements. As of June 30, 2026, the Company had not recognized any receivable or grant income related to the holdback amounts because the related conditions had not been satisfied.

During the three and six months ended June 30, 2026, the Company received initial CIRM disbursements of approximately $3.8 million. Amounts recognized in earnings were recorded as a reduction of research and development expense, while amounts received in advance of qualifying expenditures were recorded as a government grant liability. As of June 30, 2026, the Company recorded a government grant liability of approximately $1.8 million within accrued expenses and other current liabilities for amounts received in advance of qualifying expenditures.

**8. Convertible Note Payable**

On May 11, 2023, the Company issued an 8% convertible promissory note (the "Lilly Note") to Eli Lilly and Company with an aggregate principal amount of $30.0 million. The Lilly Note accrued interest at a rate of 8% per annum and matured on May 11, 2026. The Lilly Note contained provisions for conversion into the Company's equity securities upon the occurrence of certain financing, public offering or change-in-control events. Upon maturity on May 11, 2026, the Lilly Note became contractually convertible into shares of the Company's common stock in accordance with its terms. Because the related shares were not issued until after June 30, 2026, the outstanding principal and accrued interest remained reflected in the Company's balance sheet as of June 30, 2026. Subsequent to June 30, 2026, on July 17, 2026, the Company issued approximately 1.05 million shares of common stock in settlement of the outstanding principal and accrued interest associated with the Lilly Note. Following the issuance of such shares, the Lilly Note was fully extinguished and no amounts remained outstanding under the arrangement.

The fair value of the convertible note was $37.8 million and $35.3 million as of June 30, 2026 and December 31, 2025, respectively.

## 9. Commitments and Contingencies

### *Guarantees and Indemnifications*

In the normal course of business, the Company enters into agreements that contain a variety of representations and provide for general indemnification. The Company's exposure under these agreements is unknown because it involves claims that may be made against the Company in the future.

The Company has entered into indemnification agreements with certain directors and officers that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. As of June 30, 2026 and December 31, 2025, the Company does not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded related liabilities.

### *Legal Contingencies*

From time to time, the Company may become involved in legal proceedings arising from the ordinary course of business. The Company records a liability for such matters when it is probable that future losses will be incurred and that such losses can be reasonably estimated. Significant judgment by the Company is required to determine both probability and the estimated amount. Management is currently not aware of any legal matters that could have a material adverse effect on financial position, results of operations or cash flows.

### *Research and Development Agreements*

The Company enters into contracts in the normal course of business with third-party vendors for preclinical studies, supplies and other services and products for operating purposes. These contracts generally provide for termination on notice or may have a potential termination fee if a purchase order is cancelled within a specified time. As of June 30, 2026 and December 31, 2025, there were no amounts accrued related to termination and cancellation charges as the Company has not determined cancellation to be probable.

### *License Agreements*

The Company entered into the Regents Agreement and Acuitas Agreements (Note 6), which require it to pay milestones contingent upon the meeting of specific events and royalties on future sales. As of June 30, 2026 and December 31, 2025, no milestones were due and payable.

## 10. Redeemable Convertible Preferred Stock

In October 2018, the Company issued 2,051,741 shares of its Series A redeemable convertible preferred stock at a price of $9.77 per share for gross cash proceeds of $20.0 million and issued 26,338 shares of its Series A-1 redeemable convertible preferred stock upon the conversion of the outstanding convertible notes and accrued interest. In January 2019, the Company issued 46,630 shares of its Series A-2 redeemable convertible preferred stock at a price of $10.72 per share for gross cash proceeds of $0.5 million.

In March 2021, the Company issued 2,790,590 shares of Series B redeemable convertible preferred stock at a price of $35.83 per share for gross cash proceeds of $100.0 million.

Redeemable convertible preferred stock consists of the following (in thousands, except share and per share amounts):

| | December 31, 2025 and June 30, 2026 | | | | |
|---|---|---|---|---|---|
| | Shares Authorized | Original Issue Price | Shares Issued and Outstanding | Carrying Value | Liquidation Preference |
| Series A | 12,150,003 | $ 9.77 | 2,051,741 | $ 19,869 | $ 20,000 |
| Series A-1 | 155,977 | 7.82 | 26,338 | 205 | 205 |
| Series A-2 | 276,138 | 10.72 | 46,630 | 500 | 500 |
| Series B | 16,600,000 | 35.83 | 2,790,590 | 99,782 | 100,000 |
| Total | 29,182,118 | | 4,915,299 | 120,356 | 120,705 |

The rights, preferences and privileges of the redeemable convertible preferred stock are as follows:

*Voting*—The holder of each share of Series A, A-1, A-2 and Series B redeemable convertible preferred stock has the right to one vote of each share of common stock into which such redeemable convertible preferred stock is convertible. The holders of Series B redeemable convertible preferred stock, voting as a separate class, shall be entitled to elect two directors to the Company's Board of Directors. The holders of Series A redeemable convertible preferred stock, voting as a separate class, shall be entitled to elect one director to the Company's Board of Directors. The holders of common stock, voting as a separate class, shall be entitled to elect two directors to the Company's Board of Directors.

*Dividends*—The holders of shares of redeemable convertible preferred stock, in preference to the holders of common stock, shall be entitled to receive, but only out of funds that are legally available, cash dividends at the annual per share rate of 6.0% per annum based on the original issue price. Such dividends shall be payable only when, as and if declared by the Company's Board of Directors and shall be non-cumulative. No dividends have been declared as of December 31, 2025 and June 30, 2026, respectively.

*Conversion*—Each share of redeemable convertible preferred stock shall be convertible, at the option of the holder, at any time and from time to time, and without the payment of additional consideration by the holder, into such number of shares of common stock at the conversion rate that is determined by dividing the redeemable convertible preferred stock original issue price by the redeemable convertible preferred stock conversion price in effect at the time of conversion. The conversion price shall initially be equal to the applicable original issue price subject to certain anti-dilution adjustments. As of June 30, 2026, the Company's redeemable convertible preferred stock was convertible into the Company's shares of common stock on a one-for-one basis.

Each share of redeemable convertible preferred stock is automatically converted into common stock shares at the then effective conversion rate (i) upon the closing of a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least $100.0 million of gross proceeds to the Company, or (ii) if such offering is otherwise approved by vote or written consent of at least a majority of the outstanding shares of the redeemable convertible preferred stock voting together as a single class on an as-converted basis, which majority shall include at least a majority of Series B redeemable convertible preferred stock.

*Liquidation Preference*—In the event of a liquidation, dissolution or winding up of the Company, the holders of redeemable convertible preferred stock are entitled to be paid out of the assets of the Company legally available for distribution before any distribution or payment is made to holders of the Company's common stock. In the event of a liquidation, dissolution or winding up of the Company, the holders of the redeemable convertible preferred stock are entitled to receive the amount per share of redeemable convertible preferred stock owned equal to the greater of (i) the original issue price, plus any dividends declared but unpaid thereon for any redeemable convertible preferred stock owned, or (ii) such amount per share as would have been payable had all shares of redeemable convertible preferred stock been converted into common stock immediately prior to such liquidation, dissolution, winding up or deemed liquidation event. If upon any such liquidation, dissolution or winding up of the Company or deemed liquidation event, the assets of the Company available for distribution to its stockholders are insufficient to pay the holders of shares of redeemable convertible preferred stock the full amount to which they are entitled, the holders of shares of redeemable convertible preferred stock will share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution as if all amounts payable on or with respect to such shares were paid in full.

The remaining assets of the Company are distributed among the common and redeemable convertible preferred stockholders pro rata based on the number of shares held by each holder on an as-converted basis.

*Redemption*—Upon the occurrence of certain change in control events that are outside of the Company's control, including liquidation, sale or transfer, holders of the redeemable convertible preferred stock can effectively cause redemption for cash. As a result, the Company classified the redeemable convertible preferred stock as mezzanine equity on the balance sheets as the stock is contingently redeemable.

## 11. Common Stock

The Company is authorized to issue up to 52,000,000 shares of $0.001 par value common stock as of June 30, 2026.

Common stockholders are entitled to dividends when and if declared by the Board of Directors, subject to the prior rights of the redeemable convertible preferred stockholders. The holder of each share of common stock is entitled to one vote. The common stockholders voting as a class are entitled to elect two members to the Company's Board of Directors. No dividends have been declared since the inception of the Company.

The Company had reserved common stock for future issuance as follows:

| | June 30, 2026 | December 31, 2025 |
|---|---|---|
| Conversion of redeemable convertible preferred stock | 4,915,299 | 4,915,299 |
| Exercise of outstanding stock option awards | 1,068,963 | 1,180,576 |
| Common Stock Warrants issued and outstanding | 74,324 | 74,324 |
| Restricted stock issued and outstanding-2018 Equity Incentive Plan | 48,021 | 48,021 |
| Stock options available for future grant | 230,075 | 152,545 |
| **Total common stock reserved** | **6,336,682** | **6,370,765** |

***Founders' Restricted Common Stock***

In January and August 2018, the Company issued 2,195,278 restricted common stock shares to its founders at the purchase price of $0.00592 per share for their advisory and consulting services. In September 2018, the Company repurchased 211,084 such shares from one of the founders. The founders' common stock shares are subject to the Company's repurchase option if a founder terminates their services to the Company during the vesting period. The Company may repurchase any unvested restricted common stock shares at the price per share equal to the original purchase price, subject to adjustments in the event of any reorganization, recapitalization, reclassification, stock dividend, stock split or reverse stock split. The repurchase right lapses in 90 days after the termination of the founder's service or employment. During the vesting term, the holders of the founders' restricted common stock shares have the right to receive dividends and voting rights.

The restricted common stock shares vested monthly over four years from the vesting commencement date, which was the date of the initial closing of the Series A redeemable convertible preferred stock financing or October 1, 2018. The founders' shares were all fully vested as of December 31, 2024. The Company accounts for shares issued to founders as equity compensation awards and the estimated fair value at the grant date was minimal.

## 12. Equity Incentive Plan and Stock-Based Compensation

***2018 Stock Incentive Plan***

In 2018, the Company adopted the 2018 Stock Incentive Plan (the "Plan"). The Plan provides for the grants of stock options, stock appreciation rights, dividend equivalent rights, restricted stock units, restricted stock awards ("RSAs"), and other stock-based awards to employees, consultants and advisors of the Company. Options granted under the Plan may be either incentive stock options or nonqualified stock options. Incentive stock options ("ISO") may be granted only to Company employees, including officers and directors who are also employees. Nonqualified stock options ("NSO") may be granted to Company employees, consultants and advisors. As of June 30, 2026, the Company has reserved 1,672,540 shares of common stock for issuance under the Plan.

Options under the Plan may be granted for periods of up to ten years and at prices based upon the estimated fair value of the shares on the date of grant as determined by the Board of Directors, provided, however, that (i) the exercise price of an option shall not be less than 100% of the estimated fair value of the shares on the date of grant, (ii) the exercise price of an ISO granted to a greater than 10% stockholder shall not be less than 110% of the estimated fair value of the shares on the date of grant, and (iii) the term of an ISO granted to a greater than 10% stockholder should not exceed five years. Options granted generally vest over four years.

Options and RSAs granted under the Plan vest on a straight-line basis over a period of four years, with certain options vesting 25% after the first year of service and 1/48th vesting on a monthly basis thereafter. RSAs granted by the Company may be repurchased in the event that conditions specified by the administrator are not satisfied prior to the end of such applicable restriction periods established by the administrator of such awards.

On October 6, 2021, the Company granted 101,320 performance-based stock option awards to the Chief Executive Officer that only vest upon the successful completion of the first-in-human proof-of concept study. On June 26 2026, the Company's Board of Directors approved a grant of 13,239 performance-based stock option awards to the Chief Operating Officer that begin vesting upon the achievement of specified clinical development milestones. The Company recognizes stock option awards with performance vesting conditions based on the grant date fair value over the service period using the accelerated attribution method to the extent that achievement of the performance condition is probable. For the three months ended June 30, 2026, no expense has been recognized related to these performance-based stock option awards as the achievement of the performance conditions was not probable.

The Plan allows for early exercises of stock options that will be subject to a right of repurchase by the Company for any unvested shares. The repurchase rights lapse over the original vesting period of the options. The Company accounts for the cash received in consideration for the early exercised options as a liability included in accrued and other current liabilities, which is then reclassified to stockholders' equity as the options vest. As of June 30, 2026, the Company had no shares of common stock subject to an early exercise option repurchase provision under the Plan.

As of June 30, 2026, there were 1,672,540 shares reserved for issuance as stock option awards under the Plan.

***Stock Option Activity***

Stock options activity under the Plan is set forth below:

| | | Outstanding Awards | | | |
|---|---|---|---|---|---|
| | Number of Shares Available for Grant | Options issued and outstanding | Weighted Average Exercise Price | Weighted Average Remaining Contractual Term (in years) | Aggregate Intrinsic Value (in thousands) |
| **Outstanding on December 31, 2025** | 152,545 | 1,180,576 | $ 6.28 | 6.57 | $ 896 |
| Options granted | (99,340) | 99,340 | $ 19.13 | | |
| Options exercised | | (34,083) | $ 6.62 | | |
| Options forfeited or cancelled | 176,870 | (176,870) | $ 7.61 | | |
| **Outstanding on June 30, 2026** | 230,075 | 1,068,963 | $ 7.24 | 6.25 | $ 12,708 |
| Vested and Exercisable at June 30, 2026 | | 808,434 | $ 7.21 | | |

The weighted-average grant-date fair value of stock options granted to employees during the six months ended June 30, 2026 and 2025 was $20.98 per share and $5.87 per share, respectively. The total intrinsic value of stock options exercised during the six months ended June 30, 2026 was $0.4 million. As of June 30, 2026, the total unrecognized stock-based compensation related to unvested stock options was $2.7 million, which the Company expects to recognize over a remaining weighted-average period of 1.22 years.

The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying stock options and the fair value of the Company's common stock for stock options that were in-the-money at June 30, 2026.

The Company estimated the fair value of stock options using the Black-Scholes option pricing model. The fair value of employee stock options is being amortized on a straight-line basis over the requisite service period of the awards. The fair value of stock options was estimated using the following weighted-average assumptions:

| | Six Months Ended June 30, | |
|---|---|---|
| | 2026 | 2025 |
| Expected volatility | 84.56% - 88.16% | 85.54% - 86.74% |
| Risk-free interest rate | 3.87% - 4.17% | 4.06% - 4.11% |
| Dividend yield | 0% | 0% |
| Expected term | 4.99 - 6.01 years | 5.97 - 6.08 years |

***Restricted Stock Award Activity***

As of June 30, 2026 and December 31, 2025, the Company had 48,021 RSAs that were issued and outstanding to non-employee consultants. The outstanding RSAs had a weighted average grant date fair value of $2.84 per share. From the total RSAs issued and outstanding, zero shares were unvested as of June 30, 2026 and December 31, 2025. The awards are legally issued and are considered outstanding as of the grant date. The fair value of RSAs granted is equal to the estimated

fair value of the Company's common stock on the grant date. Stock-based compensation expense recognized during the three and six months ended June 30, 2026 and 2025 was zero.

***Stock Based Compensation***

No stock-based compensation related to performance-based awards was recognized in the three and six months ended June 30, 2026 and 2025 as the performance conditions were not probable.

The following table is a summary of total employee and non-employee stock-based compensation (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2026 | 2025 | 2026 | 2025 |
| Research and development | $ 164 | $ 418 | $ 1,104 | $ 868 |
| General and administrative | $ 63 | $ 258 | $ 1,093 | $ 479 |
| Total | $ 227 | $ 676 | $ 2,197 | $ 1,347 |

**13. Income Taxes**

In determining quarterly provisions for income taxes, the Company uses the annual estimated effective tax rate applied to the actual year-to-date profit or loss, adjusted for discrete items arising in that period. The Company's annual estimated effective tax rate differs from the U.S. federal statutory rate primarily as a result of changes in its valuation allowance against its deferred tax assets. For the three and six months ended June 30, 2026, the Company recorded an income tax provision of $0.2 million and $0.4 million, compared to an income tax benefit of $0.7 million and $1.0 million for the three and six months ended June 30, 2025. During the three months ended June 30, 2026, there were no material changes to the Company's unrecognized tax benefits. The Company does not have any tax audits or other issues pending.

**14. Net Loss Per Share Attributable to Common Stockholders**

The following table sets forth the computation of the basic and diluted net loss per share (in thousands, except share and per share amounts):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2026 | 2025 | 2026 | 2025 |
| Numerator: | | | | |
| Net loss attributable to common stockholders | $ (6,477) | $ (9,903) | $ (23,824) | $ (13,328) |
| Denominator: | | | | |
| Weighted-average shares outstanding subject to repurchase | 2,475,898 | 2,429,130 | 2,468,343 | 2,423,315 |
| Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted | 2,475,898 | 2,429,130 | 2,468,343 | 2,423,315 |
| Net loss per share attributable to common stockholders, basic and diluted | $ (2.62) | $ (4.08) | $ (9.65) | $ (5.50) |

Since the Company was in a loss position for all periods presented, basic net loss per share is the same as diluted net loss per share as the inclusion of all potential dilutive securities would have been anti-dilutive. Potentially dilutive securities that were not included in the diluted per share calculations because they would be anti-dilutive were as follows:

| | As of June 30, | |
|---|---|---|
| | 2026 | 2025 |
| Convertible preferred stock on an as if converted basis | 4,915,299 | 4,915,299 |
| Options to purchase common stock | 1,068,963 | 1,216,424 |
| Common stock warrants | 74,324 | 74,324 |
| Convertible note payable(1) | 1,055,054 | 1,161,478 |
| **Total** | **7,113,640** | **7,367,525** |

(1) The conversion of the convertible note payable into common stock is dependent on the price of shares that may be issued in connection with an IPO, Special Purpose Acquisition Company transaction or Qualified Financing, as well

as the completion of such transactions. The number of shares herein is calculated based on the conversion of the convertible note's outstanding principal and accrued and unpaid interest as of June 30, 2026 and 2025 into the Company's common stock at the price of $35.83 per share.

**15. Segment Information**

The Company operates as one operating and reportable segment focused on researching and developing genetic medicines using CRISPR-based tools to address the root cause of diseases, with a primary focus on cardiometabolic conditions. The Company's chief executive officer serves as the chief operating decision maker ("CODM"). The CODM evaluates segment performance and allocates resources based on net loss and operating expenses, which are also reported in the statement of operations. This measure is used to monitor spending and compare budgeted versus actual results. Factors considered in determining the single reportable segment include the nature of the Company's operating activities, its organizational and reporting structure, and the type of financial information reviewed by the CODM. All material long-lived assets and revenues are located in the United States.

The CODM reviews cash, cash equivalents and investments as a measure of segment assets. As of June 30, 2026 and December 31, 2025, the Company's cash, cash equivalents and investments were $43.0 million and $58.0 million, respectively.

The table below summarizes the segment's profit or loss, along with significant expense categories which are reviewed by the CODM, for the periods presented (in thousands).

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2026 | 2025 | 2026 | 2025 |
| Collaboration Revenue | $ 1,918 | $ 4,898 | $ 4,151 | $ 22,023 |
| Significant operating expenses: | | | | |
| Research and development: | | | | |
| Internal research expenses | (669) | (1,699) | (1,496) | (3,974) |
| External research and development | (3,822) | (5,058) | (7,870) | (10,037) |
| Employee-related expenses | (2,559) | (4,791) | (7,306) | (11,104) |
| Facilities and overhead costs | (1,567) | (1,945) | (3,030) | (3,908) |
| Professional and consulting fees | (206) | (391) | (426) | (853) |
| General and administrative | (2,490) | (2,641) | (5,844) | (9,332) |
| Total Operating expenses | (11,313) | (16,525) | (25,972) | (39,208) |
| Interest income and other income (expense), net | 383 | 880 | 898 | 1,944 |
| Interest expense | (276) | (598) | (875) | (1,197) |
| Change in fair value of Convertible Note | 3,023 | 730 | (1,585) | 2,083 |
| Loss before income taxes | (6,265) | (10,615) | (23,383) | (14,355) |
| (Provision for) / benefit from income taxes | (212) | 712 | (441) | 1,027 |
| **Net Loss** | **$ (6,477)** | **$ (9,903)** | **$ (23,824)** | **$ (13,328)** |

**16. Subsequent Events**

On July 17, 2026, the Company amended its amended and restated certificate of incorporation and effected a reverse stock split pursuant to which every 5.9218 shares of the Company's common stock issued and outstanding were automatically reclassified into one new share of common stock, subject to the treatment of fractional shares as previously described, without any action on the part of the holders.

On July 17, 2026, the Company entered into an amendment to the 2023 Sanofi License Agreement under which it extended the nomination period for Sanofi to select one additional target under the license and expanded the license to include the Company's ELXR Platform for that target.

On July 23, 2026, the Company's stockholders approved the 2026 Equity Incentive Plan (the "2026 Plan"), and the 2026 Plan became effective upon the effectiveness of the Company's registration statement in connection with its initial public offering. The 2026 Plan increased the aggregate number of shares of common stock reserved for issuance under the Company's equity compensation plans by 2,420,000 shares.

On July 23, 2026, grants of stock options previously approved by the Board of Directors on July 16, 2026 became effective upon the effectiveness of the Company's registration statement. The grants covered an aggregate of

2,066,997 shares of common stock to certain employees and members of the Board of Directors. Certain awards are subject to service-based vesting requirements and certain awards are subject to both market-based vesting conditions tied to the future trading price of the Company's common stock and service-based vesting requirements. The exercise price of the options equals the initial public offering price of $15.00 per share. The Company is evaluating the accounting impact of these awards, including the determination of grant-date fair value and resulting stock-based compensation expense to be recognized in future periods.

On July 23, 2026, the Securities and Exchange Commission declared effective the Company's Registration Statement on Form S-1 relating to its initial public offering. On July 27, 2026, the Company completed its initial public offering of 9,867,000 shares of common stock at a public offering price of $15.00 per share, including the full exercise by the underwriters of their option to purchase 1,287,000 additional shares.

On July 17, 2026, Sanofi agreed to purchase 500,000 shares of the Company's common stock in a concurrent private placement at a purchase price equal to the initial public offering price of $15.00 per share. The private placement closed on July 27, 2026 concurrently with the completion of the Company's initial public offering and generated aggregate gross proceeds of approximately $7.5 million. Aggregate gross proceeds from the initial public offering and concurrent private placement were approximately $155.5 million before underwriting discounts, commissions and offering expenses.

**Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.**

*You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our financial statements and the related notes and other financial information included elsewhere in this Quarterly Report. This discussion and analysis and other parts of this Quarterly Report contain forward-looking statements based upon our current plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, objectives, expectations, intentions and beliefs. Our actual results and the timing of events could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under the section titled "Risk Factors" and elsewhere in this Quarterly Report. You should carefully read the section titled "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section titled "Special Note Regarding Forward-Looking Statements."*

**Overview**

We are a clinical-stage biotechnology company engineering purpose-built in vivo CRISPR technologies designed to extend healthy lifespan through disease prevention and durable therapeutic intervention. Our proprietary CRISPR by Design approach combines computational engineering, machine learning, and high-throughput experimental validation to develop genetic medicines optimized for specific therapeutic applications. Through this approach, we have engineered a portfolio of proprietary CRISPR technologies, including our Epigenetic Long-Term X-Repressor, or ELXR, platform for durable epigenetic silencing and our X-Editor, or XE, platform for precision gene editing.

Our research and development efforts are currently focused on cardiometabolic diseases, including atherosclerotic cardiovascular disease, or ASCVD, where significant unmet need persists despite the availability of existing therapies. We are developing genetic medicines intended to provide durable therapeutic benefit following a single administration, with the goal of overcoming limitations associated with chronic treatment paradigms, including poor adherence, treatment burden, and suboptimal long-term outcomes.

Our lead product candidate, STX-1150, is a clinical-stage CRISPR-based epigenetic silencing therapy designed to reduce low-density lipoprotein cholesterol, or LDL-C, through repression of PCSK9 expression without inducing permanent DNA sequence modifications. STX-1150 utilizes our proprietary ELXR technology and is designed to deliver durable LDL-C lowering through epigenetic regulation of gene expression. In 2026, we initiated a first-in-human Phase 1 clinical trial of STX-1150 in adults with elevated LDL-C and increased risk of ASCVD. The trial is designed to evaluate safety, tolerability, and LDL-C lowering activity, following administration of STX-1150.

During the second quarter of 2026, we presented late-breaker preclinical data at the European Atherosclerosis Society Congress highlighting the potential of STX-1150. Data presented demonstrated potent and durable PCSK9 suppression of up to approximately 90% and LDL-C reduction of up to approximately 68% in non-human primates following a single administration. At a therapeutically relevant dose of 0.75 mg/kg, a prototype of STX-1150 produced durable LDL-C reductions of greater than 50%, sustained for two years. Additional findings demonstrated no detectable off-target transcriptional changes in primary human hepatocytes, and no significant liver safety signals at therapeutically relevant exposures. We believe these findings support the potential of ELXR-mediated epigenetic silencing to provide durable therapeutic benefit while avoiding permanent genomic changes.

Beyond STX-1150, we are advancing two wholly owned cardiometabolic product candidates based on our XE gene editing technology. STX-1200 is designed as a single-dose gene editing therapy targeting the LPA gene to durably lower lipoprotein(a), or Lp(a), in patients with genetically elevated Lp(a) and associated cardiovascular risk. STX-1400 targets APOC3 and is being developed to reduce triglyceride-rich lipoproteins and address diseases driven by severe hypertriglyceridemia, including familial chylomicronemia syndrome, multifactorial chylomicronemia syndrome, and severe hypertriglyceridemia. During 2026, the California Institute for Regenerative Medicine awarded us approximately $25.7 million of multi-year grant funding to support the advancement of STX-1200 and STX-1400 toward clinical development.

In addition to our wholly owned pipeline, we have established strategic collaborations with leading biopharmaceutical companies, Eli Lilly and Sanofi, to apply our precision-engineered CRISPR technologies in therapeutic areas outside of cardiometabolic disease. These arrangements provide a combination of upfront payments, research funding, development and commercial milestone opportunities, and potential future royalties. We believe these collaborations further validate the versatility of our technology platform while providing non-dilutive funding to support our development activities.

Since our inception, we have devoted substantially all of our resources to technology development, research

activities, preclinical and clinical development, manufacturing process development, intellectual property protection, organizational growth, and capital raising activities. We have generated revenue primarily through collaboration and license agreements and have incurred significant operating losses and negative cash flows from operations. As of June 30, 2026, we had not generated any revenue from product sales. We expect to continue to incur substantial operating losses for the foreseeable future as we advance our clinical and preclinical programs, expand our research and development activities, invest in manufacturing and clinical capabilities, and support the infrastructure required to operate as a public company.

***Financial Overview***

Since the commencement of our operations, we have devoted substantially all of our resources to conducting research and development activities, establishing and maintaining our intellectual property portfolio, establishing our corporate infrastructure, raising capital and providing general and administrative support for these operations. We have funded our operations to date primarily from proceeds received under collaboration and license agreements with our collaboration partners , the issuance and sale of redeemable convertible preferred stock, the issuance of a convertible note and, subsequent to June 30, 2026, the completion of our initial public offering. We do not expect to generate product revenue unless and until we successfully develop and obtain approval for the commercialization of a product candidate, and we cannot assure that we will ever generate significant revenue or profits.

Since inception, we have incurred significant losses and negative cash flows from operations. As of June 30, 2026, we had an accumulated deficit of $181.5 million. We incurred net losses of $6.5 million and $23.8 million for the three and six months ended June 30, 2026, respectively. Our losses have resulted primarily from costs incurred in connection with research and development activities, including platform development, product candidate advancement and personnel-related costs, as well as general and administrative expenses associated with supporting our operations and preparing to operate as a public company.

We do not expect to generate any revenue from commercial product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years, if ever. The net losses we incur may fluctuate significantly from quarter-to-quarter and year-to-year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We anticipate that our expenses will increase substantially if, and as, we:

- advance our lead product candidate, STX-1150, through clinical trials and conduct later stage clinical trials;
- initiate clinical trials for our STX-1200 and STX-1400 developmental programs;
- manufacture, or have manufactured, preclinical, clinical and potentially commercial supplies of any product candidates;
- further develop our CRISPR-based technologies, ELXR and XE, as well as engineer new technologies;
- begin or continue preclinical development of any future cardiometabolic programs;
- identify and develop future product candidates;
- experience any delays in our preclinical studies and clinical trials, if any, due to unforeseen events;
- obtain, expand, maintain, defend, and enforce our intellectual property portfolio;
- seek regulatory approvals for any product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any product candidates, if approved;
- comply with our obligations under the agreements governing our licenses, collaborations, and strategic partnerships;
- establish additional licenses, collaborations, or strategic partnerships;
- acquire or in-license intellectual property and technologies, work with strategic partners to support and expand our research and discovery, and initiate and conduct preclinical and clinical programs;
- hire additional clinical, scientific, and management personnel, as well as administrative staff to support the growth of our business;
- add operational, financial, and management information systems and personnel; and

- incur additional legal, accounting, and other costs associated with operating as a public company.

Even if we succeed in developing our lead programs and identifying potential product candidates, we may never achieve commercialization, and we may continue to incur substantial research and development expenses and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays, and other unknown factors that may adversely affect our business, financial condition, results of operations, and prospects. The size of our future losses will depend, in part, on the rate of future growth of our expenses and our ability to generate product revenue, if any. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We expect our operating expenses to increase over time as we continue to invest in the advancement of our therapeutic pipeline, including the progression of STX-1150 through clinical development, advancement of additional development programs, manufacturing activities, preclinical and clinical studies, intellectual property protection and business development activities. We also expect general and administrative expenses to increase as a result of costs associated with operating as a publicly traded company, including legal, accounting, compliance, investor relations, insurance and other corporate governance expenses.

Our net losses and cash flows may fluctuate significantly from period to period, depending on, among other things, variations in the level of expense related to the ongoing development of our product candidates or future development programs; the delay, addition or termination of clinical trials; and the execution of any additional collaboration, licensing or similar arrangements, and the timing of payments we may make or receive under such arrangements.

As of June 30, 2026, we had $43.0 million in cash, cash equivalents, and investments. Based on our current operating plan, we believe our cash, cash equivalents and investments as of June 30, 2026, together with the net proceeds from our initial public offering which closed on July 27, 2026, will be sufficient to fund our planned operating expenses and capital expenditure requirements into the first half of 2029. Our future capital requirements will depend on many factors, including the timing and progress of our research and development activities, clinical trials, manufacturing operations, potential strategic collaborations and the timing of milestone payments under existing collaboration agreements. We may seek additional capital through equity or debt financings, collaborations, licensing arrangements or other strategic transactions in the future.

***Macroeconomic Trends***

Economic conditions, such as rising inflation, higher interest rates, instability at banking and financial institutions, international trade tensions and tariffs, changes in regulatory laws and monetary exchange rates, and government fiscal policies, can also have a significant effect on operations. Moreover, negative macroeconomic conditions could adversely impact our ability to obtain financing in the future on terms acceptable to us, or at all. In addition, geopolitical instability and any related sanctions could have a significant impact on global financial markets, including volatility in the United States and global financial markets.

**Components of Results of Operations**

***Collaboration Revenue***

We have no products approved for commercial sale and have not generated any revenue from the sale of products to date and do not expect to generate any revenue from the sale of products in the near future.

Our revenue to date has been generated from payments received pursuant to collaboration and license arrangements with strategic partners. Collaboration revenue consists of revenue received from full-time equivalent and out-of-pocket reimbursements, upfront, milestone and contingent payments received from our collaborators.

In addition to receiving upfront payments, we may also be entitled to milestones and other contingent payments upon achieving predefined objectives. If a milestone is considered probable of being reached, and if it is probable that a significant revenue reversal would not occur, the associated milestone amount would also be included in the transaction price.

We expect that any collaboration revenue we generate from our current collaboration and license agreements, and from any future collaboration partners, will fluctuate in the future as a result of the timing and amount of upfront, milestone and other collaboration agreement payments and other factors.

***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred for the discovery and development of our product candidates. We expense both internal and external research and development expenses to operations in the periods in which they are incurred. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and as services are performed. We do not currently track our research and development expenses by individual projects or product candidates.

Internal research and development costs include:

- costs to acquire reagents, chemicals, for in-house lab research and preclinical studies;
- expenses related to laboratory supplies and services; and
- expenses related to laboratory related software.

External research and development consist primarily of costs incurred for the development of our product candidates and include:

- fees paid to third parties such as contract research organizations to conduct our discovery, research and development programs, preclinical activities, animal trials and regulatory operations;
- costs to acquire, develop and manufacture supplies for clinical trials and preclinical studies, including fees paid to third parties such as contract manufacturing organizations; and
- the cost to obtain and maintain licenses to intellectual property, such as fees paid under the UCB Exclusive License Agreement and fees paid to Acuitas.

Employee-related expenses include payroll and personnel expenses, including benefits and stock-based compensation expenses. Facilities and overhead costs include depreciation of research and development equipment, allocated overhead and other facilities-related expenses. Professional and consulting fees include fees paid to third-party consultants and contractors supporting research, development, and regulatory activities.

We historically do not track our research and development costs by project category, primarily because we use our employee and infrastructure resources across multiple research and development programs that we are advancing in parallel, and therefore we do not allocate salaries, stock-based compensation, employee benefit expenses or other indirect costs related to our research and development to specific product candidates.

We expect our research and development expenses to increase substantially for the foreseeable future as we identify product candidates, conduct further preclinical studies, Investigational New Drug application, or IND-enabling, studies and

clinical trials for any such product candidates, continue to invest in research and development activities for discovery programs and preclinical studies, pursue regulatory approvals and expand our pipeline. The process of conducting the necessary preclinical and clinical research to obtain regulatory approvals is costly and time-consuming. To the extent that any product candidates advance to, and continue to advance through, clinical trials, our research and development expenses will continue increasing substantially and may become more variable. The actual probability of success for such product candidates may be affected by a variety of factors, including the safety and efficacy of such product candidates, investment in our clinical programs, the ability of collaborators to successfully develop our licensed product candidates, manufacturing capability, competition with other products and commercial viability. As a result of these variables, we are unable to determine if, when and to what extent we will generate revenue from the commercialization and sale of any potential product candidates. We may never succeed in achieving regulatory approval for any product candidates.

***General and Administrative Expenses***

General and administrative expenses consist primarily of payroll and personnel expenses, including benefits and stock-based compensation, facilities-related expenses and professional fees for legal, accounting, consulting and audit, tax services, consulting fees related to human resources, intellectual property and business development. We expect our general and administrative expenses to increase for the foreseeable future as we continue to grow, improve our infrastructure and operate as a public company. This will include additional expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and listing standards applicable to companies listed on a national securities exchange, director and officer insurance premiums, investor relations activities and other administrative and professional services. We also expect our intellectual property expenses to increase as we expand our intellectual property portfolio.

***Research Funding from Government Grants***

In 2026, we entered into grant agreements with the California Institute for Regenerative Medicine, or CIRM, to support the development of our STX-1400 and STX-1200 programs. We account for these awards as income-related government grants and present amounts recognized in earnings as a reduction of research and development expense. Accordingly, the benefit recognized from these grants may fluctuate between periods based on the timing of qualifying research and development expenditures, achievement of program milestones and receipt of grant funding.

***Interest Income and Other Income, Net***

Interest and other income, net primarily consists of interest earned on our cash, cash equivalents, and available-for-sale investments securities. We expect interest income to vary each reporting period depending on our average bank deposit, money market fund, and marketable securities balances during the period and market interest rates.

***Interest Expense***

Interest expense consists of coupon interest accrued on the convertible note that we issued to Eli Lilly and Company in May 2023.

***Change in Fair Value of Convertible Note***

Change in fair value of convertible note consists of fair value adjustments on the convertible note at each reporting period.

***Provision for Income Taxes***

The provision for income taxes primarily consists of estimates for federal taxes payable and reserves for unrecognized tax benefits and state taxes. We have generated net operating losses, or NOLs, since inception and have established a full valuation allowance against our deferred tax assets due to the uncertainty surrounding the realization of such assets.

**Results of Operations**

***Comparison of the Three Months Ended June 30, 2026 and 2025***

The following table summarizes our results of operations for the three months ended June 30, 2026 and 2025 (in thousands):

| | Three Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Collaboration revenue | $ 1,918 | $ 4,898 | $ (2,980) | (61)% |
| Operating expenses: | | | | |
| Research and development | 8,823 | 13,884 | (5,061) | (36)% |
| General and administrative | 2,490 | 2,641 | (151) | (6)% |
| Total operating expenses | 11,313 | 16,525 | (5,212) | (32)% |
| Loss from operations | (9,395) | (11,627) | 2,232 | (19)% |
| Interest income and other income (expense), net | 383 | 880 | (497) | (56)% |
| Interest expense | (276) | (598) | 322 | (54)% |
| Change in fair value of convertible note | 3,023 | 730 | 2,293 | 314% |
| Net loss before provision for income taxes | (6,265) | (10,615) | 4,350 | (41)% |
| (Provision for) / benefit from income taxes | (212) | 712 | (924) | (130)% |
| Net loss | $ (6,477) | $ (9,903) | $ 3,426 | (35)% |

*Collaboration Revenue*

The following table summarizes our collaboration revenue for the three months ended June 30, 2026 and 2025 (dollars in thousands):

| | Three Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Lilly | $ 1,918 | $ 4,778 | $ (2,860) | (60)% |
| Sanofi | — | 120 | (120) | (100)% |
| Total collaboration revenue | $ 1,918 | $ 4,898 | $ (2,980) | (61)% |

Collaboration revenue was $1.9 million for the three months ended June 30, 2026 and $4.9 million for the three months ended June 30, 2025. The decrease of $3.0 million, or 61%, was primarily due to reduced reimbursable research and development activities with Lilly. No revenue was recognized under the Sanofi collaboration agreement during the three months ended June 30, 2026, compared to $0.1 million for the three months ended June 30, 2025. The decrease was due to the completion of research activities under the applicable workplan during the prior-year period.

*Research and Development Expenses*

The following table summarizes our research and development expenses for the three months ended June 30, 2026 and 2025 (dollars in thousands):

| | Three Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Internal research expenses | $ 669 | $ 1,699 | $ (1,030) | (61)% |
| External research and development | 3,822 | 5,058 | (1,236) | (24)% |
| Employee-related expenses | 2,559 | 4,791 | (2,232) | (47)% |
| Facilities and overhead costs | 1,567 | 1,945 | (378) | (19)% |
| Professional and consulting fees | 206 | 391 | (185) | (47)% |
| Total research and development | $ 8,823 | $ 13,884 | $ (5,061) | (36)% |

Research and development expenses decreased by $5.1 million, or 36%, from $13.9 million for the three months ended June 30, 2025 to $8.8 million for the three months ended June 30, 2026. The decrease was primarily driven by a $2.2 million decrease in employee-related expenses, a $1.2 million decrease in external research and development expenses, a

$1.0 million decrease in internal research expenses, and a $0.4 million decrease in facilities and overhead costs. The decrease in employee-related expenses was primarily due to lower salaries, wages and stock-based compensation expense following our reduction in force and continued program prioritization efforts. The decrease in external research and development expenses was primarily due to lower external manufacturing costs and the recognition of government grant income as a reduction of research and development expense, partially offset by increased research milestone, clinical trial and animal study costs. The decrease in internal research expenses reflected lower laboratory consumables, supplies, shipping and other internal research activities compared to the prior-year period.

*General and Administrative Expenses*

General and administrative expenses decreased by $0.2 million, or 6%, from $2.6 million for the three months ended June 30, 2025 to $2.5 million for the three months ended June 30, 2026. The decrease was primarily driven by lower professional and consulting fees, including lower intellectual property legal fees, accounting, tax and audit fees and general legal costs, partially offset by modest increases in other general and administrative costs.

*Interest Income and Other Income, Net*

Interest income and other income, net decreased by $0.5 million, or 56%, from $0.9 million for the three months ended June 30, 2025 to $0.4 million for the three months ended June 30, 2026. The decrease was primarily due to lower average cash, cash equivalent and short-term investment balances available to earn interest, together with lower market interest rates.

*Interest Expense and Change in Fair Value of Convertible Note*

Interest expense and the change in fair value of the convertible note increased by $2.6 million, from $0.1 million of net income for the three months ended June 30, 2025 to $2.7 million of net income for the three months ended June 30, 2026. The increase was driven primarily by changes in the fair value assumptions used to estimate the expected conversion of the Lilly convertible note.

*Provision for / benefit from Income Taxes*

Income taxes changed by $0.9 million, from a $0.7 million benefit for the three months ended June 30, 2025 to a $0.2 million expense for the three months ended June 30, 2026. The income tax benefit recognized in the 2025 period was primarily attributable to the ability to carry back research and development tax credits generated in the period against taxable income recognized in prior years. In contrast, the 2026 period reflects an income tax expense related to interest accrued on unrecognized tax benefits. We have recorded a full valuation allowance against our deferred tax assets.

***Comparison of the Six Months Ended June 30, 2026 and 2025***

Our results of operations for the six months ended June 30, 2026 and 2025 are summarized as follows (dollars in thousands):

| | Six Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Collaboration revenue | $ 4,151 | $ 22,023 | $ (17,872) | (81)% |
| Operating expenses: | | | | |
| Research and development | 20,128 | 29,876 | (9,748) | (33)% |
| General and administrative | 5,844 | 9,332 | (3,488) | (37)% |
| Total operating expenses | 25,972 | 39,208 | (13,236) | (34)% |
| Loss from operations | (21,821) | (17,185) | (4,636) | 27% |
| Interest income and other income (expense), net | 898 | 1,944 | (1,046) | (54)% |
| Interest expense | (875) | (1,197) | 322 | (27)% |
| Change in fair value of convertible note | (1,585) | 2,083 | (3,668) | (176)% |
| Loss before provision for / benefit from income taxes | (23,383) | (14,355) | (9,028) | 63% |
| (Provision for) / benefit from income taxes | (441) | 1,027 | (1,468) | (143)% |
| Net loss | $ (23,824) | $ (13,328) | $ (10,496) | 79% |

*Collaboration Revenue*

The following table summarizes our collaboration revenue for the six months ended June 30, 2026 and 2025 (dollars in thousands):

| | Six Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Lilly | $ 4,151 | $ 21,568 | $ (17,417) | (81)% |
| Sanofi | — | 455 | (455) | (100)% |
| Total collaboration revenue | $ 4,151 | $ 22,023 | $ (17,872) | (81)% |

Collaboration revenue was $4.2 million for the six months ended June 30, 2026 and $22.0 million for the six months ended June 30, 2025. The decrease of $17.9 million, or 81%, was primarily due to the absence of license revenue recognized in the prior-year period related to the achievement of a milestone as well as lower other collaboration revenue as fewer projects were underway in 2026. Revenue was also lower in the six months ended June 30, 2026 due to the nonrecurring revenue recognition in 2025 of consideration allocated to a material substitution right. The decrease also reflected differences in the timing and level of activities performed under our collaboration arrangements. During the six months ended June 30, 2026, collaboration revenue reflected revenue recognition under a single workplan with Lilly whereas the prior-year period included revenue recognition across multiple research and development workplans as well as Sanofi collaboration activities.

*Research and Development Expenses*

The following table summarizes our research and development expenses for the six months ended June 30, 2026 and 2025 (dollars in thousands):

| | Six Months Ended June 30, | | Change | Change |
|---|---|---|---|---|
| | 2026 | 2025 | $ | % |
| Internal research expenses | $ 1,496 | $ 3,974 | $ (2,478) | (62)% |
| External research and development | 7,870 | 10,037 | (2,167) | (22)% |
| Employee-related expenses | 7,306 | 11,104 | (3,798) | (34)% |
| Facilities and overhead costs | 3,030 | 3,908 | (878) | (22)% |
| Professional and consulting fees | 426 | 853 | (427) | (50)% |
| Total research and development | $ 20,128 | $ 29,876 | $ (9,748) | (33)% |

Research and development expenses decreased by $9.7 million, or 33%, from $29.9 million for the six months ended June 30, 2025 to $20.1 million for the six months ended June 30, 2026. The decrease was primarily driven by a $2.2 million decrease in external research and development costs, a $3.8 million decrease in employee-related expenses, a $2.5

million decrease in internal research expenses, a $0.9 million decrease in facilities and overhead costs and a $0.4 million decrease in professional and consulting fees. The decrease in external research and development costs was primarily driven by lower external manufacturing costs and offsetting government grant income recorded as a contra-expense, partially offset by higher research milestones and clinical-trial CRO costs as STX-1150 advanced into the clinic. The decrease in employee-related expenses was primarily due to lower salaries, bonuses, severance, payroll taxes and benefits following the prior-year reduction in force, partially offset by higher stock-based compensation. The decrease in internal research expenses reflected a reduction in year-over-year spend on STX-1150 as the program progressed into the clinic.

*General and Administrative expenses*

General and administrative expenses decreased by $3.5 million, or 37%, from $9.3 million for the six months ended June 30, 2025 to $5.8 million for the six months ended June 30, 2026. The decrease was primarily driven by a $3.9 million decrease in professional and consulting fees, including the absence of nonrecurring expensed deferred offering costs recognized in the prior-year period. The decrease in professional and consulting fees was primarily attributable to lower general legal, accounting, tax and audit, other professional and intellectual property legal costs. This decrease was partially offset by higher employee-related and other general and administrative costs.

*Interest Income and Other Income, Net*

Interest income and other income, net decreased by $1.0 million, or 54%, from $1.9 million for the six months ended June 30, 2025 to $0.9 million for the six months ended June 30, 2026. The decrease was primarily due to lower average cash, cash equivalent and short-term investment balances available to earn interest and lower market interest rates.

*Interest Expense and Change in Fair Value of Convertible Note*

Interest expense and the change in fair value of the convertible note changed by $3.3 million, from $0.9 million of net income for the six months ended June 30, 2025 to $2.5 million of net expense for the six months ended June 30, 2026. The movement was driven primarily by changes in the fair value assumptions used to estimate the expected conversion of the Lilly convertible note.

*Provision for / benefit from Income Taxes*

Income taxes changed by $1.5 million, from a $1.0 million benefit for the six months ended June 30, 2025 to a $0.4 million expense for the six months ended June 30, 2026. The benefit recognized in the 2025 period was primarily attributable to the carryback of research and development tax credits against taxable income recognized in prior years, while the 2026 period reflects an income tax expense related to interest accrued on unrecognized tax benefits. We have recorded a full valuation allowance against our deferred tax assets.

**Liquidity, Capital Resources and Capital Requirements**

***Sources of Liquidity***

Since our inception, we have incurred significant operating losses and negative cash flows from operations. To date, we have funded our operations primarily through proceeds from collaboration and license agreements, the issuance of equity securities, our initial public offering, and convertible debt financings.

As of June 30, 2026, we had $43.0 million in cash, cash equivalents, and short-term available-for-sale investments, $37.8 million in outstanding indebtedness and an accumulated deficit of $181.5 million. Our cash, cash equivalents, and available-for-sale investments are primarily invested in highly liquid investment-grade securities and money market funds. Our primary uses of capital are funding research and development activities, including the advancement of our product candidates and technology platforms, manufacturing activities, clinical development activities, and general and administrative expenses to support our operations.

In July 2026, we completed our initial public offering and concurrent private placement, generating aggregate net proceeds of approximately $140.7 million after underwriting discounts, commissions and offering expenses. We believe our existing cash, cash equivalents and available-for-sale investments, together with the net proceeds received from the initial public offering and concurrent private placement, will be sufficient to fund our planned operating expenses and capital expenditure requirements for at least the next 12 months and is expected to be sufficient into the first half of 2029. Our estimate is based on assumptions that may prove to be incorrect, and we could utilize our available capital resources sooner than we currently expect. We will continue to monitor our capital requirements and may pursue additional financing opportunities, strategic collaborations, or other business development transactions to support the advancement of our pipeline and technology platforms.

On July 17, 2026, the outstanding principal balance and accrued interest related to the 8% convertible promissory note issued to Eli Lilly and Company (the "Lilly Note") automatically converted into approximately 1.05 million shares of our common stock. The Lilly Note had an outstanding principal balance of $30.0 million and accrued interest of approximately $7.8 million at maturity. No cash payments were required in connection with the conversion, and the Lilly Note was fully extinguished following the conversion. As a result, we no longer have indebtedness associated with the Lilly Note.

In December 2025, the California Institute for Regenerative Medicine ("CIRM") awarded us grants totaling up to approximately $25.7 million to support the development of our STX-1200 and STX-1400 programs. We accepted the awards during the second quarter of 2026. Funding is subject to the achievement of specified operational milestones and compliance with the terms and conditions of the grant agreements. We expect future grant disbursements to support the continued advancement of these programs.

***Future Funding Requirements***

We will need substantial additional funding to support our continuing operations and pursue our long-term business plan. Our primary uses of cash are to fund our operations, which consist primarily of research and development expenditures related to our programs and, to a lesser extent, general and administrative expenditures. We expect to continue to incur significant operating losses for the foreseeable future as we advance our lead product candidate, STX-1150, through clinical development, progress our STX-1200 and STX-1400 programs, continue to invest in our ELXR and XE platforms, expand our development capabilities, and operate as a public company. Our future capital requirements will depend on numerous factors, including the pace and scope of our research and development activities, the timing and results of clinical trials, manufacturing costs, regulatory requirements, the timing of milestone payments under existing and future collaboration agreements, and the level of investment required to support future commercialization activities.

Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable, accrued expenses, and prepaid expenses.

Our future funding requirements will depend on many factors, including the following:

- the timing, results, cost and progress of preclinical studies and clinical trials for our lead product candidate, STX-1150, our STX-1200 and STX-1400 development programs and any other future product candidates;
- the number and scope of preclinical and clinical programs we decide to pursue;
- the costs of manufacturing any product candidates;
- the progress and success of developing our ELXR and XE technologies;
- the cost of regulatory submissions and timing of regulatory approvals, if any;
- the progress of the development efforts of parties with whom we have entered into licenses, collaborations, and strategic partnerships;
- the timing and amount of milestone and other payments we are obligated to make under our licensing agreements, including the UCB Exclusive License Agreement, Acuitas Agreement and any future license agreements;
- the cash requirements of any future acquisitions of, and the discovery of, product candidates;
- our ability to establish and maintain collaborations, strategic partnerships or marketing, distribution, licensing or other strategic arrangements with third parties on favorable terms, if at all;

- our ability to achieve sufficient market acceptance, adequate coverage and reimbursement from third-party payors and adequate market share and revenue for any approved product candidates;
- the costs involved in prosecuting and enforcing patent and other intellectual property claims;
- the cost of commercialization activities if any product candidates are approved for sale, including marketing, sales and distribution costs;
- our efforts to enhance operational systems and hire additional personnel, including personnel to support development of any product candidates;
- potential delays in our preclinical studies and clinical trials, if any, due to unforeseen events; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems to satisfy our obligations as a public company.

Although we believe our existing cash resources as of June 30, 2026 and the net proceeds from our initial public offering and concurrent private offering will fund our operations into the first half of 2029, we may seek additional capital earlier due to favorable market conditions, strategic opportunities, or changes in operating plans. Until we generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, licensing arrangements, government grants and other strategic transactions. There are no assurances that we will be successful in obtaining an adequate level of financing to support our business plans when needed, on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration or licensing arrangements with third parties or other strategic transactions, we may have to relinquish rights to our intellectual property, future revenue streams, research programs, or product candidates or grant licenses on terms that may not be favorable to us. If additional funding is required and is unavailable on acceptable terms, we may be required to delay, reduce or discontinue portions of our development programs or other strategic initiatives.

***Cash Flows***

The following table summarizes our cash flows for the periods presented (in thousands):

| | For the Six Months Ended June 30, | |
|---|---|---|
| | 2026 | 2025 |
| Cash used in operating activities | $ (12,892) | $ (20,252) |
| Cash provided by investing activities | 37,470 | 23,579 |
| Cash (used in) / provided by financing activities | (1,788) | 51 |
| Net change in cash, cash equivalents and restricted cash | $ 22,790 | $ 3,378 |

*Operating Activities*

Cash used in operating activities was $12.9 million for the six months ended June 30, 2026, compared to $20.3 million for the six months ended June 30, 2025. The decrease in cash used in operating activities was primarily attributable to lower cash operating expenditures resulting from reduced research and development activities and lower general and administrative spending compared to the prior-year period.

For the six months ended June 30, 2026, cash used in operating activities consisted of our net loss of $23.8 million, adjusted for $6.4 million of non-cash charges, cash received from government grants of $3.8 million and net cash inflows of $0.7 million from changes in operating assets and liabilities. Non-cash charges consisted primarily of stock-based compensation expense, depreciation and amortization, non-cash interest expense, and the change in fair value of the Lilly convertible note. Net cash inflows from changes in operating assets and liabilities were primarily driven by the collection of collaboration receivables, partially offset by decreases in deferred revenue and accrued liabilities.

For the six months ended June 30, 2025, cash used in operating activities consisted of our net loss of $13.3 million, adjusted for $6.2 million of non-cash charges, and $13.1 million of net cash outflows from changes in operating assets and liabilities. Net cash outflows from changes in operating assets and liabilities were primarily attributable to decreases in

deferred revenue and increases in collaboration receivables, partially offset by increases in accounts payable and accrued liabilities.

*Investing Activities*

Cash provided by investing activities was $37.5 million for the six months ended June 30, 2026, compared to $23.6 million for the six months ended June 30, 2025.

For the six months ended June 30, 2026, investing activities primarily reflected maturities of short-term available-for-sale investments of approximately $42.3 million, partially offset by purchases of short-term available-for-sale investments of approximately $4.8 million.

For the six months ended June 30, 2025, investing activities primarily reflected maturities of short-term available-for-sale investments of approximately $56.2 million, partially offset by purchases of short-term available-for-sale investments of approximately $31.7 million and capital expenditures related to property and equipment purchases. The increase in cash provided by investing activities in 2026 was primarily due to higher net proceeds from investment maturities.

*Financing Activities*

Cash used in financing activities was $1.8 million for the six months ended June 30, 2026, compared to cash provided by financing activities of less than $0.1 million for the six months ended June 30, 2025.

For the six months ended June 30, 2026, financing activities primarily consisted of payments of deferred offering costs associated with our initial public offering, partially offset by proceeds from stock option exercises.

For the six months ended June 30, 2025, financing activities consisted primarily of proceeds received from stock option exercises.

**Recent Accounting Pronouncements**

A description of recently issued and adopted accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our audited annual financial statements for the periods ending December 31, 2024, and 2025 and to our unaudited interim condensed financial statements included elsewhere in this Quarterly Report.

**Critical Accounting Estimates**

Significant accounting policies are described in Note 2 to the audited financial statements included in our final prospectus filed pursuant to Rule 424(b)(4) on July 24, 2026 and Note 2 to the unaudited condensed financial statements included in this Quarterly Report on Form 10-Q. There were no material changes to these accounting policies during the three months ended June 30, 2026.

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect reported amounts and related disclosures. We consider an accounting estimate to be critical if it requires management to make assumptions about matters that are highly uncertain at the time the estimate is made and if different estimates reasonably could have been used that would have a material impact on our financial statements.

There have been no material changes to our critical accounting estimates during the three months ended June 30, 2026 from those disclosed in the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Estimates" included in our final prospectus filed pursuant to Rule 424(b)(4) on July 24, 2026.

**Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are a smaller reporting company and are not required to provide the information otherwise required by this Item 3.

**Item 4. Controls and Procedures.**

**Evaluation of Disclosure Controls and Procedures**

Our disclosure controls and procedures are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the Securities and Exchange Commission's rules and forms and that such information is accumulated and communicated to our management, including the Chief Executive Officer and the Chief Financial Officer, to allow timely decisions regarding required disclosures. As of June 30, 2026, our management, with the participation of our Chief Executive Officer and Chief Financial Officer, performed an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act. Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of June 30, 2026.

**Changes in Internal Control Over Financial Reporting**

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) or 15d-15(d) that occurred during the quarter ended June 30, 2026, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

From time to time, we may be involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, in the opinion of management, would have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputation harm, and other factors.

### Item 1A. Risk Factors.

*Investing in our common stock involves a high degree of risk. Before making your decision to invest in shares of our common stock, you should carefully consider the risks described below, together with the other information contained in Quarterly Report on Form 10-Q, including in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and in our condensed financial statements and the related notes included in this Quarterly Report on Form 10-Q. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of or that we deem immaterial may also become important factors that adversely affect our business. We cannot assure you that any of the events discussed below will not occur. These events could have a material adverse impact on our business, financial condition, results of operations, and prospects.*

### Risk Factors Summary

The following is a summary of the principal risks we face. This summary does not address all of the risks that we face, and is qualified in its entirety by, and should be read together with, the more detailed discussion of risks and uncertainties set forth under this Part II, Item 1A, "Risk Factors," and the other information in this Quarterly Report. These risks include, but are not limited to, the following:

- We are a clinical-stage biotechnology company with a limited operating history and no products approved for commercial sale; we have incurred significant losses since inception, have never generated product revenue, and expect to continue to incur losses for the foreseeable future, and we may never achieve or sustain profitability.
- We will need substantial additional funding that may not be available on acceptable terms, or at all, to advance our programs.
- We are early in our development efforts, and it will be many years, if ever, before we have an approved product; genetic medicine and epigenetic modification are novel and complex, and adverse public perception of gene editing or modification could harm our prospects.
- Clinical drug development is lengthy, expensive, and uncertain; results of preclinical studies and earlier trials, including preliminary, interim, or topline data, may not be predictive of later results, and STX-1150, STX-1200, STX-1400, or our other product candidates may fail to demonstrate safety and efficacy, cause undesirable side effects, or fail to gain acceptance by physicians, patients, or payors.
- We may experience delays or difficulties in patient enrollment, manufacturing, or the durability of epigenetic modulation in humans, any of which could delay or prevent regulatory approval and commercialization.
- If we are unable to establish sales and marketing capabilities, we may not successfully commercialize any approved products.
- We rely, and expect to continue to rely, on third parties — including collaborators, licensors, clinical trial partners, and single-source manufacturers and suppliers — and the failure of these parties to perform, or conflicts within these relationships, could delay or harm our development and commercialization efforts.
- Our success depends on our ability to attract and retain key management, scientific, and clinical personnel and to manage our anticipated growth, and our relationships with our co-founders may create the appearance of conflicts of interest.

- We depend on the uninterrupted operation of our information technology systems and are subject to stringent and evolving data privacy and security laws, a significant risk of product liability, potential misconduct by employees or other agents, and competition in an environment of rapid technological change.

- Changes in tax laws, or limitations on our ability to use net operating loss carryforwards and other tax attributes, and natural disasters, geopolitical events, or other business disruptions could adversely affect our business and financial condition.

- If we are unable to obtain, maintain, protect, and enforce patent and other intellectual property protection for our technologies and product candidates — including our CRISPR-based ELXR and XE technologies, which are subject to license and other agreements — our competitive position could be harmed.

- We may become subject to costly intellectual property litigation, including third-party infringement claims, challenges to the inventorship or ownership of our intellectual property, or claims that our personnel misappropriated third-party trade secrets, and changes in patent law could diminish the value of our patents.

- Gene editing and modification are subject to significant and evolving regulation, and the regulatory approval processes of the FDA and comparable foreign authorities are lengthy, unpredictable, and may be affected by disruptions at government agencies; we may not obtain or maintain orphan drug, Breakthrough Therapy, Fast Track, or similar designations or realize their anticipated benefits.

- Even if approved, our product candidates will remain subject to ongoing regulatory obligations and may face competition from biosimilars or lower-cost alternatives, and our operations and relationships with healthcare providers and payors are subject to healthcare, fraud and abuse, export and import, and legislative and regulatory requirements that could change.

- Our operating results may fluctuate significantly and the market price of our common stock is likely to be highly volatile; our principal stockholders and management own a significant percentage of our stock and can exert significant control, sales of substantial shares could depress our stock price, and we do not intend to pay dividends.

- We are an "emerging growth company" and a "smaller reporting company" subject to reduced disclosure requirements; if we fail to maintain effective internal control over financial reporting and disclosure controls, or if anti-takeover and exclusive forum provisions apply, our stock and our stockholders' interests could be adversely affected.

- Unfavorable macroeconomic conditions or market volatility, a lack of or unfavorable analyst coverage, the increased costs of operating as a public company, and potential securities litigation could each adversely affect our business, financial condition, and stock price.

**Risks related to our financial position, limited operating history and need for additional capital**

***We have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

We are a clinical-stage biotechnology company with a limited operating history on which to base your investment decision. Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk, and we have yet to complete any clinical trials. We commenced operations in 2017, and our operations to date have been limited primarily to business planning, raising capital, acquiring certain intellectual property rights, developing and engineering our two proprietary technologies, the Epigenetic Long-Term X-Repressor, or ELXR, and X-Editor, or XE, identifying potential product candidates, and advancing our pipeline programs. All of our programs are in the research and discovery, preclinical or clinical stage of development and their risk of failure is high. To date, we have devoted substantially all of our resources to identifying, acquiring and developing our product candidates, building our pipeline, conducting preclinical studies and our clinical trial, organizing and staffing our company, business planning, establishing and maintaining our intellectual property portfolio, raising capital, and providing general and administrative support for these operations.

We have not yet demonstrated an ability to successfully complete any clinical trials, obtain regulatory approvals, manufacture a clinical or commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. As a result, particularly in light of the rapidly evolving field of genetic medicine, it may be more difficult for you to accurately evaluate the performance of our business to date or to predict our likelihood of success and viability than it would be if we had a longer operating history.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays, and other known and unknown factors and risks frequently experienced by clinical-stage biotechnology companies developing targeted product candidates for cardiometabolic diseases.

***We have a history of operating losses, we have never generated any product revenue, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future.***

We have incurred significant net losses in each reporting period since our inception, have not generated any product revenue to date, and have financed our operations principally through private placements of our preferred stock and collaboration revenue. Our net losses were $23.8 million and $13.3 million for the six months ended June 30, 2026 and 2025, respectively. As of June 30, 2026, we had an accumulated deficit of $181.5 million. Substantially all of our losses have resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of our lead program, product candidates, and our technology. The net losses we incur may fluctuate significantly from quarter-to-quarter and year-to-year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We anticipate that our expenses will increase substantially if, and as, we:

- advance our lead product candidate, STX-1150, through clinical trials and conduct later stage clinical trials;
- initiate clinical trials for our STX-1200 and STX-1400 developmental programs;
- manufacture, or have manufactured, preclinical, clinical and potentially commercial supplies of any product candidates;
- further develop our proprietary technologies, ELXR and XE, as well as engineer new technologies;
- begin or continue preclinical development of our other undisclosed cardiometabolic programs;
- identify and develop potential product candidates;
- experience any delays in our preclinical studies and clinical trials, if any, due to unforeseen events;
- obtain, expand, maintain, defend, and enforce our intellectual property portfolio;
- seek regulatory approvals for any product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any product candidates, if approved;
- comply with our obligations under the agreements governing our licenses, collaborations, and strategic partnerships;
- establish additional licenses, collaborations, or strategic partnerships;
- acquire or in-license intellectual property and technologies, work with strategic partners to support and expand our research and discovery, and initiate and conduct preclinical and clinical programs;
- hire additional clinical, scientific, and management personnel, as well as administrative staff to support the growth of our business;
- add operational, financial, and management information systems and personnel; and
- incur additional legal, accounting, and other costs associated with operating as a public company.

Even if we succeed in developing STX-1150 or product candidates in any of our other programs, we may never achieve commercialization, and we may continue to incur substantial research and development expenses and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties,

complications, delays, and other unknown factors that may adversely affect our business, financial condition, results of operations, and prospects. The size of our future losses will depend, in part, on the rate of future growth of our expenses and our ability to generate product revenue, if any. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

***Substantial doubt as to our ability to continue as a going concern existed as of June 30, 2026 and was alleviated only as a result of our initial public offering. If substantial doubt recurs and we cannot continue as a going concern, our stockholders may lose some or all of their investment in our company.***

Our unaudited interim financial statements, included elsewhere in this Quarterly Report, were prepared assuming that we will continue as a going concern. The going concern basis of presentation assumes that we will continue in operation for the foreseeable future and will be able to realize our assets and satisfy our liabilities in the normal course of business and do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or amounts and classification of liabilities that may result from our inability to continue as a going concern. As of June 30, 2026, we had cash, cash equivalents and marketable securities of $43.0 million. We have incurred recurring losses since inception, including a net loss of $23.8 million for the six months ended June 30, 2026. As of June 30, 2026, we had an accumulated deficit of $181.5 million. Based on our capital resources on hand as of June 30, 2026, which consists of cash, cash equivalents and marketable securities, we did not have sufficient cash to fund our then-current and planned operations for at least twelve months from the date of issuance of our financial statements. However, substantial doubt about our ability to continue as a going concern was alleviated by the completion of our initial public offering and concurrent private placement in July 2026. Substantial doubt could recur in future periods if we use our capital resources sooner than expected or are unable to obtain additional funding when needed.

Additionally, if we are unable to obtain funding, through equity financings, debt financings, or other capital sources, we could be forced to delay, reduce, or eliminate some or all of our programs, product candidates, development of our platform, or commercialization efforts. Any such actions could adversely affect our business prospects or our ability to continue as a going concern. There is no assurance that we will be successful in obtaining sufficient funding on terms acceptable to fund continuing operations, if at all. Even if we are able to raise additional capital, there is no guarantee the proceeds would be sufficient to support our operating plans for at least the next twelve months from the date of issuance of our financial statements. If we cannot continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our combined financial statements, and it is likely that our stockholders may lose some or all of their investment in us.

***We will need substantial additional funds to pursue our business objectives, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our development initiatives and programs or other operations.***

Identifying, developing, and continuing the research and development of targets and product candidates is a time-consuming, capital-intensive, and uncertain process that takes years to complete. If we identify any product candidates and initiate any preclinical studies or clinical trials, development will require substantial additional funds, and such products or studies may not be successful or may require us to significantly expand or create our development, regulatory, manufacturing, marketing, and sales capabilities. We have used substantial amounts of cash since inception to develop our proprietary technologies, ELXR and XE, and because we have limited financial and managerial resources, we have prioritized our research and discovery programs in specific indications. Further, we will require significant funds to conduct further research and development and initiate preclinical testing and clinical trials for any product candidates, to seek regulatory approvals for any product candidates, and to manufacture and market products, if any, which are approved for commercial sale. In addition, as a result of our initial public offering and the concurrent private placement, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. We may also need to raise additional funds sooner if we choose to pursue additional indications or markets for any product candidates or otherwise expand more rapidly than we presently anticipate.

The development of genetic medicines through our proprietary technologies, ELXR and XE, including the initiation of clinical trials and preclinical studies for our pipeline programs targeting PCSK9, LPA, and APOC3 and any product candidates, will require substantial funds. As of June 30, 2026, we had $43.0 million in cash, cash equivalents, and investments. Based on our current operating plan, we believe that our cash, cash equivalents, and investments as of June 30, 2026, together with the net proceeds from our initial public offering and the concurrent private placement, will be sufficient to fund our operating expenses and capital expenditure requirements into the first half of 2029. Substantial doubt about our

ability to continue as a going concern existed as of June 30, 2026 and was alleviated as a result of the completion of our initial public offering and the concurrent private placement, which resulted in aggregate net proceeds of approximately $140.7 million. Our operating plan is based on assumptions that may prove to be incorrect, however, and we could exhaust our available capital resources sooner than we expect. If we are unable to obtain additional funding when needed, substantial doubt about our ability to continue as a going concern could recur in future periods, and we may be forced to delay, reduce, or eliminate some or all of our programs, product candidates, development of our platform, or commercialization efforts.

However, our future capital requirements and the period for which we expect our existing resources to support our operations, fund continued growth of our operations, research and development of product candidates, or otherwise respond to competitive pressures, may vary significantly from what we expect and we may need to seek additional funds sooner than planned. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Our spending levels vary based on new and ongoing research and development and other corporate activities. Because of the length of time and challenges associated with research and development of genetic medicines using genetic modification technologies, including through our proprietary technologies, ELXR and XE, the development and success of our lead product candidate, development programs or any other product candidates, is highly uncertain. We are unable to estimate the actual funds we will require for development and any marketing and commercialization activities for any approved products in the future. Our funding requirements for our proprietary technologies, ELXR and XE, any product candidates and our ongoing operations, both near- and long-term, will depend on many factors, including, but not limited to:

- the timing, results, cost and progress of preclinical studies and clinical trials for our STX-1150 product candidate, our STX-1200 and STX-1400 development programs and any other future product candidates;
- the progress and success of developing CRISPR by Design and our ELXR and XE technologies;
- the cost of regulatory submissions and timing of regulatory approvals, if any;
- the number and scope of preclinical and clinical programs we decide to pursue;
- the progress of the development efforts of parties with whom we have entered into licenses, collaborations, and strategic partnerships;
- the timing and amount of milestone and other payments we are obligated to make under our licensing agreements, including the UCB Exclusive License Agreement (as defined herein), Acuitas Agreement (as defined herein) and any future license agreements;
- the cash requirements of any future acquisitions of, or discovery of, product candidates;
- our ability to establish and maintain collaborations, strategic partnerships or marketing, distribution, licensing or other strategic arrangements with third parties on favorable terms, if at all;
- our ability to achieve sufficient market acceptance, adequate coverage and reimbursement from third-party payors and adequate market share and revenue for any approved product candidates;
- the costs involved in prosecuting and enforcing patent and other intellectual property claims;
- the costs of manufacturing any product candidates;
- the cost of commercialization activities if any product candidates are approved for sale, including marketing, sales and distribution costs;
- our efforts to enhance operational systems and hire additional personnel, including personnel to support development of any product candidates;
- potential delays in our preclinical studies and clinical trials, if any, due to unforeseen events; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems to satisfy our obligations as a public company.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may have to delay, reduce, or terminate our research and development programs and preclinical studies or clinical trials, limit strategic opportunities or undergo reductions in our workforce or other corporate restructuring activities. We do not expect to realize revenue from sales of commercial products or royalties from licensed products in the foreseeable future, if at all, and, in no event, before any product candidates are clinically tested, approved for commercialization and successfully marketed.

We will be required to seek additional funding in the future and currently intend to do so through public or private equity offerings or debt financings, additional licensing agreements and/or collaborations, credit or loan facilities, government grants, or a combination of one or more of these funding sources. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Our future debt financings, if available, are likely to involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of our equity securities receive any distribution of our corporate assets. If we raise additional funds through licensing or collaboration arrangements with third parties, we may have to relinquish valuable rights to product candidates or grant licenses on terms that are not favorable to us. Our license and collaboration agreements and any future collaboration or other agreements may also be terminated if we are unable to meet the payment or other obligations under the agreements. We also could be required to seek collaborators for product candidates at an earlier stage than otherwise would be desirable or relinquish our rights to product candidates or technologies that we otherwise would seek to develop or commercialize ourselves. Failure to obtain capital when needed on acceptable terms, or at all, may force us to delay, limit, or terminate our product development and commercialization of our current or future product candidates, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

**Risks related to discovery, development, and commercialization**

***We are early in our development efforts and we expect it will be many years before we commercialize any product candidate, if ever. If we are unable to advance any product candidates into and through clinical trials, obtain regulatory approval and ultimately commercialize any product candidates, or experience significant delays in doing so, our business will be materially harmed.***

The success of our business depends primarily upon our ability to identify, develop, and commercialize product candidates using our ELXR and XE technologies. We have initiated clinical development of our lead product candidate, STX-1150, and are in preclinical development for our STX-1200 and STX-1400 development programs. Our future success depends heavily on the successful identification and development of product candidates using our ELXR and XE technologies. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will be a result of the successful development and eventual commercialization of our product candidates, which may never occur. Any product candidates we develop may have adverse side effects or fail to demonstrate safety, purity or potency, which is considered by the U.S. Food and Drug Administration, or FDA, to include effectiveness. Additionally, our product candidates may have other characteristics that may make them impractical or prohibitively expensive for large-scale manufacturing. Furthermore, our product candidates may not receive regulatory approval or, if they do, they may not be accepted by the medical community or patients, or may not be competitive with other products. We currently have no product revenue and we may never be able to successfully develop or commercialize a marketable product.

All of our product candidates and development programs are still in early stages of development. Our research methodology may be unsuccessful in identifying potential targets and product candidates, our product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies, they may not show promising signals of therapeutic activity in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture or develop, unmarketable, or unlikely to receive regulatory approval. We may experience delays in initiating, conducting, or completing preclinical studies for a variety of reasons, including due to supply chain interruptions that could lead to shortages in materials or animals required for such studies. For example, there have been reports of a shortage of non-human primates, or NHPs, for biomedical research, which are used in our preclinical studies.

Commencing a clinical trial in the United States is also subject to the FDA allowing the clinical trial to proceed under an IND and finalizing the trial design based on discussions with the FDA. Even after we receive and incorporate advice from the FDA or comparable foreign regulatory authorities, these regulatory authorities could disagree that we have satisfied their requirements to commence our clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional studies or trials or impose stricter conditions on the conduct

of our clinical trials than we currently expect. There are comparable processes and risks applicable to clinical trial applications in other countries, including in Europe.

Even if we complete the clinical trials necessary to support submission of a Biologics License Application, or BLA, in the United States, or comparable marketing application in another jurisdiction, we cannot predict when, or if, we will obtain regulatory approval to commercialize our product candidates in the United States or any other jurisdiction, and any such approval may be for a more narrow indication than we seek. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product candidate testing and validation and additional administrative review periods.

Commercialization of any product candidates we may develop will also require preclinical and clinical development; regulatory review and approval in multiple jurisdictions, including by the FDA or other regulatory authorities; manufacturing supply, capacity and expertise; building of a commercial organization; and significant marketing efforts.

The success of any product candidates we may identify and develop will depend on many factors, including the following:

- market acceptance of our ELXR and XE technologies;
- timely completion of successful preclinical studies, including toxicology studies, biodistribution studies, pharmacology studies and other studies in animals, where applicable;
- being allowed to proceed with clinical trials under Investigational New Drug applications, or INDs, Clinical Trial Notifications, or CTNs, or other comparable foreign applications for our planned clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including in compliance with the FDA's good clinical practices, or GCPs, good laboratory practices, or GLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from any clinical trials that support a finding by applicable regulatory authorities of safety, purity, potency, effectiveness, and an acceptable risk-benefit profile in the intended populations;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense, and enforcement of patent, trademark, trade secret, and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;
- acceptance of any benefits and use of our product candidates we may develop, including method of administration, if and when approved, by patients, the medical community, and third-party payors;
- effective competition with other therapies;
- sufficiency of our financial and other resources;
- maintenance of a continued acceptable safety, tolerability, and efficacy profile of any product candidates we may develop following approval; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payors.

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

***Genetic medicine, and epigenetic modification in particular, are novel concepts that are not yet clinically validated for human therapeutic use. The approach we are taking to discover and develop novel therapeutics using our ELXR and XE technologies is unproven and may never lead to marketable products.***

We are focused on developing engineered genetic medicines utilizing our proprietary technologies, ELXR and XE, which are new and unproven. ELXR and XE, which we have developed and are utilizing in our research and discovery programs, have not yet been clinically tested. The scientific evidence to support the feasibility of selection of targets and development of product candidates based on genetic modification technologies is both preliminary and limited. Successful development of product candidates will require us to safely deliver product candidates using ELXR and XE into target cells, optimize the efficiency and specificity of such product candidates and ensure the therapeutic selectivity of such product candidates. We may need to address other safety issues as well, and to demonstrate the potential value of these product candidates, and, in some cases, we may need to achieve these goals with a single administration and demonstrate a permanent correction. There can be no assurance that ELXR and XE will achieve these goals, lead to the development of genetic medicine, or be successful in addressing any or all of these challenges. Additionally, while we currently expect to use lipid nanoparticles, or LNPs, for delivery of our ELXR- and XE-based product candidates, the LNPs we expect to utilize will need to be evaluated as part of the clinical trials of our investigational product candidates.

Our future success is highly dependent on the successful development of our genetic modification technologies, delivery methods, and therapeutic applications of such technologies. We may decide to alter or abandon our initial research and discovery programs as new data become available and as we gain experience in developing gene editing and genetic modification therapeutics. We cannot be sure that our technologies will yield products that are safe, potent, effective, scalable, or profitable in any indication we pursue. Adverse developments in the clinical development efforts of other gene editing and genetic modification technology companies could adversely affect our efforts or the perception of any product candidates we may develop by both investors and regulatory authorities.

Similarly, other gene editing approaches may be determined to be more attractive than our technologies. Moreover, if we decide to develop genetic modification technologies outside of ELXR and XE, we cannot be certain that such technologies will be successful or compete with other existing technologies. Any of these factors could reduce or eliminate our commercial opportunity and could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***Adverse public perception of gene editing or gene modification may negatively impact regulatory approval of, and/or demand for, our product candidates.***

Certain of our product candidates, in particular those using our XE technology, involve editing the human genome and making permanent changes. The potential clinical and commercial success of our product candidates will depend in part on public understanding and acceptance of the use of gene editing therapy for the prevention or treatment of human diseases. Public perception and related media coverage relating to the adoption of new therapeutics or novel approaches to treatment, as well as ethical concerns related specifically to gene editing, may adversely influence the willingness of subjects to participate in clinical trials, or, if any product candidate is approved, of physicians and patients to accept these treatments. Adverse events may occur in our preclinical studies or clinical trials or those of our competitors or of academic researchers utilizing gene modification technologies, even if not ultimately attributable to product candidates we may identify and develop, and negative publicity could result in increased governmental regulation, unfavorable public perception, potential delays in the testing or approval of product candidates we may identify and develop, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates.

Physicians, healthcare providers, and third-party payors often are slow to adopt new products, technologies, and treatment practices, particularly those that may also require additional upfront costs and training. Physicians may not be willing to undergo training to adopt these novel therapies, may decide the particular therapy is too complex or potentially risky to adopt without appropriate training, and may choose not to administer the therapy. Furthermore, due to health conditions, genetic profile, or other reasons, certain patients may not be candidates for the therapies. In addition, responses by federal and state agencies, Congressional committees, and foreign governments to negative public perception, ethical concerns, or financial considerations may result in new legislation, regulations, or medical standards that could limit our ability to develop or commercialize any product candidates, obtain or maintain regulatory approval, or otherwise achieve profitability. New government requirements may be established that could delay or prevent regulatory approval of any product candidates we may develop. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be. Based on these and other factors, healthcare providers and payors may decide that the benefits of these new therapies do not or will not outweigh their costs.

***Clinical drug development involves a lengthy and expensive process, with an uncertain outcome, and we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for our product candidates, and the regulatory landscape that will govern our product candidates is uncertain.***

The time required to obtain approval for any of our product candidates from the FDA or other comparable foreign regulatory authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of regulatory authorities. Clinical trials, if any, may fail to demonstrate that our product candidates are safe, pure, potent, or effective for their intended uses. Even if initial clinical trials or animal studies in any of our product candidates we may develop are successful, such product candidates may fail to show the desired quality, safety, or efficacy in later stages of clinical development despite having successfully advanced through preclinical studies and initial clinical trials. There is a high failure rate for biologics proceeding through clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and regulatory authorities may not agree with the conclusions we draw from our preclinical studies and clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later-stage clinical trials even after achieving promising results in earlier stage clinical trials.

Further, we or our collaborators may experience delays in initiating or completing clinical trials. We or our collaborators also may experience numerous unforeseen events during, or as a result of, any future clinical trials that we could conduct that could delay or prevent our ability to receive regulatory approval or commercialize our clinical product candidates or any product candidates, including:

- the FDA, Australian TGA, or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials;
- delays or failure to obtain regulatory allowance or approval to initiate our clinical trials, as well as delays or failures to obtain any necessary approvals from institutional review boards, or IRBs, or ethics committees at clinical sites;
- delays, suspension, or termination of our clinical trials by regulatory authorities, IRBs, or ethics committees;
- modification of clinical trial protocols;
- delays or failures in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites, as well as possible future breaches of such agreements;
- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials;
- failure to manufacture sufficient quantities of our product candidates for use in our clinical trials;
- failure by third parties, including contract development and manufacturing organizations, or CDMOs, CROs, and clinical trial sites to comply with regulatory requirements or trial protocols, or meet their contractual obligations to us in a timely manner, or at all;
- subjects experiencing unexpected treatment-related severe or serious adverse events;
- imposition of a temporary or permanent clinical hold for safety or other reasons, such as a result of a new safety finding in a clinical trial on a similar product by one of our competitors, that presents unreasonable risk to clinical trial participants;
- changes in regulatory requirements and/or guidance that require amending or submitting new clinical trial protocols;
- changes in the standard of care on which we developed our clinical development plan, which may require us to conduct new or additional trials;
- the number of subjects required for clinical trials of any product candidates being larger than we anticipate, subjects failing to enroll or remain in our trials at the rate we expect, or failing to return for post-treatment follow-up;
- patients choosing an alternative product for the indications for which we are developing our product candidates, or participating in competing clinical trials;
- the cost of clinical trials of our product candidates being greater than we anticipated;
- insufficient funding to continue clinical trials with our product candidates;

- the emergence of unforeseen safety issues or undesirable side effects;
- clinical trials of product candidates producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development of our product candidates;
- selection of clinical trial endpoints that require prolonged periods of observations or extended analysis of the resulting data;
- inability to establish clinical trial endpoints that applicable regulatory authorities consider clinically meaningful;
- results or reports from testing conducted by our competitors or others may raise safety or efficacy concerns about our programs; and
- interruptions, delays, or staffing shortages resulting from public health crises.

Clinical trials must be conducted in accordance with the FDA's, Australian TGA's and/or other applicable regulatory authorities' legal requirements, and remain subject to oversight by these governmental authorities and ethics committees at the medical institutions where such clinical trials are conducted. In addition, changes in regulatory requirements and policies may occur, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to regulators or to committees for reexamination, which may impact the costs, timing or successful completion of a clinical trial. In addition, regulatory agencies may require extended follow-up observation periods of patients who receive treatment using gene editing products such as the FDA's recommended 15-year follow-up observation period for such patients, which will require us to adopt such observation periods for any product candidates we develop if required by the relevant regulatory agencies, which could vary by country or region.

Further, conducting clinical trials in foreign countries, as we plan to do for our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled subjects in foreign countries to adhere to clinical protocols as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, and political and economic risks, including war, relevant to such foreign countries.

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

In addition, many of the factors that cause, or lead to, the termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate. Any resulting delays to our clinical trials could shorten any period during which we may have the exclusive right to commercialize our product candidates. In such cases, our competitors may be able to bring products to market before we do, and the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition, and prospects.

***If our product candidates or any licensed products do not achieve development milestones or commercialization in the announced or expected timeframes, the further development or commercialization of such product candidates may be delayed, and our business will be harmed.***

We have estimated, and may in the future estimate, the timing of the accomplishment of various scientific, clinical, manufacturing, regulatory, and other product development objectives. These milestones have included and may include our expectations regarding the commencement or completion of clinical trials, data readouts, the submission of regulatory filings, the receipt of regulatory approval, or the realization of other commercialization objectives. The achievement of many of these milestones may be outside of our control. All of these milestones are based on a variety of assumptions, including assumptions regarding capital resources, constraints and priorities, progress of and results from development activities and the receipt of key regulatory approvals or actions, any of which may cause the timing of achievement of the milestones to vary considerably from our estimates. If we or our collaborators fail to achieve announced milestones in the expected timeframes, the commercialization of the product candidates may be delayed, our credibility may be undermined, our business and results of operations may be harmed, and the trading price of our common stock may decline.

***If we experience delays or difficulties in the enrollment of patients in clinical trials, our clinical development activities and our receipt of necessary regulatory approvals could be delayed or prevented.***

Patient enrollment is a significant factor in the timing and completion of clinical trials, and the timing of our clinical trials will depend, in part, on the speed at which we can recruit patients to participate in our trials, as well as completion of required follow-up periods. We or our collaborators may not be able to conduct clinical trials for any product candidates we identify or develop if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or other comparable regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. If patients are unwilling to participate in our gene editing trials because of negative publicity from adverse events related to the biotechnology, gene therapy, or gene editing fields, competitive clinical trials for similar patient populations, clinical trials in competing products, or for other reasons, the timeline for recruiting patients, conducting studies and trials, and obtaining regulatory approval of any product candidates we may develop may be delayed. Moreover, some of our competitors currently and may in the future have ongoing clinical trials for product candidates that treat the same indications as the product candidates we are developing and may develop in the future, and patients who would otherwise be eligible for our clinical trials may instead choose to enroll in clinical trials of our competitors' product candidates. Furthermore, risks related to patient enrollment are heightened in longer clinical trials.

Clinical trial patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- availability and efficacy of approved medications for the disease under investigation;
- availability of genetic testing for potential patients;
- ability to obtain and maintain patient informed consent;
- risk that enrolled patients will drop out before completion of the trial;
- eligibility and exclusion criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- perceived risks and benefits of gene editing or gene modification as a therapeutic approach;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

In addition, our ability to successfully initiate, enroll, and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with CROs and physicians;
- different standards for the conduct of clinical trials;
- different standard-of-care for patients with a particular disease;
- difficulty in locating qualified local consultants, physicians, and partners; and
- potential burden of complying with a variety of foreign laws, medical standards, and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment of gene editing technologies.

Enrollment delays in our clinical trials may result in increased development costs for any product candidates we may develop, which would cause the value of our company to decline and limit our ability to obtain additional financing. If we or our collaborators have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit, or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations, and prospects.

***The field of genetic medicines is relatively new and is evolving rapidly, making us subject to additional development challenges and risks. We are focusing our research and development efforts on genetic modification using our ELXR and XE technologies, but other technologies may be discovered that provide significant advantages, which could materially harm our business.***

To date, we have focused our efforts on developing engineered CRISPR-based medicines through our proprietary technologies, ELXR and XE. However, there are numerous other companies advancing gene editing and gene therapy product candidates that are in preclinical or clinical development. Some of these other companies have previously undertaken research and development of gene editing technologies using other forms of CRISPR protein, or other forms such as base editing, zinc finger nucleases, engineered meganucleases, and transcription activator-like effector nucleases, and at least one of these companies has obtained regulatory approval for a product candidate. There can be no certainty that our technologies will lead to the development of genetic medicines or that other genetic modification technologies will not be considered better or more attractive for the development of therapies. For example, transposons, or "jumping genes," can insert themselves into different places in the genome and carry specific DNA sequences to specific sites without the need for making double-stranded breaks in DNA, although such methods currently cannot target specific locations.

Other new gene editing technologies that have not been discovered yet may be determined to be more attractive than ELXR, XE, or other technologies we develop. Moreover, if we decide to develop CRISPR-based technologies other than ELXR or XE, we cannot be certain we will be able to secure patents or obtain licensing rights to such technologies. Although two of our co-founders who currently provide consulting and advisory services to us in the area of gene editing technologies have entered into agreements with us pursuant to which they assign to us any inventions with respect to the services they perform for us, such obligations are subject to limitations and do not extend to their work in other fields or to the intellectual property arising from their employment with their respective academic and research institutions. To obtain intellectual property rights assigned by these co-founders to such institutions, such as the University of California, Berkeley, we would need to enter into license agreements with such institutions, which may not be available on commercially reasonable terms or at all. In addition, other companies may use certain technologies to develop product candidates in areas they believe are not covered under our foundational licensed issued patents, patent applications or know-how. There are also a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs, using approaches other than gene editing approaches. Any of these factors could reduce or eliminate our commercial opportunity, and could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Moreover, because the *in vivo* application of ELXR and XE may involve genetic modification across multiple cell and tissue types, we are subject to many of the challenges and risks that other gene editing therapeutics and gene therapies face, including evolving regulatory guidance governing gene and gene editing therapy products, the potential risk of improper modulation of a gene sequence, and extended follow-up observation periods that may be required by regulatory agencies.

***The durability of epigenetic modulation may not translate from preclinical models to humans***

Our epigenetic silencing platform is designed to achieve durable modulation of gene expression without permanently altering DNA. While we and others have observed the sustained maintenance from mother cell to daughter cell of targeted epigenetic silencing effects in cell models, mouse models and NHP studies, the durability of such effects in humans has not yet been established and may differ materially from preclinical observations.

Epigenetic modifications are regulated by complex and dynamic cellular processes that may vary across species, tissues, disease states, and individual patients. As a result, epigenetic repression achieved in preclinical models may diminish over time in humans due to endogenous chromatin remodeling, cellular turnover, or other regulatory mechanisms, potentially requiring re-dosing or resulting in a transient therapeutic benefit.

While we have demonstrated two years of durability in NHPs to date, durable epigenetic silencing over multi-year time horizons has not been conclusively demonstrated in human clinical studies for our programs or similar approaches. If epigenetic effects in humans are less durable than anticipated, our product candidates may fail to achieve their intended clinical profile, may require additional dosing, or may be less competitive relative to alternative therapeutic approaches, any of which could adversely affect our business, prospects, and results of operations.

***Any favorable results we may have in our preclinical studies or clinical trials may not be predictive of results that may be observed in later preclinical studies or clinical trials. If any of our product candidates cause serious adverse events, undesirable side effects, or unexpected characteristics, such results could delay or prevent regulatory approval, limit the***

***commercial potential, or result in significant negative consequences following any potential regulatory approval of such product candidates.***

We are developing certain proprietary technologies to support our product candidates. This has and will continue to lead to significant challenges to develop a corresponding set of technical capabilities in support of these technologies. A variety of serious adverse events, undesirable side effects or unexpected characteristics may occur. Such events, side effects or characteristics could delay or prevent regulatory approval, limit the commercial potential, or result in significant negative consequences following any potential regulatory approval of any product candidates we may develop. In addition, ELXR, XE, or any other technologies that we develop may lead to other issues, such as inability to deliver the desired efficacy or safety-related consequences, as it is tested in clinical trials.

Any favorable results we may have in our preclinical studies or clinical trials we conduct may not be predictive of results that may be observed in later preclinical studies or clinical trials. Furthermore, we have not generated any clinical trial results to date. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain regulatory approval of their product candidates. Many product candidates that initially showed promise in early-stage testing for treating a variety of diseases have later been found to lack efficacy or to cause side effects that prevented further clinical development of the product candidates.

There have been a limited number of clinical trials involving the use of genetic modification technologies. It is impossible to predict when or if any product candidates we may develop will demonstrate adequate safety in humans. In the genetic therapy field more broadly, there have been several significant adverse events related to gene therapy in the past, including both the impact of the technology and the delivery methods used to convey the gene therapy technology. These include a variety of safety concerns, including reported cases of leukemia, other cancers, significant morbidities, and death. We currently utilize LNPs for delivery of our ELXR- and XE-based product candidates. Adverse events that could occur with this delivery method include, among others, liver toxicity and/or enzyme elevation, which could limit the effectiveness of the treatment. The occurrence of these events could trigger review and/or monitoring of trial data by a clinical data safety monitoring board or safety review committee, and/or reporting to regulatory agencies such as the FDA, which could result in reviews, delays, pauses, or halts in dosing or enrollment, or require modifications to the clinical trial design. Such actions, in turn, may adversely affect our business, financial condition, results of operations, and prospects. There can be no assurance that technologies such as ELXR and XE or the delivery methods we are using or plan to use will not cause such undesirable side effects.

We cannot be sure that any targets identified or any of our planned delivery methods will not result in adverse effects in the long-term, such as improper editing of a patient's DNA that leads to lymphoma, leukemia, other cancers, or other aberrantly functioning cells or other as yet unidentified findings. Many times, side effects manifest or are only detectable after investigational products are tested in larger scale, pivotal clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. FDA guidance advises that patients treated with genome editing products undergo long-term follow-up observation for identification of potential adverse events for as long as 15 years. If additional clinical or long-term follow-up experience indicates that any of our potential product candidates have side effects or cause 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 or limited. It is also possible that serious or life-threatening side effects may cause significant delay or altered perception of any product candidates we may develop, even if we are able to later show these effects are unrelated to our product candidates. Any adverse events may cause us to delay, limit, or terminate other planned clinical trials, including any that use a similar delivery method or those that use similar aspects of ELXR or XE, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

A significant risk in any gene editing product candidate is the result of "off-target" edits in the case of genome editing and transcriptional changes in the case of epigenetic changes. Off-target changes outside the intended site of gene modification, or unintended consequences of on- and off-target modification, may occur, which could cause serious adverse events, undesirable side effects, or unexpected characteristics. Although we engineered ELXR and XE to reduce the risk of off-target edits, we have not yet conducted clinical trials with potential product candidates for both ELXR and XE, so it is possible that we will detect off-target edits or other unintended consequences of on- or off-target modifications. Current information is limited and we cannot be certain that any product candidates we identified or may identify and develop will not cause off-target editing or that other unintended consequences of on- or off-target editing will not occur and cause serious adverse events in any of our future clinical trials. During clinical development, regulators may require, or we may determine to include, nonclinical studies of off-target gene expression in additional human cell types if warranted by biodistribution findings, observations of on-target treatment-dependent methylation in extrahepatic cells or tissues in future studies, or

evolving regulatory expectations. Furthermore, the lack of observed serious side effects in any preclinical studies does not guarantee that such side effects will not occur in human clinical trials of any potential product candidates, which would adversely impact our development programs and business.

There is also the potential risk of delayed adverse events following exposure to genetic modification due to other components of product candidates used to carry the genetic material. These risks also apply to "on-target" mis-edits or modifications that are not intended but occur at the target site of gene correction, which might also have all of the above consequences, as well as future unforeseen adverse effects.

Although we have demonstrated the ability to engineer technologies that are designed to improve the specificity of edits in a laboratory setting, we cannot be sure that our engineering efforts will result in the same changes or improvements in a clinical setting or will not lead to adverse effects. We also cannot be sure that any of our planned delivery methods will not result in adverse effects such as improper editing of a patient's DNA that leads to lymphoma, leukemia, other cancers, or other aberrantly functioning cells or other as yet unidentified findings. It is also possible that our technologies will result in significant immunogenicity that may lead to adverse effects and could also prevent any chance of reapplication of a delivery method, or gene editing method in the future, if needed.

***If STX-1150, STX-1200, STX-1400, or any of our future product candidates receives regulatory approval, and we or others later identify undesirable side effects caused by such product candidates, a number of potentially significant negative consequences could result.***

If any of our product candidates receives regulatory approval, and we or others later identify undesirable side effects caused by such product candidates, a number of potentially significant negative consequences could result. For example, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits of treatment with such product candidate outweigh the risks for each potential patient, which may include, among other things, a communication plan to healthcare practitioners, patient education, extensive patient monitoring, or distribution systems and processes that are highly controlled, restrictive, and more costly than what is typical for the industry. In addition to adopting a REMS, we may also be required to adopt or engage in similar actions, such as patient education, certification of healthcare professionals, or specific monitoring, if we or others later identify undesirable side effects caused by any product candidate that we develop. Other potentially significant negative consequences associated with adverse events include:

- we may be required to suspend marketing of a product, or we may decide to remove such product from the marketplace;
- regulatory authorities may withdraw or limit their approvals of a product;
- regulatory authorities may require additional warnings in the labeling or limit access of a product to selective specialized centers with additional safety reporting and with requirements that patients be geographically close to these centers for all or part of their treatment;
- we may be required to create a medication guide outlining the risks of a product for patients, or to conduct post-marketing studies;
- we may be required to change the way a product is administered;
- we could be subject to fines, injunctions, or the imposition of criminal or civil penalties, or be sued and held liable for harm caused to subjects or patients;
- a product may become less competitive; and
- our reputation may suffer.

Any of these events could diminish the usage or otherwise limit the commercial success of our product candidates and prevent us from achieving or maintaining market acceptance of our product candidates, if approved by the FDA or other comparable foreign regulatory authorities.

***Data from our preclinical studies or preliminary, interim, or topline data from our clinical trials that we announce or publish from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.***

From time to time, we may publicly disclose data from our completed preclinical studies or preliminary, interim, or topline data from prespecified analyses from our preclinical studies and clinical trials. Such data are based on analyses of

then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, topline, preliminary, or interim results that we report may differ from future results of the same studies or trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the topline or preliminary data we previously published. As a result, topline and preliminary data should be viewed with caution until the final data are available.

Data from interim analyses from clinical trials that we may complete are further subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between topline, preliminary, or interim data and final data could significantly harm our business prospects. Further, disclosure of such data by us or by our competitors could result in volatility in the price of our common stock.

In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the topline, preliminary, or interim data that we report differ from final results, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects, or financial condition.

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

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals, biological, and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws, regulations, and permitting requirements. These current or future laws, regulations, and permitting requirements may impair our research, development, or production efforts. Failure to comply with these laws, regulations, and permitting requirements also may result in substantial fines, penalties, or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health, and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***Genetic medicines are novel, and any product candidates we develop may be complex and difficult to manufacture. We could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of our product candidates we may develop, or otherwise harm our business.***

Any product candidates we may develop will likely require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as the product candidates we intend to develop generally cannot be fully characterized. As a result, assays of the finished product candidate may not be sufficient to ensure that the product candidate will perform in the intended manner. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, insufficient inventory, or potentially delay progression of our potential IND or BLA submissions. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. Our current product candidate and programs utilize LNPs for their delivery modalities, which introduces additional complexities in the manufacturing process.

In addition, if any product is approved, the FDA or other regulatory authorities may require us to submit samples of any lot of such approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations, and prospects. We also may encounter problems hiring and retaining the experienced scientific, quality control, and manufacturing personnel needed to manage our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. The scientific evidence to support the feasibility of developing product candidates based on this technology is both preliminary and limited, and has yet to be produced at scale.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of any product candidates we may develop could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations, and prospects.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party manufacturing processes or facilities also could restrict our ability to ensure sufficient clinical material for any clinical trials we may be conducting or are planning to conduct and meet market demand for any product candidates we develop and commercialize.

***If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product we may develop, we may not be successful in commercializing those products if and when they are approved.***

We do not have a sales or marketing infrastructure and have no experience in the sales, marketing, or distribution of any product candidates. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, factors that may inhibit our efforts to commercialize any approved product candidates include:

- the inability to recruit and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs, and other support personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of decision makers to utilize any future approved product candidates;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price our product candidates at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our product candidates to segments of the patient population;
- the lack of complementary product candidates to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product candidate lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our sales revenue or the profitability of sales revenue may be lower than if we were to market and sell any product candidates we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our product candidates effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates if approved.

***Due to the novel nature of our technologies and the potential for any product candidates we may develop to offer therapeutic benefit in a single administration or limited number of administrations, we face uncertainty related to pricing and reimbursement for these product candidates.***

We expect the cost of a single administration of genetic medicines, such as those we are seeking to develop, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any such product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of any product candidates we may develop will be paid by government authorities, private health plans, and other third-party payors. Payors may not be willing to pay high prices for a single administration. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

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

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical, and cost-effectiveness data. There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates we

may develop. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

Moreover, the downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new product candidates such as ours. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any product candidates we may develop will be harmed.

**Risks related to our reliance on third parties**

***We have entered, and may in the future seek to enter, into collaborations with third parties for the development and commercialization of programs and product candidates using our technologies. If we fail to enter into such collaborations, or such collaborations are not successful, we may not be able to capitalize on the market potential of our ELXR and XE technologies and resulting product candidates.***

We currently are parties to collaboration agreements with Sanofi and Lilly, and we may in the future seek additional third-party collaborators for research, development, and commercialization of other therapeutic technologies or product candidates. Biopharmaceutical companies are our prior and likely future collaborators for any marketing, distribution, development, licensing, or broader collaboration arrangements. With respect to our existing collaboration agreements, and what we expect will be the case with any future collaboration agreements, we have and would expect to have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Moreover, our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our technology currently pose, and will continue to pose, the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may de-emphasize or not pursue development and commercialization of any product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, including as a result of a sale or disposition of a business unit or development function, or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product, if approved, relative to other products;
- collaborators may not properly obtain, maintain, defend, or enforce our intellectual property rights or may use our proprietary information and intellectual property in such a way as to invite litigation or other intellectual property related proceedings that could jeopardize or invalidate our proprietary information and intellectual property or expose us to potential litigation or other intellectual property related proceedings;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or, if approved, commercialization of any product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or, if approved, commercialization of the applicable product candidates;
- collaboration agreements may not lead to development or, if approved, commercialization of product candidates in the most efficient manner or at all; and
- if a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or, if approved, commercialization program could be delayed, diminished or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one or more of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under such collaboration. Furthermore, even if we receive such payments, they will likely result in payment obligations under license agreements with our licensors, which could be substantial. If we do not receive the funding we expect under these collaboration agreements, or if the funding is substantially offset by payment obligations to our licensors, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one or more of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected.

As a result of the foregoing, our current and any future collaboration agreements may not lead to development or commercialization of our product candidates in the most efficient manner or at all.

Moreover, if a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished, or terminated. Any failure to successfully develop or commercialize our product candidates pursuant to our current or any future collaboration agreements could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***If we fail to successfully research, develop, and commercialize as required to achieve the milestone, royalty, and other payments in our collaboration and license agreements, we will not receive any milestone or royalty payments under such agreements.***

Certain of our collaboration and license agreements include substantial milestone and royalty payments in the event we achieve specified development, regulatory, and commercial targets. If we fail to successfully research, develop, and commercialize as required to achieve those milestone and royalty payments, we will not be entitled to such payments. Further, certain of our agreements, such as our 2023 Sanofi License Agreement (as defined herein), provide for nomination, selection, development, regulatory, and/or commercial milestone payments on a per licensed target or licensed product basis. If any of such licensed targets fail to develop into a licensed product, we will not receive certain of such milestone payments and will fail to realize the full economic value under such agreements. In certain cases, our counterparties may also terminate the agreements if we fail to achieve specified milestones, or may otherwise terminate in their sole discretion regardless of the performance of our platform and products. In many instances, our receipt of milestone or royalty payments depends on the performance of our counterparties and we have little, if any, control regarding whether such targets are achieved. In addition to other adverse effects on our business that may result from unsuccessful research and product candidates, any failure to achieve milestones under our current, or future, collaboration and license agreements may have a material adverse effect on our business, financial condition, results of operations, and prospects.

***If conflicts arise between us and our collaborators, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.***

If conflicts arise between our collaborators and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Our collaborators may develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of partner support for any product candidates we may develop.

Our collaborators could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, prevent us from obtaining timely regulatory approvals, terminate their agreements with us prematurely, or fail to devote sufficient resources to the collaboration efforts, including development, delivery, manufacturing and commercialization of products. Any of these developments could harm our company and product development efforts.

***We expect to rely on third parties to conduct our clinical trials and some aspects of our research, as well as some aspects of our delivery methods, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.***

We currently, and expect to continue to, rely on third parties, such as CROs, clinical data management organizations, medical institutions, preclinical laboratories, and clinical investigators, to conduct some aspects of our research. For example, we rely on Acuitas Therapeutics, Inc., or Acuitas, to supply LNPs pursuant to our development and option agreement with

them and on various third parties to conduct some of our preclinical animal experiments. Any of these third parties may terminate their engagements with us at any time under certain criteria. If we need to enter into alternative arrangements, it may delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA and other regulatory authorities require us and the study sites and investigators we work with to comply with regulations and standards, commonly referred to as GLPs, cGMPs, and GCPs, for conducting, recording and reporting the results of preclinical studies and clinical trials to assure, among other things, that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. Regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators, and trial sites. If we or any of our CROs, CDMOs, or trial sites fail to comply with applicable GLPs, cGMPs, GCPs, or other regulatory requirements, the data generated in our preclinical studies or clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional studies or trials before approving our marketing applications, if ever. Failure to comply with these regulations may require us to repeat preclinical studies or clinical trials, which would delay the regulatory approval process.

Although we intend to design the clinical trials for our potential product candidates, CROs will conduct some or all aspects of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct preclinical studies and future clinical trials will also result in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Among other reasons that may delay or impact the development of our potential product candidates, outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; and
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs and other third parties do not perform such preclinical studies and future clinical trials in a satisfactory manner, breach their obligations to us, or fail to comply with regulatory requirements, the development, regulatory approval, and commercialization of our potential product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our potential product candidates, or our development programs may be materially and irreversibly harmed.

In addition, our CROs have the right to terminate their agreements with us in the event of an uncured material breach and under other specified circumstances. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms or at all. Switching or adding additional CROs, investigators, and other third parties involves additional costs and requires our management's time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we work to carefully manage our relationships with our CROs, investigators, and other third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

If we are unable to rely on preclinical and clinical data collected by our CROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

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

***We rely on third-party manufacturers and suppliers to supply components for ELXR, XE, and other technologies we develop. The loss of our third-party manufacturers or suppliers, or our or their failure to comply with applicable regulatory requirements or to supply sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.***

We do not own or operate facilities for drug manufacturing, storage, distribution, or quality testing. We currently rely, and may continue to rely, on CDMOs, including in the United States, to manufacture bulk drug substances, drug products, raw materials, samples, components, or other materials and reports. Reliance on CDMOs may expose us to different risks than if we were to manufacture product candidates ourselves. There can be no assurance that our preclinical and clinical development product supplies will not be limited, interrupted, or terminated, be of satisfactory quality, or continue to be available at acceptable prices. In particular, any replacement of our CDMOs could require significant effort and expertise because there may be a limited number of qualified replacements.

The manufacturing process for a product candidate is subject to FDA and other foreign regulatory authority review. We, and our suppliers and manufacturers, must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory requirements, such as Current Good Manufacturing Practices, or cGMPs. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA and other foreign regulatory authorities. If our contract manufacturers are unable to maintain a compliance status acceptable to the FDA and other foreign regulatory authorities, our product candidates may not be approved. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or comparable foreign regulatory authorities, we may not be able to rely on their manufacturing facilities for the manufacture of components of any product candidates. Moreover, although we do not control the manufacturing process at our contract manufacturers and are completely dependent on them for compliance with current regulatory requirements, we are nonetheless responsible for ensuring that any product candidates are manufactured in accordance with applicable laws and regulatory requirements. In the event that any of our manufacturers fails to comply with such requirements or to perform its obligations in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture any product candidates may be unique or proprietary to the original contract manufacturer and we may have difficulty transferring the manufacturing of any product candidates to another third party. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to enable us, or to have another third party, manufacture any product candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines, and we may be required to repeat some of the development program. Any delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget. In addition, we may not be able to demonstrate sufficient comparability between products manufactured at different facilities to allow for inclusion of the clinical results from participants treated with products from these different facilities, in our product registrations.

We expect to continue to rely on CDMOs if we receive regulatory approval for any product candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. Any manufacturing facilities used to produce any product candidates will be subject to periodic review and inspection by the FDA and other foreign regulatory authorities, including for continued compliance with cGMP requirements, quality control, quality assurance and corresponding maintenance of records and documents. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize any product candidates, if approved. Our or a third party's failure to execute on our manufacturing requirements, to comply with cGMPs, or to maintain a compliance status acceptable to the FDA or other foreign regulatory authorities could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials of product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, if any, for product candidates;
- loss of the cooperation of future collaborators;
- subjecting third-party manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of any product candidates; and

- in the event of approval to market and commercialize a product candidate, an inability to meet commercial demands for our products.

In addition, we do not have any long-term commitments or supply agreements with any third-party manufacturers. We may be unable to establish any long-term supply agreements with third-party manufacturers or to do so on acceptable terms, which increases the risk of failing to timely obtain sufficient quantities of our product candidates or such quantities at an acceptable cost. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third party;
- tariffs (including tariffs that have been or may in the future be imposed by the United States or other countries), trade protection measures, import or export licensing requirements, trade embargoes, sanctions (including those administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury), other trade barriers (including further legislation or actions taken by the United States or other countries that restrict trade), and protectionist or retaliatory measures taken by the United States or other countries;
- failure to manufacture our product candidates according to our specifications;
- failure to manufacture our product according to our schedule or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Additionally, our contract manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our contract manufacturers were to encounter any of these difficulties, our ability to provide any product candidates to patients in preclinical and clinical trials, or to provide products for treatment of patients, if approved and commercialized, would be jeopardized.

***We expect to depend on single-source suppliers for some of the components and materials used in our product candidates.***

We expect to depend on single-source suppliers for some of the components and materials used in any future product candidate we develop. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes, and finished goods exposes us to several risks, including disruptions in supply, price increases, or late deliveries. There are, in general, relatively few alternative sources of supply for substitute components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sales. Establishing additional or replacement suppliers for these components, materials, and processes could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any single-source supplier or service provider could lead to supply delays or interruptions, which would damage our business, financial condition, results of operations, and prospects.

If we have to switch to a replacement supplier, the manufacture and delivery of any product candidates we may develop could be interrupted for an extended period, which could adversely affect our business. Establishing additional or replacement suppliers, if required, may not be accomplished quickly. If we are able to find a replacement supplier, the replacement supplier would need to be qualified and may require additional regulatory authority approval, which could result in further delays. While we seek to maintain adequate inventory of the single-source components and materials used in our products, any interruption or delay in the supply of components or materials, or our inability to obtain components or materials from alternate sources at acceptable prices in a timely manner, could impair our ability to meet the demand for our product candidates.

**Risks related to our business and operations**

***Our future performance depends on our ability to retain our President and Chief Executive Officer, other key executives, and other key employees and to attract, retain, and motivate qualified personnel and manage our human capital.***

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries largely depends upon our ability to attract, motivate and retain highly qualified managerial, scientific, and medical personnel. We are highly

dependent on the scientific and management expertise of Dr. Oakes, our President and Chief Executive Officer and the other members of our management team and other key employees and advisors. We currently do not maintain key person insurance on these individuals.

The loss of one or more members of our management team or other key employees or advisors could delay our research and development programs and have a material adverse effect on our business, financial condition, results of operations, and prospects. The relationships that our key managers have cultivated within our industry make us particularly dependent upon their continued employment with us. We are dependent on the continued service of our technical personnel, because of the highly technical nature of gene editing and epigenetic modification technologies and of our product candidates, and the specialized nature of the regulatory approval process. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty.

We primarily conduct our operations at our facility in Alameda, California. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market, and nationally, is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. We also face competition for personnel from other companies, universities, public and private research institutions, government entities, and other organizations. Our future performance will depend in large part on our continued ability to attract and retain highly qualified scientific, technical, and management personnel, as well as personnel with expertise in clinical testing, manufacturing, governmental regulation, and commercialization. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can discover and develop product candidates will be limited, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***Our relationships with our co-founders may create the appearance of conflicts of interest.***

Two of our co-founders, Dr. Doudna and Dr. Savage, are pioneers in CRISPR-based genome editing technology and were key contributors to our founding. While we do not rely on Dr. Doudna and Dr. Savage for our day-to-day operations and they are not currently employed by us, we do continue to consult with them as appropriate on strategic matters. However, each of Dr. Doudna and Dr. Savage may be engaged by entities other than us, and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us, as well as other third-party advisors and consultants.

Dr. Doudna and Dr. Savage serve on our Scientific Advisory Board and as our paid consultants. Dr. Doudna and Dr. Savage's existing positions at UCB could result in, or may create the appearance of, conflicts of interest related to our license of intellectual property rights from UCB and other contractual relationships we may enter into from time to time. Additionally, Dr. Doudna is a co-founder of the gene editing companies Azalea Therapeutics, Caribou Biosciences, Inc., or Caribou, Editas Medicine, Inc., Evercrisp Biosciences, or Evercrisp, Intellia Therapeutics Inc., or Intellia, and Mammoth Biosciences, Inc., or Mammoth. Dr. Doudna also continues to serve as a scientific advisory board member of Caribou, Evercrisp, Intellia, and Mammoth. While our agreements with Dr. Doudna and Dr. Savage include (i) confidentiality obligations, (ii) a certification that they will not enter into obligations that would preclude them from complying with the terms of these agreements, and (iii) a notification requirement to us if any potential conflict arises, including due to commencement of a new employment, consulting or business relationship or a change in the business interests of other entities which such person advises, these other relationships could still result in, or may create the appearance of, conflicts of interest to the extent we may be seen as competitors. We do not maintain a separate conflict of interest policy, but pursuant to the terms of our agreements with Dr. Doudna and Dr. Savage, in the event any conflict arises as a result of these agreements, we have the right to modify either agreement in writing. Further, if there is a conflict between these agreements and the terms of the Howard Hughes Medical Institute Uniform Consulting Agreement Provisions that each of Dr. Doudna and Dr. Savage are party to, or the Uniform Provisions, the Uniform Provisions shall govern.

***We expect to significantly expand our development, clinical, and regulatory capabilities and operations as we grow, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

As of June 30, 2026, we had 82 full-time employees. We expect to increase the number of our employees and the scope of our operations, particularly in the areas of platform development, preclinical development, clinical development, clinical operations, manufacturing, late-stage regulatory affairs, finance, accounting, business operations, public company compliance, communications, and other corporate development functions, and, if any of our product candidates receive regulatory approval, sales, marketing, and distribution capabilities. If we enter into additional collaborations, we may have to further expand our employee base beyond our current projections, which may include further preclinical research and

development or later-stage regulatory operations. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational, and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth and with developing sales, marketing, and distribution infrastructure, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources.

Further, we currently rely, and for the foreseeable future will continue to rely, in substantial part on certain third-party contract organizations, advisors and consultants to provide certain services, including assuming substantial responsibilities for the conduct of our discovery programs, preclinical development, any future clinical trials and the manufacturing of any product candidates. We cannot assure you that the services of such third-party contract organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by our third-party contract organizations, advisors or consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of any product candidates or otherwise advance our business. We cannot assure you that we will be able to properly manage our existing third-party contract organizations, advisors or consultants or find other competent outside third-party contract organizations, advisors and consultants on economically reasonable terms, or at all.

If we are not able to effectively manage growth and expand, we may not be able to successfully implement the tasks necessary to further develop our research and discovery programs or any product candidates and, accordingly, we may not achieve our research, development, and commercialization goals.

***Our business depends on the efficient and uninterrupted operation of our information technology systems, and such systems and those of our third-party vendors, contractors or consultants may fail or suffer security breaches, cyberattacks, loss or leakage of data and other disruptions, which could result in a material disruption of our development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.***

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property and proprietary business data). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. We also have outsourced elements of our information technology systems and operations to third parties, and as a result we rely on and manage a number of third-party vendors and other contractors and consultants who have access to our confidential information. We may be unable to adequately protect our information technology systems from cyberattacks, system failures or outages, and such events could compromise our ability to perform these functions in a timely manner or result in the disclosure of confidential information, which could harm our ability to conduct business, delay our financial reporting, and subject us to significant financial and legal exposure.

Despite the implementation of security measures, our information technology systems and those of our third-party vendors and other contractors and consultants are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, accidents by our employees or third-party service providers, natural disasters, terrorism, war, global pandemics, and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, third-party vendors, contractors, consultants, business partners and/or other third parties, including theft, fraud or unauthorized access to or use of our information technology systems, or attack or damage from hacking, cyberattacks or supply chain attacks by malicious third parties and sophisticated nation-state and nation-state-supported actors (including the deployment of harmful computer viruses and malware, ransomware, denial or degradation-of-service attacks, software bugs, phishing attacks and other social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure, or that of our third-party vendors and other contractors and consultants, or lead to data leakage. The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity, and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, nor implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations, or hostile foreign governments or agencies. There can also be no assurance that our cybersecurity risk management program and processes, including our policies, controls or procedures, will be fully implemented, complied with or effective in protecting our information technology systems and confidential information. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to

attackers increasingly using tools and techniques (including artificial intelligence) that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. Any breach, loss or compromise of confidential information may also subject us to liability, including litigation exposure, regulatory action or investigation and civil fines and penalties. If the information technology systems of our third-party vendors and other contractors and consultants become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. Additionally, any integration of artificial intelligence in our or any third party's operations, products or services is expected to pose new or unknown cybersecurity risks and challenges. Remote and hybrid working arrangements at our company (and at many third-party providers) also increase cybersecurity risks due to the challenges associated with managing remote computing assets and security vulnerabilities that are present in many non-corporate and home networks.

We and certain of our service providers are from time to time subject to cyberattacks and security incidents. While we do not believe we have experienced any such system failure, accident or security breach to date, we cannot assure you that our data protection efforts and our investment in information technology will prevent significant breakdowns, data leakages, breaches in our systems, or those of our third-party vendors and other contractors and consultants, or other cyber incidents that could have a material adverse effect upon our reputation, business, operations, or financial condition. Significant disruptions of our information technology systems or those of our third-party vendors and other contractors and consultants, or security breaches could result in the loss, misappropriation and/or unauthorized access, use, or disclosure of, or the prevention of access to, confidential information (including trade secrets or other intellectual property or proprietary business information) and claims (including class actions) by our counterparties that we have failed to comply with legal or contractual obligations, which could result in financial, legal, business, and reputational harm to us.

We cannot assure you that our CROs, contract manufacturing organizations, or CMOs, or other third-party service providers with access to our or our suppliers', manufacturers', and employees' sensitive data in relation to which we are responsible will not breach contractual obligations imposed by us, or that they will not experience data security incidents or other interruptions, which could have a corresponding effect on our business, including under privacy laws and regulations or which could in turn adversely affect our business, financial condition, results of operations, and prospects. Furthermore, there can be no assurance that the limitations of liability in our contracts would be enforceable or adequate to protect us from liabilities and damage and we may not have adequate insurance coverage to cover losses, or all types of costs, expenses, and losses, we could incur with respect to security breaches or disruptions. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that our existing insurance coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

***We are, or may in the future be, subject to stringent and changing obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; loss of customers or sales; and other adverse business consequences.***

The global data protection landscape is rapidly evolving and our data processing activities subject us to numerous data privacy and security obligations, such as various state, federal and foreign laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements and other obligations that govern the processing of sensitive data by us and on our behalf, and we may be subject to new or additional obligations related to data privacy and security and face increased scrutiny from regulatory authorities as our business grows. In the ordinary course of business, we process personal information and other sensitive information, including our proprietary and confidential business data, trade secrets, intellectual property, data which we expect to collect about trial participants in connection with clinical trials, and other sensitive data. Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts and other obligations that govern the processing of sensitive or confidential information by us and on our behalf, and we may be subject to new or additional data protection laws and regulations and face increased scrutiny from regulators as our business grows. The legislative and regulatory landscape for data privacy and security continues to evolve in jurisdictions worldwide, and there has been an increasing focus on these issues with the potential to affect our business.

We and our partners may be subject to federal, state, and foreign laws and regulations that govern data privacy and security. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), comprehensive consumer privacy laws, sector-specific privacy laws, data breach notification laws, laws regarding marketing, and other similar laws governing

the processing of sensitive data that we are or may in the future be required to comply with. Certain states have also adopted comparable privacy and security laws and regulations, which govern the privacy, processing, and protection of health-related and other personal information. For example, the California Consumer Privacy Act of 2018 (as amended by the California Privacy Rights Act of 2020), or collectively, the CCPA, imposes certain obligations on businesses that process the personal information of California residents (including employees based in California), such as the obligation to provide specific disclosures in privacy notices, and affords California residents certain rights related to their personal information, including a private right of action in the event of a data breach. Although the CCPA exempts certain personal information processed in the context of clinical trials, the CCPA could increase compliance costs and potential liability. Similar laws have been enacted in a number of other states, and we expect more states to pass similar laws in the future. While these states exempt some data processed in the context of clinical trials, these developments may further complicate compliance efforts and increase legal risk and compliance costs for us and the third parties with whom we work. Certain states have also adopted specific privacy and security laws and regulations which govern the privacy, processing, and protection of health-related personal information. Such laws and regulations will likely be subject to interpretation by various courts and other governmental authorities, creating potentially complex compliance issues for us and our future customers and strategic partners. In addition to government activity, privacy advocacy groups and technology and other industries continue to consider new or revised self-regulatory standards related to privacy and security that may place additional burdens on us.

Outside the United States, an increasing number of laws, regulations, and industry standards govern data privacy and security. For instance, the European Union's General Data Protection Regulation, or EU GDPR, and the United Kingdom's GDPR, or UK GDPR, together the GDPR, impose strict requirements for processing the personal data of individuals. For example, under the GDPR, government regulators may impose temporary or definitive bans on data processing, as well as fines of up to €20 million or 4% of annual global revenue, whichever is greater. Further, individuals may initiate litigation related to our processing of their personal data. Among other requirements, the GDPR (and certain other foreign jurisdictions) regulate the cross-border transfer of personal data, which could make it more difficult to transfer information across jurisdictions (such as transferring or receiving personal data that originates in the European Union, or EU, or the United Kingdom to countries such as the United States which are not considered by the EU or United Kingdom to provide adequate protection of personal data). Case law from the Court of Justice of the European Union states that reliance on the standard contractual clauses—a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism—alone may not necessarily be sufficient in all circumstances and that transfers must be assessed on a case-by-case basis. We expect the existing legal complexity and uncertainty regarding international personal data transfers to continue, and international transfers to the United States and to other jurisdictions more generally to continue to be subject to enhanced scrutiny by regulators. As the regulatory guidance and enforcement landscape in relation to data transfers continue to develop, we could suffer additional costs, complaints and/or regulatory investigations or fines; we may have to stop using certain tools and vendors and make other operational changes; we may have to implement revised standard contractual clauses for existing arrangements within required time frames; and/or it could adversely affect our business, operations and financial condition.

Complying with these complex and often evolving privacy and security related obligations can be expensive, difficult, time consuming, and subject to inconsistent application and interpretation. Any actual or perceived failure to comply with any such obligations, whether by us, or by our CROs, CMOs, partners or other third parties with whom we work, could result in significant adverse consequences, including: investigation costs; material fines and penalties; compensatory, special, punitive, or statutory damages; litigation (including class actions) and mass arbitration demands; government enforcement actions; requirements to provide notices, credit monitoring or other services to impacted individuals; adverse actions against our licenses; bans or restrictions on processing personal information; required changes to our services, technologies, systems, or practices (or those of our partners); reputational damage; imprisonment of company officials; injunctive relief; and other consequences that could adversely affect our business, financial condition, results of operations, and prospects.

***Our business entails a significant risk of product liability and our ability to obtain sufficient insurance coverage could have a material adverse effect on our business, financial condition, results of operations, and prospects.***

When we conduct clinical trials of our product candidates, if ever, we may be exposed to significant product liability risks inherent in the development, testing, manufacturing and marketing of therapeutic treatments. Product liability claims could delay or prevent completion of our development programs. If we succeed in marketing products, if approved, such claims could result in an FDA investigation of the safety and effectiveness of our products, our manufacturing processes and facilities or our marketing programs and potentially a recall of our products or more serious enforcement action, limitations on the approved indications for which they may be used or suspension or withdrawal of approvals. Regardless of the merits or eventual outcome, liability claims may also result in decreased demand for our products, termination of clinical trial sites

or entire trial programs, withdrawal of clinical trial participants, injury to our reputation and significant negative media attention, significant costs to defend the related litigation, a diversion of management's time and our resources from our business operations, substantial monetary awards to trial participants or patients, loss of revenue, the inability to commercialize any products that we may develop, and a decline in our stock price. We currently maintain general liability insurance. We may, however, need to obtain higher levels of product liability insurance for later stages of clinical development or marketing any of our product candidates. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners, and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to comply with FDA or other similar foreign regulations, provide true, complete, and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we may establish, comply with healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. If we obtain FDA or other regulatory approval of any product candidates and begin commercializing those products in the United States, our potential exposure under these laws will increase significantly, and our costs associated with compliance with these laws will likely increase. In particular, sales, marketing, and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing, and promotion, sales commission, customer incentive programs, and other business arrangements. Employee misconduct 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. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply 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 material adverse effect on our business, financial condition, results of operations, and prospects, including the imposition of significant civil, criminal, and administrative penalties, damages, fines, disgorgement, imprisonment, the curtailment or restructuring of our operations, loss of eligibility to obtain approvals from the FDA or other foreign regulatory body exclusion from participation in government contracting, healthcare reimbursement, or other government programs, including Medicare and Medicaid, integrity oversight and reporting obligations, or reputational harm.

***Changes in tax laws or regulations that are applied adversely to us may have a material adverse effect on our business, cash flow, financial condition or results of operations.***

New income, sales, use, or other tax laws, statutes, rules, regulations, or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, enacted many significant changes to the U.S. tax laws. For our 2022 through 2024 tax years, the Tax Cuts and Jobs Act eliminated the option to immediately deduct research and development expenditures and required taxpayers to amortize domestic expenditures over five years and foreign expenditures over fifteen years. Beginning with our 2025 tax year, the One Big Beautiful Bill Act, or OBBBA, restored immediate deductibility of domestic expenditures, while foreign expenditures will continue to be capitalized and amortized over fifteen years. Future changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted, changed, modified, or applied adversely to us.

Further, we are subject to U.S. federal, state, and local income taxes and other taxes in the United States and will be subject to income taxes, withholding taxes, transaction taxes, and other taxes in any foreign jurisdictions in which we currently do business or may do business in the future. Due to the expanding scale of our international business activities, we may become subject to taxation in additional foreign jurisdictions. Moreover, changes to our corporate structure, including increased headcount and expanded functions outside of the United States, as well as changes to the tax laws in the

jurisdictions in which we do business, could impact our worldwide effective tax rate and adversely affect our operating results and financial condition.

***Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.***

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. As of December 31, 2025, we had U.S. federal and state net operating loss carryforwards of approximately $49.0 million and $59.9 million, respectively, of which the state net operating loss carryforwards begin to expire in 2043. The Company has federal and state research and development tax credits of $3.1 million and $6.7 million, respectively. The federal research and development tax credits begin to expire in 2044 unless previously utilized, and the state credit carryforwards do not expire. These carryforwards could be further limited if we experience an "ownership change" as described below. Under the Tax Cuts and Jobs Act, as modified by the CARES Act, unused U.S. federal net operating losses generated in tax years beginning after December 31, 2017, will not expire and may be carried forward indefinitely but the deductibility of such federal net operating losses for any year is limited to no more than 80% of the excess, if any, of current year taxable income (without regard to certain deductions) over any federal net operating losses from taxable years beginning before January 1, 2018. Federal net operating losses generated in tax years beginning before January 1, 2018, may be carried forward for up to 20 taxable years and are not subject to the 80% of taxable income limitation. In addition, both our current and our future unused losses and other tax attributes may be subject to limitation under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, if we undergo, or have undergone, an "ownership change," generally defined as a greater than 50 percentage point change (by value) in our equity ownership by certain stockholders or groups of stockholders over a three-year period. The Company has completed an analysis of ownership changes through March 31, 2023, and as a result, the Company experienced an ownership change in October 2018. Such a change did not have a significant impact on the Company's ability to utilize net operating losses ("NOLs") and credits that existed as of that date. It is possible that we underwent an additional ownership change as a result of our initial public offering and the concurrent private placement. Because our common stock is now publicly traded, we may undergo further shifts in the ownership of our capital stock as a result of purchases and sales by our stockholders, which are largely outside of our control and which may further limit our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes, as applicable. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. As a result, even if we attain profitability, we may be unable to use all or a material portion of our net operating losses and other tax attributes, which could adversely affect our future cash flows.

***We or the third parties we depend on may be adversely affected by natural disasters, terrorist activity, pandemics and other events beyond our control, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemic, terrorist activity, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, or the manufacturing facilities of our CDMOs, may have a material adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Extreme weather conditions or other natural disasters could further disrupt our operations and have a material adverse effect on our business, financial condition, results of operations, and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our research facilities or the manufacturing facilities of our CDMOs, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time, if at all.

Our employees often conduct business outside of any facilities leased by us. These locations may be subject to additional security and other risk factors due to the limited control of our employees. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur additional and substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities, or the manufacturing facilities of our CDMOs, are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any

business interruption could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are more effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.***

The development and commercialization of new drug products is highly competitive. Moreover, the genetic medicine field is characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We will face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent or other intellectual property protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs. Such large companies have significantly greater infrastructure, resources across drug development, large-scale manufacturing, global regulatory engagement, and commercial execution than we do. As a result, they may be able to advance their programs or other competing genetic medicine approaches more rapidly or efficiently than we can, including by initiating or completing clinical trials sooner, investing more heavily in manufacturing scale-up, engaging earlier or more extensively with regulatory authorities, and deploying established global commercial and market-access capabilities. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, while others are based on entirely different approaches. Even where competing therapies are based on different technological approaches or involve different design trade-offs, large pharmaceutical and biotechnology companies may be able to achieve earlier market entry, broader physician adoption, or greater commercial penetration due to their scale and resources.

There are several companies utilizing CRISPR-Cas9 technology including CRISPR Therapeutics AG, Editas Medicine, Inc., Intellia Therapeutics, Inc. and Caribou Biosciences, Inc. In addition, companies using Cas attachment or novel nuclease technologies include Beam Therapeutics Inc., Prime Medicine, Inc., Tessera Therapeutics, Inc., Mammoth Biosciences, Inc., Arbor Biotechnologies, Inc., and Metagenomi Technologies, Inc., among others. More recently, several companies have emerged focusing on epigenetic modification to modulate protein expression without directly editing DNA, such as nChroma Bio, Inc., Epicrispr Biotechnologies, Inc. and Tune Therapeutics, Inc. We are aware of a number of genetic medicine companies with operations outside of the United States with active cardiometabolic programs, including AccurEdit Therapeutics, CorrectSequence Therapeutics, Epigenic Therapeutics Co., Ltd., Yoltech Therapeutics Co., Ltd., among others. Several additional companies utilize alternative nuclease-based epigenetic modification technologies, including ZFNs, engineered meganucleases and TALENs. In addition, we face competition from companies utilizing small interfering RNA, or siRNA, oligonucleotides, cell therapy therapeutic approaches and LNP delivery technologies to create therapeutics, including Corsera Health Inc.

Our lead product candidate, STX-1150, and development programs, STX-1200 and STX-1400, target genetic risk factors of ASCVD. There are several approved products for LDL-C lowering or cardiovascular risk reduction, such as statins, ezetimibe, bempedoic acid, lomitapide, mipomersen and icosapent ethyl. There are also several approved products that target PCSK9 protein as a mechanism to lower LDL-C and reduce the risk of ASCVD, including evolocumab, which is a monoclonal antibody, or mAb, marketed as Repatha® by Amgen Inc., alirocumab, which is marketed as PRALUENT® by both Sanofi and Regeneron Pharmaceuticals, Inc., inclisiran, which is a siRNA marketed as LEQVIO® by Novartis, and enlicitide, which is an oral peptide marketed as LIPFENDRA® by Merck & Co.

We are also aware of other gene editing programs for LDL-C lowering in development by Verve Therapeutics, a subsidiary of Eli Lilly and Company, and Editas Medicine, Inc.. Additionally, there are other investigational therapies targeting PCSK9, including AstraZeneca PLC's laroprovstat.

Further, several investigational medicines designed to reduce Lp(a) are currently in clinical development. These include pelacarsen, an antisense oligonucleotide licensed by Novartis, olpasiran, an investigational siRNA medicine targeting Lp(a) licensed by Amgen, zerlasiran, an investigational siRNA medicine being developed by Silence Therapeutics plc., lepodisiran, an investigational siRNA medicine being developed by Eli Lilly and Company, and muvalaplin, an oral small molecule also being developed by Lilly. In addition, CRISPR Therapeutics AG and Verve Therapeutics, a subsidiary of Eli Lilly and Company, have disclosed programs targeting Lp(a) in development.

In addition, several medicines designed to reduce APOC3 are currently approved and in clinical development. These include olezarsen, an antisense oligonucleotide marketed as TRYNGOLZA™ by Ionis Pharmaceuticals, Inc., approved for familial chylomicronemia syndrome, or FCS, and severe hypertriglyceridemia, or sHTG, and plozasiran, an siRNA medicine targeting *APOC3* marketed as REDEMPLO® by Arrowhead Pharmaceuticals, Inc., and approved for FCS.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates. This may include gene editing companies with other approaches, as well as other types of therapies.

Many of our competitors, either alone or in combination with their respective strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, regulatory approval processes, and marketing than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and gene editing industries may result in resources becoming increasingly concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. We also compete with these companies to recruit and retain qualified scientific and management personnel. We will also face significant competition in other areas as we approach commercialization of any product candidates, including establishing clinical trial sites, recruiting patients for clinical trials, establishing sales and marketing networks and producing technologies complementary to, or necessary for, our programs.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer or more effective, particularly if they represent cures, or are better tolerated, more convenient, or less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, or may establish more effective sales and marketing platforms. The key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience and availability of reimbursement, as well as our ability to effectively market our products.

In addition, as a result of the expiration or successful challenge of our patent or other intellectual property rights, we could face risks relating to our ability to successfully prevent or delay launch of competitors' products. The availability of our competitors' products could limit the demand and the price we are able to charge for any product candidates that we may develop and commercialize.

**Risks related to intellectual property**

***If we are unable to obtain and maintain patent and other intellectual property protection for our technology and products in the United States or other countries, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively.***

We rely upon a combination of patents, trademarks, trade secret protection, and confidentiality agreements to protect the intellectual property related to our CRISPR-based technologies, ELXR and XE. Our commercial success will depend in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our CRISPR-based medicines, ELXR and XE, and other product candidates and technologies, and their respective methods of use and manufacture. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our current or future product candidates, whether those applications are company owned or in-licensed. The patent prosecution process is expensive and time-consuming, and we may not be able to file, prosecute, or maintain all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development in a manner timely enough to obtain patent protection.

In addition, we may not pursue or obtain patent protection in all relevant countries. Filing, prosecuting and defending patents covering our current or future product candidates and technologies throughout the world would be prohibitively expensive. 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 countries where we may obtain or have patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products and technologies in jurisdictions where we do not have any issued patents, and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. The effects of geopolitical tensions could significantly limit our ability to enforce our patents in those affected jurisdictions.

The patent applications we own or in-license may fail to result in issued patents with claims that protect our current or future product candidates or technologies in the United States or in other foreign countries. There is also no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from being issued from a pending patent application or be used to invalidate an issued patent. Even if patents do successfully issue and even if such patents cover our current or future product candidates or technologies, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of our current or future product candidates and technologies. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If the patent applications we own or have in-licensed with respect to our current or future product candidates or technologies fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current or future product candidates or technologies, it could dissuade companies from collaborating with us to develop gene editing technologies and threaten our ability to commercialize our future product or technology offerings. Any such outcome could have a materially adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has been and will continue to be the subject of litigation and new legislation. The field of CRISPR-based gene editing has already been the subject of extensive patenting activity and litigation.

In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, many countries restrict the patentability of methods of treatment of the human body. Many companies have encountered significant problems in protecting and defending intellectual property rights in countries outside the United States. 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 intellectual property and proprietary rights generally. In addition, certain countries do not allow for patent protection regarding methods of treatment.

Many countries outside the United States also have compulsory licensing laws under which a patent owner in certain circumstances may be compelled to grant licenses to third parties. In addition, many countries outside the United States limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are 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.

Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result of these and other factors, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or other proceeding challenging our owned or licensed patent rights at the USPTO or patent offices in other countries. The costs of defending our owned or licensed patents in these types of administrative proceedings and litigation matters can be substantial and the outcome can be uncertain. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our current or future product candidates or technologies and compete directly with us, without payment to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop, or commercialize our product candidates or technologies.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated, or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Generally, issued patents are granted a term of 20 years from the earliest claimed non-provisional filing date. In certain instances, patent term can be adjusted to recapture a portion of delay by the USPTO in examining the patent application (patent term adjustment) or extended to account for term effectively lost as a result of the FDA regulatory review period (patent term extension), or both. The scope of patent protection may also be limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing, and regulatory review of new gene editing technologies, patents protecting our product 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.

***Our rights to develop our CRISPR-based technologies, ELXR and XE, and to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.***

We have licensed and are dependent on certain patent rights and proprietary technology from third parties that are important or necessary to the development of our CRISPR-based technologies and product candidates. For example, we are a party to the UCB Exclusive License Agreement with the Regents of the University of California, or the Regents, pursuant to which we license patents and patent applications that relate to our CRISPR-based technologies. The UCB Exclusive License Agreement imposes various diligence, milestone payment, royalty, insurance, indemnification and other obligations on us. If we breach any material obligation, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the Regents may have the right to terminate the license. If the license is terminated, we may be unable to develop, manufacture, sell, or use our CRISPR-based technologies and products that are covered by the patents licensed under the UCB Exclusive License Agreement, and the Regents may allow a competitor to license the covered technology instead.

Our licenses may not provide us with exclusive rights to use the licensed intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our CRISPR-based gene editing technologies and product candidates in the future. Some licenses granted to us are subject to certain exclusivity restrictions or preexisting rights. Further, our out-license agreements generally include exclusivity terms limiting our ability to develop product candidates that may compete with the relevant licensed target or product. If such exclusivity restrictions prevent us from developing or commercializing our technologies in a way that we deem necessary to gain or maintain our competitive advantage, it may have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

We do not have complete control in the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. It is possible that our licensors' enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, or may not be conducted in accordance with our best interests. We cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our licensors fail to prosecute, maintain, enforce, and defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors from making, using, and selling competing products.

Furthermore, inventions contained within our in-licensed patent applications from the Regents under the UCB Exclusive License Agreement were made using U.S. government funding. We rely on the Regents to ensure compliance with applicable obligations arising from such funding, such as timely reporting, an obligation associated with our in-licensed patents and patent applications. The failure of the Regents to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents. For example, the U.S. government could have certain rights in such in-licensed patent applications, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology to third parties and to exercise march-in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may also exercise its march-in rights if it

determines that action is necessary because we or our licensor failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed U.S. government-funded inventions may be subject to certain requirements to manufacture product candidates embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations, and prospects significantly.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights to our in-licensed patents, the license granted to us in jurisdictions where the consent of a co-owner is necessary to grant such a license may not be valid and such co-owners may be able to license such patents to our competitors, and our competitors could market competing products and technology. In addition, our rights to our in-licensed patents and patent applications are dependent, in part, on inter-institutional or other operating agreements between the joint owners of such in-licensed patents and patent applications. If one or more of such joint owners breaches such inter-institutional or operating agreements, our rights to such in-licensed patents and patent applications may be adversely affected. Any of these events could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

***We may not be successful in obtaining additional rights necessary to commercialize our current and future product candidates and technologies, and even if we are successful in obtaining such rights, if we fail to comply with our obligations under the agreements governing those rights, we may be required to pay damages or could lose intellectual property rights that are necessary for developing our current and future product candidates and technologies.***

The field of gene editing is competitive and growing, and we may need to obtain licenses to additional intellectual property from others in order to commercialize our CRISPR-based technologies and any product candidates. We may also need to obtain licenses from third parties covering our product candidates or technologies, or covering auxiliary technologies that are still required for the development and commercialization for our product candidates, such as certain delivery methods. For example, we are aware of certain third-party patents and patent applications in the gene repression space that may be related to our future products that incorporate ELXR. We may find it necessary or prudent to obtain licenses from such third-party intellectual property holders, or to challenge the patentability of claims in these third-party patents and patent applications in re-examination, post-grant review, *inter partes* review, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings).

In addition, with respect to any patents we may co-own with third parties, we may require licenses to such co-owners' interest in such patents. However, we may be unable to secure any of these licenses or otherwise acquire the intellectual property rights from third parties that we identify as necessary to develop and commercialize our product candidates and technologies or may be unable to secure such rights on commercially reasonable terms. The licensing or acquisition of third-party intellectual property rights is a highly competitive area, and a number of more established companies may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. If we are unable to successfully obtain rights to required third-party intellectual property rights, that could have a material adverse effect on our business.

Similarly, even if we were to obtain licenses or otherwise acquire intellectual property rights from third parties that we identify as necessary to develop and commercialize our products, if, for any reason, these agreements are terminated or we otherwise lose those rights, it could adversely affect our business. These agreements are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution, and enforcement or other obligations on us.

If we breach any material obligation, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor(s) may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell products that are covered by the licensed technology or enable a competitor to gain access to the licensed technology. It is possible that we may be unable to obtain additional licenses to such intellectual property rights at a reasonable cost or on reasonable terms. In such event, we may have to expend significant time and resources to redesign our product candidates or technologies, which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize certain product candidates or expand our platform capabilities, which could harm our business.

Furthermore, disputes may arise regarding the intellectual property rights that are subject to such license agreements that may limit the scope of our rights under those agreements. These disputes may include challenges regarding the scope of rights granted under the license agreement, disputes about sublicensing of patent rights to third parties, disputes regarding diligence obligations, disputes regarding inventorship, and/or disputes regarding ownership. Any such dispute will likely be time-consuming and costly, and if we are unsuccessful, may result in a loss of ability to commercialize our product candidates, or may increase our financial obligations.

***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 noncompliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees, and other government fees on any issued patent are due to be paid to the USPTO and other foreign patent agencies in several stages over the lifetime of the issued patent. The USPTO and various foreign national or international patent agencies also require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In certain circumstances, we rely on our licensor to pay these fees and ensure proper compliance with the procedural and documentary provisions, and do not have direct control over such compliance. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance 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. Noncompliance events that could result in abandonment or lapse of patent rights include, but are not limited to, failure to timely file national and regional stage patent applications based on our international patent application, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our current or future product candidates or technologies, our competitors might be able to enter the market with similar or identical products or technology, which would have an adverse effect on our business.

***If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.***

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent term extension of up to five years as compensation for patent term lost during the FDA regulatory review process. However, patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, or five years from the expiration date of the patent to be extended. Only one patent per product may be extended and only those claims covering the approved biologic, a method for using it, or a method for manufacturing it may be extended. Moreover, even if we were to seek patent term extension, it may not be granted because of, for example, the failure to exercise due diligence during the testing phase or regulatory review process, the failure to apply for the extension within applicable deadlines, the failure to apply prior to expiration of relevant patents, or any other failure to satisfy applicable requirements. Moreover, the extension afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

***Third party claims or litigation alleging infringement of patents or other proprietary rights, or seeking to invalidate our patents or other proprietary rights, may delay or prevent the development and commercialization of our current or future product candidates or technologies.***

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. The intellectual property landscape around gene editing technology is highly dynamic and there is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology industry, and in the gene editing space in particular. Potential litigation could include patent infringement lawsuits, derivation and administrative law proceedings, *inter partes* review and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. As the gene editing field continues to expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization, and we may not be successful in challenging the validity and/or enforceability

of such third-party patents. Also, 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. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates or technologies may infringe.

Defense of third-party claims of patent infringement or violation of intellectual property rights involves substantial litigation expense and would be a substantial diversion of management and employee time and resources from our business. Some third parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations, and prospects. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Any of the foregoing events could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, third parties may obtain patent rights in the future and claim that use of our product candidates or other technologies infringe upon these rights. If any third-party patents were held by a court of competent jurisdiction to cover our product candidates, or any aspect of their manufacture or use, the holders of any such patents may be able to block our ability to commercialize such product candidate or technology unless we obtain a license under the applicable patents, or until such patents expire. Such a license may not be available on commercially reasonable terms, or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products or technologies, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms.

The scope of a patent claim is a legal determination made by the courts. It is informed by the written disclosure of a patent, the patent's prosecution history, and other intrinsic and extrinsic factors. Our interpretation of a patent claim may not be adopted during a patent litigation alleging infringement by our products. If a court does not adopt our claim interpretation and determines that our product candidates are covered by a third-party patent, we may be held liable for damages. Similarly, we may incorrectly predict whether a third-party patent application will issue with claims that cover one or more of our product candidates. If our claim interpretations are not adopted by the USPTO during our challenge of a third-party patent application, or by a court in a patent infringement dispute, our ability to develop and market our product candidates may be harmed.

Moreover, we, or one of our licensors, may have to participate in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. If we or our licensors are unsuccessful in any validity (including any patent oppositions) or inventorship disputes to which we or they are subject, we may lose valuable intellectual property rights through the loss of one or more of our owned, licensed, or optioned patents, or such patent claims may be narrowed, invalidated, or held unenforceable, or through loss of exclusive ownership of or the exclusive right to use our owned or in-licensed patents. In the event of loss of patent rights as a result of any of these disputes, we may be required to obtain licenses from third parties, including parties involved in any such proceedings. If we are unable to obtain such licenses, we may need to cease the development, manufacture, and commercialization of one or more of the product candidates or technologies we may develop. The loss of exclusivity or the narrowing of our patent claims could limit our ability to stop others from using or commercializing similar or identical technology and product candidates. Even if we or our licensors are successful in such a proceeding, it could result in substantial costs and be a distraction to management and other employees.

Furthermore, the gene editing patent landscape is crowded and highly competitive. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates, including gene editing, guide nucleic acids, protospacer adjacent motif, or PAM, sequence variants, and gene repression technology, and they may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. Ongoing research and development in the gene editing space is taking place by several companies, universities, and other institutions. There can be no assurance that our operations do not, or will not in the future, infringe, misappropriate or otherwise violate existing or future third-party patents or other intellectual property rights. Identification of third-party patent rights that may be relevant to our operations is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and publication timelines. We cannot guarantee that any patent searches we may conduct are complete or thorough enough to identify every third-party patent and pending application in the United States and/or abroad that is relevant to or necessary for the development and commercialization of our product candidates in any country.

There has already been significant intellectual property activity in the CRISPR-based gene editing space, and we expect that to continue. The extensive patent filings related to CRISPR related technologies make it difficult for us to assess the full extent of potentially relevant patents that may cover some aspect of our product candidates. Even in the absence of litigation, we may need to obtain licenses from third parties in order to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly. We cannot provide any assurances that third party patents do not exist which might be enforced against our product candidates resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation to third parties.

***We may become involved in lawsuits to protect or enforce our owned or licensed intellectual property rights against others, which could be expensive, time-consuming and unsuccessful.***

Competitors may infringe or otherwise violate our patents, the patents of our licensors, or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal 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 licensor is not valid or is unenforceable, 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 or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter-claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge may be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement, written description, or lack of patentable subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as reexaminations, *inter partes* review or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

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. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

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

Changes in either the patent laws or the interpretation of patent laws could increase the uncertainties and costs surrounding the prosecution of patent applications and/or the enforcement or defense of our issued patents. The United States has enacted and implemented wide-ranging patent reform legislation. For example, the United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the United States Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future.

Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future. For example, the complexity and uncertainty of European patent laws have also increased in recent years. In Europe, a new unitary patent system took effect June 1, 2023, which will significantly impact European patents, including those granted before the introduction of such a system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court, or the UPC. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. Patents granted before the implementation of the UPC have the option of opting out of the jurisdiction of the UPC over the first seven years of the court's existence 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 that are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

***Any trademarks we may obtain may be infringed or successfully challenged, resulting in harm to our business.***

We expect to rely on trademarks as one means to distinguish product candidates that are approved for marketing from the products of our competitors. We have not yet selected trademarks for our product candidates and have not yet begun the process of applying to register trademarks for our current or future product candidates. Once we select trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose our trademark applications or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our products, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks, and we may not have adequate resources to enforce our trademarks. If we are unable to establish brand recognition based on our trademarks and trade names, we might not be able to compete effectively in the marketplace and our business may be harmed.

In addition, any proprietary name we propose to use with our current or future product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of the potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable proprietary product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

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

In addition to seeking patents for our technology and product candidates, we also rely on know-how and trade secret protection, as well as confidentiality agreements, non-disclosure agreements and invention assignment agreements with our employees, consultants and third parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable.

It is our policy to require our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed by or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties, except in certain specified circumstances. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access

to our trade secrets or proprietary technology and processes. Any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable.

In addition to contractual measures, we try to protect the confidential nature of our proprietary information through other appropriate precautions, such as physical and technological security measures. However, trade secrets and know-how can be difficult to protect. These measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and any recourse we might take against this type of misconduct may not provide an adequate remedy to protect our interests fully. In addition, trade secrets may be independently developed by others in a manner that could prevent us from receiving legal recourse. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any of that information was independently developed by a competitor, our competitive position could be harmed.

Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws within the United States. We may need to share our trade secrets and proprietary know-how with current or future partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors, and those affiliated with or controlled by state actors. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. In addition, some courts inside and outside the United States are sometimes less willing or unwilling to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. Even if we are successful, these types of lawsuits may consume our time and other resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators, and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and 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, valuable intellectual property. Even if we are successful, litigation could result in substantial costs and be a distraction to our management and other employees.

**Risks related to government regulation**

***Gene editing and modification, which are novel and distinct subsets of gene therapy, and the regulatory landscape that will govern any product candidates we may develop are uncertain and may change. As a result, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.***

The regulatory requirements that will govern any novel gene editing or epigenetic modification product candidates we develop are not entirely clear and may change. Within the broader genetic medicines field, we are aware of a limited number of gene therapy products that have received marketing authorization from the FDA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene editing products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene editing products and cell therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products, or OTP, within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene editing and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene editing clinical trials may also be subject to review and oversight by an Institutional Biosafety Committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Although the FDA decides whether individual gene editing protocols may

proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

Adverse developments in post-marketing experience or in clinical trials conducted by others of gene editing products, cell therapy products, or products developed through the application of a base editing or other gene editing technology may cause the FDA and other regulatory bodies to revise the requirements for development or approval of any product candidates we may develop or limit the use of products utilizing gene editing technologies, either of which could materially harm our business. In addition, the clinical trial requirements of the FDA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for novel product candidates such as the product candidates we may develop can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene editing technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

***The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.***

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing, and distribution of our product candidates are subject to extensive regulation by the FDA in the United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market our product candidates until we receive regulatory approval of a BLA from the FDA. The process of obtaining such regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity, and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and the FDA and comparable foreign regulatory authorities have substantial discretion in the approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval of a product candidate is never guaranteed. Of the large number of biologics in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized.

Prior to obtaining approval to commercialize a biological product candidate in the United States or abroad, we must demonstrate with evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe, pure, and potent for their intended uses. This process also requires that we demonstrate substantial evidence of effectiveness of such product candidates for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe available preclinical or clinical data support the safety, purity, potency or effectiveness of our product candidates, such data may not be sufficient to obtain approval from the FDA and comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or may object to elements of our clinical development program.

The FDA or comparable foreign regulatory authorities can delay, limit or deny approval of a product candidate for many reasons, including:

- such authorities may disagree with the design or execution of our clinical trials;
- negative or ambiguous results from our clinical trials or results may not meet the level of statistical significance or persuasiveness required by the FDA or comparable foreign regulatory agencies for approval;

- serious and unexpected treatment-related side effects may be experienced by participants in our clinical trials or by individuals using therapies similar to our product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety and efficacy in the full population for which we seek approval;
- such authorities may not accept clinical data from trials that are conducted at clinical facilities or in countries where the standard of care is potentially different from that of their own country;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- such authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- such authorities may not agree that the data collected from clinical trials of our product candidates are acceptable or sufficient to support the submission or filing of a BLA in the United States or other submission or to obtain regulatory approval elsewhere, and such authorities may require additional preclinical studies or clinical trials;
- such authorities may disagree with us regarding the formulation, labeling and/or the product specifications of our product candidates;
- such authorities may disagree with us that our potency assays for our product candidates are adequate;
- approval may be granted only for indications that are significantly more limited than those sought by us, and/or may include significant restrictions on distribution and use;
- such authorities may find deficiencies in the manufacturing processes or facilities of the third-party manufacturers with which we contract for clinical and commercial supplies;
- such authorities may not accept a submission due to, among other reasons, the content or formatting of the submission; or
- the approval regulations or policies of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

With respect to foreign markets, approval procedures vary among countries and, in addition to the foregoing risks, may involve additional product testing, administrative review periods and agreements with pricing authorities.

Even if we eventually complete clinical trials and receive approval of a BLA or comparable foreign marketing application for our product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials and/or the implementation of a REMS, which may be required because the FDA believes it is necessary to ensure that the benefits outweigh the risks of the product after approval. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and could have a material adverse impact on our business and prospects.

***Disruptions at the FDA and other government agencies caused by, among other factors, funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, prevent new or modified products from being developed, reviewed, approved or commercialized in a timely manner or at all, or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA and foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, government shutdowns, statutory, regulatory, and policy changes, the FDA's or foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's or foreign regulatory authorities' ability to perform routine functions. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new biologics or modifications to approved biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times, most recently in late 2025, and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. In addition, the current U.S. Presidential administration has issued certain policies and Executive Orders directed toward reducing the employee headcount and costs associated with U.S. administrative agencies, including the FDA, and it remains unclear the degree to which these efforts may limit or otherwise adversely affect the FDA's ability to conduct routine activities. Recent and future changes in FDA staffing,

including leadership, could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all.

If a prolonged government shutdown occurs, or if funding shortages, staffing limitations or similar factors hinder or prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, such events could significantly impact the ability of the FDA or other such regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

***We may in the future conduct clinical trials for current or future product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.***

We may in the future conduct clinical trials for current or future product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials. The acceptance of data from clinical trials conducted outside the United States by the FDA or other comparable foreign regulatory authorities may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for regulatory approval in the United States, the FDA will generally not approve the application unless (i) the data are applicable to the U.S. population and U.S. medical practice and (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations, and the data may be considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCPs and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. Conducting clinical trials outside the United States also exposes us to additional risks, including risks associated with foreign exchange fluctuations, compliance with foreign manufacturing, customs, shipment and storage requirements, and cultural differences in medical practice and clinical research, and diminished protection of intellectual property in some countries.

There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. For example, for FDA acceptance, we will have to demonstrate that the foreign data are applicable to the U.S. population and U.S. medical practice. If the FDA or other comparable foreign regulatory authorities do not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop being delayed or not receiving approval for commercialization in the applicable jurisdiction.

***We may seek orphan drug designation for one or more of our product candidates. We may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates and, even if we do, that exclusivity may not prevent the FDA or other comparable foreign regulatory authorities from approving other competing products.***

Regulatory authorities in some jurisdictions may designate drugs for relatively small patient populations as orphan drugs. In the United States, orphan drug designation entitles a party to financial incentives such as tax advantages and user fee waivers or exemptions. In addition, if a product receives the first FDA approval for the condition for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same condition for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity.

We may pursue orphan drug designation for one or more of our product candidates. However, obtaining an orphan drug designation can be difficult, and we may not be successful in doing so. Even if we obtain orphan drug designation, we may not be able to maintain such designation. Orphan drug designation neither shortens the development time or regulatory review time of a product candidate nor gives the product candidate any advantage in the regulatory review or approval process. Even if we obtain orphan drug designation for our product candidates in specific conditions, we may not be the first to obtain regulatory approval of these product candidates for the orphan-designated condition and therefore we may not be eligible for orphan drug exclusivity in the U.S. In addition, exclusive marketing rights in the United States may not be awarded if we seek approval for an indication broader than the orphan-designated condition or, if awarded, may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure

sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Furthermore, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan product is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Our inability to obtain orphan drug designation for any product candidates and/or our inability to maintain that designation for the duration of the applicable exclusivity period, could reduce our ability to make sufficient sales of the applicable product candidate to balance our expenses incurred to develop it.

***A Breakthrough Therapy, Fast Track, or Regenerative Medicine Advanced Therapy, or RMAT, designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive regulatory approval.***

We may seek breakthrough therapy, fast track, or RMAT designation for some or all of our product candidates. A breakthrough therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug, or biologic in our case, may demonstrate substantial improvement over existing therapies with respect to one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Fast track designation is granted for products that are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. A product candidate can receive RMAT designation if (1) the product candidate is a regenerative medicine therapy; (2) the product candidate is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (3) preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such a disease or condition.

Breakthrough therapy, fast track, or RMAT designation is within the discretion of the FDA. Accordingly, even if we believe, after completing early clinical trials, that one of our product candidates meets the criteria for designation, the FDA may disagree and instead determine not to make such designation. Even if we receive such designation for other product candidates or indications in the future, we may not experience a faster development process, review or approval compared to drugs or biologics considered for approval under conventional FDA procedures and such a designation does not assure ultimate approval by the FDA. Even if one or more of our product candidates qualify for breakthrough therapy, fast track, or RMAT designation, the FDA may later decide that such product candidates no longer meet the conditions for qualification.

***Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.***

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction. For example, even if the FDA or a comparable foreign regulatory authority grants regulatory approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. However, a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we or any partner we work with fail to comply with the regulatory requirements in international markets or fail to receive applicable regulatory approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

***Even if we receive regulatory approval for any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense.***

Any regulatory approvals that we obtain for any of our product candidates may also be subject to limitations on the approved uses for which a product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, post-approval monitoring and adverse event reporting, storage, import, export, advertising,

promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. The FDA has significant post-market authority, including the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate safety risks related to the use of a product or to require withdrawal of the product from the market. The FDA also has the authority to require a REMS after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug.

The manufacturing facilities we use to make a product, if any, will also be subject to periodic review and inspection by the FDA and other regulatory agencies, including for continued compliance with cGMP requirements. Any product promotion and advertising will also be subject to regulatory requirements and continuing regulatory review.

Subsequent discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our CDMOs or manufacturing processes, or our failure to comply with regulatory requirements, may result in, among other things:

- holds on clinical trials;
- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market or voluntary or mandatory product recalls;
- restrictions on product distribution or use, or requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- refusal by the FDA to approve pending applications or supplements to approved applications;
- suspension or revocation of product approvals or licenses;
- product seizure or detention or refusal to permit the import or export of products; and
- injunctions, fines or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity. The FDA policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of any of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability, which would adversely affect our business.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. If any legislation, executive orders, personnel changes, or lapses in agency funding impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

***Our operations and relationships with healthcare providers, healthcare organizations, customers and third-party payors will be subject to applicable anti-bribery, anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations, which could expose us to, among other things, enforcement actions, criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.***

Our current and future arrangements with healthcare providers, healthcare organizations, third-party payors and customers expose us to broadly applicable anti-bribery, fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research as well as market, sell and distribute any of our product candidates. Restrictions under applicable federal and state anti-bribery and healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, individuals and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, 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 or state healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the federal criminal and civil false claims and civil monetary penalties laws, including the federal False Claims Act, which can be enforced through civil whistleblower or qui tam actions against individuals or entities, prohibit, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Moreover, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act;
- HIPAA, which prohibits, among other things, knowingly and willfully executing, or attempting to execute a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the Physician Payments Sunshine Act, which requires certain manufacturers of covered drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with certain exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members, with the information made publicly available on a searchable website;
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; and
- certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and drug pricing information, and state and local laws that require the registration of pharmaceutical sales representatives.

Efforts to ensure that our current and future business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including certain advisory agreements we have entered into with physicians who are paid, in part, in the form of stock or stock options, do not comply with current or future statutes, regulations, agency guidance, or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any such requirements, we may be subject to significant penalties, including civil, criminal, and administrative penalties, damages, fines, disgorgement, imprisonment, the curtailment or restructuring of our operations, loss of eligibility to obtain approvals from the FDA, exclusion from participation in government contracting, healthcare reimbursement, or other government programs, including Medicare and Medicaid, integrity oversight and reporting obligations, or reputational harm, any of which could adversely affect our financial results. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time, and resources.

***Our product candidates for which we intend to seek approval may face competition from biosimilars approved through an abbreviated regulatory pathway.***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. The BPCIA created an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as interchangeable based on its similarity to an existing reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the reference product is approved under a BLA.

We believe that if any of our product candidates is approved as a biological product under a BLA, it should qualify for the 12-year period of exclusivity. However, there is a risk that the FDA will not consider any of our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, an interchangeable biosimilar, once approved, may be substituted under existing law for any one of our products determined to be reference products in a way that is similar to traditional generic substitution; any non-interchangeable biosimilar products may also be substituted by a healthcare provider but, under existing law, will not be automatically substituted at the pharmacy. The extent of the impact of such substitution will depend on a number of marketplace and regulatory factors that are still developing.

***We may face difficulties from healthcare legislative and regulatory reform measures.***

Existing laws and regulatory policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of any of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any regulatory approval that we may have obtained, or may face penalties for any approved products, and we may not achieve or sustain profitability.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. Among other things, the ACA, enacted in 2010, increased manufacturers' rebate liability under the Medicaid Drug Rebate Program and imposed a significant annual fee on companies that manufacture or import branded prescription drug products.

Recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several presidential executive orders, Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, reduce the costs of drugs under Medicare, and reform government program reimbursement methodologies for drug products.

These initiatives recently culminated in the enactment of the Inflation Reduction Act, or IRA, in August 2022, which, among other things, requires the Secretary of the Department of Health and Human Services, or HHS, to negotiate the selling price of certain drugs and biologics that CMS reimburses under Medicare Part B and Part D, although this only applies to high-expenditure single-source biologics that have been approved for at least 11 years (7 years for drugs). The negotiated prices will be capped at a statutory ceiling price representing a significant discount from average prices to wholesalers and direct purchasers. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product was announced. CMS has also selected and negotiated the maximum fair price for 15 additional Medicare Part D drugs, which will first be effective in 2027. For 2028, CMS has selected an additional 15 drugs, comprised of drugs covered under Medicare Part D and, for the first time, drugs payable under Medicare Part B. For 2029 and subsequent years, 20 Part B or Part D drugs will be selected. Currently, a drug or biological product that has an orphan drug designation for only one rare disease or condition is excluded from the IRA's price negotiation requirements, as long as the drug is approved only for an indication within that disease or condition. However, as a result of a statutory amendment enacted in July 2025, beginning with the 2028 negotiated price applicability year, a drug may be designated for more than one rare disease or condition and still be excluded from price negotiation, as long as the only approved indications are for such rare diseases or conditions. The constitutionality of the IRA's drug price negotiation program provisions is currently subject to ongoing litigation. The outcome of this litigation cannot yet be fully determined.

The IRA also penalizes drug manufacturers that increase prices of Medicare Part B and Part D drugs at a rate greater than the rate of inflation. In addition, the law eliminates the "donut hole" under Medicare Part D beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost through a newly established manufacturer discount program. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA permits the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. While it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry.

More recently, the One Big Beautiful Bill Act, which was enacted in July 2025, imposes significant reductions in the funding of the Medicaid program. Such reductions are expected to decrease the number of persons enrolled in Medicaid and

reduce the services covered by Medicaid, which could adversely affect our sales of any product candidate that we commercialize.

The current administration has indicated that it plans to pursue additional policies aimed at lowering prescription drug costs. For example, in May 2025, the administration published an executive order regarding most favored nation, or MFN, drug pricing, which is sometimes referred to as international reference pricing. This executive order directs the Secretary of HHS to communicate MFN price targets to pharmaceutical manufacturers, and if significant progress toward MFN pricing is not delivered, to propose a rule-making plan to impose MFN pricing. HHS is currently developing a proposed rule to establish a demonstration model under the auspices of CMS's Center for Medicare and Medicaid Innovation that will require MFN pricing, but the proposed rule has not yet been published, so it is not yet known which drugs will be covered, how long the model will be in effect, or how pricing will be determined. If that rule or other MFN pricing rules are finalized, they are likely to mandate reduced prices of at least some drugs in the United States, if they are also sold in comparator countries. The scope, timing, and potential impact of current and future policy initiatives remain uncertain, and accordingly, we cannot predict how such legal and regulatory changes may affect our business, operations, or financial condition. However, if MFN drug pricing is implemented, the U.S. list price of our products that are also being commercialized outside of the United States could be substantially reduced, which could negatively impact our U.S. product sale revenues and the overall U.S. market opportunity. Even if we do not market products outside of the United States, we will be indirectly affected if our products competed with products that are reduced by MFN pricing.

At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including restrictions or prohibitions on certain marketing practices, reporting of specified categories of remuneration provided to healthcare practitioners, and reporting and justification of price increases greater than a specified level. In some cases, states have designed programs to encourage importation from other countries and bulk purchasing. For example, on January 5, 2024, the FDA approved Florida's Section 804 Importation Program, or SIP, proposal to import certain drugs from Canada for specific state healthcare programs. On December 20, 2024, FDA granted an extension of Florida's SIP authorization for an additional period of six months, until July 6, 2025, and, on June 2, 2025, FDA granted an additional extension until November 6, 2025. It is unclear how and whether this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the United States or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for pharmaceuticals and other healthcare products and services, which could result in reduced demand for any product candidates or additional pricing pressures.

Other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

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

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, or FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Export controls and trade sanctions laws and regulations may restrict or prohibit altogether the provision, sale, or supply of any product candidates to certain governments, persons, entities, countries and territories, including those that are the target of comprehensive sanctions or an embargo. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents and contractors from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties outside the United States to, among other things, conduct clinical trials and obtain necessary permits, licenses, patent registrations and other regulatory approvals. We or our third parties may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations, such as state-owned entities. Although we currently only maintain operations in the United States, we have not historically had trainings, policies or manuals regarding these laws and regulations, and have not implemented processes to screen and review our actions or the

actions of our employees, agents, contractors, or other partners to monitor compliance with such laws and regulations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, or other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

Detecting, investigating, and resolving actual or alleged violations of these laws and regulations can require a significant diversion of time, resources, and attention from management. In addition, noncompliance with such laws and regulations could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, enforcement actions, fines, damages, other civil or criminal penalties, injunctions, suspension or debarment from contracting with certain persons, reputational harm, adverse media coverage, and other collateral consequences. If any subpoenas are received or investigations are launched, or governmental or other sanctions are imposed, or if we do not prevail in any possible civil or criminal proceeding, our business, operating results, and financial condition could be materially harmed. In addition, responding to any action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees.

**Risks related to our common stock**

***Our quarterly and annual operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.***

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- timing and results of our clinical trials, preclinical studies, or the addition or termination of future preclinical studies and clinical trials or funding support by us, or existing or future collaborators or licensing partners;
- timing and variations in the level of expense related to the ongoing development of STX-1150, STX-1200, STX-1400, any other future product candidate or our CRISPR-based technologies, ELXR and XE;
- our execution of any additional collaboration, licensing or similar arrangements, and the timing of payments we may make or receive under existing or future arrangements or the termination or modification of any such existing or future arrangements;
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us or our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;
- if any of our product candidates receives regulatory approval, the terms of such approval and market acceptance and demand for such product candidates;
- future accounting pronouncements or changes in our accounting policies;
- regulatory developments affecting any product candidates or those of our competitors;
- the timing and cost to establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval and intend to commercialize on our own or jointly with current or future collaborators;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- effects of macro events, such as inflation, geopolitical conflicts, pandemics, natural disasters, tariffs, trade tensions and supply chain issues, on our business and operations; and
- changes in general market and economic conditions.

If our quarterly or annual operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly or annual fluctuations in our operating results may, in turn, cause the price of our common stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

***The market price of our common stock is likely to be highly volatile, and you could lose all or part of your investment.***

The trading price of our common stock is likely to continue to be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. As a result of this volatility, investors may not be able to sell their common stock at or above the price initially paid for the stock. The market price for our common stock may be influenced by many factors, including the other risks described in this "Risk Factors" section and the following:

- results of preclinical studies and future clinical trials of any product candidates, or those of our competitors or our existing or future collaborators;
- regulatory or legal developments in the United States or other countries, especially changes in federal or global health policies, laws or regulations applicable to any product candidates, including the review and oversight functions of federal health regulatory bodies;

- the success or failure of competitive products or technologies;
- introductions and announcements of new product candidates by us, any future commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to any product candidates, clinical studies, and, if approved, manufacturing process or sales and marketing terms;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to identify, acquire or in-license additional technologies or product candidates;
- developments concerning any future collaborations, including but not limited to those with development and commercialization partners if any product candidates are approved;
- market conditions in the pharmaceutical and biotechnology sectors;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures or capital commitments;
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for any product candidates;
- our ability or inability to raise additional capital and the terms on which we are able to raise it, if at all;
- our ability to effectively manage our growth;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- actual or anticipated changes in earnings estimates, development timelines or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- our failure or the failure of our competitors to meet analysts' projections or guidance that we or our competitors may give to the market;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- announcement and expectation of additional financing efforts;
- speculation in the press or investment community;
- fluctuations of trading volume of our common stock;
- sales of our common stock by us, insiders or our stockholders;
- the concentrated ownership of our common stock;
- expiration of market stand-off or lock-up agreements;
- changes in accounting principles;
- actions instituted by activist shareholders or others;
- terrorist acts, acts of war or periods of widespread civil unrest;
- natural disasters and other calamities, including global pandemics;
- general economic, industry and market conditions, including changes in tariffs and trade restrictions, fluctuating interest rates and inflation; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations that have been often unrelated or disproportionate to the operating performance of the issuer. Furthermore, the trading price of our common stock may be adversely affected by third parties trying to drive down the market price. Short sellers and others, some of whom post anonymously on social media, may be positioned to profit if our stock declines and their activities can negatively affect our stock price. These broad market and industry factors may seriously harm the market price of our common stock, regardless of

our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this "Risk Factors" section, could have a dramatic and adverse impact on the market price of our common stock.

***A sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell in the public market before or after the lock-up and other legal restrictions on resale lapse in connection with our IPO, the market price of our stock could decline significantly. Each of our officers, directors and holders of substantially all of our outstanding equity securities have entered into lock-up agreements that restrict their ability to sell or transfer their shares. The lock-up agreements will expire on January 19, 2027. However, Leerink Partners LLC and Goldman Sachs & Co. LLC may, in their sole discretion, permit our officers, directors and other current stockholders who are subject to the contractual lock-up to sell shares prior to January 19, 2027.

In addition, pursuant to our amended and restated investors' rights agreement, certain stockholders have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or our stockholders. We also have registered shares of common stock that we may issue under our equity incentive plans. These shares are freely tradable in the public market upon issuance, subject to the 180-day lock-up period under the lock-up agreements described above.

We cannot predict what effect, if any, sales of our shares in the public market or the availability of shares for sale will have on the market price of our common stock. However, future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of our outstanding options, or the perception that such sales may occur, could adversely affect the market price of our common stock.

We also expect that significant additional capital may be needed in the future to continue our planned operations. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. To the extent that additional capital is raised through the sale and issuance of shares of common stock or other securities convertible into shares of common stock, our stockholders will be diluted. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares of common stock, could reduce the market price of our common stock.

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

Based on the beneficial ownership of our common stock as of July 10, 2026, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 38.3% of our voting stock. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our Company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our Company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

***We do not currently intend to pay dividends on our common stock and, consequently, our stockholders' ability to achieve a return on their investment will depend on appreciation of the value of our common stock.***

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to support operations and to finance the growth and development of our business. We do not intend to declare or pay any cash dividends on our capital stock in the foreseeable future. As a result, any investment return on our common stock will depend upon increases in the value of our common stock, which is not certain.

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

We are an "emerging growth company" as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (i) being permitted to present only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this Quarterly Report; (ii) reduced disclosure about our executive compensation arrangements; (iii) not being required to hold advisory votes on executive compensation or to obtain stockholder approval of any golden parachute arrangements not previously approved; (iv) an exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act; and (v) an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on the financial statements.

We may take advantage of these exemptions until December 31, 2031 or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.235 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of our IPO; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

We have taken advantage of reduced reporting requirements in this Quarterly Report. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. Additionally, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption and, therefore, while we are an emerging growth company, we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies. As a result of this election, our financial statements may not be comparable to those of other public companies that comply with new or revised accounting pronouncements as of public company effective dates. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies. Until the date that we are no longer an "emerging growth company" or affirmatively and irrevocably opt out of the exemption provided by Section 7(a)(2)(B) of the Securities Act, upon issuance of a new or revised accounting standard that applies to our financial statements and that has a different effective date for public and private companies, we will disclose the date on which adoption is required for non-emerging growth companies and the date on which we will adopt the recently issued accounting standard.

We are also a "smaller reporting company," meaning that the market value of our common stock held by non-affiliates is less than $700.0 million and our annual revenue is less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either (i) the market value of our common stock held by non-affiliates is less than $250.0 million or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements, we are not required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

***If we fail to establish and maintain proper and effective internal control over financial reporting in the future, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.***

Pursuant to Section 404 of the Sarbanes-Oxley Act, we are required to furnish a report by our management on our internal control over financial reporting within our Annual Report on Form 10-K. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming

effort that will need to be frequently evaluated. Our failure to maintain the effectiveness of our internal controls in accordance with the requirements of the Sarbanes-Oxley Act could have a material adverse effect on our business. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on Nasdaq.

As we grow, we expect to hire additional personnel and may utilize external temporary resources to implement, document and modify policies and procedures to maintain effective internal controls. However, it is possible that we may identify deficiencies and weaknesses in our internal controls. If material weaknesses or deficiencies in our internal controls exist and go undetected or unremediated, our financial statements could contain material misstatements that, when discovered in the future, could cause us to fail to meet our future reporting obligations and cause the price of our common stock to decline.

***Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.***

We are subject to the periodic reporting requirements of the Exchange Act of 1934, as amended, or the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected. In addition, we do not have a formal risk management program for identifying and addressing risks to our business in other areas.

***Anti-takeover provisions in our charter documents and under Delaware law could prevent or delay an acquisition of us, which may be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management.***

Our amended and restated certificate of incorporation and our amended and restated bylaws contain provisions that could delay or prevent a change in control of our Company. These provisions could also make it difficult for stockholders to elect directors who are not nominated by current members of our board of directors or take other corporate actions, including effecting changes in our management. These provisions:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit only the board of directors to establish the number of directors and fill vacancies on the board;
- provide that directors may only be removed “for cause” and only with the approval of two-thirds of our stockholders;
- require super-majority voting to amend some provisions in our amended and restated certificate of incorporation and amended and restated bylaws;
- authorize the issuance of “blank check” preferred stock that our board of directors could use to implement a stockholder rights plan;
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting; and
- establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

In addition, Section 203 of the Delaware General Corporation Law, or DGCL, may discourage, delay or prevent a change in control of our Company. Section 203 imposes certain restrictions on mergers, business combinations and other transactions between us and holders of 15% or more of our common stock.

***The exclusive forum provisions in our organizational documents may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or employees, or the underwriters of any offering giving rise to such claim, which may discourage lawsuits with respect to such claims.***

Our amended and restated bylaws, to the fullest extent permitted by law, provide that the Court of Chancery of the State of Delaware is the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws; or any action asserting a claim that is governed by the internal affairs doctrine. This exclusive forum provision does not apply to suits brought to enforce a duty or liability created by the Exchange Act. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision. This choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or other employees, or the underwriters of any offering giving rise to such claims, which may discourage lawsuits with respect to such claims. Alternatively, if a court were to find the choice of forum provisions contained in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, financial condition, results of operations, and prospects.

Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all claims brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. Our amended and restated bylaws provide that the federal district courts of the United States will, to the fullest extent permitted by law, be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, or the Federal Forum Provision, including for all causes of action asserted against any defendant named in such complaint. For the avoidance of doubt, this provision is intended to benefit and may be enforced by us, our officers and directors, the underwriters to any offering giving rise to such complaint, and any other professional person or entity whose profession gives authority to a statement made by that person or entity and who has prepared or certified any part of the documents underlying the offering. Our decision to adopt a Federal Forum Provision followed a decision by the Supreme Court of the State of Delaware holding that such provisions are facially valid under Delaware law. While federal or other state courts may not follow the holding of the Delaware Supreme Court or may determine that the Federal Forum Provision should be enforced in a particular case, application of the Federal Forum Provision means that suits brought by our stockholders to enforce any duty or liability created by the Securities Act must be brought in federal court and cannot be brought in state court, and our stockholders cannot waive compliance with the federal securities laws and the rules and regulations thereunder. Section 27 of the Exchange Act creates exclusive federal jurisdiction over all claims brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. In addition, neither the exclusive forum provision nor the Federal Forum Provision applies to suits brought to enforce any duty or liability created by the Exchange Act. Accordingly, actions by our stockholders to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder must be brought in federal court, and our stockholders cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

Any person or entity purchasing or otherwise acquiring or holding any interest in any of our securities shall be deemed to have notice of and consented to our exclusive forum provisions, including the Federal Forum Provision. These provisions may limit a stockholder's ability to bring a claim, and may result in increased costs for a stockholder to bring such a claim, in a judicial forum of their choosing for disputes with us or our directors, officers, other employees, or agents, which may discourage lawsuits against us and our directors, officers, other employees, or agents.

**General risk factors**

***Unfavorable macroeconomic conditions or market volatility resulting from geopolitical developments or national or global economic conditions, including those affecting the financial services industry, could adversely affect our business, financial condition or results of operations.***

Adverse macroeconomic conditions or market volatility resulting from national or global economic developments, political unrest, high inflation, rising interest rates, international tariffs, changes in international trade relationships and military conflicts, such as the ongoing conflict between Russia and Ukraine, recent military conflicts and geopolitical instability in the Middle East, the potential for significant changes in U.S. policies or regulatory environment, or other factors, could materially and adversely affect our business operations. Sanctions imposed by the U.S. and other countries in

response to such conflicts may also continue to adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability. Tariffs levied by the U.S. and other countries also may adversely affect financial markets and the global economy. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. For instance, actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. Investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. In addition, any further deterioration in the macroeconomic economy or financial services industry could lead to losses or defaults by our suppliers, which, in turn, could have a material adverse effect on our current and/or planned business operations and our current or projected results of operations and financial condition. For example, there has been recent U.S. legislation that may restrict the ability of U.S. biopharmaceutical companies to purchase services or products from, or otherwise collaborate with, certain Chinese biotechnology companies of concern without losing the ability to contract with, or otherwise receive funding from, the U.S. government. We continue to assess the law to determine whether it could have an effect on our contractual relationships. Also, current inflationary trends in the global economy may impact salaries and wages, costs of goods and transportation expenses, among other things, and recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures may create market and economic instability.

A severe or prolonged economic downturn or additional global financial crises could result in a variety of risks to our business, including weakened demand for any product candidates we develop or our ability to raise additional capital when needed on acceptable terms, if at all.

Further, U.S. government appropriations have been affected by larger U.S. government budgetary issues and related legislation. In addition, in the past, U.S. debt ceiling and budget deficit concerns have increased the possibility of additional credit-rating downgrades and economic slowdowns, or a recession in the U.S. Although U.S. lawmakers passed legislation to raise the federal debt ceiling on multiple occasions, ratings agencies have lowered or threatened to lower the long-term sovereign credit rating on the U.S. The impact of this or any further downgrades to the U.S. government's sovereign credit rating or its perceived creditworthiness could adversely affect the U.S. and global financial markets and economic conditions. As a result, government spending levels are difficult to predict beyond the near term due to numerous factors, including the external threat environment, future government priorities and the state of government finances. Significant changes in government spending or changes in U.S. government priorities, policies and requirements could have a material adverse effect on our results of operations, financial condition or liquidity.

Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

***If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.***

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not have any control over the industry or securities analysts, or the content and opinions included in their reports. If no or few securities or industry analysts continue or commence coverage of us, the trading price for our common stock could be impacted negatively. In the event we obtain securities or industry analyst coverage, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our preclinical studies and clinical trials and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of such analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause a decline in our stock price or trading volume.

***We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.***

As a public company, and particularly after we are no longer an emerging growth company or smaller reporting company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, the listing requirements of Nasdaq and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance

practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. The increased costs may require us to reduce costs in other areas of our business. Moreover, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

***We may be subject to securities litigation, which is expensive and could divert management attention.***

The market price of our common stock is likely to be highly volatile. The stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

**Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities.**

**Recent Sales of Unregistered Securities**

On July 17, 2026, we entered into a stock purchase agreement with Aventis, Inc., a Sanofi company, together with Genzyme Corporation, to purchase $7,500,000 of our common stock in a concurrent private placement to our IPO. This private placement closed on July 27, 2026, concurrently with our IPO, and Aventis, Inc. purchased 500,000 shares of our common for an aggregate purchase price of $7,500,000. This transaction was exempt from registration requirements of the Securities Act in reliance upon Section 4(a)(2) of the Securities Act or Regulation D promulgated under the Securities Act.

**Use of Initial Public Offering Proceeds**

On July 23, 2026, our registration statement on Form S-1 (File No. 333-297246) relating to the initial public offering of our common stock was declared effective by the Securities and Exchange Commission. Our initial public offering closed on July 27, 2026, at which time we issued and sold 9,867,000 shares of common stock at a price to the public of $15.00 per share, including 1,287,000 shares sold pursuant to the underwriters' exercise in full of their option to purchase additional shares, resulting in aggregate gross proceeds to us of approximately $148.0 million. Leerink Partners LLC and Goldman Sachs & Co. LLC acted as representatives of the underwriters for the offering.

We received aggregate net proceeds of approximately $140.7 million from the initial public offering and the concurrent private placement, after deducting underwriting discounts and commissions and offering expenses payable by us of approximately $14.8 million in the aggregate. None of the expenses incurred in connection with the offering were direct or

indirect payments to our directors or officers, to persons owning 10% or more of any class of our equity securities, or to any of our affiliates, other than payments in the ordinary course of business to our officers for salaries.

There has been no material change in the planned use of proceeds from our initial public offering from that described in the final prospectus dated July 23, 2026, filed with the Securities and Exchange Commission pursuant to Rule 424(b) under the Securities Act.

**Issuer Purchases of Equity Securities**

We did not repurchase any shares of our common stock during the three months ended June 30, 2026.

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

Not applicable.

**Item 5. Other Information.**

**Rule 10b5-1 Trading Arrangements**

None of our directors or officers adopted or terminated a “Rule 10b5-1 trading arrangement” or a “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K, during the three months ended June 30, 2026.

**Item 6. Exhibits.**

| Exhibit Number | Description | Form | File No. | Exhibit | Filing Date | Filed Herewith |
|---|---|---|---|---|---|---|
| 3.1 | Amended and Restated Certificate of Incorporation. | 8-K | 001-43409 | 3.1 | July 27, 2026 | |
| 3.2 | Amended and Restated Bylaws. | 8-K | 001-43409 | 3.2 | July 27, 2026 | |
| 4.1 | Form of Common Stock Certificate. | S-1 | 333-297246 | 4.1 | July 2, 2026 | |
| 10.1 | Form of Indemnity Agreement. | S-1/A | 333-297246 | 10.1 | July 10, 2026 | |
| 10.2 | 2026 Equity Incentive Plan, and forms of award agreements. | S-1/A | 333-297246 | 10.3 | July 20, 2026 | |
| 10.3 | 2026 Employee Stock Purchase Plan, and forms of award agreements. | S-1/A | 333-297246 | 10.4 | July 20, 2026 | |
| 10.4 | Non-Employee Director Compensation Policy. | S-1/A | 333-297246 | 10.8 | July 20, 2026 | |
| 10.5† | Notice of Award, dated May 27, 2026, between the Registrant and California Institute for Regenerative Medicine. | S-1 | 333-297246 | 10.16 | July 2, 2026 | |
| 10.6† | Notice of Award, dated June 1, 2026, between the Registrant and California Institute for Regenerative Medicine. | S-1 | 333-297246 | 10.17 | July 2, 2026 | |
| 10.7 | Stock Purchase Agreement, dated July 17, 2026, between the Registrant and Aventis, Inc. (a Sanofi affiliate). | S-1/A | 333-297246 | 10.18 | July 20, 2026 | |
| 31.1* | Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. | | | | | X |
| 31.2* | Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. | | | | | X |
| 32.1* | Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. | | | | | X |
| 32.2* | Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. | | | | | X |
| 101.INS | Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document. | | | | | |
| 101.SCH | Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents | | | | | |
| 104 | Cover Page Interactive Data File (embedded within the Inline XBRL document) | | | | | |

† The Registrant has omitted portions of the exhibit (indicated by "[*]") as permitted under Item 601(b)(10) of Regulation S-K.

* This certification is deemed not filed for purposes of Section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act or the Exchange Act.

**SIGNATURES**

Pursuant to the requirements 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.

**Scribe Therapeutics Inc.**

Date: September 2, 2026

By: /s/ Benjamin L. Oakes

Benjamin L. Oakes, Ph.D.
*President, Chief Executive Officer and Director*
*(Principal Executive Officer)*

Date: September 2, 2026

By: /s/ David L. Parrot

David L. Parrot
*Chief Financial Officer*
*(Principal Financial and Accounting Officer)*

**Exhibit 31.1**

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Benjamin L. Oakes, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scribe Therapeutics Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 2, 2026

By: /s/ Benjamin L. Oakes

Benjamin L. Oakes, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

**Exhibit 31.2**

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, David L. Parrot, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scribe Therapeutics Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 2, 2026

By: /s/ David L. Parrot
David L. Parrot
Chief Financial Officer
(Principal Financial Officer)

**Exhibit 32.1**

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report of Scribe Therapeutics Inc. (the "Company") on Form 10-Q for the three months ending June 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Benjamin L. Oakes, Chief Executive Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: September 2, 2026

By: /s/ Benjamin L. Oakes
Benjamin L. Oakes
Chief Executive Officer
(Principal Executive Officer)

**Exhibit 32.2**

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report of Scribe Therapeutics Inc. (the "Company") on Form 10-Q for the three months ending June 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, David L. Parrot, Chief Financial Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: September 2, 2026

By: /s/ David L. Parrot
David L. Parrot
Chief Financial Officer
(Principal Financial Officer)