UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 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 September 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 001-39402

ANNEXON, INC.

(Exact name of Registrant as specified in its Charter)

Delaware	**27-5414423**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

1400 Sierra Point Parkway, Bldg C, Suite 200
Brisbane, California 94005
(Address of principal executive offices including zip code)

Registrant's telephone number, including area code: (650) 822-5500

Former name, former address and former fiscal year, if changed since last report: Not applicable

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	ANNX	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, 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 ☒

The number of shares of the Registrant's Common Stock outstanding as of November 5, 2026 was 190,763,984. This number does not include 14,745,093 shares of Common Stock issuable upon the exercise of pre-funded warrants (which are immediately exercisable at an exercise price of $0.001 per share of Common Stock, subject to beneficial ownership limitations). See Note 7—*Stockholders' Equity* to the Registrant's unaudited condensed consolidated financial statements.

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In this Quarterly Report on Form 10-Q, "we," "our," "us," "Annexon" and the "Company" refer to Annexon, Inc. and its consolidated subsidiary. Annexon, Annexon, Inc., the Annexon logo and other trade names, trademarks or service marks of Annexon are the property of Annexon, Inc. This report contains references to our trademarks and to trademarks belonging to other entities. Trade names, trademarks and service marks of other companies appearing in this report are the property of their respective holders. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

ANNEXON, INC.
Condensed Consolidated Balance Sheets
(in thousands)

	September 30, 2026 (Unaudited)	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 102,389	$ 162,051
Short-term investments	110,985	76,294
Prepaid expenses and other current assets	4,525	3,846
Total current assets	217,899	242,191
Restricted cash	1,032	1,032
Property and equipment, net	9,165	10,617
Operating lease right-of-use assets	13,882	15,185
Other non-current assets	8,430	8,546
Total assets	$ 250,408	$ 277,571
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 15,910	$ 14,931
Accrued and other current liabilities	18,512	24,791
Operating lease liabilities, current	3,238	2,908
Total current liabilities	37,660	42,630
Term loan, non-current	49,340	—
Operating lease liabilities, non-current	20,613	23,293
Total liabilities	107,613	65,923
Commitments and contingencies (Note 6)		
Stockholders' equity:		
Common stock	190	149
Additional paid-in capital	1,210,856	1,128,917
Accumulated other comprehensive loss	(154)	(29)
Accumulated deficit	(1,068,097)	(917,389)
Total stockholders' equity	142,795	211,648
Total liabilities and stockholders' equity	$ 250,408	$ 277,571

See accompanying notes to unaudited condensed consolidated financial statements.

ANNEXON, INC.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 41,368	$ 49,700	$ 123,744	$ 142,039
General and administrative	10,870	7,318	31,774	24,110
Total operating expenses	52,238	57,018	155,518	166,149
Loss from operations	(52,238)	(57,018)	(155,518)	(166,149)
Interest expense	(833)	—	(833)	—
Interest and other income, net	1,853	2,096	5,643	7,715
Net loss	(51,218)	(54,922)	(150,708)	(158,434)
Deemed dividend on modification of common stock warrants	—	—	—	(1,857)
Net loss attributable to common stockholders	$ (51,218)	$ (54,922)	$ (150,708)	$ (160,291)
Net loss per share, basic and diluted	$ (0.25)	$ (0.37)	$ (0.75)	$ (1.08)
Weighted-average shares used in computing net loss per share, basic and diluted	204,652,743	149,123,701	199,990,325	148,521,489

See accompanying notes to unaudited condensed consolidated financial statements.

ANNEXON, INC.
Condensed Consolidated Statements of Comprehensive Loss
(in thousands)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Net loss	$ (51,218)	$ (54,922)	$ (150,708)	$ (158,434)
Other comprehensive loss:				
Foreign currency translation adjustment	—	—	1	2
Unrealized (loss) gain on available-for-sale securities	(72)	17	(126)	(88)
Comprehensive loss	$ (51,290)	$ (54,905)	$ (150,833)	$ (158,520)

See accompanying notes to unaudited condensed consolidated financial statements.

ANNEXON, INC.
Condensed Consolidated Statements of Stockholders' Equity
(in thousands, except share amounts)
(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Cost				
Balances as of December 31, 2025	149,362,800	$ 149	$ 1,128,917	$ (29)	$ (917,389)	$ 211,648
Exercise of stock options	61,306	—	229	—	—	229
Restricted stock vested in the period	501,341	—	—	—	—	—
Issuance of common stock, net of issuance costs $909	6,049,762	6	32,688	—	—	32,694
Exercise of pre-funded warrants	6,526,508	7	(7)	—	—	—
Stock-based compensation	—	—	4,156	—	—	4,156
Other comprehensive loss	—	—	—	(52)	—	(52)
Net loss	—	—	—	—	(44,142)	(44,142)
Balances as of March 31, 2026	162,501,717	162	1,165,983	(81)	(961,531)	204,533
Exercise of stock options	179,664	1	563	—	—	564
Issuance of common stock, net of issuance costs $1,150	5,734,361	6	31,007	—	—	31,013
Exercise of pre-funded warrants	5,998,871	6	(6)	—	—	—
Issuance of common stock per Employee Stock Purchase Plan purchase	189,585	—	487	—	—	487
Stock-based compensation	—	—	4,813	—	—	4,813
Other comprehensive loss	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(55,348)	(55,348)
Balances as of June 30, 2026	174,604,198	175	1,202,847	(82)	(1,016,879)	186,061
Exercise of stock options	105,137	—	398	—	—	398
Issuance of common stock, net of issuance costs $103	485,754	—	2,516	—	—	2,516
Exercise of pre-funded warrants	14,897,630	15	(15)	—	—	—
Stock-based compensation	—	—	5,110	—	—	5,110
Other comprehensive loss	—	—	—	(72)	—	(72)
Net loss	—	—	—	—	(51,218)	(51,218)
Balances as of September 30, 2026	190,092,719	$ 190	$ 1,210,856	$ (154)	$ (1,068,097)	$ 142,795

See accompanying notes to unaudited condensed consolidated financial statements.

ANNEXON, INC.
Condensed Consolidated Statements of Stockholders' Equity
(in thousands, except share amounts)
(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Cost				
Balances as of December 31, 2024	109,381,556	$ 109	$ 1,003,685	$ 10	$ (710,699)	$ 293,105
Exercise of stock options	43,113	—	62	—	—	62
Restricted stock vested in the period	289,735	—	—	—	—	—
Stock-based compensation	—	—	5,078	—	—	5,078
Other comprehensive loss	—	—	—	(101)	—	(101)
Net loss	—	—	—	—	(54,356)	(54,356)
Balances as of March 31, 2025	109,714,404	109	1,008,825	(91)	(765,055)	243,788
Issuance of common stock per Employee Stock Purchase Plan purchase	117,743	1	181	—	—	182
Stock-based compensation	—	—	4,205	—	—	4,205
Other comprehensive loss	—	—	—	(2)	—	(2)
Net loss	—	—	—	—	(49,156)	(49,156)
Balances as of June 30, 2025	109,832,147	110	1,013,211	(93)	(814,211)	199,017
Exercise of stock options	1,289	—	3	—	—	3
Restricted stock vested in the period	53,187	—	—	—	—	—
Issuance of common stock, net of issuance costs $697	5,235,959	5	13,487	—	—	13,492
Stock-based compensation	—	—	3,835	—	—	3,835
Other comprehensive income	—	—	—	17	—	17
Net loss	—	—	—	—	(54,922)	(54,922)
Balances as of September 30, 2025	115,122,582	$ 115	$ 1,030,536	$ (76)	$ (869,133)	$ 161,442

See accompanying notes to unaudited condensed consolidated financial statements.

ANNEXON, INC.
Condensed Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2026	**2025**
Operating activities:		
Net loss	$ (150,708)	$ (158,434)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,604	1,623
Accretion of discount on available-for-sale securities	(1,479)	(3,581)
Non-cash interest expense	132	—
Stock-based compensation	14,079	13,118
Reduction in the carrying amount of right-of-use assets	1,303	1,118
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(679)	343
Other non-current assets	116	(5,312)
Accounts payable	778	7,756
Accrued and other current liabilities	(6,279)	4,973
Operating lease liabilities	(2,350)	(2,034)
Net cash used in operating activities	(143,483)	(140,430)
Investing activities:		
Purchases of property and equipment	(137)	(137)
Purchases of available-for-sale securities	(126,338)	(147,190)
Proceeds from maturities of available-for-sale securities	93,000	363,900
Net cash (used in) provided by investing activities	(33,475)	216,573
Financing activities:		
Proceeds from the exercise of common stock options	1,191	65
Proceeds from Employee Stock Purchase Plan purchases	487	182
Proceeds from the issuance of common stock	68,385	14,189
Proceeds from the issuance of term loan, net of debt discount	49,387	—
Payment of financing costs	(2,155)	(660)
Net cash provided by financing activities	117,295	13,776
(Decrease) increase in cash, cash equivalents and restricted cash	(59,663)	89,919
Effect of exchange rate changes on cash, cash equivalents and restricted cash	1	2
Cash, cash equivalents and restricted cash		
Beginning of period	163,083	50,530
End of period	$ 103,421	$ 140,451
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 356	$ —
Cash paid for amounts included in the measurement of lease liability	$ 3,909	$ 2,518
Non-cash investing and financing activities:		
Exercise of pre-funded warrants	$ 28	$ —
Debt issuance costs included in accounts payable and accrued liabilities	$ 179	$ —
Deemed dividend on modification of common stock warrants	$ —	$ 1,857

See accompanying notes to unaudited condensed consolidated financial statements.

1. Organization

Annexon, Inc., or the Company, is a biopharmaceutical company advancing the next generation platform of targeted immunotherapies aimed at complement-mediated neuroinflammatory diseases that collectively impact nearly 10 million people worldwide. The Company is located in Brisbane, California and was incorporated in Delaware in March 2011.

The Company's wholly-owned subsidiary, Annexon Biosciences Australia Pty Ltd, or the Subsidiary, is a proprietary limited company incorporated in 2016 and domiciled in Australia.

Liquidity

Since inception, the Company has been involved primarily in performing research and development activities, conducting clinical trials, hiring personnel, and raising capital to support and expand these activities. The Company has experienced losses and negative cash flows from operations since its inception and, as of September 30, 2026, had an accumulated deficit of approximately $1.1 billion and cash and cash equivalents and short-term investments of $213.4 million.

The Company has historically funded its operations through the issuance of shares of its common stock and warrants. On July 30, 2026, the Company also received $50.0 million in gross proceeds under a loan agreement with Oxford Finance LLC and Oxford Finance Credit Fund IV, LP and has up to $150.0 million of additional borrowing capacity, subject to specified milestones and other conditions, to support ongoing operations, collectively the Debt Financing. See Note 5—*Debt.* Based on projected activities, management projects that existing cash and cash equivalents and short-term investments will enable the Company to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these financial statements. The Company's future viability beyond that point is dependent on its ability to achieve development and regulatory milestones and obtain additional funding. Management expects to continue to incur losses and negative cash flows from operations for at least the next several years. There are uncertainties associated with the Company's ability to (1) obtain additional equity or debt financing on terms that are favorable to the Company, (2) enter into collaborative agreements with strategic partners, and (3) succeed in its future operations. If the Company is not able to obtain the required funding for its operations or is not able to obtain funding on terms that are favorable to the Company, it could be forced to delay, reduce or eliminate its research and development programs and its business could be materially harmed.

2. Basis of Presentation and Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP, and applicable rules and regulations of the Securities and Exchange Commission, or SEC, regarding interim financial reporting.

The condensed consolidated balance sheet as of September 30, 2026, the condensed consolidated statements of operations, comprehensive loss, and stockholders' equity for the three and nine months ended September 30, 2026 and 2025 and the condensed consolidated statements of cash flows for the nine months ended September 30, 2026 and 2025 are unaudited. These unaudited condensed consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's consolidated financial position, results of operations and cash flows for the interim period presented. The financial data and other financial information contained in these notes to the condensed consolidated financial statements related to the three month periods are also unaudited. The results of operations for the three and nine months ended September 30, 2026 are not necessarily indicative of the results to be expected for the year ending December 31, 2026 or for any other future annual or interim period. The condensed consolidated balance sheet as of December 31, 2025 included herein was derived from the audited consolidated financial statements as of that date. These unaudited condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025, filed with the SEC on March 30, 2026.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported expenses during the reporting period. Management evaluates its estimates, including but not limited to the fair value of

investments, incremental borrowing rate for operating lease right-of-use assets and liabilities, valuation of deferred tax assets and uncertain tax positions, clinical trial accruals and stock-based compensation. The Company evaluates its estimates and assumptions on an ongoing basis using historical experience and other factors and adjusts those estimates and assumptions when facts and circumstances dictate. Actual results could differ from those estimates.

Principles of Consolidation

The condensed consolidated financial statements include the operations of Annexon, Inc. and its wholly-owned subsidiary and include the results of operations and cash flows of these entities. All intercompany balances and transactions have been eliminated in consolidation.

Summary of Significant Accounting Policies

Reference is made to Note 2—*Summary of Significant Accounting Policies*, in the Company's 2025 Form 10-K filed on March 30, 2026 for a detailed description of significant accounting policies. Except for the policy on debt issuance costs, debt discounts, and final payment described below, there have been no significant changes to the Company's accounting policies as disclosed in its 2025 Form 10-K.

Debt Issuance Costs, Debt Discounts, and Final Payment

Debt issuance costs include legal fees and other direct costs incurred in connection with the execution of the Company's Debt Financing. Debt discounts represent costs paid to the lenders. Debt issuance costs and debt discounts are deducted from the carrying amount of the debt liability and are amortized to interest expense over the term of the related debt using the effective interest method. Final payment obligations represent amounts due to lenders upon repayment or maturity of the related debt and are accreted to interest expense over the term of the related debt using the effective interest method.

Recently Issued Accounting Pronouncements

In November 2024, the FASB issued ASU 2024-03, *Income Statement-Reporting Comprehensive Income-Expense Disaggregation Disclosures: Disaggregation of Income Statement Expenses*, which requires public entities to disclose additional information about specific expense categories in the notes to the financial statements on an interim and annual basis. This guidance is effective for annual reporting periods beginning after December 15, 2026, and interim periods beginning after December 15, 2027. Early adoption is permitted. The Company is currently evaluating the impact of this guidance on its consolidated financial statements.

3. Fair Value Measurements

The Company utilizes valuation techniques that maximize the use of observable inputs and minimize the use of unobservable inputs to the extent possible. The Company determines fair value based on assumptions that market participants would use in pricing an asset or liability in the principal or most advantageous market. When considering market participant assumptions in fair value measurements, the following fair value hierarchy distinguishes between observable and unobservable inputs, which are categorized in one of the following levels:

- *Level 1 Inputs:* Unadjusted quoted prices in active markets for identical assets or liabilities accessible to the reporting entity at the measurement date.
- *Level 2 Inputs:* Other than quoted prices included in Level 1 inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the asset or liability.
- *Level 3 Inputs:* Unobservable inputs for the asset or liability used to measure fair value to the extent that observable inputs are not available, thereby allowing for situations in which there is little, if any, market activity for the asset or liability at measurement date.

On a recurring basis, the Company measures certain financial assets and liabilities at fair value. The following tables summarize the fair value of the Company's financial assets measured at fair value on a recurring basis by level within the fair value hierarchy (in thousands):

		September 30, 2026			
	Valuation Hierarchy	Amortized Cost Basis	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value
Assets:					
Cash equivalents:					
Money market funds	Level 1	$ 52,710	$ —	$ —	$ 52,710
Government bonds	Level 2	40,913	—	(3)	40,910
Total cash equivalents		93,623	—	(3)	93,620
Short-term investments:					
Government bonds	Level 2	111,055	1	(71)	110,985
Total short-term investments		111,055	1	(71)	110,985
		$ 204,678	$ 1	$ (74)	$ 204,605

		December 31, 2025			
	Valuation Hierarchy	Amortized Cost Basis	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value
Assets:					
Cash equivalents:					
Money market funds	Level 1	$ 116,181	$ —	$ —	$ 116,181
Government bonds	Level 2	43,844	13	—	43,857
Total cash equivalents		160,025	13	—	160,038
Short-term investments:					
Government bonds	Level 2	76,252	42	—	76,294
Total short-term investments		76,252	42	—	76,294
		$ 236,277	$ 55	$ —	$ 236,332

All of the investments held as of September 30, 2026, had original maturities of less than two years. As of September 30, 2026, all of the investments are scheduled to mature within 12 months. During the three and nine months ended September 30, 2026, the Company did not recognize any credit losses. The Company determined that the decline in fair value of debt securities was not due to credit-related factors, and no allowance for expected credit losses was recorded as of September 30, 2026. There were nominal unrealized losses as of September 30, 2026, and no investments have been in the loss position for more than 12 months. However, the Company is planning to hold these securities until maturity and expects to recover the amortized cost basis.

For the three and nine months ended September 30, 2026 and 2025, the Company recognized no material realized gains or losses on financial instruments.

The Company's term loan is not measured at fair value on a recurring basis. As of September 30, 2026, the Company's term loan of $49.3 million approximates fair value using Level 2 inputs, as the debt bears interest at a floating interest rate.

4. Balance Sheet Components

Cash, Cash Equivalents and Restricted Cash

The Company considers all highly liquid instruments with an original maturity of three months or less at time of purchase to be cash equivalents. Cash equivalents, which include amounts invested in money market funds and government bonds, are stated at fair value.

Restricted cash as of September 30, 2026 relates to the letters of credit established for the Company's office leases.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the unaudited condensed consolidated balance sheets that sum to the total of the same amounts shown in the unaudited condensed consolidated statements of cash flows (in thousands):

	September 30, 2026	December 31, 2025
Cash	$ 8,769	$ 2,013
Cash equivalents	93,620	160,038
Cash and cash equivalents	102,389	162,051
Restricted cash	1,032	1,032
Cash, cash equivalents and restricted cash	$ 103,421	$ 163,083

Property and Equipment, Net

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

	September 30, 2026	December 31, 2025
Leasehold improvements	$ 17,256	$ 17,262
Laboratory equipment	1,928	1,878
Furniture and fixtures	820	730
Computer equipment and software	110	92
Total property and equipment, gross	20,114	19,962
Less: accumulated depreciation	(10,949)	(9,345)
Total property and equipment, net	$ 9,165	$ 10,617

Accrued and Other Current Liabilities

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

	September 30, 2026	December 31, 2025
Accrued research and development expenses	$ 11,492	$ 16,285
Accrued compensation	5,750	7,517
Accrued professional services	656	480
Other accrued and current liabilities	614	509
Total accrued and other current liabilities	$ 18,512	$ 24,791

5. Debt

On July 30, 2026, the Company entered into a Loan and Security Agreement, or Oxford Loan Agreement, with Oxford Finance LLC, as collateral agent, and Oxford Finance Credit Fund IV, LP, or collectively, Oxford. The Oxford Loan Agreement provides for term loans of up to an aggregate principal amount of $200.0 million to be funded in multiple tranches. The tranches include (i) a $50.0 million Term A Loan funded on July 30, 2026, (ii) a Term B Loan of up to $25.0 million available through September 30, 2027 upon achievement of a specified clinical milestone, (iii) a Term C Loan of up to $25.0 million, plus up to $15.0 million of unused Term B Loan, available through December 31, 2027 upon receipt of specified regulatory approval, (iv) a Term D Loan of up to $50.0 million available through December 31, 2028 upon receipt of specified regulatory approval, and (v) an additional discretionary Term E Loan of up to $50.0 million available at the Company's request and subject to Oxford's approval. As of September 30, 2026, $150.0 million of borrowing capacity remained available under the Oxford Loan Agreement.

Outstanding borrowings under the Oxford Loan Agreement bear interest at a floating rate equal to the greater of (i) the 1-Month CME Term SOFR, or (ii) 3.00%, plus 4.60%, subject to a minimum interest rate of 7.60%. The Company is required to make monthly interest-only payments until September 1, 2029, or the Amortization Date. Beginning on the Amortization Date, principal is also payable in equal monthly installments through the maturity date of July 1, 2031. Upon achievement of specified clinical and regulatory

milestones, the Amortization Date may be extended to September 1, 2031, with a corresponding extension of the maturity date to July 1, 2032.

Upon the earliest to occur of (i) the maturity date, (ii) the acceleration of any tranche, or (iii) prepayment of any tranche, the Company will be required to make a final payment ranging from 2.5% to 6.75% of the original principal amount, depending on the applicable tranche and timing of repayment. The Company has the option to prepay all or a portion of the outstanding borrowings prior to the maturity date, subject to a prepayment fee ranging from 1.0% to 2.0%, depending on the timing of the prepayment. As of September 30, 2026, the applicable final payment on the outstanding Term A Loan was 5.0% of the original principal amount, or approximately $2.5 million.

As of September 30, 2026, the estimated future principal payments and final fee payments are due as follows (in thousands):

	(in thousands)
2026 (remaining three months)	$ —
2027	—
2028	—
2029	8,696
2030	26,087
2031	17,717
Total	52,500
Less: unamortized debt discount, issuance costs and other debt related adjustments	(3,160)
Total term loan, non-current	$ 49,340

All obligations under the Oxford Loan Agreement are secured by a first-priority perfected lien on, and security interest in, substantially all present and future assets of the Company. The Oxford Loan Agreement includes customary affirmative and negative covenants, including restrictions on the ability to pay dividends and make other distributions. In addition, the Oxford Loan Agreement contains events of default, including the occurrence of a material adverse change, that may permit the lenders to accelerate the outstanding amounts. The Oxford Loan Agreement also includes minimum cash and minimum revenue covenants that become applicable upon specified dates or triggering events. As of September 30, 2026, the minimum cash and minimum revenue covenants were not yet applicable.

Interest expense under the Oxford Loan Agreement is calculated using the effective interest method and includes noncash amortization of the debt discount and debt issuance costs and accretion of the final payment over the life of the term loan at an effective interest rate of 9.6%. During the three and nine months ended September 30, 2026, interest expense was $0.8 million.

6. Commitments and Contingencies

Leases

The Company leases its offices and laboratory in Brisbane, California, under a ten-year noncancelable lease agreement that ends in October 2031 with a ten-year renewable option.

As of September 30, 2026, the operating lease right-of-use assets were $13.9 million and lease liabilities were $23.9 million on the unaudited condensed consolidated balance sheet. The weighted-average remaining lease term is 5.1 years.

The weighted-average incremental borrowing rate used to measure the operating lease liability is 8.4%.

Operating lease costs were $1.0 million for each of the three months ended September 30, 2026 and 2025, and $2.9 million for each of the nine months ended September 30, 2026 and 2025. Variable lease payments were $0.1 million and $0.6 million for the three months ended September 30, 2026 and 2025, respectively, and $1.2 million and $2.0 million for the nine months ended September 30, 2026 and 2025, respectively.

Future minimum lease payments and related lease liabilities as of September 30, 2026, were as follows:

	(in thousands)
2026 (remaining three months)	$ 1,333
2027	5,425
2028	5,615
2029	5,812
2030 and thereafter	11,173
Total undiscounted lease payments	29,358
Less: Imputed interest	(5,507)
Total lease liabilities	$ 23,851

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. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. As of September 30, 2026, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded related liabilities.

7. Stockholders' Equity

Pre-Funded and Common Warrants

The Company issued pre-funded warrants in connection with public offerings of common stock in July 2022, December 2023, June 2024 and November 2025. Each pre-funded warrant has an exercise price of $0.001 per share, does not expire and is immediately exercisable subject to beneficial ownership limitations. The pre-funded warrants are classified as equity.

During the nine months ended September 30, 2026, the Company issued an aggregate of 27,423,009 shares of common stock upon the cashless exercise of pre-funded warrants to purchase 27,427,778 shares of common stock.

The Company issued common warrants to purchase up to 8,427,508 shares of common stock in connection with an equity offering in July 2022. The common warrants had an exercise price of $5.807 per share and an original expiration date of June 30, 2025. In June 2025, the Company and holders of common warrants to purchase 6,877,622 shares agreed to extend the expiration date to June 30, 2026 and common warrants to purchase the remaining 1,226,993 shares expired unexercised on June 30, 2025. The Company recorded the modification as a $1.9 million deemed dividend, which increased net loss attributable to common stockholders for the nine months ended September 30, 2025. On June 30, 2026, the amended common warrants to purchase 6,877,622 shares expired unexercised. No common warrants remained outstanding as of September 30, 2026.

The following summarizes warrant activity during the nine months ended September 30, 2026 and 2025:

	Number of Common Warrants	Number of Pre-funded Warrants	Weighted-Average Exercise Price
Balances as of December 31, 2025	6,877,622	42,293,577	
Issued	—	—	$ —
Exercised	—	(27,427,778)	$ 0.001
Expired	(6,877,622)	—	$ 5.807
Balances as of September 30, 2026	—	14,865,799	

	Number of Common Warrants	Number of Pre-funded Warrants	Weighted-Average Exercise Price
Balances as of December 31, 2024	8,104,615	38,543,577	
Issued	—	—	$ —
Exercised	—	—	$ —
Expired	(1,226,993)	—	$ 5.807
Balances as of September 30, 2025	6,877,622	38,543,577	

Subsequent to September 30, 2026 and through the date of issuance of these interim unaudited condensed consolidated financial statements, the Company issued an aggregate of 120,677 shares of common stock upon the cashless exercise of pre-funded warrants to purchase 120,706 shares of common stock.

At the Market (ATM) Program

In March 2026, the Company entered into a sales agreement with TD Securities (USA) LLC, or TD Cowen, as sales agent, pursuant to which the Company may offer and sell, from time to time through TD Cowen, at its option, shares of its common stock having an aggregate offering price of up to $150.0 million, or the 2026 ATM program. The Company agreed to pay TD Cowen a commission of up to 3.0% of the aggregate gross proceeds for the common stock sold through the 2026 ATM program. During the nine months ended September 30, 2026, the Company sold 6,220,115 shares of common stock under the 2026 ATM program for net proceeds of approximately $33.5 million, after deducting commissions paid to TD Cowen and other offering costs. As of September 30, 2026, approximately $115.2 million remained available under the 2026 ATM program. Subsequent to September 30, 2026 and through the date of issuance of these interim unaudited condensed consolidated financial statements, the Company sold 639,068 shares of common stock under the 2026 ATM program for net proceeds of approximately $2.7 million, after deducting commissions paid to TD Cowen.

In March 2024, the Company entered into a sales agreement with TD Cowen, as sales agent, pursuant to which the Company was permitted to offer and sell shares of its common stock having an aggregate maximum offering price of up to $100.0 million, or 2024 ATM program. During the nine months ended September 30, 2026 and 2025, the Company sold 6,049,762 and 5,235,959 shares of its common stock under the 2024 ATM program for net proceeds of approximately $32.7 million and $13.5 million, respectively, after deducting commissions paid to TD Cowen and other offering costs. In May 2026, the Company terminated the 2024 ATM program. Approximately $33.3 million remained available under the 2024 ATM program at the time of termination; however, no further shares of common stock may be sold under the 2024 ATM program.

Common Stock

The Company reserved the following shares of common stock for issuance as follows:

	September 30, 2026	December 31, 2025
Stock options issued and outstanding	23,487,204	18,645,325
Stock options reserved for 2020 Incentive Award Plan	1,535,659	1,184,877
Unvested restricted stock units outstanding	2,318,396	1,252,847
Common stock reserved for 2024 ATM program	—	14,509,176
Common stock reserved for 2026 ATM program	23,779,885	—
Common stock reserved for Employee Stock Purchase Plan	4,104,593	2,800,550
Common stock reserved for 2022 Employment Inducement Award Plan	2,444,057	3,585,793
Common stock reserved for pre-funded warrants	14,865,799	42,293,577
Common stock reserved for common warrants	—	6,877,622
Total common stock reserved	72,535,593	91,149,767

8. Equity Incentive Plans

In July 2020, the Company's board of directors and stockholders adopted and approved the 2020 Incentive Award Plan, or the 2020 Plan, and the Employee Stock Purchase Plan, or the ESPP, which became effective in connection with the Company's initial public offering, or the IPO.

The Company may not grant any additional awards under the 2011 Equity Incentive Plan, or the 2011 Plan. The 2011 Plan will continue to govern outstanding equity awards granted thereunder.

2020 Incentive Award Plan

The number of shares of common stock reserved for issuance under the 2020 Plan automatically increases on the first day of January, in an amount equal to 4% of the total number of shares of the Company's capital stock outstanding on the last day of the preceding year, or a lesser number of shares determined by the Company's board of directors.

Awards granted under the 2020 Plan expire no later than ten years from the date of grant. For Incentive Stock Options, or ISOs, and Nonstatutory Stock Options, or NSOs, the option price shall not be less than 100% of the estimated fair value on the date of grant. Options granted typically vest over a four-year period but may be granted with different vesting terms. As of September 30, 2026, there were 1,535,659 shares available for issuance under the 2020 Plan.

2022 Employment Inducement Award Plan

In July 2022, the Company's board of directors adopted the Annexon, Inc. 2022 Employment Inducement Award Plan, as amended, or the Inducement Plan, and together with the 2011 Plan and the 2020 Plan, the Plans. The Inducement Plan was adopted by the Company's board of directors without stockholder approval pursuant to Nasdaq Marketplace Rule 5635(c)(4), or Rule 5635(c)(4). In accordance with Rule 5635(c)(4), awards made under the Inducement Plan may only be granted to newly hired employees as an inducement material to the employees entering into employment with the Company. Awards granted under the Inducement Plan expire no later than ten years from the date of grant. An aggregate of 7,850,000 shares of common stock were reserved for issuance under the Inducement Plan. As of September 30, 2026, there were 2,444,057 shares available for issuance under the Inducement Plan.

Stock Options

The following table presents stock option activity under the Plans for the period:

	Number of Shares	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Balances as of December 31, 2025	18,645,325	$ 6.69	7.53	$ 17,234
Stock options granted	6,610,313	$ 5.17		
Stock options exercised	(346,960)	$ 3.44		
Stock options forfeited	(1,421,474)	$ 5.84		
Balances as of September 30, 2026	23,487,204	$ 6.36	7.32	$ 10,257
Vested and Exercisable as of September 30, 2026	12,869,471	$ 7.95	6.06	$ 4,871

The total intrinsic value of options exercised was $0.7 million and $0.1 million for the nine months ended September 30, 2026, and 2025, respectively. The intrinsic value is the difference between the fair value of the Company's common stock at the time of exercise and the exercise price of the stock option.

The weighted-average grant date fair value of options granted to employees during the nine months ended September 30, 2026 and 2025 was $3.97 and $1.97 per share, respectively.

As of September 30, 2026, the total unrecognized stock-based compensation expense related to outstanding unvested stock options was $35.1 million, which the Company expects to recognize over an estimated weighted-average period of 2.8 years.

Restricted Stock Units

RSUs are share awards that entitle the holder to receive freely tradeable shares of the Company's common stock upon vesting. The RSUs cannot be transferred and the awards are subject to forfeiture if the holder's employment terminates prior to the release of the vesting restrictions. The RSUs generally vest over a three-year period in equal amounts on an annual basis, provided the employee remains continuously employed with the Company. The fair value of the RSUs is equal to the closing price of the Company's common stock on the grant date.

A summary of RSU activity under the Company's equity incentive plan and related information is as follows:

	Number of Shares	Weighted-Average Grant Date Fair Value Per Share
Unvested as of December 31, 2025	1,252,847	$ 3.31
Granted	1,855,563	5.38
Vested	(501,341)	3.69
Cancelled	(288,673)	4.65
Unvested as of September 30, 2026	2,318,396	$ 4.72

As of September 30, 2026, unrecognized stock-based compensation expense related to outstanding unvested RSUs was $8.3 million, which is expected to be recognized over a weighted-average period of 2.2 years.

Employee Stock Purchase Plan

The ESPP enables eligible employees to purchase shares of the Company's common stock at the end of each offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower. Eligible employees generally include all employees. Share purchases are funded through payroll deductions of at least 1%, and up to 15% of an employee's eligible compensation for each payroll period. The number of shares reserved for issuance under the ESPP increases automatically on the first day of each fiscal year, by a number equal to, 1% of the shares of common stock outstanding on the last day of the immediately preceding fiscal year, or such number of shares determined by the Company's board of directors. As of September 30, 2026, 4,104,593 shares were available for future purchase. The ESPP generally provides for six-month consecutive offering periods beginning on May 16 and November 16 of each year. The ESPP is a compensatory plan as defined by the authoritative guidance for stock compensation. As such, stock-based compensation expense has been recorded for the three and nine months ended September 30, 2026.

The stock-based compensation expense related to the ESPP was $0.1 million for each of the nine months ended September 30, 2026 and 2025.

Stock-Based Compensation Expense

The total stock-based compensation expense recognized was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 2,630	$ 2,380	$ 7,661	$ 7,897
General and administrative	2,480	1,455	6,418	5,221
Total stock-based compensation expense	$ 5,110	$ 3,835	$ 14,079	$ 13,118

To determine the value of stock option awards for stock-based compensation purposes, the Company uses the Black-Scholes option pricing model and the assumptions discussed below. Each of these inputs is subjective and generally requires significant judgment.

Fair Value of Common Stock—The fair value of each share of underlying common stock is based on the closing price of the Company's common stock as reported on the date of grant on the Nasdaq Global Select Market.

Expected Term—The expected term represents the period that the stock-based awards are expected to be outstanding and is determined using the simplified method. The Company continues using the simplified method as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term due to the limited period of time its equity shares have been publicly traded.

Expected Volatility—Beginning in 2026, the expected volatility assumption is estimated based solely on the historical volatility of the Company's share trading price for its common stock and is no longer derived from a weighted average that includes comparable publicly traded life sciences companies. Prior to 2026, the expected volatility was estimated based on a weighted volatility using both

the Company's trading history for its common stock and the average volatility for comparable publicly traded life sciences companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on similar size, stage in life cycle, or area of specialty.

Risk-Free Interest Rate—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of the option.

Dividend Yield—The Company has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

The fair value of each stock option issued was estimated on the date of grant using the Black-Scholes option pricing model with the following assumptions:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Expected term (in years)	5.77 - 6.08	6.08	5.50 - 6.08	5.50 - 6.08
Expected volatility	89.60% - 90.70%	93.30%	89.60% - 92.30%	92.30% - 93.80%
Risk-free interest rate	4.32% - 4.87%	3.95%	3.72% - 4.87%	3.95% - 4.49%
Dividend yield	—	—	—	—

9. Net Loss Per Share

The Company calculates basic net loss per share by dividing net loss by the weighted-average number of shares of common stock outstanding, excluding restricted common stock. The weighted-average number of shares of common stock used in the basic and diluted net loss per share calculation include pre-funded warrants to purchase up to 14,865,799 shares of common stock, as the pre-funded warrants are exercisable at any time for nominal cash consideration. The Company has generated a net loss in all periods presented, so the basic and diluted net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

The following outstanding potentially dilutive shares have been excluded from the calculation of diluted net loss per share due to their anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Stock options to purchase common stock	23,487,204	19,381,754	23,487,204	19,381,754
Shares subject to Employee Stock Purchase Plan	58,339	183,953	58,339	183,953
Unvested restricted stock units	2,318,396	1,308,942	2,318,396	1,308,942
Common warrants	—	6,877,622	—	6,877,622
Total	25,863,939	27,752,271	25,863,939	27,752,271

10. Segment Reporting

The Company has one reportable segment, which is related to the research and development of its product candidates focused on complement-mediated diseases of the body, brain and eye for which there is significant unmet medical need. The accounting policies of the one reportable segment are the same as those described in the summary of significant accounting policies. See Note 2—*Basis of Presentation and Significant Accounting Policies.*

The Company's chief operating decision maker, or CODM, is its Chief Executive Officer. The segment is managed on a consolidated basis and the CODM uses total operating expenses and consolidated net loss to assess performance, forecast future financial results and allocate resources. In assessing the Company's financial performance and making strategic decisions, the CODM regularly

reviews operating expenses by function. This includes a review of budget versus actual expenses and direct program spend, which includes clinical costs, consultant fees, manufacturing expenses, and other direct external costs.

The following table presents the operations for the reportable segment for the three and nine months ended September 30, 2026 and 2025 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development - external expenses[1]	$ 29,964	$ 37,733	$ 88,632	$ 103,509
Research and development - personnel	6,919	7,916	21,281	25,338
General and administrative - personnel	2,804	2,193	8,407	6,686
Other general and administrative expenses[2]	6,916	4,795	21,515	15,875
Depreciation expense	525	546	1,604	1,623
Stock-based compensation	5,110	3,835	14,079	13,118
Total operating expense	52,238	57,018	155,518	166,149
Loss from operations	(52,238)	(57,018)	(155,518)	(166,149)
Interest expense	(833)	—	(833)	—
Interest and other income, net	1,853	2,096	5,643	7,715
Consolidated segment net loss	$ (51,218)	$ (54,922)	$ (150,708)	$ (158,434)

(1) Research and development - external expenses include program expenses, other non-program specific expenses and other research expenses.
(2) Other general and administrative expenses include consulting and professional services fees for business development, legal, accounting, tax, and facilities costs.

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 unaudited condensed consolidated financial statements and the related notes and other financial information included elsewhere in this Quarterly Report on Form 10-Q and our consolidated financial statements and related notes thereto for the year ended December 31, 2025, included in our Annual Report on Form 10-K for the year ended December 31, 2025, filed with the Securities and Exchange Commission, or the SEC, on March 30, 2026.

In addition to historical financial information, this discussion and other parts of this report contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, based upon current expectations about us and our industry that involve substantial risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the section titled "Risk Factors" under Part II, Item 1A below. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "position," "potential," "predict," "seek," "should," "target," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms. Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause actual results and events to differ from those anticipated. 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, like all statements in this report, speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

Overview

We are a biopharmaceutical company advancing a next generation platform of targeted immunotherapies aimed at complement-mediated neuroinflammatory diseases that collectively impact nearly 10 million people worldwide. Building on more than a decade of expertise stopping acute and chronic neuroinflammation at its source, we have demonstrated robust target engagement in the body, brain and eye, and clinical proof of concept in multiple diseases.

We are advancing two late-stage registrational programs, tanruprubart toward our first approval in Guillain-Barré Syndrome, or GBS, and vonaprument toward pivotal data in dry age-related macular degeneration, or AMD, with geographic atrophy, or GA, as well as developing ANX1502, a novel oral small molecule for autoimmune conditions.

Tanruprubart

Tanruprubart is an investigational targeted immunotherapy delivered in a single infusion to rapidly halt aggressive neuroinflammation and damage in GBS, an acute, rare, neuromuscular emergency that annually affects ~150,000 people worldwide. There are currently no therapies approved by the FDA for GBS and no substantial evidence of effectiveness from the current standard of care. In the placebo-controlled Phase 3 trial, approximately 90% of GBS patients treated with tanruprubart improved by week 1 and more than twice as many treated patients achieved a normal state of health at week 26. Tanruprubart has consistently demonstrated rapid and sustained functional improvements across a comprehensive data package. We continue to engage with applicable EU and U.S. regulators to advance tanruprubart towards registration worldwide. Our Marketing Authorization Application, or MAA, filed with the European Medicines Agency, or EMA, in January 2026 for tanruprubart for the treatment of GBS is under review, and we continue to engage with EU regulators through the MAA review process. Tanruprubart has been granted orphan designation from the EMA. Tanruprubart has also been granted Fast Track and orphan drug designation for the treatment of GBS from the FDA.

The currently ongoing open-label U.S./Europe FORWARD study is designed to broaden Western experience with tanruprubart. Eighteen patients have been treated to date, representing a broad spectrum of disease from moderate to severe in both male and female patients ranging in age from 12 to 78 years old. Functional outcomes, complete target engagement and a generally well-tolerated safety profile were observed in FORWARD, consistent with Phase 3 and real-world evidence studies. FORWARD findings provide additional evidence of the consistency and reproducibility of tanruprubart treatment effect across a broad range of GBS patients with different disease neurotypes, severities, ages and geographies, which further strengthen our comprehensive data package for tanruprubart in GBS. We plan to engage with the FDA with the goal of reaching alignment on our submission of a biologics license application, or BLA, anticipated in the fourth quarter of 2026.

Vonaprument

Vonaprument is an investigational neuroprotective inhibitor of C1q and the classical complement cascade delivered intravitreally

for GA, a leading cause of blindness affecting more than eight million people worldwide. There are no approved therapies for GA targeting the preservation of vision. Vonaprument is the only investigational therapy in GA to show significant vision preservation on assessments of best corrected visual acuity, or BCVA, and low luminance visual acuity, demonstrating significant protection from vision loss in both normal and low light conditions, as well as significant preservation of central retinal photoreceptors necessary for visual acuity. In the Phase 2 ARCHER trial, vonaprument also reduced risk of 15-letter vision loss by more than 70%.

The global, sham-controlled, double-masked Phase 3 ARCHER II trial is ongoing in 659 patients with GA. ARCHER II is designed to demonstrate clinically meaningful vision protection at Months 15 and 24. Given the linear progression of vision loss, among other things, we believe longer follow-up provides greater potential for sustained treatment benefit and increased separation between vonaprument treatment and sham over time. ARCHER II enrolled a patient population similar to Phase 2, which showed consistent, dose-dependent vision protection across a broad spectrum of disease. Notably, ARCHER II is larger and longer in duration and better powered than the Phase 2, and we believe is well-positioned for success on the primary endpoint of BCVA 15-letter loss. Study execution reflects strong physician and patient engagement. All eligible patients have received at least 15 months of treatment with patient retention at 90%, and masked vision-loss events have tracked closely with projections.

We have established a global registration path with the FDA and EMA, which supports the potential of vonaprument to be the first treatment approved in both Europe and the U.S. for the protection of vision in patients with GA. The ARCHER II single overall study will be analyzed for EU registration and allocated into two pre-defined sub-studies that will be analyzed for U.S. registration in accordance with the FDA's two-trial recommendation. An independent Data Monitoring Committee (DMC) will assess the ARCHER II single study Month 15 primary endpoint in the fourth quarter of 2026. If the DMC recommends that the endpoint has been met, the DMC will evaluate the two prespecified sub-studies, with results expected in the first quarter of 2027. Alternatively, the DMC may recommend continuing the trial through the Month 24 primary endpoint analysis. ARCHER II completion, including Month 24 analyses, is expected in the third quarter of 2027. Upon completion of Month 24, all patients will have the option to receive monthly vonaprument treatment in the open-label extension (OLE) study, designed to evaluate the longer-term profile of vonaprument for inclusion in the label. Vonaprument is the first and only therapeutic candidate for the treatment of GA to receive Priority Medicine, or PRIME, designation by the EMA, which provides early and proactive support to developers of promising medicines that may offer a major therapeutic advantage over existing treatments or benefit to patients without treatment options. Vonaprument was also selected by the EMA for the Product Development Coordinator Pilot launched in July 2025 to help PRIME designation holders efficiently navigate regulatory interactions including expedited scientific advice, MAA submission readiness activities, and ad-hoc queries throughout the development program.

ANX1502

ANX1502 is a novel oral small molecule inhibiting the activated form of C1s, an enzyme carried by C1q to initiate the classical cascade, which we believe is first-in-kind and has the potential to offer the advantages of selective upstream classical complement inhibition with the convenience and flexibility of oral administration. In a Phase 1 single-ascending dose and multiple-ascending dose clinical trial in healthy volunteers designed to evaluate the safety, tolerability, PK and PD, ANX1502 was generally well tolerated across cohorts with no serious adverse events, achieved target levels of active drug and showed supportive impact on a PD biomarker of complement activity. We recently completed a POC study evaluating an enteric-coated tablet formulation of ANX1502 in nine patients with cold agglutinin disease, or CAD. No significant safety signals were observed. Drug activity, including observed reductions in disease biomarkers such as bilirubin and LDH, supports ANX1502's potential for further development. Formulation optimization is underway to improve absorption of the enteric-coated tablet prior to executing studies in clinically validated, potentially blockbuster diseases, including neuromuscular disorders such as generalized myasthenia gravis.

We were incorporated in March 2011 and commenced operations later that year. To date, we have focused primarily on performing research and development activities, hiring personnel and raising capital to support and expand these activities. We do not have any products approved for sale, and we have not generated any revenue from product sales. We have incurred net losses each year since our inception. Our net losses were $51.2 million and $54.9 million for the three months ended September 30, 2026 and 2025, respectively, and $150.7 million and $158.4 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, we had an accumulated deficit of approximately $1.1 billion and cash and cash equivalents and short-term investments of $213.4 million.

Components of Operating Results

Revenue

Our product candidates are not approved for commercial sale. We have not generated any revenue from sales of our product candidates and do not expect to do so in the foreseeable future and until we complete clinical development, submit regulatory filings and receive approvals from applicable regulatory bodies for such product candidates, if ever.

Operating Expenses

Research and Development

Research and development expenses account for a significant portion of our operating expenses. Research and development expenses consist primarily of direct and indirect costs incurred for the development of our product candidates.

Direct expenses include:

- preclinical and clinical outside service costs associated with discovery, preclinical and clinical testing of our product candidates;
- professional services agreements with third party contract organizations, investigative clinical trial sites and consultants that conduct research and development activities on our behalf;
- contract manufacturing costs to produce clinical trial materials and commercial materials to support our planned regulatory package submissions to FDA and other foreign regulatory agencies; and
- laboratory supplies and materials.

Indirect expenses include:

- compensation and personnel-related expenses (including stock-based compensation);
- allocated expenses for facilities and depreciation; and
- other indirect costs.

We record research and development expenses as incurred. Payments made to other entities are under agreements that are generally cancelable by us. Advance payments for goods or services to be received in future periods for use in research and development activities are deferred as prepaid expenses. The prepaid amounts are then expensed as the related services are performed. At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates.

We expect our future research and development expenses to vary from period to period as we pursue regulatory approval of our product candidates, continue to advance our product candidates through late-stage clinical trials, invest in capabilities to prepare for commercialization including manufacturing, and hire additional personnel to support our organization. The process of conducting the necessary clinical research, development and manufacturing to obtain regulatory approval is costly and time-consuming, and the successful development and approval of our product candidates is highly uncertain.

General and Administrative

General and administrative expenses consist primarily of compensation and personnel-related expenses (including stock-based compensation) for our personnel in executive, finance and other administrative functions. General and administrative expenses also include professional fees paid for accounting, legal and tax services, allocated expenses for facilities and depreciation and other general and administrative costs.

We expect our general and administrative expenses to vary from period to period as we continue to support our research and development activities, grow our business, advance our product candidates in late-stage clinical trials and toward regulatory approval and commercialization activities, and operate as a public company.

Interest Expense

Interest expense consists primarily of interest incurred on our term loan, including non-cash amortization of debt issuance costs and debt discounts and non-cash accretion of final payment obligations.

Interest and Other Income, Net

Interest and other income, net, primarily consists of interest income earned on our cash equivalents and short-term investments.

Results of Operations

Comparison of the Three Months Ended September 30, 2026 and 2025

The following tables summarize our results of operations for the periods presented:

	Three Months Ended September 30,		Dollar Change	% Change
	2026	2025		
	(in thousands)			
Operating expenses:				
Research and development	$ 41,368	$ 49,700	$ (8,332)	(17%)
General and administrative	10,870	7,318	3,552	49%
Total operating expenses	52,238	57,018	(4,780)	(8%)
Loss from operations	(52,238)	(57,018)	4,780	(8%)
Interest expense	(833)	—	(833)	*
Interest and other income, net	1,853	2,096	(243)	(12%)
Net loss	$ (51,218)	$ (54,922)	$ 3,704	(7%)

*Not meaningful change for the periods presented.

Research and Development Expenses

	Three Months Ended September 30,		Dollar Change	% Change
	2026	2025*		
	(in thousands)			
Direct costs:				
Clinical and nonclinical outside services	$ 16,851	$ 16,969	$ (118)	(1%)
Contract manufacturing	5,923	14,911	(8,988)	(60%)
Consulting and professional services	6,217	4,814	1,403	29%
Laboratory supplies and materials	116	142	(26)	(18%)
Indirect costs:				
Compensation and personnel-related (including stock-based compensation)	10,079	10,844	(765)	(7%)
Facilities and depreciation	1,364	1,725	(361)	(21%)
Other	818	295	523	177%
Total research and development expenses	$ 41,368	$ 49,700	$ (8,332)	(17%)

*Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassifications had no impact on total research and development expenses.

Research and development expenses decreased by $8.3 million, or 17%, for the three months ended September 30, 2026 compared to the same period in 2025. The change was primarily attributable to a decrease of $9.0 million in completed contract manufacturing activity supporting the tanruprubart European MAA filing. Compensation and personnel-related expenses (including stock-based compensation) decreased by $0.8 million, reflecting lower headcount. These decreases were partially offset by a $1.4 million increase in consulting and professional services expenses associated with the vonaprument Phase 3 ARCHER II trial in GA.

General and Administrative Expenses

	Three Months Ended September 30,			
	2026	2025*	Dollar Change	% Change
	(in thousands)			
Compensation and personnel-related (including stock-based compensation)	$ 5,516	$ 3,775	$ 1,741	46%
Consulting and professional services	4,186	2,149	2,037	95%
Facilities and depreciation	521	572	(51)	(9%)
Other	647	822	(175)	(21%)
Total general and administrative expenses	$ 10,870	$ 7,318	$ 3,552	49%

*Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassifications had no impact on total general and administrative expenses.

General and administrative expenses increased by $3.6 million, or 49%, for the three months ended September 30, 2026 compared to the same period in 2025, primarily driven by a $2.0 million increase in consulting and professional services costs for corporate affairs activities supporting late-stage tanruprubart and vonaprument programs. In addition, compensation and personnel-related expenses were higher by $1.7 million primarily due to stock-based compensation expense and increased headcount.

Interest expense

Interest expense increased by $0.8 million for the three months ended September 30, 2026. The increase was due to the term loan entered into in July 2026.

Interest and other income, net

Interest and other income, net, decreased by $0.2 million, or 12%, for the three months ended September 30, 2026 compared to the same period in 2025. The decrease was primarily due to lower average interest rates.

Comparison of the Nine Months Ended September 30, 2026 and 2025

The following tables summarize our results of operations for the periods presented:

	Nine Months Ended September 30,			
	2026	2025	Dollar Change	% Change
	(in thousands)			
Operating expenses:				
Research and development	$ 123,744	$ 142,039	$ (18,295)	(13%)
General and administrative	31,774	24,110	7,664	32%
Total operating expenses	155,518	166,149	(10,631)	(6%)
Loss from operations	(155,518)	(166,149)	10,631	(6%)
Interest expense	(833)	—	(833)	*
Interest and other income, net	5,643	7,715	(2,072)	(27%)
Net loss	$ (150,708)	$ (158,434)	$ 7,726	(5%)

*Not meaningful change for the periods presented.

Research and Development Expenses

	Nine Months Ended September 30,			
	2026	2025*	Dollar Change	% Change
	(in thousands)			
Direct costs:				
Clinical and nonclinical outside services	$ 46,190	$ 44,050	$ 2,140	5%
Contract manufacturing	23,435	37,672	(14,237)	(38%)
Consulting and professional services	15,945	17,962	(2,017)	(11%)
Laboratory supplies and materials	414	523	(109)	(21%)
Indirect costs:				
Compensation and personnel-related (including stock-based compensation)	30,840	35,260	(4,420)	(13%)
Facilities and depreciation	4,784	5,578	(794)	(14%)
Other	2,136	994	1,142	115%
Total research and development expenses	$ 123,744	$ 142,039	$ (18,295)	(13%)

*Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassifications had no impact on total research and development expenses.

Research and development expenses decreased by $18.3 million, or 13%, for the nine months ended September 30, 2026 compared to the same period in 2025. The change was primarily attributable to a decrease of $14.2 million in completed contract manufacturing activity supporting the tanruprubart European MAA filing, a decrease of $4.4 million in compensation and personnel-related expenses (including stock-based compensation) reflecting lower headcount, and a decrease of $2.0 million in consulting and professional services expenses following the MAA submission for tanruprubart in January 2026. These decreases were partially offset by a $2.1 million increase in direct clinical and nonclinical outside services costs associated with the ongoing tanruprubart FORWARD study in GBS and the launch of the vonaprument OLE study in GA.

General and Administrative Expenses

	Nine Months Ended September 30,			
	2026	2025*	Dollar Change	% Change
	(in thousands)			
Compensation and personnel-related (including stock-based compensation)	$ 15,409	$ 12,311	$ 3,098	25%
Consulting and professional services	12,901	7,247	5,654	78%
Facilities and depreciation	1,742	1,842	(100)	(5%)
Other	1,722	2,710	(988)	(36%)
Total general and administrative expenses	$ 31,774	$ 24,110	$ 7,664	32%

*Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassifications had no impact on total general and administrative expenses.

General and administrative expenses increased by $7.7 million, or 32%, for the nine months ended September 30, 2026 compared to the same period in 2025, primarily driven by a $5.7 million increase in consulting and professional services costs for corporate affairs activities supporting late-stage tanruprubart and vonaprument programs. In addition, compensation and personnel-related expenses were higher by $3.1 million primarily due to stock-based compensation expense and increased headcount.

Interest expense

Interest expense increased by $0.8 million for the nine months ended September 30, 2026. The increase was due to the term loan entered into in July 2026.

Interest and other income, net

Interest and other income, net, decreased by $2.1 million, or 27%, for the nine months ended September 30, 2026 compared to the same period in 2025. The decrease was primarily due to lower average interest rates as well as lower average cash and investment balances.

Liquidity and Capital Resources

Sources of Liquidity

Due to our significant research and development expenditures, we have generated operating losses each year since our inception.

To date, we have funded our operations primarily through the sale of equity securities including, most recently in November 2025, the public offering of approximately $86.3 million of shares of common stock and pre-funded warrants. In addition, on March 30, 2026, we entered into a sales agreement with TD Cowen pursuant to which we may offer and sell, from time to time through TD Cowen, at our option, shares of our common stock having an aggregate offering price of up to $150.0 million. On July 30, 2026, we also received $50.0 million in gross proceeds under a Loan and Security Agreement, or Oxford Loan Agreement, with Oxford Finance LLC, as collateral agent, and Oxford Finance Credit Fund IV, LP, or collectively, Oxford, and have up to $150.0 million of additional borrowing capacity, subject to specified milestones and other conditions, to support our ongoing operations. As of September 30, 2026, we had available cash and cash equivalents and short-term investments of $213.4 million and an accumulated deficit of approximately $1.1 billion.

Historical Cash Flows

	Nine Months Ended September 30,	
	2026	2025
	(in thousands)	
Net cash used in operating activities	$ (143,483)	$ (140,430)
Net cash (used in) provided by investing activities	(33,475)	216,573
Net cash provided by financing activities	117,295	13,776
(Decrease) increase in cash, cash equivalents and restricted cash	$ (59,663)	$ 89,919

Cash Flow from Operating Activities

Cash used in operating activities for the nine months ended September 30, 2026 was $143.5 million, which consisted of a net loss of $150.7 million and a net change of $8.4 million in our operating assets and liabilities, partially offset by $15.6 million in non-cash charges. The non-cash charges consisted of stock-based compensation of $14.1 million, depreciation and amortization of $1.6 million, a reduction in the carrying amount of right-of-use assets of $1.3 million, and non-cash interest expense of $0.1 million, partially offset by accretion of discount on available-for-sale securities of $1.5 million.

Cash used in operating activities for the nine months ended September 30, 2025 was $140.4 million, which consisted of a net loss of $158.4 million, partially offset by $12.2 million in non-cash charges and a net change of $5.7 million in our operating assets and liabilities. The non-cash charges consisted of stock-based compensation of $13.1 million, depreciation and amortization of $1.6 million, and a reduction in the carrying amount of right-of-use assets of $1.1 million, partially offset by accretion of discount on available-for-sale securities of $3.6 million.

Cash Flow from Investing Activities

Cash used in investing activities for the nine months ended September 30, 2026 was $33.5 million, which consisted of $126.3 million of purchases of available-for-sale securities and $0.1 million of purchases of property and equipment, partially offset by $93.0 million of proceeds from maturities of available-for-sale securities.

Cash provided by investing activities for the nine months ended September 30, 2025 was $216.6 million, which consisted of $363.9 million of proceeds from maturities of available-for-sale securities, partially offset by $147.2 million of purchases of available-for-sale securities.

Cash Flow from Financing Activities

Cash provided by financing activities for the nine months ended September 30, 2026 was $117.3 million, which consisted of $66.2 million of net proceeds from the aggregate issuance of common stock under our 2024 ATM program and 2026 ATM program, $49.4 million from the issuance of term loan, net of debt discount, and $1.7 million of net proceeds from the exercise of common stock options and employee stock purchase plan purchases.

Cash provided by financing activities for the nine months ended September 30, 2025 was $13.8 million, which consisted of $13.5 million of net proceeds from the issuance of common stock under our 2024 ATM program and $0.2 million of net proceeds from the exercise of common stock options and employee stock purchase plan purchases.

Funding Requirements

We use our cash to fund operations, primarily to fund our clinical trials, research and development expenditures and related personnel costs. We expect our future research and development expenses to increase as we pursue regulatory approval of our product candidates, continue to advance our product candidates through late-stage clinical trials, invest in capabilities to prepare for commercialization including manufacturing, and hire additional personnel to support our organization. In addition, we expect our general and administrative expenses to increase as we continue to support our research and development activities, grow our business, advance our product candidates in late-stage clinical trials and toward regulatory approval and commercialization activities, and operate as a public company. The timing and amount of our operating expenditures will depend on many factors, including:

- the scope, progress, results and costs of researching and developing our current product candidates or any other future product candidates we choose to pursue, and conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining feedback from regulators on our clinical trials and regulatory approvals for our lead product candidates or any future product candidates;
- the number and characteristics of any additional product candidates we develop or acquire;
- the timing and amount of any milestone, royalty and/or other payments we are required to make pursuant to our current or any future license or collaboration agreements;
- the cost of manufacturing our lead product candidates or any future product candidates and any products we successfully commercialize;
- the cost of building a sales force in anticipation of product commercialization;
- the cost of commercialization activities of our product candidates, if approved for sale, including marketing, sales and distribution costs;
- our ability to establish strategic collaborations, licensing or other arrangements and the financial terms of any such agreements, including the timing and amount of any future milestone, royalty or other payments due under any such agreement;
- any product liability or other lawsuits related to our products;
- the expenses needed to attract, hire and retain skilled personnel;
- the costs associated with operating as a public company;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property portfolio; and
- the timing, receipt and amount of sales of any future approved products.

Based upon our current operating plan, we believe that our existing cash and cash equivalents and short-term investments will enable us to fund operations into 2028. We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. We will be required to seek additional funding in the future until such time, if ever, as we can generate substantial product revenue, and currently intend to do so through public or private equity offerings or debt financings, credit or loan facilities, collaborations or a combination of one or more of these funding sources. We may also need to seek additional funds sooner than planned as result of changes in our development plans and regulatory requirements to support registration of our product candidates. Additional funds may not be available to us on acceptable terms or at all. If we fail to obtain necessary capital when needed on acceptable terms, or at all, we could be forced to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations. 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. Debt financing, if available, is 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 received any distribution of our corporate assets.

Oxford Loan Agreement

On July 30, 2026, we entered into the Oxford Loan Agreement with Oxford. The Oxford Loan Agreement provides for term loans of up to $200.0 million to be funded in multiple tranches, including a $50.0 million Term A Loan funded at closing and up to $150.0 million of additional borrowing capacity subject to specified conditions. The Oxford Loan Agreement matures on July 1, 2031 and bears interest at a floating annual rate equal to the greater of 1-Month CME Term SOFR or 3.00%, plus 4.60%, for a minimum of 7.60%. We are required to make monthly interest-only payments through September 1, 2029, after which monthly payments of principal and interest will be due. Based on the scheduled maturity without extension, estimated future principal and final fee payments are $8.7 million in 2029, $26.1 million in 2030 and $17.7 million in 2031. The Oxford Loan Agreement includes customary affirmative and negative covenants, including restrictions on the ability to pay dividends and make other distributions. As of September 30, 2026, $150.0 million of borrowing capacity remained available under the Oxford Loan Agreement and we were in compliance with all covenants and have not received any notification or indication from Oxford of an intent to declare the loan due prior to maturity.

Pre-Funded and Common Warrants

We have issued pre-funded warrants in connection with public offerings of our common stock in July 2022, December 2023, June 2024 and November 2025. Each pre-funded warrant has an exercise price of $0.001 per share, does not expire and is immediately exercisable subject to beneficial ownership limitations. The pre-funded warrants are classified as equity.

During the nine months ended September 30, 2026, we issued an aggregate of 27,423,009 shares of common stock upon the cashless exercise of pre-funded warrants to purchase 27,427,778 shares of common stock.

In July 2022, we issued common warrants to purchase up to 8,427,508 shares of common stock in connection with an equity offering. The common warrants had an exercise price of $5.807 per share and an original expiration date of June 30, 2025. In June 2025, we and holders of common warrants to purchase 6,877,622 shares agreed to extend the expiration date to June 30, 2026. We recorded the modification as a $1.9 million deemed dividend, which increased net loss attributable to common stockholders for the nine months ended September 30, 2025. Common warrants to purchase the remaining 1,226,993 shares expired unexercised on June 30, 2025. On June 30, 2026, the amended common warrants to purchase 6,877,622 shares expired unexercised. No common warrants remained outstanding as of September 30, 2026.

The following summarizes warrant activity during the nine months ended September 30, 2026 and 2025:

	Number of Common Warrants	Number of Pre-funded Warrants	Weighted-Average Exercise Price
Balances as of December 31, 2025	6,877,622	42,293,577	
Issued	—	—	$ —
Exercised	—	(27,427,778)	$ 0.001
Expired	(6,877,622)	—	$ 5.807
Balances as of September 30, 2026	—	14,865,799	

	Number of Common Warrants	Number of Pre-funded Warrants	Weighted-Average Exercise Price
Balances as of December 31, 2024	8,104,615	38,543,577	
Issued	—	—	$ —
Exercised	—	—	$ —
Expired	(1,226,993)	—	$ 5.807
Balances as of September 30, 2025	6,877,622	38,543,577	

Subsequent to September 30, 2026 and through the date of issuance of these interim unaudited condensed consolidated financial statements, we issued an aggregate of 120,677 shares of common stock upon the cashless exercise of pre-funded warrants to purchase 120,706 shares of common stock.

At the Market (ATM) Program

In March 2026, we entered into a sales agreement with TD Securities (USA) LLC, or TD Cowen, as sales agent, pursuant to which we may offer and sell, from time to time through TD Cowen, at our option, shares of our common stock having an aggregate offering price of up to $150.0 million, or the 2026 ATM program. We agreed to pay TD Cowen a commission of up to 3.0% of the aggregate gross proceeds for the common stock sold through the 2026 ATM program. During the nine months ended September 30, 2026, we sold 6,220,115 shares of common stock under the 2026 ATM program for net proceeds of approximately $33.5 million, after deducting commissions paid to TD Cowen and other offering costs. As of September 30, 2026, approximately $115.2 million remained available under the 2026 ATM program. Subsequent to September 30, 2026 and through the date of issuance of these interim unaudited condensed consolidated financial statements, we sold 639,068 shares of common stock under the 2026 ATM program for net proceeds of approximately $2.7 million, after deducting commissions paid to TD Cowen.

In March 2024, we entered into a sales agreement with TD Cowen, as sales agent, pursuant to which we were permitted to offer and sell shares of our common stock having an aggregate maximum offering price of up to $100.0 million, or 2024 ATM program. During the nine months ended September 30, 2026 and 2025, we sold 6,049,762 and 5,235,959 shares of our common stock under the 2024 ATM program for net proceeds of approximately $32.7 million and $13.5 million, respectively, after deducting commissions paid to TD Cowen and other offering costs. In May 2026, we terminated the 2024 ATM program. Approximately $33.3 million remained available under the 2024 ATM program at the time of termination; however, no further shares of common stock may be sold under the 2024 ATM program.

Critical Accounting Policies and Estimates

Management's discussion and analysis of our financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these unaudited condensed consolidated financial statements requires us to make estimates and assumptions for the reported amounts of assets, liabilities, expenses and related disclosures. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions and any such differences may be material.

During the quarter ended September 30, 2026, there were no material changes to our critical accounting policies or in the methodology used for estimates from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Recent Accounting Pronouncements Not Yet Adopted

See Note 2—*Basis of Presentation and Significant Accounting Policies* to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for information about recent accounting pronouncements, the timing of their adoption, and our assessment, to the extent we have made one yet, of their potential impact on our financial condition or results of operations.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Not applicable.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our Chief Executive Officer and our Chief Financial Officer, has evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that, as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls and procedures are effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

Changes in Internal Control over Financial Reporting

There has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended September 30, 2026 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

We are not party to any material legal proceedings at this time. From time to time, we may become involved in various legal proceedings that arise in the ordinary course of our business.

Item 1A. Risk Factors.

RISK FACTORS

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

Risk Factor Summary

The following summarizes the most material risks that make an investment in our securities risky or speculative. If any of the following risks occur or persist, our business, financial condition and results of operations could be materially harmed and the price of our common stock could significantly decline.

- We are a clinical-stage biopharmaceutical company with a limited operating history and no products approved for commercial sale. We have incurred significant losses since our inception, and we anticipate that we will continue to incur significant losses for the foreseeable future, which, together with our limited operating history, makes it difficult to assess our future viability.
- We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations.
- Our business is heavily dependent on the successful development, regulatory approval and commercialization of our product candidates, some of which are in early stages of clinical development.
- Research and development of biopharmaceutical products is inherently risky. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.
- We may encounter substantial delays in our clinical trials or may not be able to conduct or complete our clinical trials on the timelines we expect, if at all.
- Adverse events or undesirable side effects caused by, or other unexpected properties of, any of our product candidates could halt their clinical development, delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.
- We conduct, and in the future plan to conduct, clinical trials for product candidates outside the United States, and the Food and Drug Administration, or FDA, and comparable foreign regulatory authorities may not accept data from such trials.
- We rely on third-party suppliers to manufacture our product candidates, and we intend to rely on third parties to produce commercial supplies of any approved product. The loss of these suppliers, or their failure to comply with applicable regulatory requirements or to provide us with sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.
- The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish adequate coverage, reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

- Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our product candidates.
- If we are unable to obtain, maintain and enforce intellectual property protection directed to our current and any future technologies that we develop, others may be able to make, use or sell products substantially the same as ours, which could adversely affect our ability to compete in the market.
- Our stock price has been volatile, and could in the future be volatile, and you may not be able to resell shares of our common stock at or above the price you paid.
- Actual or perceived failure to comply with applicable data protection laws, regulations, standards, contractual obligations and other requirements related to data privacy and security could lead to government enforcement actions as well as civil and criminal penalties, private litigation (including class actions), adverse publicity and otherwise could negatively affect our results of operations and business.

Risks Related to Our Limited Operating History, Financial Condition and Capital Requirements

We are a clinical-stage biopharmaceutical company with a limited operating history and no products approved for commercial sale. We have incurred significant losses since our inception, and we anticipate that we will continue to incur significant losses for the foreseeable future, which, together with our limited operating history, makes it difficult to assess our future viability.

We are a clinical-stage biopharmaceutical company, and we have only a limited operating history upon which you can evaluate our business and prospects. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have no products approved for commercial sale and have not generated any revenue from sales of our product candidates and have incurred losses in each year since our inception in March 2011. We have only a limited operating history upon which you can evaluate our business and prospects. In addition, we have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical, biopharmaceutical and biotechnology industry.

We have had significant operating losses since our inception. Our net loss for the years ended December 31, 2025 and 2024 was approximately $206.7 million and $138.2 million, respectively, and our net loss for the three and nine months ended September 30, 2026 was approximately $51.2 million and $150.7 million, respectively. As of September 30, 2026, we had an accumulated deficit of approximately $1.1 billion. 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 continue to incur losses for the foreseeable future, and we anticipate these losses will continue as we develop our product candidates, conduct clinical trials and pursue research and development activities. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations.

Since our inception, we have invested a significant portion of our efforts and financial resources in research and development activities. Our product candidates will require additional clinical development, and if approved, will require additional commercialization efforts. We also intend to conduct additional research and development activities to discover and develop new product candidates, including conducting preclinical studies and clinical trials, all of which will require substantial additional funds. We will continue to expend significant resources for the foreseeable future in connection with these activities. These expenditures will include costs associated with conducting preclinical studies and clinical trials, obtaining regulatory approvals and manufacturing and supply, as well as marketing and selling any products approved for sale. In addition, other unanticipated costs may arise. Because the outcome of any preclinical study or clinical trial is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates or any future product candidates.

As of September 30, 2026, we had capital resources consisting of cash and cash equivalents and short-term investments of approximately $213.4 million. We expect our existing capital resources to fund planned operations into 2028. However, our operating plans may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned through public or private equity offerings or debt financings or other sources, such as strategic collaborations. We may also need to seek additional funds sooner than planned as a result of changes in our development plans and regulatory requirements to support registration of our product candidates. Such financing may result in dilution to our stockholders, imposition of burdensome debt covenants and repayment obligations, or other restrictions that may affect our business. 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 future capital requirements depend on many factors, including:

- the scope, progress, results and costs of researching and developing our current product candidates or any other future product candidates we choose to pursue, conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining feedback from regulators on our clinical trials and regulatory approvals for our product candidates or any future product candidates;
- the number and characteristics of any additional product candidates we develop or acquire;
- the timing and amount of any milestone, royalty and/or other payments we are required to make pursuant to our current or any future license or collaboration agreements;
- the cost of manufacturing our product candidates or any future product candidates and any products we successfully commercialize;
- the cost of building a sales force in anticipation of product commercialization;
- the cost of commercialization activities of our product candidates, if approved for sale, including marketing, sales and distribution costs;
- our ability to establish strategic collaborations, licensing or other arrangements and the financial terms of any such agreements, including the timing and amount of any future milestone, royalty or other payments due under any such agreement;
- any product liability or other lawsuits related to our products;
- the expenses needed to attract, hire and retain skilled personnel;
- the costs associated with operating as a public company;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property portfolio; and
- the timing, receipt and amount of sales of any future approved products.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions, macroeconomic factors, including recent and potential bank failures, increasing inflation and interest rates, funding shortages at governmental and regulatory agencies on which we rely, exchange rate fluctuations and supply chain disruptions, geopolitical conflicts, such as the war in Ukraine and conflicts in the Middle East, and disruptions to and volatility in the credit and financial markets in the United States and worldwide. If adequate funds are not available to us on a timely basis, we may be required to:

- delay, limit, reduce or terminate preclinical studies, clinical trials or other development activities for our product candidates or any future product candidate;
- delay, limit, reduce or terminate our research and development activities; or
- delay, limit, reduce or terminate our efforts to establish manufacturing and sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates or any future product candidate or reduce our flexibility in developing or maintaining our sales and marketing strategy.

We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to some of our technologies or product candidates that we would otherwise pursue on our own. We do not expect to realize revenue from sales of products or royalties from licensed products in the foreseeable future, if at all, and unless and until our product candidates are clinically tested, approved for commercialization and successfully marketed. To date, we have primarily financed our operations through the sale of equity securities and warrants to purchase our equity securities. For example, in March 2026, we entered into a sales agreement (as amended from time to time), or Sales Agreement, with TD Securities (USA) LLC, or TD Cowen, pursuant to which we may offer and sell, from time to time through TD Cowen, at our option, shares of our common stock having an aggregate offering price of up to $150 million under an at-the-market offering program. In July 2026, we also incurred indebtedness of $50.0 million pursuant to a loan agreement with Oxford Finance LLC, as collateral agent, and certain other lenders from time to time. 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, credit or loan facilities, collaborations or a combination of one or more of these funding sources. Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities including pursuant to sales under the Sales Agreement, 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. Debt financing, if available, is 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 received any distribution of our corporate assets.

Our debt obligations under the Loan and Security Agreement place restrictions on our operating and financial flexibility and could adversely affect our financial condition.

On July 30, 2026, we entered into a Loan and Security Agreement, or Loan Agreement, with Oxford Finance LLC, as collateral agent, or Collateral Agent, and certain lenders from time to time party thereto, or Lenders, providing for term loans in an aggregate principal amount of up to $200.0 million, or Term Loans. Under the terms of the Loan Agreement, the Lenders made an initial term loan of $50.0 million funded on the effective date of the Loan Agreement. Three additional tranches in an aggregate principal amount of up to $100.0 million are available upon the achievement of specified milestones and conditions related to our vonaprument and tanruprubart registrational programs, and one additional uncommitted tranche in an aggregate principal amount of up to $50.0 million is available upon the mutual agreement of the Company and the Lenders. The Term Loans bear interest at a floating per annum rate equal to the greater of (i) the 1-Month CME Term SOFR plus 4.6% and (ii) 7.60%. The Term Loans mature on July 1, 2031, or July 1, 2032, depending on the achievement of certain milestones. We are required to make monthly payments of interest only until September 1, 2029, which can be extended to September 1, 2030 or September 1, 2031, depending on the achievement of certain milestones, after which monthly payments of both principal and interest will be due. The Term Loans may be prepaid, in whole or in part, at our option, subject to customary prepayment fees. We are also obligated to pay other customary fees for a loan facility of this size and type, including a final payment fee upon repayment of the Term Loans (whether at maturity, upon acceleration or by prepayment or otherwise). We granted the Collateral Agent a security interest in substantially all of our assets to secure our obligations under the Loan Agreement.

Our debt obligations under the Loan Agreement could adversely impact us as follows:

- require us to use a large portion of our cash flow to repay the outstanding principal balance, accrued and unpaid interest, prepayment fees, a final payment fee and other fees, if applicable, which will reduce the amount of cash flow available to fund working capital, capital expenditures, product candidate development and other general corporate purposes;
- adversely impact our credit rating, which could increase borrowing costs and reduce our ability to raise funds on favorable terms;
- subject us to restrictive covenants, including financial covenants that require us to maintain certain liquidity levels and/or minimum revenue levels upon the occurrence of certain events, that may limit our future ability to take certain corporate actions, incur additional indebtedness, create liens, make investments or pay dividends or distributions, or to raise funds for capital expenditures, strategic acquisitions or business opportunities, R&D, and other general corporate requirements;
- increase our vulnerability to adverse economic, industry and market conditions; and
- increase our exposure to rising interest rates from variable rate indebtedness.

Our ability to meet our payment obligations under the Loan Agreement depends on our ability to generate significant cash flows or obtain external financing in the future. This, to some extent, is subject to market, economic, financial, competitive, legislative, and regulatory factors as well as other factors that are beyond our control. There can be no assurance that our business will generate cash flow from operations, or that additional capital will be available to us, in amounts sufficient to enable us to meet our debt payment obligations and to fund other liquidity needs. Additionally, events and circumstances may occur which would cause us to not be able to satisfy applicable draw-down conditions and access the additional loan amounts available under the Loan Agreement. If we are unable to generate sufficient cash flows to service our payment obligations or satisfy our covenants under the Loan Agreement, we may need to refinance, restructure, or amend the terms of our debt, sell assets, reduce or delay capital investments, or seek to raise additional capital. If we are unable to implement one or more of these alternatives, we may be unable to meet our debt payment obligations which could result in an event of default. If we default under the Loan Agreement, the Lenders may accelerate all of our repayment obligations and exercise all of their rights and remedies under the Loan Agreement and applicable law, including foreclosing on substantially all of our assets, which secure our obligations under the Loan Agreement, potentially requiring us to renegotiate the terms of our indebtedness on terms less favorable to us. Further, if we are liquidated, the Lenders' right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. The Loan Agreement includes events of default, including, but not limited to, nonpayment of principal, interest, fees or other amounts; material inaccuracy of a representation or warranty; failure to perform or observe covenants; cross-defaults with certain other indebtedness; bankruptcy and insolvency events; material monetary judgment defaults; the occurrence of a material adverse change; impairment of the Lenders' security interest in the collateral; and delisting of our common stock. Upon the occurrence of an event of default (subject, in certain cases, to notice and grace periods), obligations under the Loan Agreement may be accelerated, thereby requiring us to repay the loans immediately. Any declaration by the

Lenders of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. Additionally, if we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

Due to the significant resources required for the development of our product candidates, we must prioritize development of certain product candidates and/or certain disease indications. We may expend our limited resources on candidates or indications that do not yield a successful product and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We seek to maintain a process of prioritization and resource allocation among our programs to balance time, risk and cost, due to the significant resources required for the development of our product candidates. Our resources are primarily focused on advancing tanruprubart in GBS, and vonaprument in GA. If sufficient funding is not available, we may not be able to complete our planned clinical trials, or on the timelines we currently anticipate, and we may need to redesign, reduce the scope of or terminate some of our programs.

Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates or therapeutic areas may not lead to the development of any viable commercial product and may divert resources away from better opportunities. Similarly, any decision to delay, terminate or collaborate with third parties in respect of certain programs may subsequently also prove to be suboptimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the viability or market potential of any of our programs or product candidates or misread trends in the pharmaceutical, biopharmaceutical or biotechnology industry, our business, financial condition and results of operations could be materially adversely affected. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases and disease pathways that may later prove to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain development and commercialization rights.

Conducting a global Phase 3 program for vonaprument in patients with dry AMD with GA will be expensive and time consuming, and we may need additional capital to complete the Phase 3 clinical program and even if favorable, the FDA and comparable foreign regulatory authorities may not accept data from our Phase 3 program.

Recent regulatory engagement regarding the Phase 3 ARCHER II trial design has established a global registration path for vonaprument in the U.S. and Europe. As a result, we are conducting ARCHER II, a global sham-controlled double-masked, Phase 3 trial. The single-study program will be analyzed as two sub-studies for the U.S. in accordance with the FDA's two-trial recommendation. In August 2026, we expanded the ARCHER II program to add a Month 24 dual primary endpoint, complementing the original Month 15 primary endpoint, and to launch an open-label extension, or OLE, study in which patients completing the Month 24 assessment will have the option to receive monthly vonaprument treatment. There can be no assurance that the FDA and comparable regulatory authorities will accept the data from our Phase 3 program, including the amended dual primary endpoint design or the OLE study, or that such data will be determined sufficient to support approval.

Conducting large Phase 3 trials in multiple jurisdictions is expensive and can take many years to complete, and we cannot guarantee that clinical trials will be conducted as planned or completed timely, if at all. Our timeline and costs for ARCHER II could be substantially longer and larger than we initially planned, including as a result of the recently added Month 24 primary endpoint and OLE study, which extend the trial's duration and increase its complexity. Under the dual primary endpoint design, ARCHER II may achieve statistical success at either the Month 15 or Month 24 timepoint. However, there is no assurance that either timepoint will show a statistically significant treatment effect, that the FDA, EMA or other regulators will agree that success at one timepoint is sufficient to support approval, or that the design amendment itself will not require additional discussion, data or time to align with regulators. We may need additional capital to complete the expanded Phase 3 clinical program and the OLE study for vonaprument and may not be able to raise sufficient capital in a timely manner which could delay or prevent us from completing our clinical trial, prevent us from seeking FDA approval of vonaprument, if ever, and could delay or prevent commercialization of vonaprument.

Our results of operations may fluctuate significantly, which makes our future results of operations difficult to predict and could cause our results of operations to fall below expectations.

Our quarterly and annual results of operations may fluctuate significantly, which makes it difficult for us to predict our future results of operations. These fluctuations may occur due to a variety of factors, many of which are outside of our control and may be difficult to predict, including:

- the timing and cost of, and level of investment in, research, development and, if approved, commercialization activities relating to our product candidates, which may change from time to time;

- the timing and status of enrollment for our clinical trials;
- the cost of manufacturing our product candidates, as well as building out our supply chain, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we may incur to acquire, develop or commercialize additional product candidates and technologies;
- timing and amount of any milestone, royalty or other payments due under any collaboration or license agreement;
- future accounting pronouncements or changes in our accounting policies;
- the timing and success or failure of preclinical studies and clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- the timing of receipt of approvals for our product candidates from regulatory authorities in the United States and internationally;
- coverage and reimbursement policies with respect to our product candidates, if approved, and potential future drugs that compete with our products; and
- the level of demand for our product candidates, if approved, which may vary significantly over time.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual results of operations. As a result, comparing our results of operations on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or results of operations fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if any forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

Risks Related to Our Business

Our business is heavily dependent on the successful development, regulatory approval and commercialization of our product candidates, some of which are in early stages of clinical development.

We have no products approved for sale, and some of our product candidates are in early stages of clinical development. The success of our business, including our ability to finance our company and generate revenue in the future, will primarily depend on the successful development, regulatory approval and commercialization of our product candidates and, in particular, the advancement of our current clinical-stage product candidates. However, given the stage of development of our product candidates, it may be many years, if we succeed at all, before we have demonstrated the safety, purity, potency and/or efficacy of a product candidate sufficient to warrant approval for commercialization. We cannot be certain that our product candidates will receive regulatory approval or be successfully commercialized even if we receive regulatory approval.

While inhibition of the complement pathway has been validated as a therapeutic approach, C1q inhibition is a novel therapeutic approach, which exposes us to certain risks. For example, we may discover that our product candidates do not possess certain properties required for therapeutic effectiveness, or even if found to be effective in one type of disease, they are not effective in other types of disease. In addition, given the novel nature of this therapeutic approach, designing preclinical studies and clinical trials to demonstrate the effect of the product candidates is complex and exposes us to risks, including that our biomarker-driven approach may not translate into therapeutic effectiveness.

In the future, we may also become dependent on other product candidates that we may develop or acquire. The clinical and commercial success of our product candidates and future product candidates will depend on a number of factors, including the following:

- our ability to raise any additional required capital on acceptable terms, or at all;
- our ability to complete an investigational new drug application, or IND, enable studies and successfully submit INDs or comparable applications;

- timely completion of our preclinical studies and clinical trials, which may be significantly slower or cost more than we currently anticipate and will depend substantially upon the performance of third-party contractors;
- whether we are required by the FDA, or similar foreign regulatory agencies to conduct additional clinical trials or other studies beyond those planned to support the approval and commercialization of our product candidates or any future product candidates;
- acceptance of our proposed indications and primary endpoint assessments relating to the proposed indications of our product candidates by the FDA and similar foreign regulatory authorities;
- our ability to demonstrate to the satisfaction of the FDA and similar foreign regulatory authorities the safety, efficacy and acceptable risk to benefit profile of our product candidates or any future product candidates;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates or future approved products, if any;
- the timely receipt of necessary marketing approvals from the FDA and similar foreign regulatory authorities;
- achieving and maintaining, and, where applicable, ensuring that our third-party contractors achieve and maintain compliance with our contractual obligations and with all regulatory requirements applicable to our product candidates or any future product candidates or approved products, if any;
- the ability of third parties with whom we contract to manufacture adequate clinical trial and commercial supplies of our product candidates or any future product candidates remain in good standing with regulatory agencies and develop, validate and maintain commercially viable manufacturing processes that are compliant with current good manufacturing practices, or cGMPs;
- our ability to successfully develop a commercial strategy and thereafter commercialize our product candidates or any future product candidates in the United States and internationally, if approved for marketing, reimbursement, sale and distribution in such countries and territories, whether alone or in collaboration with others;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- the convenience of our treatment or dosing regimen;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of our product candidates or any future product candidates, if approved, including relative to alternative and competing treatments;
- the willingness of physicians, operators of clinics and patients to utilize or adopt any of our product candidates or any future product candidates, if approved;
- patient demand for our product candidates, if approved, including the willingness of patients to pay out-of-pocket for any approved products in the absence of coverage and/or adequate reimbursement from third-party payors;
- our ability to establish and enforce intellectual property rights in and to our product candidates or any future product candidates; and
- our ability to avoid third-party patent interference, intellectual property challenges or intellectual property infringement claims.

These factors, many of which are beyond our control, could cause us to experience significant delays or an inability to obtain regulatory approvals or commercialize our product candidates. Even if regulatory approvals are obtained, we may never be able to successfully commercialize any of our product candidates. Accordingly, we cannot provide assurances that we will be able to generate sufficient revenue through the sale of our product candidates or any future product candidates to continue our business or achieve profitability.

Public health crises such as pandemics or similar outbreaks have, and could in the future, materially and adversely affect our preclinical and clinical trials, business, financial condition and results of operations.

As a result of public health crises, including the COVID-19 pandemic, we have experienced, and may in the future experience, disruptions that could materially and adversely impact our clinical trials, business, financial condition and results of operations. These disruptions include but are not limited to:

- delays or difficulties in enrolling patients in our clinical trials;

- delays or difficulties in initiating or expanding clinical trials, including delays or difficulties with clinical site initiation and recruiting clinical site investigators and clinical site staff;
- increased rates of patients withdrawing from our clinical trials following enrollment as a result of health conditions;
- interruption of key clinical trial activities, such as clinical trial site data monitoring and efficacy, safety and translational data collection, processing and analyses, due to limitations on travel imposed or recommended by federal, state or local governments, employers and others or interruption of clinical trial subject visits, which may impact the collection and integrity of subject data and clinical study endpoints;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- delays or disruptions in preclinical experiments and IND-enabling studies due to restrictions of on-site staff and unforeseen circumstances at contract research organizations, or CROs, and vendors;
- interruption or delays in the operations of the FDA and comparable foreign regulatory agencies;
- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems;
- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;
- limitations on employee or other resources that would otherwise be focused on the conduct of our clinical trials and pre-clinical work, including because of sickness of employees or their families, the desire of employees to avoid travel or contact with large groups of people, an increased reliance on working from home, school closures or mass transit disruptions;
- changes in regulations as part of a response to pandemics or similar outbreaks, which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue such clinical trials altogether;
- delays in necessary interactions with regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government or contractor personnel; and
- refusal of the FDA to accept data from clinical trials in affected geographies outside the United States.

The extent to which any future outbreak may affect our clinical trials, business, financial condition, results of operations and clinical development timelines and plans will depend on factors including the duration of the outbreak, rates of infection in the locations in which we and our CROs, third-party manufacturers, regulatory authorities and other third parties with whom we do business operate, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease. Future developments in these and other areas present material uncertainty and risk with respect to our clinical trials, business, financial condition and results of operations.

Research and development of biopharmaceutical products is inherently risky. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

Our future success is dependent on our ability to successfully develop, obtain regulatory approval for and successfully commercialize our product candidates, and we may fail to do so for many reasons, including the following:

- our product candidates may not successfully complete preclinical studies or clinical trials;
- a product candidate may be shown to have harmful side effects or other characteristics that indicate it does not meet applicable regulatory criteria;
- our competitors may develop therapeutics that render our product candidates obsolete or less attractive;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable or commercially attractive;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all;
- if a product candidate obtains regulatory approval, we may be unable to establish sales and marketing capabilities, or successfully market such approved product candidate; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a product candidate or candidates, which would have a material adverse effect on our business and could potentially cause us to cease operations. Failure of a product candidate

may occur at any stage of preclinical or clinical development, and we may never succeed in developing marketable products or generating product revenue.

We may not be successful in our efforts to further develop our current and future product candidates. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. Each of our product candidates will require significant additional clinical development, management of preclinical, clinical and manufacturing activities, regulatory approval, adequate manufacturing supply, a commercial organization and significant marketing efforts before we generate any revenue from product sales, if at all. Any clinical studies that we may conduct may not demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates. If the results of our ongoing or future clinical studies are inconclusive with respect to the efficacy of our product candidates, if we do not meet the clinical endpoints with statistical significance or if there are safety concerns or adverse events associated with our product candidates, we may be prevented or delayed in obtaining marketing approval for our product candidates.

The FDA or other regulatory agencies may not agree with our clinical development plan and require that we conduct additional clinical trials to support our regulatory submissions. Regulatory agencies, including the FDA may require that we conduct more than one pivotal trial in order to gain approval.

Furthermore, clinical trials must be conducted in accordance with the laws, rules and regulations, guidelines and other requirements of the FDA, the EMA, and other applicable regulatory authorities outside of those jurisdictions and are subject to oversight by these regulatory authorities and institutional review boards, or IRBs, or ethics committees at the medical institutions where such clinical trials are conducted. Further, conducting global clinical trials, as we do for GBS and GA, may require that we coordinate among the legal requirements and guidelines of regulatory authorities across a number of jurisdictions, including the United States, the EU, and countries outside of those jurisdictions, which could require that we amend clinical trial protocols or determine not to conduct a trial in one or more jurisdictions or to run separate trials in various jurisdictions due to the inability, cost or delay in harmonizing divergent requests from such regulatory authorities, all of which could increase costs. In addition, clinical trials that are conducted in countries outside the United States and the EU may subject us to risks associated with the engagement of non-United States and non-EU CROs who are unknown to the FDA or the EMA, or the EU member states' regulatory authorities and may have different standards of diagnosis, screening and medical care, as well as risks associated with further delays and expenses as a result of increased shipment costs (including as a result of local quality release or in-country testing of a product candidate supply produced in a different jurisdiction for our clinical trials) and political and economic risks relevant to such countries outside the United States and the EU.

If any of our product candidates successfully completes clinical trials, we plan to seek regulatory approval to market our product candidates in the United States, the EU, and in additional foreign countries where we believe there is a viable commercial opportunity. We may never receive regulatory approval to market any product candidates even if such product candidates successfully complete clinical trials, which would adversely affect our viability. To obtain regulatory approval in countries outside the United States, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical trials, commercial sales, pricing and distribution of our product candidates. We may also rely on collaborators or partners to conduct the required activities to support an application for regulatory approval and to seek approval for one or more of our product candidates. We cannot be sure that any such collaborators or partners will conduct these activities successfully or do so within the timeframe we desire. Even if we or any future collaborators or partners are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. If we are unable to obtain approval for our product candidates in multiple jurisdictions, our revenue and results of operations could be negatively affected.

Even if we receive regulatory approval to market any of our product candidates, we cannot assure you that any such product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. Any approval we may obtain could be for indications or patient populations that are not as broad as intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We may also be required to perform additional or unanticipated clinical trials to obtain approval or be subject to additional post-marketing testing requirements to maintain approval. In addition, regulatory authorities may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a Risk Evaluation and Mitigation Strategy, or REMS. The failure to obtain timely regulatory approval of product candidates, any product marketing limitations or a product withdrawal would negatively impact our business, results of operations and financial condition.

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

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. We cannot be sure that submission of an IND or

a clinical trial application, or CTA, will result in the FDA or other regulatory authority, as applicable, allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our future clinical trials may not be successful. Clinical trials can be delayed or terminated for a variety of reasons, including delays or failures related to:

- generating sufficient preclinical, toxicology or other in vivo or in vitro data to support the initiation or continuation of clinical trials;
- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials;
- delays in obtaining regulatory authorization to commence a trial;
- reaching agreements on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- identifying, recruiting and training suitable clinical investigators;
- obtaining IRB approval at each trial site;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND or amendment, or equivalent foreign application or amendment;
- new safety findings that present unreasonable risk to clinical trial participants;
- a negative finding from an inspection of our clinical trial operations or study sites;
- recruiting an adequate number of suitable patients to participate in a trial;
- having subjects complete a trial or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol or dropping out of a trial;
- addressing subject safety concerns that arise during the course of a trial;
- adding a sufficient number of clinical trial sites; or
- obtaining sufficient product supply of product candidates for use in preclinical studies or clinical trials from third-party suppliers.

We may experience numerous adverse or unforeseen events during, or as a result of, preclinical studies and clinical trials which could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- we may receive feedback from regulatory authorities that requires us to modify the design of our clinical trials or require that we submit additional data or information before allowing a clinical trial to be initiated;
- clinical studies of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements, fail to maintain adequate quality controls or be unable to provide us with sufficient product supply to conduct and complete preclinical studies or clinical trials of our product candidates in a timely manner, or at all;
- we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the quality of our product candidates or other materials necessary to conduct preclinical studies or clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements and add new requirements for approving our product candidates, or such requirements may not be as we anticipate; and

- any future collaborators may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

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

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

For example, we intend to seek FDA approval of tanruprubart for the treatment of patients with GBS. Our data package is based on completed placebo-controlled studies conducted in Southeast Asia. We filed a MAA with the EMA for tanruprubart for the treatment of GBS in January 2026, and we continue to engage with EU regulators through the MAA review process. Tanruprubart has been granted orphan designation from the EMA. Tanruprubart has also been granted Fast Track and orphan drug designation for the treatment of GBS from the FDA. The currently ongoing open-label FORWARD study in the U.S. and Europe is designed to broaden Western experience with tanruprubart. Eighteen patients have been treated to date, representing a broad spectrum of disease from moderate to severe in both male and female patients ranging in age from 12 to 78 years old. Functional outcomes, complete target engagement and a generally well-tolerated safety profile were observed in FORWARD, consistent with Phase 3 and real-world evidence studies. FORWARD findings provide additional evidence of the consistency and reproducibility of tanruprubart treatment effect across a broad range of GBS patients with different disease neurotypes, severities, ages and geographies, which further strengthen our comprehensive data package for tanruprubart in GBS. We plan to engage with the FDA with the goal of reaching alignment on our submission of a biologics license application, or BLA, anticipated in the fourth quarter of 2026. In our discussions to date with the FDA, the FDA has indicated that the generalizability package may not be sufficient to support BLA approval absent additional patient data. While we expect that the inclusion of U.S. and European results from the FORWARD study will support a BLA submission in GBS, it is possible the FDA determines that our current package, including the FORWARD study results, is not sufficient or requires us to provide additional data in GBS patients that are not feasible to obtain. Our inability to satisfy such additional requirements by the FDA may result in the FDA failing to approve tanruprubart in GBS, which would prevent the commercialization of tanruprubart in GBS in the United States, and limit the size of our commercial market opportunity and our potential future revenues, and could otherwise have a material adverse effect on our business.

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

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

Principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or a regulatory authority concludes that the financial relationship may have affected the interpretation of the trial, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of the marketing application we submit. Any such delay or rejection could prevent or delay us from commercializing our current or future product candidates.

If we experience delays in the completion, or termination, of any preclinical study or clinical trial of our product candidates, the commercial prospects of our product candidates may be harmed, and our ability to generate revenues from any of these product

candidates will be delayed or not realized at all. In addition, any delays in completing our clinical trials may increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may materially and adversely affect our business, financial condition, results of operations and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. If one or more of our product candidates proves to be ineffective, unsafe or commercially unviable, our business, financial condition, results of operations and prospects may be materially and adversely affected.

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

We may not be able to initiate or continue clinical trials on a timely basis or at all 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 the trials as required by applicable regulations or as needed to provide appropriate statistical power for a given trial. The timely completion of clinical trials in accordance with their protocols depends on, among other things, our ability to enroll a sufficient number of patients who remain in the study until its conclusion. We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The enrollment of patients depends on many factors, including:

- the severity and difficulty of diagnosing the disease under investigation;
- the patient eligibility and exclusion criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- the existing body of safety and efficacy data with respect to the study drug and safety concerns;
- patient referral practices of physicians;
- risk that enrolled subjects will drop out before completion of the trial, including as a result of health conditions or being forced to quarantine;
- ability to monitor patients adequately during and after treatment;
- availability and efficacy of approved medications or therapies, or other clinical trials, for the disease or condition under investigation;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating; and
- our ability to obtain and maintain patient consents.

In addition, our clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. In addition, patients may not opt to enroll in our trials because of the availability of approved therapeutics for their disease. Because the number of qualified clinical investigators is limited, we may conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site. Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

Adverse events or undesirable side effects caused by, or other unexpected properties of, any of our product candidates could halt their clinical development, delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. We have experienced adverse events during clinical trials, and may in the future experience, adverse or unforeseen events during, or as a result of, clinical trials. If unacceptable side effects arise in the development of our product candidates, we, the FDA, the IRBs at the institutions in which our studies are conducted or the DSMB could suspend or terminate our

clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete any of our clinical trials or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may materially and adversely affect our business, financial condition, results of operations and prospects.

In addition, early clinical trials may only include a limited number of subjects and limited duration of exposure to our product candidates. In particular, we are pursuing a novel approach to inhibiting upstream molecules of the classical complement pathway, primarily C1q, and as a result, our product candidates may cause unforeseen safety events when evaluated in larger patient populations. Further, clinical trials may not be sufficient to determine the effect and safety consequences of taking our product candidates over a multi-year period.

If any of our product candidates receives marketing approval, and we or others later identify undesirable and unforeseen side effects caused by such product, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- we may be required to conduct additional clinical trials or post-approval studies;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product or any component thereof;
- regulatory authorities may require the addition of labeling statements, such as a "black box" warning or a contraindication, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to implement a REMS or create a Medication Guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients;
- we may be subject to fines, injunctions or the imposition of criminal penalties;
- the product may become less competitive; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and result in the loss of significant revenues to us, which would materially and adversely affect our results of operations and business. In addition, if one or more of our product candidates prove to be unsafe, our business, financial condition, results of operations and prospects may be materially and adversely affected.

Interim, "top-line" and preliminary data from studies or 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 publicly disclose interim, "top-line" or preliminary data from preclinical studies or clinical trials. Interim data are subject to the risk that one or more of the outcomes may materially change as more data become available. 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 when we publish such data. As a result, the "top-line" results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results once additional data have been received and fully evaluated. "Top-line" or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, "top-line" and preliminary data should be viewed with caution until the final data are available. From time to time, we also disclose interim data from our clinical studies. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between interim, "top-line" or preliminary data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. 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 you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure. Any information we determine not to disclose may ultimately be deemed significant by you or others with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the "top-line," preliminary or interim data that we report differ from final results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, product candidates may be harmed, which could significantly harm our business prospects.

Even if our current or future product candidates obtain regulatory approval, they may fail to achieve the broad degree of physician and patient adoption and use necessary for commercial success.

Even if one or more of our product candidates receive FDA or other regulatory approvals, the commercial success of any of our current or future product candidates will depend significantly on the broad adoption and use of the resulting product by physicians and patients for approved indications. Our product candidates may not be commercially successful. For a variety of reasons, including, among other things, competitive factors, pricing or physician preference, reimbursement by insurers, the degree and rate of physician and patient adoption of our current or future product candidates, if approved, will depend on a number of factors, including:

- the clinical indications for which the product is approved and patient demand for approved products that treat those indications;
- the safety and efficacy of our product as compared to other available therapies; for example, with respect to vonaprument, physicians may prescribe or patients may prefer recently approved therapies for the treatment of GA;
- the availability of coverage and adequate reimbursement from managed care plans, insurers and other healthcare payors for any of our product candidates that may be approved;
- acceptance by physicians, operators of clinics and patients of the product as a safe and effective treatment;
- physician and patient willingness to adopt a new therapy over other available therapies to treat approved indications;
- overcoming any biases physicians or patients may have toward particular therapies for the treatment of approved indications;
- proper training and administration of our product candidates by physicians and medical staff;
- public misperception regarding the use of our therapies, if approved for commercial sale;
- patient satisfaction with the results and administration of our product candidates and overall treatment experience, including, for example, the convenience of any dosing regimen;
- the cost of treatment with our product candidates in relation to alternative treatments and reimbursement levels, if any, and willingness to pay for the product, if approved, on the part of insurance companies and other third-party payors, physicians and patients;
- the revenue and profitability that our products may offer a physician as compared to alternative therapies;
- the prevalence and severity of side effects;
- limitations or warnings contained in the FDA-approved labeling for our products;
- the willingness of physicians, operators of clinics and patients to utilize or adopt our products as a solution;
- any FDA requirement to undertake a REMS;
- the effectiveness of our sales, marketing and distribution efforts;
- adverse publicity about our products or favorable publicity about competitive products; and
- potential product liability claims.

We cannot assure you that our current or future product candidates, if approved, will achieve broad market acceptance among physicians and patients. Any failure by our product candidates that obtain regulatory approval to achieve market acceptance or commercial success would adversely affect our results of operations.

We have received Orphan Drug designation for tanruprubart for the treatment of GBS and HD in the United States and for GBS in Europe, and we may seek Orphan Drug designation for certain future product candidates. We may be unable to obtain such designations or to maintain the benefits associated with Orphan Drug designation, including market exclusivity, which may cause any revenue from product sales to be reduced.

We have received Orphan Drug designation in the United States for tanruprubart for the treatment of GBS and HD and for the treatment of GBS in Europe from the EMA. Although we may seek Orphan Drug designation for some or all of our other product candidates, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a drug or biologic product as an Orphan Drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan Drug designation must be requested before submitting a BLA, or new drug application, or NDA. In the EU, the EMA's Committee for Orphan Medicinal Products, or COMP, grants Orphan Drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the EU. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, Orphan Drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and application fee waivers. After the FDA grants Orphan Drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA.

In addition, if a product receives the first FDA approval for the indication 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 disease or 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 to the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective. In the EU, Orphan Drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the Orphan Drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable to not justify maintenance of market exclusivity.

Even if we obtain Orphan Drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain Orphan Drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an Orphan Drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective or makes a major contribution to patient care. Orphan Drug designation neither shortens the development time or regulatory review time of a drug or biologic nor gives the drug or biologic any advantage in the regulatory review or approval process.

A Breakthrough Therapy 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 it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek a Breakthrough Therapy designation for our product candidates if the clinical data support such a designation for one or more 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 may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs and biologics designated as breakthrough therapies by the FDA may also be eligible for priority review and rolling review of BLA or NDA submissions.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited FDA review procedures and

does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

A Fast Track designation by the FDA or PRIME designation by the EMA, 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 marketing approval.

The FDA has granted Fast Track designation for tanruprubart in GBS and for vonaprument in GA, and the EMA has granted PRIME designation for vonaprument in GA, and, in the future, we may seek Fast Track designation or PRIME designation for our product candidates. If a drug or biologic is intended for the treatment of a serious or life-threatening condition and the drug or biologic demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for Fast Track designation. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once a BLA or NDA is submitted, the application may be eligible for priority review. A Fast Track product candidate may also be eligible for rolling review, where the FDA may consider for review sections of the BLA or NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA or NDA, the FDA agrees to accept sections of the BLA or NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA or NDA. The FDA has broad discretion whether or not to grant this designation.

PRIME is a program launched by the EMA to enhance support for research on and development of medicines that have demonstrated preliminary safety and efficacy and thus the potential to target a significant unmet medical need and bring a major therapeutic advantage to patients. This regulatory program offers developers of promising medicines enhanced interaction and early dialogue with the EMA and is designed to optimize development plans and speed evaluation ensuring these medicines reach patients as early as possible. The EMA has broad discretion whether or not to grant this designation.

Even if we believe a particular product candidate is eligible for these designations, we cannot assure you that the FDA, EMA or similar regulatory agency would decide to grant them. Fast Track and PRIME designations may not result in a faster development process, review or approval compared to conventional FDA or EMA procedures, respectively. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. The Fast Track and PRIME designations do not assure ultimate regulatory approval by the FDA or the EMA. Many drugs and biologics that have received Fast Track or PRIME designation have failed to obtain approval.

Disruptions at the FDA and other government agencies or foreign regulatory authorities caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and/or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. 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 or comparable foreign regulatory authorities may also slow the time necessary for new drugs and 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 and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. Similar considerations are applicable to foreign regulatory authorities.

If a prolonged government shutdown occurs, or if new or existing global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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

We conduct clinical trials of our product candidates outside the United States, and plan to continue to do so. For example, we conducted our Phase 1b GBS clinical trial of tanruprubart in Bangladesh, and completed our Phase 3 GBS clinical trial of tanruprubart in Bangladesh and the Philippines. We are also conducting a global Phase 3 program for vonaprument in dry AMD with GA. The

acceptance of study data from clinical trials conducted outside the United States or the applicable jurisdiction by the FDA and comparable foreign regulatory authorities may be subject to certain conditions, or may not be accepted at all.

In particular, where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, regardless of whether such trials were conducted under an IND, the FDA will not approve the application on the basis of foreign data alone unless those data are applicable to the United States population and United States medical practice, the trials were performed by clinical investigators of recognized competence and the data are 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. For trials that are conducted only at sites outside of the United States and not subject to an IND, the FDA requires the clinical trial to have been conducted in accordance with good clinical practice, or GCP, requirements, and the FDA must be able to validate the data from the clinical trial through an on-site inspection if it deems such inspection necessary. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory bodies have similar approval requirements. In addition, foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA, EMA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials or the amendment of ongoing trials, which would be costly and time-consuming and delay aspects of our business plan or receiving approval or clearance for commercialization in the applicable jurisdiction.

To support data from clinical trials conducted in foreign jurisdictions, applicants may submit clinical evidence, clinical trials, patient registries or other sources of RWE, such as electronic health records or the collection of larger confirmatory data sets. In particular, because all of our studies to date for tanruprubart in GBS have been conducted at sites outside the United States, we conducted a RWE study to assess comparability of disease populations in the US and ex-US using a large natural history database from IGOS. Published data from IGOS presents baseline characteristics of GBS patients in various jurisdictions and patient outcomes at certain timepoints over the course of their disease. We filed a MAA with the EMA for tanruprubart for the treatment of GBS in January 2026, and we continue to engage with EU regulators through the MAA review process. Tanruprubart has been granted orphan designation from the EMA. Tanruprubart has also been granted Fast Track and orphan drug designation for the treatment of GBS from the FDA. The currently ongoing open-label U.S./EU FORWARD study in the U.S. and Europe is designed to broaden Western experience with tanruprubart. Eighteen patients have been treated to date, representing a broad spectrum of disease from moderate to severe in both male and female patients ranging in age from 12 to 78 years old. Functional outcomes, complete target engagement and a generally well-tolerated safety profile were observed in FORWARD, consistent with Phase 3 and real-world evidence studies. FORWARD findings provide additional evidence of the consistency and reproducibility of tanruprubart treatment effect across a broad range of GBS patients with different disease neurotypes, severities, ages and geographies, which further strengthen our comprehensive data package for tanruprubart in GBS. We plan to engage with the FDA with the goal of reaching alignment on our submission of a biologics license application, or BLA, anticipated in the fourth quarter of 2026. In our discussions with the FDA, the FDA has indicated that the generalizability package may not be sufficient to support BLA approval absent additional patient data. While we expect that the inclusion of U.S. and European results from the FORWARD study will support a BLA submission in GBS, it is possible the FDA determines that our current package, including the FORWARD study results, is not sufficient or requires us to provide additional data in GBS patients that are not feasible to obtain. Our inability to satisfy such additional requirements by the FDA may result in the FDA failing to approve tanruprubart in GBS, which would prevent the commercialization of tanruprubart in GBS in the United States, and limit the size of our commercial market opportunity and our potential future revenues, and could otherwise have a material adverse effect on our business.

In addition, we are conducting a global registrational program of vonaprument for the treatment of dry AMD with GA. To accomplish this objective, we must obtain and maintain regulatory approvals and comply with regulatory requirements in each jurisdiction. We are in ongoing discussions with the relevant regulatory authorities. While we believe we have designed a global clinical development program that could satisfy the regulators in all of our target markets, there is no assurance that our efforts will be successful or that the various regulators will be aligned or accept the data from the Phase 3 program are sufficient to warrant approval of vonaprument.

If the product candidates that we develop receive regulatory approval in the United States or another jurisdiction, they may never receive approval in other jurisdictions, which would limit market opportunities for our product candidates and adversely affect our business.

Approval of a product candidate in the United States by the FDA or by the requisite regulatory agencies in any other jurisdiction does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions. The approval process varies among countries and may limit our or any future collaborators' ability to develop, manufacture, promote and sell product candidates internationally. Failure to obtain marketing approval in international jurisdictions would prevent the product candidates from being marketed outside of the jurisdictions in which regulatory approvals have been received. In order to market and sell product candidates in the EU, and many other jurisdictions, we and any future collaborators must obtain separate marketing approvals and

comply with numerous and varying regulatory requirements. The approval procedure varies among countries and may involve additional preclinical studies or clinical trials both before and after approval. In many countries, any product candidate for human use must be approved for reimbursement before it can be approved for sale in that country. In some cases, the intended price for such product is also subject to approval. Further, while regulatory approval of a product candidate in one country does not ensure approval in any other country, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. If we or any future collaborators fail to comply with the regulatory requirements in international markets or to obtain all required marketing approvals, the target market for a particular potential product will be reduced, which would limit our ability to realize the full market potential for the product and adversely affect our business.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or the BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own pre-clinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

We rely on third-party suppliers to manufacture our product candidates, and we intend to rely on third parties to produce commercial supplies of any approved product. The loss of these suppliers, or their failure to comply with applicable regulatory requirements or to provide us with sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.

We do not have the ability, and we do not plan to build or acquire the infrastructure or capability internally, to manufacture supplies of our product candidates or the materials necessary to produce our product candidates for use in the conduct of our preclinical studies or clinical trials, and we lack the internal resources and the capability to manufacture any of our product candidates on a preclinical, clinical or commercial scale. The facilities used by our contract manufacturers to manufacture our product candidates are subject to various regulatory requirements and may be subject to the inspection of the FDA or other regulatory authorities. We do not control the manufacturing processes of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements, known as cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or comparable regulatory authorities in foreign jurisdictions, we may not be able to rely on their manufacturing facilities for the manufacture of our product candidates. In addition, we have limited control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority finds these facilities inadequate for the manufacture of our product candidates or if such facilities are subject to enforcement action in the future or are otherwise inadequate, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates.

We currently intend to supply our product candidates in all territories for our clinical development programs, and rely on third parties at key stages in our supply chain. For instance, the supply chains for our product candidates involve several manufacturers that specialize in specific operations of the manufacturing process, specifically, raw materials manufacturing, drug substance manufacturing and drug product manufacturing. As a result, the supply chain for the manufacturing of our product candidates is complicated, and we expect the logistical challenges associated with our supply chain to grow more complex as our product candidates are further developed.

We do not have any control over the process or timing of the acquisition or manufacture of materials by our manufacturers. We generally do not begin preclinical or clinical trials unless we believe we have access to a sufficient supply of a product candidate to complete such study. In addition, any significant delay in, or quality control problems with respect to, the supply of a product candidate, or the raw material components thereof, for an ongoing study could considerably delay completion of our preclinical or clinical trials, product testing and potential regulatory approval of our product candidates.

We have initiated discussions with contract manufacturers for the commercial supply of our product candidates. Although we intend to enter into such agreements prior to commercial launch of any of our product candidates, we may be unable to enter into any such agreement or do so on commercially reasonable terms, which could have a material adverse impact upon our business. Moreover, if there is a disruption to one or more of our third-party manufacturers' or suppliers' relevant operations, or if we are unable to enter into arrangements for the commercial supply of our product candidates, we will have no other means of producing our product candidates until they restore the affected facilities or we or they procure alternative manufacturing facilities or sources of supply. Our ability to progress our preclinical and clinical programs could be materially and adversely impacted if any of the third-party suppliers upon which we rely were to experience a significant business challenge, disruption or failure due to issues such as financial difficulties or bankruptcy, issues relating to other customers such as regulatory or quality compliance issues, or other financial, legal, regulatory or reputational issues. Additionally, any damage to or destruction of our third-party manufacturers' or suppliers' facilities or equipment may significantly impair our ability to manufacture our product candidates on a timely basis.

In addition, to manufacture our product candidates in the quantities we believe would be required to meet anticipated market demand, our third-party manufacturers would likely need to increase manufacturing capacity and we may need to secure alternative sources of commercial supply, which could involve significant challenges and may require additional regulatory approvals. In addition, the development of commercial-scale manufacturing capabilities may require us and our third-party manufacturers to invest substantial additional funds and hire and retain the technical personnel who have the necessary manufacturing experience. Neither we nor our third-party manufacturers may successfully complete any required increase to existing manufacturing capacity in a timely manner, or at all. If our manufacturers or we are unable to purchase the raw materials necessary for the manufacture of our product candidates on acceptable terms, at sufficient quality levels or in adequate quantities, if at all, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of such product candidates, if approved.

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

We currently do not have the ability to independently conduct preclinical studies or clinical trials that comply with the regulatory requirements known as GLP requirements or GCP requirements, respectively. The FDA and regulatory authorities in other jurisdictions require us to comply with GCP requirements for conducting, monitoring, recording and reporting the results of clinical trials, in order to ensure that the data and results are scientifically credible and accurate and that the trial subjects are adequately informed of the potential risks of participating in clinical trials. We rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct GLP-compliant preclinical studies and GCP-compliant clinical trials on our product candidates properly and on time. While we have agreements governing their activities, we control only certain aspects of their activities and have limited influence over their actual performance. The third parties with whom we contract for execution of our GLP-compliant preclinical studies and our GCP-compliant clinical trials play a significant role in the conduct of these studies and the subsequent collection and analysis of data. These third parties are not our employees and, except for restrictions imposed by our contracts with such third parties, we have limited ability to control the amount or timing of resources that they devote to our programs. Although we rely on these third parties to conduct our GLP-compliant preclinical studies and GCP-compliant clinical trials, we remain responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

Many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If the third parties conducting our preclinical studies or our clinical trials do not adequately perform their contractual duties or obligations, experience significant business challenges, disruptions or failures, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the data they obtain is compromised due to their failure to adhere to our protocols or to GLPs or GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. This could be difficult, costly or impossible, and our preclinical studies or clinical trials may need to be extended, delayed, terminated or repeated. As a result we may not be able to obtain regulatory approval in a timely fashion, or at all, for the applicable product candidate, our business, financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If we are not successful in identifying, developing and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives would be impaired.

Although a substantial amount of our effort will focus on the continued development and potential approval of our current product candidates, a key element of our strategy is to identify, develop and commercialize a portfolio of products that address classical complement-mediated autoimmune and neurodegenerative diseases. A component of our strategy is to evaluate our product candidates

in multiple indications based, in part, on our evaluation of certain biomarkers in a disease area. For example, we intend to evaluate tanruprubart in neurodegenerative diseases, including ALS and HD; however, we are continuing to evaluate tanruprubart in these patient populations, and even if we believe we have obtained positive clinical results in patients with one of these neurodegenerative diseases, such results may not be replicated in later studies evaluating tanruprubart in patients with the same disease or across other neurodegenerative or autoimmune diseases. Even though we are currently developing a pipeline of product candidates, our development efforts may still fail to yield product candidates potentially suitable for commercialization for many reasons, including the following:

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

We therefore cannot provide any assurance that we will be able to successfully identify or acquire additional product candidates, advance any of these additional product candidates through the development process, successfully commercialize any such additional product candidates, if approved, or assemble sufficient resources to identify, acquire, develop or, if approved, commercialize additional product candidates. If we are unable to successfully identify, acquire, develop and commercialize additional product candidates, our commercial opportunities may be limited.

We face significant competition in an environment of rapid technological and scientific change, and our product candidates, if approved, will face significant competition, which may prevent us from achieving significant market penetration. Most of our competitors have significantly greater resources than we do, and we may not be able to successfully compete.

The pharmaceutical, biopharmaceutical and biotechnology industries in particular are characterized by rapidly advancing technologies, intense competition and a strong emphasis on developing proprietary therapeutics. Numerous companies are engaged in the development, patenting, manufacturing and marketing of healthcare products competitive with those that we are developing. We face competition from a number of sources, such as pharmaceutical, biopharmaceutical and biotechnology companies, generic drug companies and academic and research institutions, many of which have greater financial resources, marketing capabilities, sales forces, manufacturing capabilities, research and development capabilities, clinical trial expertise, intellectual property portfolios, experience in obtaining patents and regulatory approvals for product candidates and other resources than we do. Some of the companies also have a broad range of other product offerings, large direct sales forces and long-term customer relationships with our target physicians, which could inhibit our market penetration efforts. Mergers and acquisitions in the pharmaceutical, biopharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Certain alternative treatments offered by competitors may be available at lower prices and may offer greater efficacy or better safety profiles. Furthermore, currently approved products could be discovered to have application for the intended indication of our product candidates, which could give such products significant regulatory and market timing advantages over any of our product candidates. Our competitors also may obtain FDA, EMA, or other regulatory approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications our product candidates are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. For example, with respect to vonaprument, there are two approved products for GA which may pose competition for vonaprument, if approved. For additional information regarding our competition, see the section titled "Business—Competition" in our Annual Report on Form 10-K for the year ended December 31, 2025.

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish adequate coverage, reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and adequacy of coverage and reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford prescription medications such as our product candidates, if approved. Our ability to achieve acceptable levels of coverage and reimbursement for products by

governmental authorities, private health insurers and other organizations will have an effect on our ability to successfully commercialize our product candidates. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Even if we obtain coverage for our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. We cannot be sure that coverage and reimbursement in the United States, the EU or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical, biopharmaceutical and biotechnology products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. For example, the U.S. Department of Health and Human Services, or HHS, imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation. In addition, HHS has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least 7 years and single-source biologics that have been on the market for at least 11 years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop, which could have an adverse effect on our operating results and our overall financial condition.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice, and we believe that changes in these rules and regulations are likely.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other foreign jurisdictions have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amounts that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products, and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed health care, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

We currently have no sales organization. If we are unable to establish sales capabilities on our own or through third parties, we may not be able to market and sell our product candidates, if approved, effectively in the United States and foreign jurisdictions or generate product revenue.

We currently do not have a marketing or sales organization. In order to commercialize our product candidates in the United States and foreign jurisdictions, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make

arrangements with third parties to perform these services, and we may not be successful in doing so. We have no prior experience in the marketing, sale and distribution of pharmaceutical, biopharmaceutical and biotechnology products, and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. To build on our current resources and limited commercial experience, we intend to establish third-party relationships to successfully commercialize our products. The near and long-term commercial viability of our product candidates, if approved, may depend, in part, on our ability to successfully establish strategic collaborations with contract commercial organizations, pharmaceutical and biotechnology companies, non-profit organizations, and government agencies. Establishing and maintaining strategic collaborations is difficult and time-consuming. Potential collaborators may reject collaborations based upon their assessment of our financial, regulatory or intellectual property position or based on their internal pipeline or available resources. If we are unable to enter into such arrangements on acceptable terms or at all, we may not be able to successfully commercialize our product candidates. Even if we enter into collaborations with contract commercial organizations, there can be no assurances that it will ever result in the successful commercialization of any products for several reasons, including that:

- we may not have the ability to control the activities of our partners and cannot provide assurance that they will fulfill their obligations to us in a timely manner or at all;
- such partners may not devote sufficient resources to the commercialization of our products, if approved, properly maintain or defend our intellectual property rights (if required);
- relationships with our collaborators could also be subject to certain fraud and abuse laws if not structured properly to comply with such laws; and
- any failure on the part of our partners to perform or satisfy their obligations to us could lead to delays in the commercialization of our product candidates and affect our ability to realize product revenue.

If we fail to establish agreements as necessary, we could be required to undertake commercialization activities solely at our own expense. These activities would significantly increase our capital requirements and, given our lack of sales, marketing and distribution capabilities, significantly delay or hinder our commercial success. If we are not successful in commercializing our product candidates or any future product candidates, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses.

We will need to increase the size of our organization, and we may experience difficulties in managing growth.

As of September 30, 2026, we had 97 full-time employees. We will need to continue to expand our managerial, operational, finance and other resources in order to manage our operations and clinical trials, continue our development activities and commercialize our product candidates or any future product candidates. Our management and personnel, systems and facilities currently in place may not be adequate to support our growth. Our need to effectively execute our growth strategy requires that we:

- manage our clinical trials effectively;
- successfully commercialize tanruprubart and any of our other product candidates, if approved;
- identify, recruit, retain, incentivize and integrate additional employees, including sales personnel;
- manage our internal development and operational efforts effectively while carrying out our contractual obligations to third parties; and
- continue to improve our operational, financial and management controls, reports systems and procedures.

If management is unable to effectively manage our growth, our expenses may increase more than expected. Our future financial performance and our ability to compete effectively will depend, in part, on our ability to effectively manage any future growth.

If we fail to attract and retain senior management and key scientific personnel, our business may be materially and adversely affected.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management and clinical and scientific personnel. We are highly dependent upon members of our senior management, as well as our senior scientists. The loss of services of any of these individuals could delay or prevent the successful development of our product pipeline, initiation or completion of our planned clinical trials or the commercialization of our product candidates or any future product candidates.

We cannot guarantee that we will not face turnover in the future. Our ability to execute our business strategies may be adversely affected by the uncertainty associated with any transition and the time and management attention needed to fill any vacant role could disrupt our business.

Competition for qualified personnel in the pharmaceutical, biopharmaceutical and biotechnology field is intense due to the limited number of individuals who possess the skills and experience required by our industry. We will need to hire additional personnel as we expand our clinical development and if we initiate commercial activities. We may not be able to attract and retain quality personnel on acceptable terms, or at all. In addition, to the extent we hire personnel from competitors, we may be subject to allegations that they have been improperly solicited or that they have divulged proprietary or other confidential information, or that their former employers own their research output.

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

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

- decreased demand for our current or future product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- regulatory investigations, product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue; and
- the inability to commercialize our current or any future product candidates.

If we are unable to obtain and maintain sufficient product liability insurance at an acceptable cost and scope of coverage to protect against potential product liability claims, the commercialization of our current or any future product candidates we develop could be inhibited or prevented. We currently carry product liability insurance covering our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions and deductibles, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient funds to pay such amounts. Moreover, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses. If and when we obtain approval for marketing any of our product candidates, we intend to expand our insurance coverage to include the sale of such product candidate; however, we may be unable to obtain this liability insurance on commercially reasonable terms or at all.

Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our product candidates.

While we have not entered into any collaboration agreements to date, we may seek collaboration arrangements for the commercialization, or potentially for the development, of certain of our product candidates depending on the merits of retaining commercialization rights for ourselves as compared to entering into collaboration arrangements. For example, certain of the disease areas that we believe our product candidates address, including, among others, ophthalmic indications, require large, costly and later-stage clinical trials, which a collaboration partner may be better positioned to finance and/or conduct. In addition, a component of our strategy is to maximize the commercial value of our current and future product candidates, which may also strategically align with partnering commercial rights with partners that have larger and established sales organizations. To the extent that we decide to enter

into collaboration agreements, we may face significant competition for appropriate collaborators. Moreover, collaboration arrangements are complex and time-consuming to negotiate, document, implement and maintain and challenging to manage. We may not be successful in our efforts to enter into collaboration agreements. The terms of collaborations or other arrangements that we may establish may not be favorable to us.

The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include risks that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in their strategic focus due to their acquisition of competitive products or their internal development of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial, 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 our products or product candidates;
- collaborators with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and collaborators that cause the delay or termination of the research, development or commercialization of our current or future product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, this may result in a need for additional capital to pursue further development or commercialization of the applicable current or future product candidates;
- collaborators may own or co-own intellectual property covering products that result from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property;
- disputes may arise with respect to the ownership of any intellectual property developed pursuant to our collaborations; and
- collaborators' sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

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

Our business is susceptible to general conditions in the global economy and in the global financial markets. Global financial crises and global or regional political disruptions have caused, and could in the future cause, extreme volatility in the capital and credit markets. A severe or prolonged economic downturn, including a recession or depression, recent and potential bank failures, the current inflationary economic environment, rising interest rates, debt and equity market fluctuations, diminished liquidity and credit availability, increased unemployment rates, funding shortages at governmental and regulatory agencies on which we rely, including regulatory agencies, decreased investor and consumer confidence, supply chain challenges, natural catastrophes, the effects of climate change, regional and global conflicts and terrorist attacks or political disruption or turmoil could result in a variety of risks to our business, including weakened demand for our product candidates or any future product candidates, if approved, and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy or political disruption could also strain our manufacturers or suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our potential products. Any of the foregoing could materially and adversely affect our business, financial condition, results of operations and prospects, and we cannot anticipate all of the ways in which the political or economic climate and financial market conditions could adversely impact our business.

International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and growth prospects.

We operate in a global economy, which includes utilizing third-party suppliers in several countries outside the United States. The current international trade and regulatory environment is subject to significant ongoing uncertainty, and there is inherent risk that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations.

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates for clinical testing, as well as for manufacture of any products that we may commercialize, if approved. Currently, several of our suppliers are located outside of the United States, and our product candidates, tanruprubart and vonaprument, are manufactured in Europe. We also rely on specialized laboratory equipment, supplies, materials, and precursor compounds, all or part of which we believe may be ultimately sourced from multiple countries outside the United States, to advance our research and development efforts.

Current or future tariffs may result in increased research and development expenses, including with respect to increased costs associated with raw materials, laboratory equipment and research materials and components. In addition, such tariffs may increase our supply chain complexity, potentially disrupt our existing supply chain, and could result in delays to our development timelines. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly.

The ultimate impact of current or future tariffs and trade restrictions remains uncertain. While we actively monitor these risks, any escalation in trade tensions could materially and adversely affect our business, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this Quarterly Report on Form 10-Q.

We or the third parties upon whom we depend on may be adversely affected by earthquakes or other natural disasters, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our corporate headquarters and other facilities are located in the San Francisco Bay Area, which has experienced both severe earthquakes and the effects of wildfires. We do not carry earthquake insurance. Earthquakes, wildfires or other natural disasters could severely disrupt our operations, and could materially and adversely affect 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 or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

Furthermore, integral parties in our supply chain are similarly vulnerable to natural disasters or other sudden, unforeseen and severe adverse events. If such an event were to affect our supply chain, it could have a material adverse effect on our business.

Our employees and independent contractors, including principal investigators, consultants, any future commercial collaborators, service providers and other vendors, may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk that our employees and independent contractors, including principal investigators, consultants, any future commercial collaborators, service providers and other vendors may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate the laws and regulations of the FDA and other similar regulatory bodies, including those laws that require the reporting of true, complete and accurate information to such regulatory bodies; manufacturing standards; U.S. federal and state healthcare fraud and abuse, data privacy laws and other similar non-U.S. laws; or laws that require the true, complete and accurate reporting of financial information or data. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials, or illegal misappropriation of product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, 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. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid and other U.S. healthcare programs, other sanctions, imprisonment, contractual damages, reputational harm, diminished profits and future earnings and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Our business involves the use of hazardous materials, and we and our third-party manufacturers and suppliers must comply with environmental laws and regulations, which can be expensive and restrict how we do business.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use and disposal of hazardous materials owned by us, including the components of our product candidates and other hazardous compounds. We and any third-party manufacturers and suppliers 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 and biological and radioactive materials. Our operations also produce hazardous waste. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, and environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products.

We cannot guarantee that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials comply with the standards prescribed by these laws and regulations, nor can we eliminate the risk of accidental contamination or injury from these materials. 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. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources, and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research, product development and manufacturing efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from hazardous 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. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, results of operations and financial condition.

Risks Related to Intellectual Property

Our current and any future product candidates or products could be alleged to infringe patent rights and other proprietary rights of third parties, which may require costly litigation and, if we are not successful, could cause us to pay substantial damages and/or limit our ability to commercialize our products.

Our commercial success depends on our ability to develop, manufacture and market our current and any future product candidates that may be approved for sale, and to use our proprietary technology without infringing the patents and other proprietary rights of third parties. Intellectual property disputes can be costly to defend and may cause our business, results of operations and financial condition to suffer. We operate in an industry with extensive intellectual property litigation. As the pharmaceutical, biopharmaceutical and biotechnology industries expand and more patents are issued, the risk increases that there may be patents issued to third parties that relate to our products and technology of which we are not aware or that we may need to challenge to continue our operations as currently contemplated.

Whether merited or not, we may face allegations that we have infringed the trademarks, copyrights, patents and other intellectual property rights of third parties, including patents held by our competitors or by non-practicing entities. We may also face allegations that our employees have misappropriated the intellectual property rights of their former employers or other third parties. Litigation may make it necessary to defend ourselves by determining the scope, enforceability and validity of third-party proprietary rights, or to

establish our proprietary rights. Regardless of whether claims that we are infringing patents or other intellectual property rights have merit, such litigation can be time consuming, divert management attention and financial resources and are costly to evaluate and defend. There can be no assurance with respect to the outcome of any current or future litigation brought by or against us, and the outcome of any such litigation could have a material adverse impact on our business, results of operations and financial condition. Litigation is inherently unpredictable, and outcomes are uncertain. Further, as the costs and outcome of such litigation can vary significantly, it is difficult to estimate potential losses that may occur. As a result of such litigation, we may be required to stop treating certain conditions, obtain licenses or modify our products and features while we develop non-infringing substitutes, or may result in significant settlement costs or royalty obligations. For example, litigation can involve substantial damages for infringement, and if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees. We may also be prohibited from selling or licensing our products unless the third-party licenses rights to us, which it is not required to do at a commercially reasonable price or at all. If a license is available from a third party, we may have to pay substantial royalties or upfront fees or grant cross-licenses to intellectual property rights for our products. We may also have to redesign our products so they do not infringe third-party intellectual property rights, which may not be possible or may require substantial monetary expenditures and time, during which our products may not be available for manufacture, use or sale. Accordingly, we are unable at this time to estimate the effects of these potential future lawsuits on our financial condition, operations or cash flows.

Additionally, some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock. Any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

Although we have reviewed certain third-party patents and patent filings that we believe may be relevant to our product candidates, we have not conducted a comprehensive freedom-to-operate search or analysis for any of our product candidates, and we may not be aware of patents or pending or future patent applications that, if issued, would block us from commercializing our product candidates. Additionally, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to commercialize our product candidates. Thus, we cannot guarantee that our activities related to their product candidates, or our commercialization, do not and will not infringe any third party's intellectual property.

In addition, patent applications in the United States and many other jurisdictions are typically not published until 18 months after the filing of certain priority documents (or, in some cases, are not published until they issue as patents), and publications in the scientific literature often lag behind actual discoveries. Therefore, we cannot be certain that others have not filed patent applications or made public disclosures relating to our technology or our contemplated technology. A third party may have already filed, and may in the future file, patent applications covering our product candidates or technology similar to ours. Any such patent application may have priority over our patent applications or patents, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, depending on whether the timing of the filing date falls under certain patent laws, we may have to participate in a priority contest (such as an interference proceeding) declared by the United States Patent and Trademark Office, or the USPTO, to determine priority of invention in the United States. The costs of patent litigation and other proceedings related to the protection of our global patent position could be substantial, and it is possible that such efforts would be unsuccessful, resulting in a loss of our patent position with respect to such invention.

If we are unable to obtain, maintain and enforce intellectual property protection directed to our current and any future technologies that we develop, others may be able to make, use or sell products substantially the same as ours, which could adversely affect our ability to compete in the market.

The market for pharmaceuticals and biopharmaceuticals is highly competitive and subject to rapid technological change. Our success depends, in part, upon our ability to maintain a competitive position in the development and protection of technologies and any future products for use in these fields and upon our ability to obtain, maintain and enforce our intellectual property rights. We seek to obtain and maintain patents and other intellectual property rights to restrict the ability of others to market products that misappropriate our technology and/or infringe our intellectual property to unfairly and illegally compete with any future products. If we are unable to protect our intellectual property and proprietary rights, our competitive position and our business could be harmed, as third parties may be able to make, use or sell products that are substantially the same as any future products we may sell without incurring the sizeable development and licensing costs that we have incurred, which would adversely affect our ability to compete in the market.

We use a combination of patents, trademarks, know-how, confidentiality procedures and contractual provisions to protect our proprietary technology. However, these protections may not be adequate and may not provide us with any competitive advantage. For

example, patents may not issue from any of our currently pending or any future patent applications, and our issued patents and any future patents that may issue may not survive legal challenges to their scope, validity or enforceability, or provide significant protection for us.

We have not pursued or maintained, and may not pursue or maintain in the future, patent protection for our product candidates in every country or territory in which we may sell our products. In addition, we cannot be sure that any of our pending patent applications or pending trademark applications will issue or that, if issued, they will issue in a form that will provide adequate protection for our products. The USPTO, patent offices in other jurisdictions, or judicial or other bodies in any jurisdiction may deny or significantly narrow claims made under our patent applications, and claims in our issued patents may be invalidated, may be designed around or may otherwise be unable to provide us with protection for our products. Further, the USPTO, trademark offices in other jurisdictions, or judicial or other bodies in any jurisdiction may deny our trademark applications and, even if published or registered, these trademarks may not effectively protect our brand and goodwill. Like patents, trademarks also may be successfully opposed or challenged.

We cannot be certain that the steps we have taken will prevent unauthorized use or unauthorized reverse engineering of our technology that is material to our business. Moreover, third parties may independently develop technologies that are competitive with ours and such competitive technologies may or may not infringe our intellectual property. The enforcement of our intellectual property rights also depends on the success of any legal actions we may take against these third parties in the respective country or forum, but these actions may not be successful. As with all granted intellectual property, such intellectual property may be challenged, invalidated or circumvented, may not provide protection and/or may not prove to be enforceable in actions against specific alleged infringers.

If we or any future collaborators we may have were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates or future product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including obviousness or lack of novelty, enablement or written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before the USPTO even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to validity, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution, or that a defendant would not prevail on an assertion of invalidity based on prior art that we were aware of during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

Even if claims in our patents survive assertions of invalidity and unenforceability, they may not be broad enough to prevent others from marketing products similar to ours or designing around our patents. For example, third parties may be able to make products that are similar to ours but that are not covered by the claims of our patents. The claims of our issued patents or patent applications when issued may not cover our product candidates or any future products that we develop.

Patent law can be highly uncertain and involve complex legal and factual questions for which important principles remain unresolved. In the United States and in many other jurisdictions, policies regarding the breadth of claims allowed in patents can be inconsistent. The U.S. Supreme Court and the Court of Appeals for the Federal Circuit have made, and will likely continue to make, changes in how the patent laws of the United States are interpreted. Similarly, courts in other jurisdictions have made, and will likely continue to make, changes in how the patent laws in their respective jurisdictions are interpreted. We cannot predict future changes in the interpretation of patent laws or changes to patent laws that might be enacted into law by U.S. and international legislative bodies. Those changes may materially affect the patents and patent applications of our licensors, our existing or future patents and patent applications and our ability to obtain additional patents in the future.

Patent reform legislation in the United States could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For example, on September 16, 2011, the Leahy-Smith America Invents Act, or Leahy-Smith Act, was signed into law. The Leahy-Smith Act included a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art, may affect patent litigation and switch the U.S. patent system from a "first-to-invent" system to a "first-to-file" system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO has developed regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, only became effective on March 16, 2013. The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, which could have a material adverse effect on our business and financial condition. Any future changes in the patent laws of the United States, or even the possibility of such changes, may further increase these uncertainties and costs.

The USPTO and various patent agencies in other jurisdictions require compliance with a number of procedural, documentary, fee, annuity payment and other provisions to maintain patent applications and issued patents. Although 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 with these requirements can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction.

In addition, we have a number of patents and patent applications outside of the United States and expect to continue to pursue patent protection in many of the significant markets in which we intend to do business. The laws of some international jurisdictions do not protect intellectual property rights to the same extent as laws in the United States, and many companies have encountered significant difficulties in obtaining, protecting and defending such rights in certain jurisdictions outside of the United States. If we encounter such difficulties or we are otherwise precluded from effectively protecting our intellectual property rights in international jurisdictions, our business, financial condition, results of operations and prospects could be materially and adversely affected. Earlier patent filings in certain international countries may also permit third parties to allege priority to certain technology in those countries.

Patent terms may be shortened or lengthened in certain jurisdictions by, for example, terminal disclaimers, patent term adjustments, supplemental protection certificates and patent term extensions. PTEs and supplemental protection certificates, and the like, may be impacted by the regulatory process and may not significantly lengthen patent term. Non-payment or delay in payment of patent extension filing (including any patent term extension or adjustment filing) fees, whether intentional or unintentional, may also result in the loss of patent rights important to our business. Certain countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to other parties. In addition, many countries limit the enforceability of patents against other parties, including government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of any patents.

Monitoring unauthorized use of our intellectual property is difficult and costly. From time to time, we review our competitors' products, and may in the future seek to enforce our patents or other rights against potential infringement. However, the steps we have taken to protect our proprietary rights may not be adequate to prevent misappropriation of our intellectual property. We may not be able to detect unauthorized use of, or take appropriate steps to enforce, our intellectual property rights. Our competitors may also independently develop similar technology. Any inability to meaningfully protect our intellectual property could result in competitors offering products competitive to our products. In addition, we may need to defend our patents from third-party challenges, such as interferences, derivation proceedings, re-examination proceedings, post-grant review, inter partes review, third-party submissions, oppositions, nullity actions or other patent proceedings. We may need to initiate infringement claims or litigation.

Adverse proceedings such as litigation can be expensive, time consuming and may divert the efforts of our technical and managerial personnel, which could in turn materially and adversely affect our business, financial condition, results of operations and prospects, whether or not we receive a determination favorable to us. In addition, in an infringement proceeding, a court or other judicial body may decide that the patent we seek to enforce is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that the patent in question does not cover the technology in question or that stopping the other party would harm the public interest. An adverse result in any litigation could put one or more of our patents at risk of being invalidated or interpreted narrowly. Some of our competitors may be able to devote significantly more resources to intellectual property litigation, and may have significantly broader patent portfolios to assert against us if we assert our rights against them. Further, because of the substantial discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be disclosed or otherwise compromised during litigation.

We may not be able to correctly estimate or control our future operating expenses in relation to obtaining intellectual property, enforcing intellectual property and/or defending intellectual property, which could affect operating expenses. Our operating expenses may fluctuate significantly in the future as a result of a variety of factors, including the costs of preparing, filing, prosecuting, defending and enforcing patent and trademark claims and other intellectual property-related costs, including adverse proceedings and litigation costs.

If we are unable to prevent disclosure of our trade secrets or other confidential information to third parties, our competitive position may be impaired.

In addition to the protection afforded by patents, we rely on confidentiality agreements to protect confidential information and proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and other confidential information by maintaining

physical security of our premises and physical and electronic security of our information technology systems. Agreements or security measures may be breached and detecting the disclosure or misappropriation of confidential information and enforcing a claim that a party illegally disclosed or misappropriated confidential information is difficult, expensive and time-consuming, and the outcome is unpredictable. Further, we may not be able to obtain adequate remedies for any breach. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position. Furthermore, the laws of some other jurisdictions do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and in other jurisdictions. In addition, our confidential information may otherwise become known or be independently discovered by competitors, in which case we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. The failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We license patent rights from third-party owners. Such licenses may be subject to early termination if we fail to comply with our obligations in our licenses with third parties, which could result in the loss of rights or technology that are material to our business.

We are or may become a party to licenses that give us rights to third-party intellectual property that are necessary or useful for our business, and we may enter into additional licenses in the future. Under these license agreements, we are or may become obligated to pay the licensor fees, which may include annual license fees, milestone payments, royalties, a percentage of revenues associated with the licensed technology and a percentage of sublicensing revenue. These fees may be significant, which could make it difficult for us to achieve or maintain profitability. In addition, under certain of such agreements, we are or may become required to diligently pursue the development of products using the licensed technology. If we fail to comply with these obligations and fail to cure our breach within a specified period of time, the licensor may have the right to terminate the applicable license, in which event we could lose valuable rights and technology that are material to our business, harming our ability to develop, manufacture and/or commercialize our platform or product candidates. If the licensor retains control of prosecution of the patents and patent applications licensed to us, we may have limited or no control over the manner in which the licensor chooses to prosecute or maintain its patents and patent applications and have limited or no right to continue to prosecute any patents or patent applications that the licensor elects to abandon.

The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

Our intellectual property agreements with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

Certain provisions in our intellectual property agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could affect the scope of our rights to the relevant intellectual property or technology, or affect financial or other obligations under the relevant agreement, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact conceives or develops intellectual property that we regard as our own. Our assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

We may wish to form collaborations in the future with respect to our product candidates, but may not be able to do so or to realize the potential benefits of such transactions, which may cause us to alter or delay our development and commercialization plans.

Our product candidates may also require specific components to work effectively and efficiently, and rights to those components may be held by others. Any delays in entering into new collaborations or strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies, which could harm our business prospects, financial condition and results of operations.

We jointly own certain patent rights with third parties. Our ability to out-license these patent rights, or to prevent the third party from out-licensing these patent rights, may be limited in certain countries.

We jointly own certain patents and patent applications with third parties, and may jointly own patents and patent applications with third parties in the future. Unless we enter into an agreement with the joint owner, we will be subject to certain default rules pertaining to joint ownership. Certain countries require the consent of all joint owners to license jointly owned patents, and if we are unable to obtain such consent from the joint owner, we may not be able to license our rights under these patents and patent applications. In certain other countries, including the United States, the joint owner could license its rights under these patents and patent applications to another party without our consent and without any duty of accounting to us.

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

We may also be subject to claims that former employees, any future collaborators or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights and could even face litigation for infringing patents that we had regarded as ours. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distraction to management and other employees.

We or our licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as national governments, such that we or our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our patents, including in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

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

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented, declared generic or conflict with third-party rights. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition with potential partners, physicians or patients in our markets of interest. During trademark registration proceedings, our trademark applications may be rejected. Although we are given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties can oppose pending trademark applications and seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Furthermore, third parties may file first for our trademarks in certain countries. If they succeeded in registering such trademarks, and if we are not successful in challenging such third-party rights, we may not be able to use these trademarks to market our future products in those countries. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. In such cases, over the long term, if we are unable to establish and maintain name recognition based on our trademarks and trade names, then our commercial success abilities may be impacted.

Moreover, any name we propose to use with our product candidates in the United States or any other country must be approved by the FDA, EMA or any other relevant health authority regardless of whether we have registered it, or applied to register it, as a trademark. The FDA as well as EMA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA, EMA or any other relevant approval authority 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 substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA, EMA or any other relevant approval authority.

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

Filing and prosecuting patent applications, and defending patents, related to our product candidates in all countries throughout the world would be prohibitively expensive, and the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent protection but

enforcement is not as strong as that in the United States. These products may compete with any future products we may sell, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

Risks Related to Government Regulation

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

If our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies and submission of safety, efficacy and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMPs and adherence to commitments made in any approved marketing application. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

We will have to comply with requirements concerning advertising and promotion for any future products. Promotional communications with respect to prescription drugs and biologics are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. We may not promote products for indications or uses for which they do not have approval. The holder of an approved application must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety, purity, potency and/or efficacy of our products in general or in specific patient subsets. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from any future products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our results of operations will be adversely affected.

Moreover, the policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, 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 be subject to enforcement action and we may not achieve or sustain profitability.

Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the EU and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers.

Since its enactment, there have been amendments and judicial, Congressional and executive branch challenges to certain aspects of the ACA. For example, on July 4, 2025, the One Big Beautiful Bill Act, or the OBBBA, was signed into law, which narrowed access to ACA marketplace exchange enrollment and declined to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired ACA subsidies. It is unclear how these and other healthcare reform measures will impact our business.

In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review the relationship between pricing and manufacturer patient programs.

The current administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, the Centers for Medicare & Medicaid Services, and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturer to offer, through a direct-to-consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directives to reduce agency workforce and cut programs; (2) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation, or CMMI, to consider new payment and healthcare models to limit drug spending; (3) imposing tariffs on certain imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, the U.S. Supreme Court's Loper Bright decision greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, on June 15, 2026, the FDA approved Colorado's Section 804 Importation Program, or SIP, proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this and Florida's similar program, approved by the FDA in 2024, will be implemented and whether they will overcome potential legal, regulatory, or industry challenges in the United States and/or Canada. Legally mandated price controls on payment amounts by third-party payors or other restrictions could materially and adversely affect our business, financial condition, results of operations and

prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the EU, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the EU, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and EU, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or judicial action in the United States, the EU or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

If we develop a small molecule product candidate that obtains regulatory approval, additional competitors could enter the market with generic versions of such drugs, which may result in a material decline in sales of affected products.

Under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, a pharmaceutical manufacturer may submit an abbreviated new drug application, or ANDA, seeking approval of a generic version of an approved, small molecule innovator product. Under the Hatch-Waxman Act, a manufacturer may also submit an NDA under section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act that references the FDA's prior findings of safety and effectiveness of the small molecule innovator product. For example, a 505(b)(2) NDA product may be for a new or improved version of the original innovator product. The Hatch-Waxman Act also provides for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA filing and review) of an ANDA or 505(b)(2) NDA. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the Orange Book. If there are patents listed in the Orange Book for a product, a generic or 505(b)(2) applicant that seeks to market its product before expiration of the patents must include in their applications what is known as a "Paragraph IV" certification, challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the patent owner and NDA holder and if, within 45 days of receiving notice, either the patent owner or NDA holder sues for patent infringement, approval of the ANDA or 505(b)(2) NDA is stayed for up to 30 months.

Accordingly, if our small molecule program results in a product that is approved, competitors could submit ANDAs for generic versions of our small molecule drug products or 505(b)(2) NDAs that reference our small molecule drug products. If there are patents listed for our small molecule drug products in the Orange Book, those ANDAs and 505(b)(2) NDAs would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. We cannot predict which, if any, patents in our current portfolio or patents we may obtain in the future will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents, or the outcome of any such suit.

We may not be successful in securing or maintaining proprietary patent protection for products and technologies we develop or license. Moreover, if any of our owned or in-licensed patents that are listed in the Orange Book are successfully challenged by way of a Paragraph IV certification and subsequent litigation, the affected product could immediately face generic competition and its sales would likely decline rapidly and materially.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include, without limitation:

- the U.S. federal civil and criminal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs 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 U.S. federal false claims laws, including the False Claims Act, which can be enforced through whistleblower actions, and civil monetary penalties laws, which, among other things, impose criminal and civil penalties against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval 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 U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the Health Insurance Portability and Accountability Act of 1996, which imposes criminal and civil liability for, 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 U.S. 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;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the U.S. Physician Payments Sunshine Act and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the government information related to certain payments and other transfers of value to physicians, as defined by such law, certain non-physician providers 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;
- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state and local laws that require licenses to manufacture or distribute our products commercially and/or the registration of pharmaceutical sales representatives in the jurisdiction; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; and
- the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office and foreign political parties or officials thereof.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices such

as the provision of stock options to physicians who may influence the ordering, prescribing or use of our product candidates, if approved, as compensation for consulting services, 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 of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. Further, defending against any such actions can be costly and time-consuming and may require significant personnel resources. Even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

Risks Related to Our Common Stock

Our stock price has been volatile, and could in the future be volatile, and you may not be able to resell shares of our common stock at or above the price you paid.

The stock price of our common stock has been, and could in the future be, subject to substantial volatility and wide fluctuations in response to various factors, some of which are beyond our control. In particular, the stock prices for pharmaceutical, biopharmaceutical and biotechnology companies have been highly volatile as a result of extreme volatility and disruptions in the global economy, including rising inflation and interest rates, tariffs, declines in economic growth, international conflicts and uncertainty about economic stability, including a potential recession. These factors include those discussed in this "Risk Factors" section and others such as:

- results from, and delays in, our clinical trials for our product candidates or any other future clinical development programs;
- announcements of regulatory approval or disapproval of our current or any future product candidates;
- failure or discontinuation of any of our research and development programs;
- the termination of any of our existing license agreements;
- announcements relating to any future licensing, collaboration or development agreements;
- delays in the commercialization of our current or any future product candidates;
- public misperception regarding the use of our product candidates;
- acquisitions and sales of new products or product candidates, technologies or businesses;
- manufacturing and supply issues related to our product candidates for clinical trials or future product candidates for commercialization;
- quarterly variations in our results of operations or those of our competitors;
- changes in earnings estimates or recommendations by securities analysts;
- announcements by us or our competitors of new products or product candidates, significant contracts, commercial relationships, acquisitions or capital commitments;
- developments with respect to intellectual property rights;
- our commencement of, or involvement in, litigation;
- changes in financial estimates or guidance;
- any major changes in our board of directors or management;
- new legislation or regulation in the United States relating to the sale or pricing of pharmaceuticals;
- FDA or other U.S. or foreign regulatory actions affecting us or our industry;
- product liability claims or other litigation or public concern about the safety of our product candidates;
- market conditions in the pharmaceutical, biopharmaceutical and biotechnology sectors;
- general economic conditions in the United States and abroad, the current inflationary economic environment, rising interest rates and global health concerns;
- sales of our common stock, including sales by our officers, directors and specific existing stockholders; and
- the issuance of shares of our common stock pursuant to the exercise of outstanding warrants.

In addition, the stock markets in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme volatility that may have been unrelated to the operating performance of the issuer. These broad market fluctuations may adversely affect the stock price or liquidity of our common stock.

We will cease to be eligible to use the requirements for smaller reporting companies beginning with the Quarterly Report on Form 10-Q for the fiscal quarter ending March 31, 2027. Since we will no longer be a smaller reporting company and no longer qualify for applicable exemptions, we will be subject to additional laws and regulations affecting public companies that may increase our costs and the demands on management and could harm our operating results.

We are subject to the reporting requirements of the Exchange Act, which requires, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition as well as other disclosure and corporate governance requirements. As a "smaller reporting company", we take advantage of certain exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Once we are no longer a smaller reporting company or otherwise qualify for these exemptions, we will be required to comply with these additional legal and regulatory requirements applicable to public companies and will incur significant legal, accounting and other expenses to do so. If we are not able to comply with the requirements in a timely manner or at all, our financial condition or the market price of our common stock may be harmed. For example, if we or our independent auditor identify deficiencies in our internal control over financial reporting that are deemed to be material weaknesses we could face additional costs to remedy those deficiencies, the market price of our stock could decline or we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources. Because the aggregate market value of our common stock held by non-affiliates exceeded $700 million as of June 30, 2026, we will be ineligible to use the requirements for smaller reporting companies beginning with our Quarterly report on Form 10-Q for the quarter ending March 31, 2027, but will remain a non-accelerated filer through 2027. When we are no longer a "non-accelerated filer", we will have to provide more expansive disclosure regarding executive compensation in our periodic reports and be subject to shorter filing deadlines, which will require additional time and expense. We will also be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act.

We incur significant costs as a result of operating as a public company, and our management needs to devote substantial time to compliance initiatives. We may fail to comply with the rules that apply to public companies, which could result in sanctions or other penalties that could materially and adversely affect our business, financial condition, results of operations and prospects.

We incur significant legal, accounting and other expenses as a public company, including costs resulting from public company reporting obligations under the Exchange Act and regulations regarding corporate governance practices. The listing requirements of the Nasdaq Stock Market and the rules of the SEC require that we satisfy certain corporate governance requirements relating to director independence, filing annual and interim reports, stockholder meetings, approvals and voting, soliciting proxies, conflicts of interest and a code of conduct. Our management and other personnel needs to devote a substantial amount of time to ensure that we comply with all of these requirements. Moreover, these reporting requirements, rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors or board committees or to serve as executive officers, or to obtain certain types of insurance, including directors' and officers' insurance, on acceptable terms.

If we experience material weaknesses in the future or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately report our financial condition or results of operations which may adversely affect investor confidence in us and, as a result, the value of our common stock.

We are subject to Section 404 of the Sarbanes-Oxley Act, or Section 404, and the related rules of the SEC, which generally require our management and independent registered public accounting firm to report on the effectiveness of our internal control over financial reporting. Pursuant to Section 404(a), we are required to file with the SEC an annual management assessment of the effectiveness of our internal control over financial reporting. Because we re-qualified as a smaller reporting company and we have less than $100 million in annual revenue, as of December 31, 2025, we are a non-accelerated filer and are not required to comply with the auditor attestation requirements regarding the effectiveness of our internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act until we become an accelerated filer or large accelerated filer.

Our management's assessment needs to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. A material weakness is a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of a company's annual and interim financial statements will not be detected or prevented on a timely basis. If we identify one or more material weaknesses in our internal control

over financial reporting, we will be unable to assert that our internal controls are effective. The effectiveness of our controls and procedures may be limited by a variety of factors, including:

- faulty human judgment and simple errors, omissions or mistakes;
- fraudulent action of an individual or collusion of two or more people;
- inappropriate management override of procedures; and
- the possibility that any enhancements to controls and procedures may still not be adequate to assure timely and accurate financial control.

While we believe our internal control over financial reporting is currently effective, the effectiveness of our internal controls in future periods is subject to the risk that our controls may become inadequate because of changes in conditions. Establishing, testing and maintaining an effective system of internal control over financial reporting requires significant resources and time commitments on the part of our management and our finance staff, may require additional staffing and infrastructure investments and would increase our costs of doing business. We can give no assurance that material weaknesses in our internal control over financial reporting will not be identified in the future. Our failure to implement and maintain effective internal control over financial reporting could result in errors in our financial statements that could result in a restatement of our financial statements and cause us to fail to meet our reporting obligations. Effective internal control over financial reporting is necessary for us to provide reliable and timely financial reports and, together with adequate disclosure controls and procedures, are designed to reasonably detect and prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. An independent assessment of the effectiveness of our internal control over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal control over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from the Nasdaq Global Select Market or other adverse consequences that would materially and adversely affect our business, financial condition, results of operations and prospects.

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

As of September 30, 2026, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned 43% of our outstanding voting stock. In addition, in our 2023, 2024, and 2025, financings, certain of the holders of 5% or more of our capital stock acquired pre-funded warrants to purchase shares of our common stock (which are immediately exercisable and have an exercise price of $0.001 per share) or common warrants to purchase shares of our common stock. Until exercised, the shares issuable upon the exercise of the pre-funded warrants and the common warrants are not included in the number of our outstanding shares of common stock. If such holders exercise their warrants, then the shares of our capital stock beneficially owned by our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates would increase significantly. Therefore, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Our current shares outstanding and resulting market valuation do not reflect shares of our common stock issuable upon the exercise of pre-funded warrants that are exercisable at the discretion of the holders of such warrants. If we sell shares of our common stock in the future, stockholders may experience immediate dilution. Stockholders may be unable to compute the dilutive impact of future financings.

We may from time to time issue additional shares of common stock, and as a result, our stockholders would experience immediate dilution. In addition, as opportunities present themselves, we may enter into financing or similar arrangements in the future, including the issuance of debt securities, preferred stock or common stock. For example, in March 2024, we entered into a sales agreement with TD Cowen, as sales agent, pursuant to which we sold approximately $66.7 million of shares of our common stock under an at the market offering program as of the date of this Quarterly Report on Form 10-Q. In March 2026, we entered into a new sales agreement with TD Cowen, as sales agent, pursuant to which we may issue and sell shares of our common stock for an aggregate maximum offering price of up to $150 million under a new at-the-market offering program. As of the filing date of this Quarterly Report on Form 10-Q, we had sold $37.5 million of common stock under the program. In addition, in December 2023, June 2024, and November 2025, we closed financings which included the sale of pre-funded warrants to purchase shares of our common stock. Until exercised, the shares issuable upon the exercise of the pre-funded warrants are not included in the number of our outstanding shares of common stock. If we issue common stock or securities convertible into common stock in the future, our stockholders would experience additional dilution and such dilutive impact may be difficult to compute.

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

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the stock price of our common stock could decline. As of September 30, 2026, the number of shares of our common stock outstanding was 190,092,719. This number does not include 14,865,799 shares of common stock issuable upon the exercise of pre-funded warrants. Sales of a substantial number of shares of our common stock in the public market, or the perception that these sales might occur, may reduce the stock price of our common stock.

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

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a rolling three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income or taxes may be limited.

We completed a study through December 31, 2025, to determine whether an ownership change had occurred under Section 382 or 383 of the Code, and we determined that ownership changes occurred in 2011, 2014, 2020 and 2023. We have identified $0.1 million and $34.7 million of federal and state NOLs, respectively, that will expire unused due to ownership changes, and federal credits of $4.3 million that will not be able to be utilized due to ownership change limitation and excluded these amounts from deferred tax asset balances as of December 31, 2025. Federal NOLs of $556.1 million and state and local NOLs of $191.4 million are not expected to expire unutilized as a result of ownership changes identified through December 31, 2025.

Our ability to use NOLs, research and development credit carryforwards and other tax attributes to reduce future taxable income and tax liabilities may be further limited as a result of shifts in our stock ownership subsequent to December 31, 2025. As a result, even if we attain profitability, our ability to use our pre-change NOLs or other pre-change tax attributes to offset United States federal and state taxable income may be subject to further limitations.

Provisions in our charter documents and under Delaware law could discourage a takeover that stockholders may consider favorable and may lead to entrenchment of management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent changes in control or changes in our management without the consent of our board of directors. These provisions include the following:

- a classified board of directors with three-year staggered terms, which may delay the ability of stockholders to change the membership of a majority of our board of directors;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- the exclusive right of our board of directors to elect a director to fill a vacancy, however occurring, including by an expansion of the board of directors, which prevents stockholders from being able to fill vacancies on our board of directors;
- the ability of our board of directors to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including voting or other rights or preferences, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquiror;
- the ability of our board of directors to alter our amended and restated bylaws without obtaining stockholder approval;
- the required approval of at least 66 2/3% of the shares entitled to vote at an election of directors to adopt, amend or repeal our amended and restated bylaws or repeal the provisions of our amended and restated certificate of incorporation regarding the election and removal of directors;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- the requirement that a special meeting of stockholders may be called only by the board of directors, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors; and
- advance notice procedures that stockholders must comply with in order to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquiror from conducting a solicitation of proxies to elect the acquiror's own slate of directors or otherwise attempting to obtain control of us.

We are also subject to the anti-takeover provisions contained in Section 203 of the General Corporation Law of the State of Delaware, or DGCL. Under Section 203, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the DGCL, our amended and restated bylaws and our indemnification agreements that we have entered into with our directors and officers provide that:

- we will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful;
- we may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law;
- we are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification;
- we are not obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification;
- the rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons; and
- we may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

While we maintain a directors' and officers' insurance policy, such insurance may not be adequate to cover all liabilities that we may incur, which may reduce our available funds to satisfy third-party claims and may adversely impact our cash position.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for certain disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware (or, in the event that the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware or other state courts of the State of Delaware) is the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of 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 against us that is governed by the internal affairs doctrine; provided that, the exclusive forum provision will not apply to suits brought to enforce any liability or duty created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; and provided further that, if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware. Our amended and restated certificate of incorporation and amended and restated bylaws also provide that the federal district courts of the United States of America are the exclusive forum for the resolution of any complaint asserting a cause of action against us or any of our directors, officers, employees or agents and arising under the Securities Act. Nothing in our amended and restated certificate of incorporation and amended and restated bylaws precludes stockholders that assert claims under the Exchange Act from bringing such claims in state or federal court, subject to applicable law.

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, other employees or stockholders, which may discourage lawsuits with respect to such

claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions, and there can be no assurance that such provisions will be enforced by a court in those other jurisdictions. If a court were to find the choice of forum provision that will be contained in our amended and restated certificate of incorporation and 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 adversely affect our business and financial condition.

We do not currently intend to pay dividends on our common stock, and, consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.

We do not currently intend to pay any cash dividends on our common stock for the foreseeable future. We currently intend to invest our future earnings, if any, to fund our growth. Therefore, you are not likely to receive any dividends on your common stock for the foreseeable future. Since we do not intend to pay dividends, your ability to receive a return on your investment will depend on any future appreciation in the market value of our common stock. There is no guarantee that our common stock will appreciate or even maintain the price at which our holders have purchased it.

General Risk Factors

Changes in tax laws and regulations may have a material adverse effect on our business, financial condition and results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of any of our future domestic and foreign earnings. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. Generally, future changes in applicable U.S. and non-U.S. tax laws and regulations, or their interpretation and application, could have an adverse effect on our business, financial conditions and results of operations. We are unable to predict whether such changes will occur and, if so, the ultimate impact on our business.

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, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors and other collaborators 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 to sell our products outside the United States, to conduct clinical trials and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors and other collaborators, 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.

Cybersecurity risks and the failure to maintain the security, confidentiality, integrity, or availability of our information technology systems or data, and those maintained on our behalf, could lead to adverse consequences that materially adversely affect our business, including, without limitation, regulatory investigations or actions, a material interruption to our operations, including clinical trials, damage to our reputation and/or subject us to costs, fines and penalties or lawsuits.

We collect and maintain information in digital and other forms that is necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business. In the ordinary course of our business, we and the third parties with whom we work process sensitive data. We have established certain physical, electronic and organizational measures designed to safeguard and secure our systems in an effort to prevent a data or other compromise; there can, however, be no assurance that these measures will be or have been effective. We have also outsourced elements of our information technology infrastructure, and

as a result a number of third-party vendors have access to our sensitive data. Our information technology systems and infrastructure, and those of any future collaborators and our contractors, consultants, vendors and other third parties with whom we work, are vulnerable to and have experienced attacks, damage and interruption from cyber-attacks, malicious internet-based activity, online and offline fraud, malicious code (such as computer viruses, and worms), malware, ransomware attacks, credential stuffing, credential harvesting, supply-chain attacks, natural disasters, fire, terrorism, war, telecommunication and electrical failures, attacks enhanced or facilitated by AI, denial or degradation of service attacks, hacking, sophisticated nation-state and nation-state supported actors, phishing and other social engineering attacks (including through deep fakes, which are increasingly more difficult to identify), attachments to emails, fraud, personnel misconduct or error, server malfunctions, software or hardware failures, loss or theft of data or information technology assets, unauthorized access or use, and other similar threats. In particular, ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, loss of sensitive data, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

The risk of a security breach or disruption, particularly through cyber-attacks, including by traditional computer "hackers", threat actors, "hacktivists", organized criminal threat actors, sophisticated nation states, and nation-state supported actors, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. The prevalent use of mobile devices that access sensitive data also increases the risk of lost or stolen devices, security incidents and data security breaches, which could lead to the loss or other compromise of sensitive data. In a hybrid working environment, we also face risks of a security breach or disruption due to our reliance on information systems that we do not necessarily control and the number of our personnel who are working remotely, which creates additional opportunities for cyber criminals to exploit vulnerabilities or other weaknesses. Additionally, future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. We may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. Security breaches may remain undetected for an extended period. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We have not and may not in the future, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we have and may in the future experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities.

We rely upon third-party service providers and technologies to operate critical business systems and process sensitive data in a variety of contexts, including, without limitation, third-party providers of cloud-based infrastructure, encryption and authentication technology, employee email, and other functions. Our ability to monitor these third parties' security practices is limited, and these third parties may not have adequate security measures in place. Our third-party service providers have experienced and may experience in the future a security incident or other interruption. For example, in 2025, two of our service providers experienced potential security incidents that triggered us to conduct investigations. While we determined that these incidents did not have a material impact on our business, results of operations, or financial condition, these and similar incidents have and could lead to business interruptions and additional costs. Any significant system failure, accident or security breach and resulting interruptions in our operations or our critical third parties' operations could result in a material disruption of our product development programs, and ultimately, our financial results. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. The costs to us to prevent, investigate, mitigate and remediate security incidents, breaches, disruptions, network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures designed to protect our data security and information technology systems and sensitive data, our efforts to address these problems may not be successful, and these problems have and may in the future result in unexpected interruptions, delays, cessation of service, negative publicity and other harm to our business and our competitive position. Any security

compromise affecting us or the third parties with whom we work, or our industry, whether real or perceived, could harm our reputation, erode confidence in the effectiveness of our security measures and lead to regulatory scrutiny. Moreover, if a security breach affects our systems or results in the unauthorized access to or unauthorized use, disclosure, release or other processing of sensitive data, we may be required, or we may voluntarily choose, to notify individuals, governmental authorities, supervisory bodies, the media and other parties, or to take other actions, such as providing credit monitoring and identity theft protection services pursuant to privacy and security laws or other obligations, and our reputation could be materially damaged. We would also be exposed to a risk of loss, governmental investigations or enforcement, or litigation (including class action claims) and other potential liability, which could materially adversely affect our business, results of operations and financial condition.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We maintain cyber liability insurance; however, this insurance may not be sufficient to cover the financial, legal, business or reputational losses that may result from an interruption or breach of our systems.

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

Actual or perceived failure to comply with applicable data protection laws, regulations, standards, contractual obligations and other requirements related to data privacy and security could lead to government enforcement actions as well as civil and criminal penalties, private litigation (including class actions), adverse publicity and otherwise could negatively affect our results of operations and business.

In the ordinary course of business we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive and confidential information, including proprietary and confidential business data, trade secrets, intellectual property, data we may collect about trial participants in connection with clinical trials, sensitive third-party data, and employee data (collectively, sensitive data). Our data processing activities actually or may subject us to numerous privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security. Data privacy and security obligations are stringent and changing, with new data privacy and security laws being proposed or enacted. Preparing for and complying with these obligations requires significant resources and has in the past necessitated and may in the future necessitate changes to our information technologies, systems, and practices and to those of any third parties with whom we work. The laws and regulations that affect our ability to operate include, but may not be limited to:

- the Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and its implementing regulations, or HIPAA, which imposes, among other things, certain standards relating to the privacy, security, transmission and breach reporting of individually identifiable health information. Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA. We do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. Depending on the facts and circumstances, however, we could be subject to significant administrative, civil and criminal penalties if we obtain, use or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA;
- the California Consumer Privacy Act of 2018, or CCPA, which requires covered businesses to provide certain disclosures in privacy notices to California residents, including consumers, business representatives, and employees, and requires businesses honor certain requests of California residents to exercise certain privacy rights. The CCPA provides for administrative fines, as well as a private right of action for data breaches that has increased the likelihood of, and risks associated with data breach litigation. Further, the amendments to the CCPA expanded the CCPA's requirements, including by adding a right for individuals to correct their personal data and establishing a regulatory agency to implement and enforce the law. Additional compliance investment and business process changes may be required in an effort to address data protection laws. Similar laws have been passed in other states and are continuing to be proposed at the state and federal level, reflecting a trend toward more stringent privacy legislation in the United States. The enactment of such laws could have potentially conflicting requirements that make compliance challenging. The CCPA and other comprehensive U.S. state consumer privacy laws exempt some data processed in the context of clinical trials, but these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties with whom we work; and
- foreign data protection laws, including the European Union's General Data Protection Regulation, or EU GDPR, and the United Kingdom GDPR, or UK GDPR, and laws of other jurisdictions in which we have in the past, presently or may in the future conduct clinical trials (such as Australia, Canada, and New Zealand), which contain provisions specifically directed

at the processing of personal data and, more broadly, impose significant and complex compliance burdens on processing personal data. Under the EU and UK GDPR, government regulators may impose temporary or definitive bans on data processing, as well as fines for noncompliance of up to €20 million under the EU GDPR (£17.5 million under the UK GDPR) or 4% of annual global revenue of the noncompliant company, whichever is greater. Noncompliance with the EU and UK GDPR could also result in private litigation related to the processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. These laws such as the EU and UK GDPR impose numerous requirements for the collection, use, storage and disclosure of personal data of data subjects, including requirements relating to providing notice to and obtaining consent from data subjects, personal data breach notification, cross-border transfers of personal data, and honoring and providing for the rights of individuals in relation to their personal data, including the right to access, correct and delete their data. Among other requirements, the EU and UK GDPR regulate transfers of personal data subject to the EU and UK GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EU and the United States remains uncertain.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States and other countries. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States or other relevant countries. If there were no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data out of Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. Other jurisdictions (including the US) have adopted or may adopt stringent data localization and cross-border data transfer laws. For example, regulators in the United States have enacted certain prohibitions and restrictions on cross-border data transfers. Specifically, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which limits certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered persons (i.e., individuals and entities who are designated as such by the U.S. Attorney General or considered "foreign persons" and are majority owned by, organized under the laws of, a primary resident in, or a contractor of, a covered person or country of concern, as applicable) that has or may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours that operate in the clinical trial space and impacts our ability to transfer data in connection with certain transactions.

In addition to data privacy and security laws, clinical trial subjects about whom we or any of our potential collaborators obtain information, as well as the providers who share this information with us, have and may in the future limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could materially and adversely affect our business, financial condition, results of operations and prospects. We also publish policies, marketing materials, and other statements regarding data privacy and security. Regulators are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

Our personnel use generative artificial intelligence, or AI, and/or automated decision-making technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating AI and/or automated decision-making technologies. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use AI and/or automated decision-making technologies, it could make our business less efficient and result in competitive disadvantages.

Although we work to comply with applicable privacy and data security laws, regulations and standards, contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. Compliance

with these data protection obligations has in the past and may in the future require us to take on more onerous obligations in our contracts, require us to engage in costly compliance exercises, restrict our ability to collect, use, disclose and otherwise process personal data, or in some cases, impact our or our partners' or suppliers' ability to operate in certain jurisdictions. Any actual or perceived failure to comply by us or our personnel, representatives, contractors, consultants, collaborators, or other third parties could result in government investigations and/or enforcement actions, fines, civil and/or criminal penalties, additional reporting requirements and/or oversight, bans on processing personal data (including clinical trial data), orders to destroy or not use personal data, private litigation (including class action claims) and mass arbitration demands, adverse publicity and could otherwise negatively affect our results of operations and 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 is influenced by the research and reports that industry or securities analysts publish about us or our business. 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 clinical trials and results of operations fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these 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 our stock price or trading volume to decline.

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

In the past, companies that have experienced volatility in the market price of their securities have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Regardless of the merits or the ultimate results of such litigation, securities litigation brought against us could result in substantial costs and divert our management's attention from other business concerns.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

Trading Arrangements of Section 16 Reporting Persons.

During the three months ended September 30, 2026, the following directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, amended or terminated any "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as those terms are defined in Item 408 of Regulation S-K.

Name and Position	Action	Adoption Date	Types of Trading Arrangement		Total Shares of Common Stock to Be Sold	Total Shares of Common Stock to Be Purchased	Expiration Date
			Rule 10b5-1*	Non-Rule 10b5-1**			
Bill Carlson, M.D., Director	Adopt	07/03/2026	X			102,000	4/30/27

* Contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.
** "Non-Rule 10b5-1 trading arrangement" as defined in Item 408(a) of Regulation S-K under the Exchange Act.

Item 6. Exhibits.

EXHIBIT INDEX

Exhibit No.	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File No.	Exhibit	Filing Date	
3.1	Amended and Restated Certificate of Incorporation.	8-K	001-39402	3.1	07/28/20	
3.2	Amendment to Amended and Restated Certificate of Incorporation.	10-Q	001-39402	3.2	08/12/26	
3.3	Amended and Restated Bylaws.	8-K	001-39402	3.3	07/28/20	
10.1	Loan and Security Agreement by and among Annexon, Inc. and Oxford Finance LLC, as collateral agent, and certain lenders parties thereto, dated July 30, 2026.	8-K	001-39402	10.1	7/30/26	
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as amended.					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 amended.					X
32.1*	Certification Pursuant to 18 U.S.C. Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, as amended.					X
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document					X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Document					X
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)					X

* The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, as amended, is not deemed "filed" by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, nor shall Exhibit 32.1 be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, except to the extent that the Registrant specifically incorporates it by reference.

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.

Annexon, Inc.

Date: November 9, 2026

By: /s/ Douglas Love, Esq.

Douglas Love, Esq.
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 9, 2026

By: /s/ Jennifer Lew

Jennifer Lew
Executive Vice President and 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 AMENDED

I, Douglas Love, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Annexon, 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 and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: November 9, 2026

By: /s/ Douglas Love

Douglas Love, Esq.
President and 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 AMENDED

I, Jennifer Lew, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Annexon, 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 and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: November 9, 2026

By: /s/ Jennifer Lew

Jennifer Lew
Executive Vice President and Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER AND CHIEF FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002, AS AMENDED

In connection with the Quarterly Report on Form 10-Q of Annexon, Inc. (the "Company") for the quarter ended September 30, 2026 (the "Report") filed with the Securities and Exchange Commission on the date hereof, Douglas Love, President and Chief Executive Officer of the Company, and Jennifer Lew, Executive Vice President and Chief Financial Officer of the Company, each hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, as amended, that:

1. The Report fully complies with the requirements of Section 13(a) or 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: November 9, 2026

/s/ Douglas Love
Douglas Love, Esq.
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 9, 2026

/s/ Jennifer Lew
Jennifer Lew
Executive Vice President and Chief Financial Officer
(Principal Financial and Accounting Officer)