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 001-42504

SIONNA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**84-2801521**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
21 Hickory Drive, Suite 500, Waltham, MA	**02451**
(Address of Principal Executive Offices)	(Zip Code)

(617) 819-2020
Registrant's telephone number, including area code

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, $0.001 par value per share	SION	The Nasdaq Global Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐
Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes ☐ No ☒
The number of shares of registrant's common stock outstanding as of October 30, 2026 was 45,235,568.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (the "Quarterly Report") contains forward-looking statements about us and our industry that involve substantial risks and uncertainties, and which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements other than statements of historical facts contained in this Quarterly Report, including statements regarding our future results of operations and financial position, business strategy, product candidates, preclinical studies and clinical trials, results of preclinical studies and clinical trials, research and development costs, regulatory approvals, commercial strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements involve known and unknown risks, uncertainties and other important factors that are in some cases beyond our control and may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "would," "expect," "plan," "anticipate," "could," "intend," "target," "project," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. Forward-looking statements contained in this Quarterly Report include, but are not limited to, statements about:

- the initiation, timing, progress and results of our research and development programs, preclinical studies and clinical trials;
- the ability of clinical trials to demonstrate safety and efficacy of our product candidates, and other positive results, the ability of our earlier clinical trials and preclinical studies to predict later clinical trial results, and our interpretation of data from preclinical studies, clinical trials and post hoc or exploratory analyses, including the conclusions we draw from such data and their role in informing our development strategy;
- the timing, scope and likelihood of regulatory filings and approvals of our product candidates;
- the implementation of our business model, and strategic plans for our business, programs, and current and future product candidates;
- our expectations regarding the timing, costs and anticipated benefits of our restructuring plan, including expected cost savings, the sufficiency of retained personnel and capabilities, and the impact on our operations and cash runway;
- our ability to obtain additional cash and the sufficiency of our existing cash, cash equivalents and investments in marketable securities to fund our future operating expenses and capital expenditure requirements;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- our potential and ability to successfully manufacture and supply our current and future product candidates for clinical trials and for commercial use, if approved;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- developments relating to our competitors and our industry, including competing product candidates and therapies;
- existing regulations and regulatory developments in the U.S. and other jurisdictions, including changes in U.S. federal policy, such as trade policies or tariffs;
- expectations regarding future events under collaboration and licensing agreements, including potential future payments, as well as our plans and strategies for entering into further collaboration and licensing agreements;
- general economic, industry and market conditions, including elevated interest rates and inflation;
- our ability to attract and retain the continued service of our key personnel and to identify, hire and then retain additional qualified personnel;

- our expectations regarding the period during which we will qualify as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act");
- our expectations regarding the period during which we will qualify as a "smaller reporting company" as defined by Rule 12b-2 of the Exchange Act; and
- our anticipated use of our existing cash, cash equivalents and investments in marketable securities.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this Quarterly Report and are subject to a number of risks, uncertainties and assumptions described in the section entitled “Risk Factors” and elsewhere in this Quarterly Report. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events or otherwise.

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

You should read this Quarterly Report and the documents that we reference herein or incorporated by reference as exhibits hereto with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

NOTE REGARDING TRADEMARKS

Sionna Therapeutics, Inc. is the owner of the trademarks SIONNA and SIONNA THERAPEUTICS, as well as certain other trademarks, including design versions of some of these trademarks. The symbols ™ and ® are not used in connection with the presentation of these trademarks in this Quarterly Report and their absence does not indicate a lack of trademark rights. Certain other trademarks used in this Quarterly Report are the property of third-party trademark owners and may be presented with or without trademark symbols.

All brand names or trademarks appearing in this Quarterly Report are the property of their respective owners. Unless the context requires otherwise, references in this report to “Sionna,” the “Company,” “we,” “us” and “our” refer to Sionna Therapeutics, Inc. and its subsidiary.

SUMMARY OF MATERIAL RISKS ASSOCIATED WITH OUR BUSINESS

We are subject to numerous risks and uncertainties, including those further described below in the section entitled “Risk Factors” in this Quarterly Report, as well as in other documents that we file with the U.S. Securities and Exchange Commission (“SEC”). The following considerations, among others, may offset our competitive strengths or have a negative effect on our business strategy, which could materially adversely affect our business, financial conditions, results of operations and future growth prospects:

- We are a clinical-stage biopharmaceutical company and have incurred significant operating losses since inception and anticipate that we will continue to incur significant operating losses for the foreseeable future.
- We will need to raise substantial additional funding. We may be unable to raise capital on acceptable terms, if at all, and, as a result, we may be required to delay, reduce or eliminate our product development programs or future commercialization efforts.
- We are substantially dependent on the success of our dual combination of SION-451 + SION-2222. If we are unable to advance SION-451 + SION-2222 into our planned proof-of-concept clinical trial or later-stage clinical development or unable to obtain regulatory approval and commercialize SION-451 + SION-2222, or experience significant delays in doing so, our business will be materially harmed.
- Targeting the NBD1 domain of the cystic fibrosis transmembrane conductance regulator protein is novel, and the time, cost of development and likelihood of successful product development is difficult to predict.
- We are developing a proprietary dual combination of an NBD1 stabilizer with a complementary modulator. Developing combination treatments increases complexity and risk, including risks of drug-drug interactions, unforeseen side effects or failures in our clinical trials that could delay or prevent their regulatory approval or limit the commercial profile of an approved label.
- We may incur additional costs and experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.
- Positive results from our preclinical studies or prior clinical trials are not necessarily predictive of the results of later clinical trials of our current or future product candidates. If we cannot replicate positive results from our earlier preclinical studies or clinical trials of our current or potential future product candidates, or results of post hoc analyses thereof, or if we suffer any other significant setbacks in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our current or potential future product candidates.
- Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious or unacceptable adverse side effects or unexpected toxicology findings may be identified during the development of our product candidates, which could prevent or delay further clinical development, regulatory approvals and commercialization, impact the product’s labeling, if approved, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.
- We rely, and in the future expect to rely, on third parties to conduct our planned clinical trial(s) for our current and future product candidates. If these third parties do not successfully carry out their contractual duties, comply with regulatory requirements or meet expected deadlines, or if we experience delays or difficulties in the initiation of, or enrollment or retention of patients in, clinical trials, obtaining necessary regulatory approvals could be delayed or prevented.
- The regulatory approval processes of the U.S. Food and Drug Administration and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain the required regulatory approval for any product candidate, our business will be substantially harmed.
- We may not be successful in our efforts to identify or discover additional product candidates, or we may expend our limited resources to pursue a particular product candidate or indication 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 are dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates, if approved. If we breach any of the agreements under which we license the use, development and commercialization

rights to our product candidates from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.

- We contract with third parties for the manufacture of our product candidates for clinical drug supply and expect to continue to do so for commercialization, if our product candidates are approved. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or obtain such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.
- If we or our licensors are unable to obtain, maintain and enforce intellectual property rights relating to any of our product candidates, or if the scope of the protection obtained is not sufficiently broad, our competitors or other third parties could develop and commercialize products similar or identical to ours, our ability to successfully commercialize our product candidates may be adversely affected and we may not be able to compete effectively in our markets.
- We face substantial competition. Our main competitor in the CF market holds substantial market share and has substantially greater resources than we do. We may not be able to compete successfully in this environment and, in particular, against a much larger competitor.
- The trading price of the shares of our common stock may be volatile, and investors could lose all or part of their investment.

The summary risk factors described above should be read together with the text of the full risk factors in the section titled “Risk Factors” and the other information set forth in this Quarterly Report, as well as in other documents that we file with the SEC. The risks summarized above or described in full elsewhere in this Quarterly Report are not the only risks that we face. Additional risks and uncertainties not presently known to us, or that we currently deem to be immaterial may also materially adversely affect our business, financial condition, results of operations and future growth prospects.

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Part I - Financial Information

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data) (Unaudited)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 83,878	$ 58,451
Marketable securities, current	140,709	177,431
Prepaid expenses and other current assets	3,941	5,537
Total current assets	228,528	241,419
Property and equipment, net	1,762	2,225
Marketable securities, noncurrent	25,210	74,420
Restricted cash	963	963
Operating lease right-of-use asset	6,160	6,926
Other assets	638	—
Total assets	$ 263,261	$ 325,953
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,050	$ 769
Accrued expenses	13,936	9,668
Operating lease liability, current	1,434	1,269
Other current liabilities	—	6
Total current liabilities	16,420	11,712
Operating lease liability, noncurrent	6,317	7,408
Total liabilities	22,737	19,120
Stockholders' equity:		
Preferred stock, $0.001 par value, 10,000,000 shares authorized as of September 30, 2026 and December 31, 2025, respectively; no shares issued or outstanding as of September 30, 2026 and December 31, 2025	—	—
Common stock, $0.001 par value, 500,000,000 shares authorized as of September 30, 2026 and December 31, 2025, respectively; 45,214,369 and 44,723,585 shares issued, 45,214,369 and 44,723,585 shares outstanding as of September 30, 2026 and December 31, 2025, respectively	45	45
Additional paid-in capital	586,639	562,602
Accumulated other comprehensive (loss) income	(723)	540
Accumulated deficit	(345,437)	(256,354)
Total stockholders' equity	240,524	306,833
Total liabilities and stockholders' equity	$ 263,261	$ 325,953

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

SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 22,025	$ 16,044	$ 62,852	$ 45,095
General and administrative	12,794	7,761	34,036	20,275
Total operating expenses	34,819	23,805	96,888	65,370
Loss from operations	(34,819)	(23,805)	(96,888)	(65,370)
Other income:				
Interest income	2,407	3,456	7,814	10,123
Other income (expense), net	(6)	71	(9)	419
Total other income	2,401	3,527	7,805	10,542
Net loss	$ (32,418)	$ (20,278)	$ (89,083)	$ (54,828)
Net loss per share, basic and diluted	$ (0.72)	$ (0.46)	$ (1.98)	$ (1.43)
Weighted-average common shares outstanding, basic and diluted	45,208,940	44,255,053	45,094,349	38,387,388
Comprehensive loss:				
Net loss	$ (32,418)	$ (20,278)	$ (89,083)	$ (54,828)
Unrealized gain (loss) on marketable securities	(275)	237	(1,263)	52
Total other comprehensive gain (loss)	(275)	237	(1,263)	52
Comprehensive loss	$ (32,693)	$ (20,041)	$ (90,346)	$ (54,776)

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

SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(in thousands, except share amounts)
(Unaudited)

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Series C Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2024	3,963,963	$ 13,014	5,704,161	$ 25,235	11,370,621	$ 110,791	18,628,970	$ 181,328	4,722,763	$ 5	$ 17,000	$ 368	$ (181,086)	$ (163,713)
Conversion of convertible preferred stock to common stock upon closing of initial public offering	(3,963,963)	(13,014)	(5,704,161)	(25,235)	(11,370,621)	(110,791)	(18,628,970)	(181,328)	27,149,206	27	330,340	—	—	330,367
Issuance of common stock from initial public offering, net of issuance costs of $4,265	—	—	—	—	—	—	—	—	12,176,467	12	199,558	—	—	199,570
Vesting of restricted common stock awards	—	—	—	—	—	—	—	—	43,681	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	2,094	—	—	2,094
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	—	—	(67)	—	(67)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(16,482)	(16,482)
Balance at March 31, 2025	—	$ —	—	$ —	—	$ —	—	$ —	44,092,117	$ 44	$ 548,992	$ 301	$ (197,568)	$ 351,769
Exercise of common stock options	—	—	—	—	—	—	—	—	7,944	—	6	—	—	6
Vesting of restricted common stock awards	—	—	—	—	—	—	—	—	32,277	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	2,840	—	—	2,840
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	—	—	(118)	—	(118)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(18,068)	(18,068)
Balance at June 30, 2025	—	$ —	—	$ —	—	$ —	—	$ —	44,132,338	$ 44	$ 551,838	$ 183	$ (215,636)	$ 336,429
Exercise of common stock options	—	—	—	—	—	—	—	—	331,189	—	1,974	—	—	1,974
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	3,306	—	—	3,306
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	—	—	237	—	237
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(20,278)	(20,278)
Balance at September 30, 2025	—	$ —	—	$ —	—	$ —	—	$ —	44,463,527	$ 44	$ 557,118	$ 420	$ (235,914)	$ 321,668

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

SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(in thousands, except share amounts)
(Unaudited)

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Series C Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2025	—	$ —	—	$ —	—	$ —	—	$ —	44,723,585	$ 45	$ 562,602	$ 540	$ (256,354)	$ 306,833
Exercise of common stock options	—	—	—	—	—	—	—	—	352,066	—	2,225	—	—	2,225
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	6,553	—	—	6,553
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	—	—	(624)	—	(624)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(26,779)	(26,779)
Balance at March 31, 2026	—	$ —	—	$ —	—	$ —	—	$ —	45,075,651	$ 45	$ 571,380	$ (84)	$ (283,133)	$ 288,208
Exercise of common stock options	—	—	—	—	—	—	—	—	88,588	—	975	—	—	975
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	6,842	—	—	6,842
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	—	—	(364)	—	(364)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(29,886)	(29,886)
Balance at June 30, 2026	—	$ —	—	$ —	—	$ —	—	$ —	45,164,239	$ 45	$ 579,197	$ (448)	$ (313,019)	$ 265,775
Exercise of common stock options	—	—	—	—	—	—	—	—	50,130	—	358	—	—	358
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	7,084	—	—	7,084
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	—	—	(275)	—	(275)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(32,418)	(32,418)
Balance at September 30, 2026	—	$ —	—	$ —	—	$ —	—	$ —	45,214,369	$ 45	$ 586,639	$ (723)	$ (345,437)	$ 240,524

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

SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2026	**2025**
Cash flows from operating activities:		
Net loss	$ (89,083)	$ (54,828)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	20,479	8,240
Non-cash operating lease expense	1,470	1,471
Amortization of discount on marketable securities	(566)	(2,699)
Depreciation expense	463	495
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	1,596	(543)
Accounts payable	281	295
Accrued expenses and other current liabilities	4,207	(517)
Operating lease liabilities	(1,630)	(1,585)
Net cash used in operating activities	(62,783)	(49,671)
Cash flows from investing activities:		
Purchases of property and equipment	—	(173)
Purchases of marketable securities	(69,712)	(282,147)
Maturities of marketable securities	154,947	140,983
Net cash provided by (used in) investing activities	85,235	(141,337)
Cash flows from financing activities:		
Proceeds from initial public offering, net of underwriter discounts, commissions and offering costs paid during the period	—	202,071
Payment of deferred offering costs	(583)	—
Exercise of common stock options	3,558	1,980
Net cash provided by financing activities	2,975	204,051
Net increase in cash, cash equivalents and restricted cash	25,427	13,043
Cash, cash equivalents and restricted cash at beginning of period	59,414	38,750
Cash, cash equivalents and restricted cash at end of period	$ 84,841	$ 51,793
Supplemental disclosure of non-cash investing and financing activities:		
Conversion of convertible preferred stock into common stock upon closing of initial public offering	$ —	$ 330,368
Offering costs transferred to additional paid in capital	—	4,265
Deferred offering costs in accrued expenses and accounts payable	55	—
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 83,878	$ 50,831
Restricted cash	963	962
Total cash, cash equivalents, and restricted cash	$ 84,841	$ 51,793

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

SIONNA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. Nature of the Business

Organization

Sionna Therapeutics, Inc. (the “Company”), formerly known as Sling Therapeutics, Inc., was incorporated in Delaware in August 2019 and is headquartered in Waltham, Massachusetts. The Company is a clinical-stage biopharmaceutical company on a mission to revolutionize the current treatment paradigm for cystic fibrosis (“CF”) patients by developing novel medicines that normalize the function of the cystic fibrosis transmembrane conductance regulator protein to deliver clinically meaningful benefit to CF patients.

Restructuring

In August 2026, the Company announced topline results from the PreciSION CF trial, a Phase 2a proof-of-concept clinical trial evaluating SION-719, an NBD1 stabilizer product candidate, as an add-on to the standard of care for CF. While SION-719 was generally well tolerated in the trial, the trial did not achieve its key activity endpoint. As a result, the Company is not advancing SION-719 as an add-on to the standard of care.

In connection with these developments, on September 9, 2026, the Company's board of directors approved a restructuring plan (the "Restructuring") intended to focus the Company's resources on advancing its SION-451 and SION-2222 dual combination program and extend the Company's cash runway. The Restructuring included a reduction in the Company's workforce of approximately 46%, while retaining core clinical, regulatory, technical and corporate capabilities required to advance the dual combination program. The Company also initiated a wind-down of activities associated with the SION-719 add-on program, including the termination of certain clinical, manufacturing, and other vendor arrangements. The workforce reduction and wind-down of applicable vendor arrangements were substantially completed by the end of the third quarter of 2026, with related cash payments expected to continue into the fourth quarter of 2026.

The Company currently estimates that it will incur aggregate restructuring and related charges of approximately $6.4 million, consisting of approximately $5.2 million of employee severance and other benefits, $0.8 million of contract termination and other close-out costs and $0.4 million of stock-based compensation expense. During the three and nine months ended September 30, 2026, the Company recognized approximately $6.2 million of restructuring and related charges, consisting of $5.8 million of cash-related charges and $0.4 million of non-cash stock-based compensation expense.

Selected information related to accrued cash-related restructuring liabilities (excludes stock-based compensation expense) was as follows (in thousands):

Restructuring liabilities balance at June 30, 2026	$	—
Restructuring charges	$	5,787
Cash paid	$	(1,700)
Restructuring liabilities balance at September 30, 2026	$	4,087

Restructuring expenses were recorded within research and development expense and general and administrative expense based on the functions of the affected employees and the nature of the underlying costs. Of the $6.2 million of restructuring and related charges recognized during the three and nine months ended September 30, 2026, $3.7 million was recorded within research and development expense and $2.5 million was recorded within general and administrative expense in the condensed consolidated statements of operations and comprehensive loss. As of September 30, 2026, the Company had restructuring-related liabilities of $4.1 million, which were included in accounts payable and accrued expenses in the condensed consolidated balance sheet.

The Company evaluated the recoverability of its property and equipment and operating lease right-of-use assets in connection with the Restructuring. Based on this evaluation, the Company did not recognize any impairment charges as of September 30, 2026.

The estimates of restructuring charges and the timing of related activities are subject to a number of assumptions, and actual results may differ from the Company's current estimates. The Company will continue to evaluate the expected costs associated with the Restructuring and may incur additional charges.

Risks and Uncertainties

The Company is subject to a number of risks common to other companies in the biotechnology industry, including but not limited to, development by competitors of new technological innovations, risks of failure of preclinical studies and clinical trials, development and manufacturing of product candidates, obtaining regulatory approval for product candidates, competition from substitute products, the need to successfully commercialize and gain market acceptance of its product candidates, protection of proprietary technology, dependence on key personnel, the ability to attract and retain qualified employees, reliance on third-party organizations, compliance with government regulations, and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive clinical testing and regulatory approval, prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance reporting capabilities. Even if the Company’s development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity and Going Concern

The Company has incurred annual net operating losses and has generated negative operating cash flows in every year since inception. As of September 30, 2026, the Company had an accumulated deficit of $345.4 million. The Company expects its operating losses to continue into the foreseeable future as it continues to pursue its research and development efforts.

The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company’s ability to continue as a going concern within one year after the date that these condensed consolidated financial statements are issued. The Company believes that its existing cash, cash equivalents and marketable securities of $249.8 million as of September 30, 2026, will be sufficient to allow the Company to fund operations beyond twelve months from the date that the financial statements are issued.

In March 2026, the Company entered into a sales agreement with Leerink Partners LLC (“Leerink”), under which the Company, from time to time, may issue and sell shares of the Company’s common stock through an "at the market" equity offering program under which Leerink is acting as sales agent, for aggregate gross sale proceeds of up to $250.0 million. As of September 30, 2026, the Company has not sold any shares of common stock under the sales agreement.

2. Summary of Significant Accounting Policies

The Company’s significant accounting policies are disclosed in Note 2, *“Summary of Significant Accounting Policies,”* in the audited consolidated annual financial statements in the Annual Report on Form 10-K for the year ended December 31, 2025, which was filed with the Securities and Exchange Commission (“SEC”) on March 2, 2026 (the “Annual Report”). Since the date of those annual financial statements, there have been no changes to the Company’s significant accounting policies.

Unaudited Interim Financial Information

The accompanying condensed consolidated balance sheet as of September 30, 2026, and the condensed consolidated statements of operations and comprehensive loss, statements of convertible preferred stock and stockholders’ equity and statements of cash flows for the nine months ended September 30, 2026, and 2025, are unaudited. The condensed consolidated interim financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all

adjustments, which include only normal recurring adjustments necessary for the fair presentation of the Company's financial position as of September 30, 2026, and the results of its operations and its cash flows for the periods presented. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2026, and 2025, are also unaudited. The results for the three and nine months ended September 30, 2026, are not necessarily indicative of results to be expected for the year ending December 31, 2026, or for any other subsequent period.

Recently Adopted Accounting Pronouncements

In September 2025, the FASB issued ASU 2025-07, *Derivatives Scope Refinement*, ("ASU 2025-07"). ASU 2025-07 creates a scope exception within ASC 815 for certain contracts with underlyings tied to a party's operations or activities (such as regulatory approval, clinical, or earnings milestones), which may cause some arrangements that previously met the derivative definition to no longer be accounted for as derivatives. The guidance is effective for fiscal years beginning after December 15, 2026, including interim periods. Early adoption is permitted. The Company adopted the guidance prospectively as of January 1, 2026, for contracts entered into or modified after the adoption date. The adoption did not have a material impact on the Company's consolidated financial statements.

Recently Issued Accounting Pronouncements Not Yet Adopted

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU 2024-03"), which requires entities to disclose additional information about specific expense categories in the notes to the financial statements. ASU 2024-03 is effective for annual periods beginning after December 15, 2026 and for interim periods within fiscal years beginning after December 15, 2027. Early adoption is permitted. ASU 2024-03 may be applied retrospectively or prospectively. The Company is currently evaluating the effect of this update on its consolidated financial statements and related disclosures.

3. Marketable Securities

The following tables summarize the amortized cost and estimated fair value of the Company's current and noncurrent marketable securities (in thousands):

	September 30, 2026			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Marketable securities, current:				
U.S. Treasury securities	$ 98,618	$ —	$ (331)	$ 98,287
Commercial paper	17,877	—	(16)	17,861
Corporate debt	13,773	—	(29)	13,744
Government agency securities	10,886	—	(69)	10,817
Total marketable securities, current	141,154	—	(445)	140,709
Marketable securities, noncurrent:				
U.S. Treasury securities	15,315	—	(184)	15,131
Government agency securities	9,187	—	(84)	9,103
Corporate debt	986	—	(10)	976
Total marketable securities, noncurrent	25,488	—	(278)	25,210
Total marketable securities	$ 166,642	$ —	$ (723)	$ 165,919

	December 31, 2025			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
Marketable securities, current:				
U.S. Treasury securities	$ 135,801	$ 263	$ —	$ 136,064
Commercial paper	24,391	10	(5)	24,396
Corporate debt	11,813	3	(1)	11,815
Government agency securities	5,145	11	—	5,156
Total marketable securities, current	177,150	287	(6)	177,431
Marketable securities, noncurrent:				
U.S. Treasury securities	55,793	208	—	56,001
Government agency securities	18,368	51	—	18,419
Total marketable securities, noncurrent	74,161	259	—	74,420
Total marketable securities	$ 251,311	$ 546	$ (6)	$ 251,851

The Company has determined that there were no material changes in the credit risk, therefore the Company has not recognized any allowance for credit losses on its debt securities. As of September 30, 2026, the Company holds marketable securities with an aggregate fair value of $25.2 million that had remaining maturities between one and two years. All other securities had a contractual maturity of less than a year. There were no realized gains or losses during the three and nine months ended September 30, 2026, or 2025.

4. Fair Value Measurements

The following tables present information about the Company's financial instruments that are measured at fair value on a recurring basis and indicates the fair value hierarchy of the inputs the Company utilized to determine such fair value (in thousands):

	September 30, 2026			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 82,379	$ 82,379	$ —	$ —
U.S. Treasury securities	998	998	—	—
Marketable securities:				
U.S. Treasury securities	113,418	113,418	—	—
Government agency securities	19,920	—	19,920	—
Commercial paper	17,861	—	17,861	—
Corporate debt	14,720	—	14,720	—
Total financial assets	$ 249,296	$ 196,795	$ 52,501	$ —

	December 31, 2025			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 51,962	$ 51,962	$ —	$ —
Commercial paper	5,988	—	5,988	—
Marketable securities:				
U.S. Treasury securities	192,065	192,065	—	—
Commercial paper	24,396	—	24,396	—
Government agency securities	23,575	—	23,575	—
Corporate debt	11,815	—	11,815	—
Total financial assets	$ 309,801	$ 244,027	$ 65,774	$ —

During the three and nine months ended September 30, 2026, there were no transfers or reclassifications between fair value measurement levels of assets or liabilities. The carrying values of prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

5. Property and Equipment, net

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

	September 30, 2026	December 31, 2025
Leasehold improvements	$ 1,920	$ 1,920
Laboratory equipment	1,575	1,575
Furniture & fixtures	852	852
Total property and equipment	4,347	4,347
Less: accumulated depreciation	(2,585)	(2,122)
Property and equipment, net	$ 1,762	$ 2,225

Depreciation expense for the three months ended September 30, 2026 and 2025 was $0.2 million and $0.1 million, respectively. Depreciation expense was $0.5 million for the nine months ended September 30, 2026 and 2025.

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Accrued research and development	$ 7,715	$ 4,305
Accrued compensation and benefits	5,457	4,740
Accrued professional fees	641	522
Accrued other	68	101
Accrued deferred offering costs	55	—
Total accrued expenses	$ 13,936	$ 9,668

As of September 30, 2026, accrued compensation and benefits and accrued research and development expenses included approximately $3.6 million and $0.5 million, respectively, of liabilities associated with the Restructuring described in Note 1.

7. Commitments and Contingencies

Operating Leases

The Company leases office space under a noncancelable operating lease. There have been no material changes to the Company's lease during the nine months ended September 30, 2026. For additional information, see Note 7, "*Leases,*" in the audited consolidated annual financial statements in the Annual Report.

Legal Proceedings

The Company was not subject to any material legal proceedings as of September 30, 2026, and the Company is not aware of any material legal proceedings that are currently pending or threatened.

Other Contracts

The Company enters into agreements in the ordinary course of business with contract research organizations and contract manufacturing organizations, which generally provide for termination on notice. Amounts payable upon termination are dependent on the timing of termination and the terms of the respective agreement.

The Company evaluates such arrangements on an ongoing basis and records a liability for obligations that are probable and reasonably estimable. For matters that do not meet these criteria, the Company considers whether disclosure is appropriate.

Indemnification Agreements

In the ordinary course of business the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters arising out of the relationship between such parties and the Company. In addition, the Company has entered into indemnification agreements with members of its board of directors and senior management that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, however, the Company has not incurred any material costs as a result of such indemnifications nor experienced any losses related to them. As of September 30, 2026, the Company was not aware of any claims under indemnification arrangements and does not expect significant claims under indemnification arrangements and it has not accrued any liabilities related to such obligations.

8. License Agreements

The Company has entered into several license agreements with third parties that typically involve payments from the Company, including up-front payments, milestone payments and royalty payments. The terms and conditions as well as accounting analysis for the Company's significant license agreements are described in Note 9, "*License Agreements,*" in the audited consolidated annual financial statements in the Annual Report. There have been no material changes to the terms and conditions, or accounting conclusions, previously disclosed in the Annual Report.

9. Stock-Based Compensation

The Company's stock-based compensation plans are described in Note 11, *"Common Stock,"* in the audited consolidated annual financial statements in the Annual Report. There have been no material changes to the Company's stock-based compensation plans previously disclosed in the Annual Report.

Stock Option Activity

The following table summarizes the Company's common stock option activity for the nine months ended September 30, 2026:

	Number of Common Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term	Aggregate Intrinsic Value
			(in years)	(in thousands)
Outstanding as of December 31, 2025	5,767,799	$ 12.01	8.3	$ 168,007
Granted	3,629,558	38.60		
Exercised	(490,784)	7.25		
Cancelled or forfeited	(2,657,242)	32.99		
Outstanding as of September 30, 2026	6,249,331	$ 18.91	6.7	$ 305
Exercisable as of September 30, 2026	3,184,266	$ 12.71	5.5	$ 305

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock.

The weighted-average grant date fair value of options granted during the three and nine months ended September 30, 2026, was $29.42 per share and $27.60 per share, respectively. The total intrinsic value of stock options exercised during the three and nine months ended September 30, 2026 was $1.9 million and $15.6 million, respectively.

Restricted Stock Units

In connection with the Restructuring and as part of an employee retention program, the Company granted time-based and performance-based restricted stock units ("RSUs") to eligible retained employees. The time-based RSUs vest after nine months, while the performance-based RSUs vest following the achievement of a development milestone (the "Performance Condition").

The grant-date fair value of each RSU is based on the closing price of the Company's common stock on the grant date. Stock-based compensation expense for time-based RSUs is recognized on a straight-line basis over the requisite service period. If deemed probable, expense for performance-based RSUs is recognized through the date on which the Performance Condition is expected to be achieved.

In connection with the performance-based RSU grant, the Company concluded that the Performance Condition is probable of achievement. The estimated date on which the Performance Condition is expected to be achieved is reassessed each reporting date, and changes in that estimate may affect the timing of stock-based compensation expense recognition.

The following table summarizes the Company's RSU activity for the nine months ended September 30, 2026:

	Number of Shares	Weighted Average Grant Date Fair Value Per Share
Outstanding as of December 31, 2025	—	$ —
Granted	703,050	$ 7.18
Vested	—	$ —
Cancelled or forfeited	(3,506)	$ 7.18
Outstanding as of September 30, 2026	699,544	$ 7.18

Restructuring - Stock Option Repricing

On September 9, 2026, the Company's board of directors approved the repricing of certain outstanding vested and unvested stock options held by eligible employees, effective September 17, 2026 (the "Repricing Date"). The board determined that stock options with exercise prices exceeding $18.00 per share, together with certain new-hire stock options granted at $18.00 per share, no longer provided their intended retention and incentive value relative to the market price of the Company's common stock. To enhance retention and better align the interests of employees with those of stockholders, the board approved the repricing of these options to the fair market value of the Company's common stock on the Repricing Date, which was $7.18 per share.

The repriced stock options are subject to terms providing that any exercise prior to the expiration of a retention period shall use the original exercise price. The retention period ends on the 18-month anniversary of the Repricing Date or an earlier sales event or qualifying termination of service. The vesting schedules, expiration dates and other terms of the repriced stock options were not modified.

The repricing affected options to purchase an aggregate of 1,495,491 shares of common stock held by 36 employees. The repricing was accounted for as a modification, and the incremental fair value was measured as the excess of the fair value of the modified awards immediately after the modification over the fair value of the original awards immediately before the modification, in each case using the Black-Scholes option-pricing model. The repricing resulted in incremental stock-based compensation cost of approximately $3.4 million, of which $2.0 million is currently being recognized over the 18-month retention period. During the three and nine months ended September 30, 2026, the Company recognized approximately $47.5 thousand of incremental stock-based compensation expense. As of September 30, 2026, approximately $3.4 million remained unrecognized and is expected to be recognized over the applicable remaining requisite service periods.

Stock-Based Compensation Expense

During the three and nine months ended September 30, 2026, and 2025, the Company recorded stock-based compensation in the accompanying condensed consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development expense	$ 2,317	$ 1,104	$ 6,542	$ 2,992
General and administrative expense	4,767	2,202	13,937	5,248
Total	$ 7,084	$ 3,306	$ 20,479	$ 8,240

As of September 30, 2026, the Company had unrecognized stock-based compensation expense related to unvested stock options and RSUs of $55.9 million and $4.8 million, respectively, which is expected to be recognized over weighted-average periods of 2.5 years and 1.0 years, respectively.

10. Segment Information

The following table presents segment net loss, including significant expense categories (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development expenses:				
External research and development expenses[1]	$ 14,375	$ 11,623	$ 43,000	$ 32,892
Personnel-related expenses, excluding stock-based compensation	4,952	2,782	12,052	7,659
Facility and information technology allocated expenses	331	486	1,113	1,390
General and administrative expenses:				
Personnel-related expenses, excluding stock-based compensation	4,621	2,990	12,002	7,639
General corporate and facility expenses[2]	3,221	2,317	7,435	6,162
Other segment items[3]	4,918	80	13,481	(914)
Net loss	$ 32,418	$ 20,278	$ 89,083	$ 54,828

[1]External research and development expenses consist primarily of costs paid to third-parties including contract research organizations, contract development and manufacturing organizations, consultants, advisors and lab-related vendors.

[2]General corporate and facility expenses consists primarily of professional services fees for legal, finance, human resources, medical affairs in addition to information technology expenses and rent expense, net of sublease income.

[3]Other segment items consists primarily of interest income, partially offset by taxes and non-cash expenses, such as stock-based compensation, depreciation, and amortization of discounts and premiums on marketable securities.

11. Net Loss Per Share

The Company excluded the following shares from the computation of diluted net loss per share attributable to common stockholders as of September 30, 2026, and 2025, because including them would have had an anti-dilutive effect:

	Nine Months Ended September 30,	
	2026	2025
Options to purchase common stock	6,249,331	5,713,769
Unvested restricted stock units	699,544	—
Total	6,948,875	5,713,769

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

The following discussion and analysis of our financial condition and results of operations should be read together with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report and our Annual Report on Form 10-K for the year ended December 31, 2025, which was filed with the Securities and Exchange Commission ("SEC") on March 2, 2026 (the "Annual Report").

This discussion and analysis as well as other parts of this Quarterly Report contain forward-looking statements that involve risks and uncertainties, including but not limited to, information with respect to our plans and strategy for our business. As a result of many factors, including those set forth in the section titled "Risk Factors," our actual results could differ materially from the results described in or implied by the forward-looking statements. You should carefully read, consider and evaluate the section titled "Risk Factors" to gain an understanding of the factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section titled "Cautionary Note Regarding Forward-Looking Statements" included elsewhere in this Quarterly Report. Our historical results are not necessarily indicative of the results that may be expected for any period in the future. For convenience of presentation, some of the numbers have been rounded in the text below.

Overview

We are a clinical-stage biopharmaceutical company on a mission to revolutionize the current treatment paradigm for cystic fibrosis ("CF") by developing novel medicines that improve the function of the cystic fibrosis transmembrane conductance regulator ("CFTR") protein to deliver clinically meaningful benefit to people with CF. Our goal is to deliver differentiated medicines for people with CF that can restore their CFTR function to as close to normal as possible by directly stabilizing CFTR's nucleotide binding domain 1 ("NBD1"). We are advancing a pipeline of small molecules, NBD1 stabilizers, engineered to correct the defects caused by F508del, the most common CF mutation, which resides in NBD1. Our portfolio also includes multiple complementary CFTR modulators designed to work synergistically with our NBD1 stabilizers to improve CFTR function.

In August 2026, we announced topline results from our Phase 1 clinical trial evaluating SION-451, an NBD1 stabilizer, in proprietary dual combinations with each of SION-2222 and SION-109, two of our complementary modulator candidates. The trial achieved its safety, tolerability, and pharmacokinetic (PK) objectives. Based on the totality of the clinical, PK, and nonclinical data, we selected SION-451 in combination with SION-2222 as our dual combination. The combination of SION-451 + SION-2222 demonstrated a favorable tolerability profile in the dose ranges and regimens we plan to advance. We expect to initiate the AscenSION CF Phase 2 proof-of-concept trial in the first quarter of 2027. AscenSION CF will be an open-label trial evaluating people with CF treated with SION-451 + SION-2222 for 28 days after switching from the standard of care for CF, Vertex Pharmaceuticals Incorporated's Trikafta. The trial will assess changes in sweat chloride, safety, and PK.

Also in August 2026, we announced topline results from the PreciSION CF Phase 2a proof-of-concept trial, which evaluated SION-719, an NBD1 stabilizer, as an add-on to Trikafta. PreciSION CF did not achieve its key activity endpoint, with a -1.0 mmol/L mean placebo-adjusted sweat chloride change when SION-719 was added to Trikafta (p=0.7). Based on these results, we do not plan to advance SION-719 as an add-on to the standard of care. Following this development, in September 2026, we announced a workforce reduction of approximately 46% and other cost-saving measures designed to focus our resources on advancing the dual combination program and extend our operating runway.

Additionally, in September 2026, we announced the results of a post hoc analysis of data from PreciSION CF. The post hoc analysis identified three factors that may have confounded the observed treatment effect: (1) three participants had drug exposures at key timepoints consistent with dosing non-adherence; (2) exposures of the components of Trikafta were lower on average during SION-719 treatment periods while remaining relatively stable on average during placebo periods; and (3) a potential interaction between an NBD1 stabilizer and a potentiator, a component of Trikafta, may have blunted the additional benefit of NBD1 stabilization, an interaction that we have not observed in our preclinical studies when SION-451 is combined with SION-2222. We believe these analyses suggest that SION-719 was biologically active in the trial, but that

the identified confounding factors may have affected the observed outcome and blunted a potential treatment signal.

Findings from the post hoc analysis of PreciSION CF, together with results from our Phase 1 dual combination trial, informed the decision to advance SION-451 + SION-2222. The design of the AscenSION CF trial will incorporate key learnings from PreciSION CF.

Liquidity and Financial Condition

Since our inception, we have funded our operations primarily with proceeds from private placements of convertible preferred stock and through net proceeds from our initial public offering. In March 2026, we entered into a sales agreement with Leerink Partners LLC (“Leerink”), to issue and sell shares of our common stock, from time to time, for aggregate gross sale proceeds of up to $250.0 million through an "at the market" equity offering program under which Leerink is acting as sales agent. As of September 30, 2026, we had not sold any shares of common stock under the sales agreement. As of September 30, 2026, we raised aggregate net proceeds of $530.0 million from the sale and issuance of our preferred stock and our initial public offering. We have not generated any revenue from product sales or other sources.

Due to our significant research, development and manufacturing expenditures, we have accumulated substantial losses and negative cash flows since our inception, including net losses of $89.1 million and $54.8 million for the nine months ended September 30, 2026, and 2025, respectively. As of September 30, 2026, we had an accumulated deficit of $345.4 million.

As a result of our recently implemented restructuring plan (the "Restructuring"), including a reduction in our workforce and the wind-down of activities related to the SION-719 add-on program, we expect our expenses and operating losses to decrease in the near term compared to prior periods. The amount and timing of our future research and development expenses will depend on the scope and progress of our development activities and the product candidates and programs we elect to pursue.

Although we expect our ongoing operating expenses to decrease in the near term as a result of the Restructuring, we expect to continue to incur significant expenses and operating losses for the foreseeable future as we:

- continue to advance the clinical development of our current and future product candidates, including conducting our planned clinical trial(s);
- continue to advance our research and preclinical activities and seek to discover and develop additional product candidates;
- continue to utilize third parties to manufacture our product candidates and ensure sufficient manufacturing capabilities to produce our drug substances and drug products;
- continue to develop, maintain, expand, enforce, defend and protect our intellectual property portfolio (including intellectual property obtained through license agreements) and provide reimbursement of third-party expenses related to our patent portfolio;
- attract, hire and retain additional qualified personnel;
- seek regulatory approvals for any current or future product candidates that successfully complete clinical trials;
- make any potential milestones, royalties or other payments due under any current or future in-license, collaboration or other agreements;
- undertake any pre-commercial activities and scale-up external commercial-scale manufacturing capabilities;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may receive regulatory approval;
- add additional operational, financial, clinical, quality and management information systems; and
- incur additional audit, legal, regulatory, tax and other expenses associated with being a public company.

In addition, we have several clinical development, regulatory, and commercial milestones, as well as royalty payment obligations under our licensing arrangements. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our planned clinical trial(s) and our expenditures on other research and development activities.

We do not have any products approved for sale and have not generated any revenue from product sales. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our current and any future product candidates, which we expect will take a number of years or may never occur. As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings or other capital sources, potentially including collaborations, licenses or other strategic arrangements. See the section titled "*—Liquidity and Capital Resources.*" We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates, or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Due to the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses or the timing of when, or if, we will be able to achieve or maintain profitability. Even if we generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $249.8 million. Based upon our current operating plans, we believe that our existing cash, cash equivalents and marketable securities will be sufficient to fund our operations into the second half of 2029. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See the sections titled "*—Liquidity and Capital Resources*" and "*Risk Factors—Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital*" included elsewhere in this Quarterly Report.

License and Collaboration Agreements

We have entered into several license agreements with various third parties. The terms and conditions as well as accounting analysis for our significant license agreements are described in Note 9, "*License Agreements,*" in the audited consolidated annual financial statements in the Annual Report. There have been no material changes to the terms and conditions, or accounting conclusions, previously disclosed in the Annual Report.

Components of Results of Operations

Revenue

To date, we have not generated any revenue. In the future, we may generate revenue from product sales from any approved product, which approval we do not expect to occur for at least the next several years, if ever, as well as collaboration or license agreements we may enter into with respect to our current or future product candidates. We cannot predict if, when or to what extent we will generate revenue as we may never succeed in obtaining regulatory approval for any of our product candidates. If we fail to complete preclinical and clinical development of our current or future product candidates or fail to obtain regulatory approval for any that successfully complete clinical trials, our ability to generate future revenues, and our results of operations and financial position would be adversely affected.

Operating Expenses

Our operating expenses consist of (i) research and development expenses and (ii) general and administrative expenses.

Research and Development Expenses

Research and development expenses consist primarily of costs associated with the preclinical and clinical development of our current and potential future product candidates. In particular, our research and development expenses may include:

- personnel-related costs, including salaries, payroll tax, bonuses, benefits, severance and stock-based compensation for employees engaged in research and development functions;
- the costs to acquire in-process research and development ("IPR&D") with no alternative future use acquired in an asset acquisition;
- external expenses, including expenses incurred under arrangements with third parties, such as contract research organizations, contract development and manufacturing organizations, consultants and our scientific and clinical advisors;
- the cost of manufacturing our product candidates, including costs for laboratory supplies, research materials and reagents;
- facility costs, depreciation and other expenses, which include direct and allocated expenses; and
- the cost of obtaining and maintaining patent and trade secret protection for our product candidates.

We recognize research and development costs in the periods during which they are incurred. Most of our research and development expenses have been related to identifying and developing our product candidates. Typically, external expenses are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers as of each reporting date. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses, which are expensed as the related goods are delivered or the services are performed, or when it is no longer expected that the goods will be delivered or the services rendered. Significant judgments and estimates are made in determining the accrued or prepaid expense balances at the end of any reporting period.

External costs represent a significant portion of our research and development expenses, which we track on a program-by-program basis following the nomination of a product candidate. Our internal research and development expenses consist primarily of personnel-related expenses, including stock-based compensation expenses and allocated expenses. We do not track our internal research and development expenses on a program-by-program basis because they either relate to early-stage research expenses, such as lab supplies or our personnel expenses, consulting fees or other costs that are deployed across multiple programs.

As a result of the wind-down of the SION-719 add-on program and the reduction in our research and development workforce under the Restructuring, we expect our ongoing research and development expenses to decrease in the near term. The amount and timing of our future research and development expenses will depend on the scope and progress of our development activities and the product candidates and programs we elect to pursue.

Product candidates in later stages of development generally have higher development costs than those in earlier stages resulting from larger and more complex clinical trials, manufacturing scale-up and an increase in research and development headcount to oversee these activities. As a result, management expects that our research and development expenses will increase substantially over the next several years as we potentially advance our product candidates into later-stage development efforts.

Our future development costs may vary significantly based on a variety of factors, including:

- the timing, complexity and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- the timing and extent of manufacturing scale-up activities, including the development of later-stage manufacturing processes and production of clinical materials;
- the extent to which we in-license or acquire other product candidates and technologies to further develop our pipeline;

- the successful initiation and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to domestic and foreign regulatory authorities;
- receipt of marketing approvals, if any, from applicable regulatory authorities;
- the development of commercial-scale manufacturing and distribution processes for our current and any future product candidates;
- our ability to obtain, maintain and protect patent, trade secret protection and regulatory exclusivity for our product candidates, both in the U.S. and internationally;
- our ability to successfully recruit and retain additional employees;
- the timing and amount of milestones, royalties or other payments we must make to our licensing partners; and
- the commercialization of our product candidates, if and when approved.

A change in the outcome of any of these variables with respect to the development of any current or future product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the U.S. Food and Drug Administration ("FDA") or another regulatory authority were to delay a planned start of a clinical trial or require us to conduct clinical trials beyond those that we currently anticipate would be required for the completion of clinical development of a product candidate, or if we experience significant delays in our clinical trials due to slower than expected patient enrollment or other reasons, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate. We do not have control over many of these factors, including certain aspects of clinical development, the regulatory submissions process, potential threats to our intellectual property rights and general political and economic conditions that may negatively impact our business in the future. We may never obtain regulatory approval for any of our product candidates, and, even if we do, drug commercialization takes several years and millions of dollars in development costs.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, payroll tax, bonuses, benefits, severance and stock-based compensation charges for those individuals in executive, legal, finance, human resources, information technology and other administrative functions. Other significant costs include legal fees relating to intellectual property and corporate matters, professional fees for auditing, accounting, tax and consulting services. Lastly, corporate overhead expenses such as information technology, insurance, facilities, and depreciation that are not allocated to the Company’s research and development activities are included in general and administrative expenses. We recognize general and administrative expenses in the periods in which they are incurred.

As a result of the reduction in our general and administrative workforce and other cost-saving measures implemented under the Restructuring, we expect our ongoing general and administrative expenses to decrease in the near term. However, certain costs associated with operating as a public company may remain significant or increase, including expenses related to audit, accounting, legal, intellectual property, director and officer insurance, and investor and public relations activities. Over the longer-term, our general and administrative expenses could increase if we expand our research and development activities, undertake pre-commercial activities for our product candidates and any future product candidates and, if any product candidate receives marketing approval, commercialization activities. These increases will likely include increased costs related to the hiring of additional personnel and fees paid to outside consultants, among other expenses.

Interest Income

Interest income consists primarily of interest earned and the amortization of discount or premiums on our cash equivalents and investments in marketable securities.

Results of Operations

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

The following table summarizes our results of operations (in thousands):

	Three Months Ended September 30,		
	2026	2025	Change
Operating expenses:			
Research and development	$ 22,025	$ 16,044	$ 5,981
General and administrative	12,794	7,761	5,033
Total operating expenses	34,819	23,805	11,014
Loss from operations	(34,819)	(23,805)	(11,014)
Other income:			
Interest income	2,407	3,456	(1,049)
Other income (expense), net	(6)	71	(77)
Total other income:	2,401	3,527	(1,126)
Net loss	$ (32,418)	$ (20,278)	$ (12,140)

Research and Development Expenses

The following table summarizes our research and development expenses (in thousands):

	Three Months Ended September 30,		
	2026	2025	Change
Direct research and development expenses by program:			
NBD1 and combination development programs[1]	$ 10,510	$ 8,609	$ 1,901
Complementary modulator programs[2]	1,916	1,532	384
Unallocated research and development expenses:			
Personnel-related (including stock-based compensation)	7,270	3,886	3,384
Other R&D related costs	1,897	1,377	520
Facility, lab and depreciation	432	640	(208)
Total research and development expenses	$ 22,025	$ 16,044	$ 5,981

[1]NBD1 and combination development program costs include NBD1 stabilizer activities and development of our proprietary combination therapy.

[2]Complementary modulator program costs include manufacturing expenses for the complementary modulators.

Research and development expenses increased by $6.0 million to $22.0 million for the three months ended September 30, 2026, from $16.0 million for the three months ended September 30, 2025. The increase in research and development expenses was primarily due to:

- direct research and development expenses increased by $2.3 million primarily due to an increase in development activities and activities related to the wind-down of the SION-719 add-on program; and
- unallocated research and development expenses increased by $3.7 million primarily due to a $3.4 million increase in personnel-related expenses including stock-based compensation, and restructuring related costs associated with the workforce reduction.

General and Administrative Expenses

The following table summarizes our general and administrative expenses (in thousands):

	Three Months Ended September 30,		
	2026	2025	Change
Personnel-related (including stock-based compensation)	$ 9,387	$ 5,192	$ 4,195
Professional services & fees	2,818	2,049	769
Facility and depreciation	589	520	69
Total general and administrative expenses	$ 12,794	$ 7,761	$ 5,033

General and administrative expenses increased by $5.0 million to $12.8 million for the three months ended September 30, 2026, from $7.8 million for the three months ended September 30, 2025. The increase in general and administrative expenses was primarily due to:

- $4.2 million increase in personnel-related expenses including stock-based compensation, and restructuring related costs associated with the workforce reduction.

Interest Income

Interest income decreased by $1.0 million to $2.4 million for the three months ended September 30, 2026, from $3.5 million for the three months ended September 30, 2025, primarily due to lower average balances of cash, cash equivalents and investments in marketable securities, reflecting the use of such balances to fund operating expenses.

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

The following table summarizes our results of operations (in thousands):

	Nine Months Ended September 30,		
	2026	2025	Change
Operating expenses:			
Research and development	$ 62,852	$ 45,095	$ 17,757
General and administrative	34,036	20,275	13,761
Total operating expenses	96,888	65,370	31,518
Loss from operations	(96,888)	(65,370)	(31,518)
Other income:			
Interest income	7,814	10,123	(2,309)
Other income (expense), net	(9)	419	(428)
Total other income:	7,805	10,542	(2,737)
Net loss	$ (89,083)	$ (54,828)	$ (34,255)

Research and Development Expenses

The following table summarizes our research and development expenses (in thousands):

	Nine Months Ended September 30,		
	2026	2025	Change
Direct research and development expenses by program:			
NBD1 and combination development programs[1]	$ 32,428	$ 25,540	$ 6,888
Complementary modulator programs[2]	5,739	3,813	1,926
Unallocated research and development expenses:			
Personnel-related (including stock-based compensation)	18,593	10,650	7,943
Other R&D related costs	4,542	3,294	1,248
Facility, lab and depreciation	1,550	1,798	(248)
Total research and development expenses	$ 62,852	$ 45,095	$ 17,757

[1]NBD1 and combination development program costs include NBD1 stabilizer activities and development of our proprietary combination therapy.

[2]Complementary modulator program costs include manufacturing expenses for the complementary modulators.

Research and development expenses increased by $17.8 million to $62.9 million for the nine months ended September 30, 2026, from $45.1 million for the nine months ended September 30, 2025. The increase in research and development expenses was primarily due to:

- direct research and development expenses increased by $8.8 million primarily due to an increase in development activities and activities related to the wind-down of the SION-719 add-on program; and
- unallocated research and development expenses increased by $8.9 million primarily due to a $7.9 million increase in personnel-related expenses including stock-based compensation, and restructuring related costs associated with the workforce reduction.

General and Administrative Expenses

The following table summarizes our general and administrative expenses (in thousands):

	Nine Months Ended September 30,		
	2026	2025	Change
Personnel-related (including stock-based compensation)	$ 25,939	$ 12,889	$ 13,050
Professional services & fees	6,388	5,957	431
Facility and depreciation	1,709	1,429	280
Total general and administrative expenses	$ 34,036	$ 20,275	$ 13,761

General and administrative expenses increased by $13.8 million to $34.0 million for the nine months ended September 30, 2026, from $20.3 million for the nine months ended September 30, 2025. The increase in general and administrative expenses was primarily due to:

- $13.1 million increase in personnel-related expenses including stock-based compensation, and restructuring related costs associated with the workforce reduction.

Interest Income

Interest income decreased by $2.3 million to $7.8 million for the nine months ended September 30, 2026, from $10.1 million for the nine months ended September 30, 2025, primarily due to lower average balances of cash, cash equivalents and investments in marketable securities, reflecting the use of such balances to fund operating expenses.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from operations. Although we expect our ongoing operating expenses to decrease in the near term as a result of the Restructuring, we expect to continue to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates and maintain the infrastructure necessary to operate as a public company. Our expenses could increase over the longer term if we advance our current or future product candidates into later stages of development or toward commercialization. As a result, we will need additional capital to fund our operations, which we may obtain from additional equity or debt financings or strategic agreements.

In March 2026, we entered into a sales agreement with Leerink to issue and sell shares of our common stock, from time to time, for aggregate gross sale proceeds of up to $250.0 million through an "at the market" equity offering program under which Leerink is acting as sales agent. As of September 30, 2026, we had not sold any shares of common stock under the sales agreement.

As of September 30, 2026, we had $249.8 million in cash, cash equivalents and marketable securities.

Cash Flows

The following table sets forth a summary of the net cash flow activity (in thousands):

	Nine Months Ended September 30,	
	2026	2025
Net cash used in operating activities	$ (62,783)	$ (49,671)
Net cash provided by (used in) investing activities	85,235	(141,337)
Net cash provided by financing activities	2,975	204,051
Net increase in cash, cash equivalents and restricted cash	$ 25,427	$ 13,043

Operating Activities

For the nine months ended September 30, 2026, net cash used in operating activities was $62.8 million primarily due to our net loss of $89.1 million and changes in operating assets and liabilities of $4.5 million, partially offset by $21.8 million of net non-cash charges, which includes stock-based compensation, depreciation, non-cash operating lease expense and amortization of discounts on marketable securities.

For the nine months ended September 30, 2025, net cash used in operating activities was $49.7 million primarily due to our net loss of $54.8 million and changes in operating assets and liabilities of $2.4 million, partially offset by $7.5 million of net non-cash charges, which includes stock-based compensation, depreciation, non-cash operating lease expense and amortization of discounts on marketable securities.

Investing Activities

Net cash provided by investing activities was $85.2 million during the nine months ended September 30, 2026, which was primarily driven by maturities of marketable securities of $154.9 million, partially offset by purchases of marketable securities of $69.7 million.

Net cash used in investing activities was $141.3 million during the nine months ended September 30, 2025, which was primarily driven by purchases of marketable securities of $282.1 million, partially offset by maturities of marketable securities of $141.0 million.

Financing Activities

Net cash provided by financing activities was $3.0 million during the nine months ended September 30, 2026, primarily due to exercise proceeds totaling $3.6 million, partially offset by payments of deferred offering costs of $0.6 million related to the sales agreement entered into in March 2026.

Net cash provided by financing activities was $204.1 million during the nine months ended September 30, 2025, during which time the Company closed its initial public offering.

Future Funding Requirements

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $249.8 million. Based upon our current operating plans, we believe that our existing cash, cash equivalents and investments in marketable securities, will be sufficient to fund our operations into the second half of 2029. However, our forecast for the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. Additionally, conducting preclinical studies and clinical trials is costly, and the timing of progress and expenses in these studies and trials is uncertain. We will need to raise substantial additional capital in the future.

Our future funding requirements will depend largely on:

- the type, number, scope, progress, expansions, results, costs and timing of discovery, preclinical studies and clinical trials of our current and future product candidates;
- the costs and timing of manufacturing for our current and future product candidates and commercial manufacturing;
- the costs, timing and outcome of regulatory review of our current and future product candidates;
- the timing and amount of milestones, royalties, or other payments we may be required to make to third parties, including Sanofi SA, the Cystic Fibrosis Foundation and AbbVie, and the terms and timing of establishing and maintaining any other similar arrangements we may enter into in the future;
- the legal costs of obtaining, maintaining and enforcing our patents and other intellectual property rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company;
- the costs associated with hiring additional personnel and consultants as our clinical activities increase;
- the costs and timing of establishing or securing sales and marketing capabilities if any current or future product candidate is approved;
- 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 financial terms of any such agreement that we may enter into, including if we in-license or acquire additional product candidates or intellectual property; and
- costs to add additional operational, financial, clinical, quality and management information systems.

We have no committed sources of capital. Until such time, if ever, as we can generate substantial product revenue to support our cost structure, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, potentially including collaborations, licenses or other strategic arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. In addition, debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends. If we raise additional funds through a strategic agreement, we may have to grant rights to develop and market our current and future product candidates even if we would otherwise prefer to develop and market such product candidates ourselves. Our failure to raise capital or enter

into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies.

If we are unable to raise additional funds, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Contractual Obligations and Commitments

In connection with the Restructuring, we incurred obligations related to employee severance and other termination benefits and the wind-down of certain clinical, manufacturing, and other vendor arrangements associated with the SION-719 add-on program. As of September 30, 2026, approximately $4.1 million of these obligations were unpaid. We expect to make the related cash payments primarily through the fourth quarter of 2026. See Note 1 to our condensed consolidated financial statements for additional information regarding the Restructuring.

Other than this matter, there were no material changes to our contractual obligations and commitments described under "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" in the Annual Report.

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which are prepared in accordance with GAAP. The preparation of our consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements and accompanying notes. We base our estimates and assumptions on historical experience and other factors that we believe to be reasonable under the circumstances. We evaluate our estimates and judgments on an ongoing basis. We base our estimates on historical experience, known trends and events 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 values of assets and liabilities that are not readily apparent from other sources. Our actual results may differ from these estimates under different assumptions or conditions.

During the nine months ended September 30, 2026, there were no material changes to our critical accounting estimates described under "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" in the Annual Report.

Recent Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2, "*Summary of Significant Accounting Policies*" in our consolidated financial statements and our unaudited interim condensed consolidated financial statements included elsewhere in this Quarterly Report.

Emerging Growth Company and Smaller Reporting Company Status

We are an "emerging growth company," as defined in the JOBS Act, and we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. We may take advantage of these exemptions until we are no longer an emerging growth company. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. We have elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, our consolidated financial statements may not be comparable to companies that comply with public company effective dates. We may take advantage of these exemptions up until the time that we are no longer an "emerging growth company."

We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering, (b) in which we have total annual

gross revenue of at least $1.235 billion or (c) in which we are deemed to be a “large accelerated filer” under the rules of the SEC, which means, among other things, the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the last business day of our most recently completed second fiscal quarter and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We are also a “smaller reporting company” as defined in the Exchange Act. We may take advantage of certain of the scaled disclosures available to smaller reporting companies for so long as either (i) our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter. Based on the market value of our common stock held by our non-affiliates as of June 30, 2026, we will no longer be a “smaller reporting company” as of January 1, 2027, but remain eligible to use the requirements of a smaller reporting company until our Quarterly Report on Form 10-Q for the quarter ending March 31, 2027.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

As a "smaller reporting company," as defined by Rule 12b-2 of the Exchange Act, and pursuant to Item 305 of Regulation S-K, we are not required to provide quantitative and qualitative disclosures about market risk.

Item 4. Controls and Procedures

Management's Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Our disclosure controls and procedures are designed to ensure that information required to be disclosed by us in the reports we file under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. As required by Rule 13a-15(b) or Rule 15d-15(b) promulgated by the SEC under the Exchange Act, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this Quarterly Report. Based on the foregoing, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the period covered by this Quarterly Report on Form 10-Q that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Part II - Other Information

Item 1. Legal Proceedings

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

Item 1A. Risk Factors

Our future operating results could differ materially from the results described in this Quarterly Report due to the risks and uncertainties described below. You should consider carefully the following information about risks below in evaluating our business. If any of the following risks actually occur, our business, financial condition, results of operations and future growth prospects will likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline. In addition, we cannot assure investors that our assumptions and expectations will prove to be correct. Important factors could cause our actual results to differ materially from those indicated or implied by forward-looking statements. See "Cautionary Note Regarding Forward-Looking Statements" on page 2 of this Quarterly Report for discussion of some of the forward-looking statements that are qualified by these risk factors. Factors that could cause or contribute to such differences include those factors discussed below.

Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital

We are a clinical-stage biopharmaceutical company and have incurred significant operating losses since inception and anticipate that we will continue to incur significant operating losses for the foreseeable future. Our net losses were $89.1 million and $54.8 million for the nine months ended September 30, 2026, and 2025, respectively. We had an accumulated deficit of $345.4 million as of September 30, 2026. We may never achieve or maintain profitability.

We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred operating losses in each year since our inception. Our net losses were $89.1 million and $54.8 million for the nine months ended September 30, 2026, and 2025, respectively. We had an accumulated deficit of $345.4 million as of September 30, 2026. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. 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.

Since our inception in 2019, we have devoted substantially all of our efforts and financial resources to the development of our product candidates, the continuation of ongoing clinical trials, the commencement of new clinical trials and ongoing manufacturing to support our product candidates. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have not yet demonstrated an ability to conduct later-stage clinical trials, obtain regulatory approval, manufacture any product on a commercial scale or conduct sales and marketing activities necessary for successful product commercialization, and there is no assurance that we will accomplish any of these abilities in the future. Our limited operating history makes any assessment of our future success and viability subject to significant uncertainty. In addition, if we obtain marketing approval for any of our product candidates, we will incur significant sales, marketing and manufacturing expenses. We expect to continue to incur significant costs associated with operating as a public company. As a result, we expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

The amount of our future losses is uncertain, and our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline. Our operating losses may fluctuate significantly from quarter to quarter and from year to year. Although we expect our ongoing operating expenses to decrease in the near term as a result

of our recently implemented restructuring plan (the "Restructuring"), we expect to continue to incur significant expenses and operating losses for the foreseeable future as we:

- continue to advance clinical development of our current and future product candidates, including conducting our planned clinical trial(s);
- continue to advance our research and preclinical activities and seek to discover and develop additional product candidates;
- continue to utilize third parties to manufacture our product candidates and ensure sufficient supply of our manufacturing of drug substances and drug products;
- continue to develop, maintain, expand and protect our intellectual property portfolio (including intellectual property obtained through license agreements) and provide reimbursement of third-party expenses related to our patent portfolio;
- attract, hire and retain additional qualified personnel;
- seek regulatory approvals for any current or future product candidates that successfully complete clinical trials;
- make any potential milestones, royalties or other payments due under any current or future in-license, collaboration or other agreements;
- undertake any pre-commercial activities and scale up external commercial-scale manufacturing capabilities;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain regulatory approval;
- add additional operational, financial, clinical, quality and management information systems; and
- incur additional audit, legal, regulatory, tax and other expenses associated with being a public company.

We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

Given the numerous risks and uncertainties associated with pharmaceutical product development, it is not certain if any of our current or future product candidates will advance through late-stage development or be approved for commercial sale; therefore, we are unable to predict if or when we will generate product revenue or achieve or maintain profitability.

Even if we successfully complete development and obtain the necessary regulatory approval for commercialization for any of our product candidates, we anticipate incurring significant costs associated with launching and commercializing such products. If we fail to become profitable or do not sustain profitability on a continuing basis, we may be unable to continue our operations at planned levels and be forced to reduce or cease operations.

We will need substantial additional funding to develop and, if approved, commercialize our product candidates and identify and invest in new product candidates. We may be unable to raise capital on acceptable terms, if at all, and, as a result, we may be required to delay, reduce or eliminate our product development programs or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approvals and achieve product sales. Although we expect our ongoing operating expenses to decrease in the near term as a result of the Restructuring, we expect to continue to incur significant and increasing expenses and operating losses for the foreseeable future as we initiate and conduct clinical trials of our current and any future product candidates, scale-up and manufacture our product candidates, advance our preclinical programs, seek marketing and regulatory approvals for any product candidates that successfully complete clinical trials and commercialize our products, if approved. Because the outcome of any clinical trial or preclinical study is highly uncertain, we cannot reliably project the availability or terms of financing or estimate the actual amount of financing necessary to successfully complete the development and commercialization of any of our product candidates.

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $249.8 million. We believe that, based upon our current operating plans, our existing cash, cash equivalents and marketable securities will be sufficient to fund our operations into the second half of 2029. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including but not limited to changes in progress of our development activities, acquisitions of additional product candidates and changes in regulations. Our future capital requirements will depend on, and could increase significantly as a result of, many factors, including:

- the scope, timing, progress, costs, complexity and results of discovery, preclinical development and clinical trials for our current or future product candidates, including any additional expenses attributable to adjusting our anticipated development plans;
- the number of clinical trials required for regulatory approvals of our current or future product candidates;
- the extent to which we develop, in-license or acquire other product candidates in our pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development and, if approved, commercialization;
- the number and development requirements of product candidates that we may pursue;
- the timing and amount of the milestone, royalty or other payments we must make to Sanofi SA (“Sanofi”), the Cystic Fibrosis Foundation, AbbVie Global Enterprises Ltd. (“AbbVie”) and any other third parties;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors (or patients’ willingness to pay out-of-pocket for any approved products in the absence of such coverage) and adequate market share and revenue for any approved products;
- the revenue, if any, received from commercial sales of any product candidates for which we receive marketing approval;
- the effect of macroeconomic trends including fluctuating inflation rates, capital markets disruption, and interest rates;

- addressing any potential supply chain interruptions or delays, which may be due in part to international tariffs; and
- the costs of operating as a public company.

We will require additional capital to achieve our business objectives. Additional funds may not be available on a timely basis, on favorable terms or at all, including if results of clinical trials are not positive, and such funds, if raised, may not be sufficient to enable us to continue implementing our long-term business strategy. Further, our ability to raise additional capital may be adversely impacted by potentially worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States ("U.S.") and worldwide resulting from factors that include but are not limited to, fluctuating inflation and capital markets disruptions, international tariffs, ongoing conflicts between Russia and Ukraine and in the Middle East, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, uncertainty about economic stability and other factors. For example, the U.S. government has imposed substantial tariffs on most countries throughout the world and has further threatened to continue to broadly impose tariffs, which could lead to corresponding punitive actions by the countries with which the U.S. trades. If the equity and credit markets continue to deteriorate, it may make any necessary debt or equity financing more difficult to obtain, more costly and more dilutive. If we are unable to raise sufficient additional capital, we could be forced to curtail our planned operations and the pursuit of our growth strategy, or even cease operations.

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

Until such time, if ever, as we can generate substantial revenues from product sales, we expect to finance our cash needs through a combination of equity offerings, debt financings or other capital sources, such as grants, collaborations, licenses or other similar arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interest may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as a common stockholder. Debt financing and preferred equity financing may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt or making capital expenditures. Such restrictions could adversely impact our ability to conduct our operations and execute our business plan.

If we raise additional funds through grants, collaborations, licenses or other similar arrangements with third parties, we may be required to relinquish valuable rights to our future revenue streams, intellectual property or product candidates, grant licenses on terms that may not be favorable to us and/or that may reduce the value of our common stock or commit us to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed or on terms acceptable to us, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, or on less favorable terms than we would otherwise choose.

We maintain the majority of our cash and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at certain of these institutions exceed insured limits. Market conditions and changes in financial regulations and policies can impact the viability of these institutions. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position. In addition, changes in regulations governing financial institutions are beyond our control and difficult to predict; consequently, the impact of such changes on our business and results of operations is difficult to predict and may have an adverse effect on us.

Risks Related to the Discovery and Development of Our Product Candidates

We are substantially dependent on the success of our dual combination of SION-451 + SION-2222. If we are unable to advance SION-451 + SION-2222 into our planned proof-of-concept clinical trial or later-stage clinical development, or to obtain regulatory approval and commercialize a combination therapy

of SION-451 + SION-2222 for the treatment of cystic fibrosis, or experience significant delays in doing so, our business will be materially harmed.

To date, as an organization, we have not completed the development of any product candidates. We are substantially dependent on the success of our proprietary dual combination of SION-451, a nucleotide binding domain 1 ("NBD1") stabilizer product candidate, and SION-2222, a complementary modulator product candidate, both of which are currently in development for the treatment of cystic fibrosis ("CF"). Following the PreciSION CF trial, which did not yield positive results, we discontinued the development of SION-719 as an add-on to the standard of care. As a result, our near-term prospects depend on the outcome of the AscenSION CF Phase 2 proof-of-concept trial, which we expect to initiate in the first quarter of 2027. The timely and successful completion of the AscenSION CF trial is subject to a number of risks, including risks related to participant retention. For example, participants enrolled in the AscenSION CF trial will be required to switch from the standard of care to an investigational combination therapy of SION-451 + SION-2222. This trial design may make it more difficult to retain participants, particularly if participants who do not experience a clinical benefit or who experience adverse events elect to switch back to the standard of care and withdraw from the trial. If the AscenSION CF trial is not timely and successfully completed, we may be required to delay, scale back or discontinue our current development strategy, and we may not be able to advance alternative product candidates, which would have a material adverse effect on our business, financial condition and prospects.

The success of SION-451 + SION-2222 and any other product candidates we may advance will depend on several factors, including the following:

- successful and timely initiation and enrollment of clinical trials and completion of clinical trials with favorable results, including for our planned AscenSION CF Phase 2 proof-of-concept trial;
- the safety, tolerability and pharmacokinetic profile of our product candidates observed in clinical trials;
- acceptance of regulatory submissions by the U.S. Food and Drug Administration (“FDA”) and/ or comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates, and the proposed design of our planned clinical trials;
- the frequency and severity of adverse safety findings in nonclinical studies and adverse events (“AEs”) in clinical trials;
- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and *in vitro* dose projection studies in animals, where applicable;
- acceptance of our products, if approved, by CF patients, the medical community and third-party payors, and their perspective on the cost, safety, tolerability and efficacy and perceived advantages of alternative therapies for CF, including the standard of care;
- maintaining relationships with contract research organizations (“CROs”) and clinical sites for the clinical development of our product candidates and the ability of such CROs and clinical sites to comply with clinical trial protocols, Good Clinical Practices (“GCPs”) and other applicable requirements;
- demonstrating the safety and efficacy of our product candidates to the satisfaction of applicable regulatory authorities;
- receipt and maintenance of marketing approvals from applicable regulatory authorities for our initial target indication and label expansions to include new populations;
- our ability to establish and maintain relationships with third-party manufacturers and their ability to comply with current good manufacturing practices (“cGMPs”), as well as our ability to make arrangements with third-party manufacturers to ensure adequate supply for our clinical trials and commercial manufacturing capabilities at a cost and scale sufficient to support commercialization;
- the availability of drug substance and drug product for use in production of our product candidates;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining, establishing, maintaining and enforcing patent and any potential trade secret protection or regulatory exclusivity for our product candidates;
- maintaining an acceptable safety profile of our product candidates following regulatory approvals, if any;

- the sufficiency of our financial resources to fund our operations and our ability to raise necessary additional funds;
- the impact of competition with other products approved for the treatment of CF and product candidates in development for the treatment of CF; and
- maintaining and growing an organization of people who can develop and, if approved, commercialize, market and sell our current or future product candidates.

If we are unable to develop, receive marketing approval for and successfully commercialize our product candidates, or if we experience delays as a result of any of the above factors or otherwise, our business would be significantly harmed.

We are early in our development efforts. If we are unable to successfully develop, receive regulatory approval for and commercialize any product candidate, or experience significant delays in doing so, our business will be substantially harmed.

We are early in our development efforts. Each of our product candidates will require additional preclinical and/or clinical development and regulatory approval, and will require us to obtain sufficient manufacturing supply, capacity and expertise. Our lead product candidates, SION-451 and SION-2222, are in early clinical development and we have not completed any clinical trials in CF patients for such product candidates. Further, we will be required to build a commercial organization or successfully outsource commercialization, and make substantial investment and significant marketing efforts before we generate any revenue from product sales, if ever. Given our early stage of development, it will take several years before we can demonstrate the safety and efficacy of a product candidate sufficient to warrant approval for commercialization, if we can do so at all.

We do not have complete control over many of the factors that affect our development efforts, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for our product candidates or any future product candidate we develop or if we experience delays in such approvals, we may not be able to continue our operations.

Further, we have conducted, and may in the future conduct, clinical trials of our current or future product candidates outside the United States. Conducting clinical trials in foreign countries 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, failure to properly translate or interpret patient-reported outcome endpoints, managing additional administrative burdens associated with foreign regulatory schemes as well as political and economic risks relevant to such foreign countries. Recent policy proposals in the U.S., if enacted in the future, may make acceptance by the FDA or inclusion in a marketing application of foreign data more difficult or costly. In addition, the FDA recently signaled increased scrutiny of foreign clinical trial data submitted in support of marketing applications, indicating plans for increased foreign site inspections and increased transparency of inspection results.

The foregoing makes our ability to successfully and timely complete development of our product candidates and obtain regulatory approval for them less certain. If we are unable to develop, or obtain marketing approval for, or, if approved, successfully commercialize our product candidates, our business, financial condition, results of operations and prospects would be materially harmed.

We are developing a proprietary dual combination of an NBD1 stabilizer with a complementary modulator. Developing combination treatments increases complexity and risk, including risks of drug-drug interactions, unforeseen side effects or failures in our clinical trials that could delay or prevent their regulatory approval or limit the commercial profile of an approved label.

The current treatment paradigm in CF for all patients with the F508del mutation is based on a combination of drug therapies. We expect to initiate the AscenSION CF Phase 2 proof-of-concept trial in CF

patients in the first quarter of 2027. AscenSION CF will be an open-label trial evaluating people with CF who are homozygous for the F508del mutation treated with SION-451 + SION-2222 for 28 days after switching from Trikafta. The use of our product candidates in combination with each other may subject us to risks that we would not face if our product candidates were being developed to be administered as a monotherapy.

The combination of our product candidates with each other, or when used in patients already receiving other companies' products or product candidates, may result in adverse side effects or toxicities that the product candidates do not produce when used alone. In addition, the product candidates may interact with each other, or with other companies' products or product candidates that patients receiving our product candidates may also be receiving, in undesirable ways that could negatively impact the efficacy of our product candidates. For example, the post hoc analysis of data from PreciSION CF identified a potential interaction between SION-719, an NBD1 stabilizer, and a potentiator, a component of Trikafta, which may have blunted the potential additional benefit of NBD1 stabilization. Testing product candidates in combination with each other may increase the risk of significant adverse effects or failed clinical trials. Further, our planned AscenSION CF trial will require participants to take two separate drugs on different dosing schedules, which may increase the risk of dosing errors and reduce participant compliance. The timing, outcome and cost of developing products to be used in a combination therapy is difficult to predict and dependent on a number of factors that are outside our reasonable control. If serious adverse or unexpected side effects are identified during development and are determined to be attributed to our product candidates, we, the FDA, or comparable foreign regulatory authorities, or institutional review boards and other reviewing entities, could interrupt, delay, or halt our clinical trials and the observed effects could result in a more restrictive label or particularly narrow product indication (substantially limiting the product's commercial opportunities), a risk evaluation and mitigation strategy ("REMS") program or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities.

The occurrence of any of these risks could result in a dual combination therapy, if developed and approved, being removed from the market or being less successful commercially. If we experience safety, tolerability or toxicity issues in any of our planned combination treatment clinical trials, or the data from these trials regarding the efficacy of the combinations are not favorable, our clinical development plans could be materially negatively affected or delayed, or we may not receive regulatory approval for our product candidates, which would materially harm our business and likely cause the market price of our common stock to decline.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain the required regulatory approval for any product candidate, our business will be substantially harmed.

We are not permitted to market, commercialize, sell or promote any product candidate in the U.S. until we receive regulatory approval of a new drug application (“NDA”) for such product candidate in a specific indication from the FDA. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize our product candidates and any future product candidates in a timely manner. The time required to obtain approval or other marketing authorizations by the FDA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations and the type and amount of data necessary to gain approval may change during the course of a product candidate’s clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates in the future.

Prior to obtaining approval to commercialize any product candidate in the U.S. or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidate is safe and effective for its intended use. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs. Furthermore, because we are developing an NBD1 stabilizer product candidate in combination with a complementary modulator product candidate, we will have to satisfy the regulatory agencies’ requirements to demonstrate the contribution of each component in the combination. As

neither product candidate in this potential combination is an approved drug, the FDA and other regulatory authorities will require full demonstration of the safety of each component (alone and in combination), as well as the efficacy of the combination (and the contribution of each component). Accordingly, development of a proprietary dual combination therapy may require additional safety, efficacy and/or drug-drug interaction data relative to what would be required for a monotherapy.

Our current and future product candidates could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree as to the design or implementation of our clinical trials and interpretation of data from clinical trials or preclinical studies;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's benefits outweigh its risks;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA to the FDA or other submission to obtain regulatory approval in the European Union or elsewhere;
- the FDA or comparable foreign regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects. The FDA and comparable foreign authorities have substantial discretion in the approval process and determining when or whether regulatory approval will be granted for any product candidate that we develop. The U.S. Supreme Court's July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays or changes.

Even if we complete clinical testing and receive approval of an NDA or foreign marketing application for our current or future product candidates, the FDA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-marketing clinical trials. The FDA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited indication or patient population than we originally request, and the FDA or applicable foreign regulatory authority may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization, or failure to obtain our desired product label, would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

In addition, the FDA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations or take other actions, which may prevent or delay approval of our future product candidates under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may obtain. Further, macroeconomic and other global conditions have impacted and could in the future impact the ability of the FDA and comparable foreign regulatory authorities to provide any required approvals or marketing authorizations for our product candidates or result in the delay of such approvals or authorizations. Changes to the requirements

and policies of the FDA, the SEC and other regulatory agencies with jurisdiction over our product candidates and our business could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates. Some of these efforts have manifested to date in the form of personnel measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development, to have preapproval inspections completed in a timely fashion, and to obtain the requisite regulatory approvals in the future.

There remains general uncertainty regarding future potential policy changes at the FDA and other regulatory agencies, which could adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic products. Additionally, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by changes in regulatory policy, there could be a material adverse effect on us and our business.

Preclinical and clinical product development involves a lengthy and expensive process, with an uncertain outcome.

Our current assumptions about our product candidates' development potential are based on the data generated from preclinical studies and clinical trials; however, we may observe materially and adversely different safety results as we continue to conduct our clinical trials. In order to obtain FDA approval to market a new drug product, we must demonstrate the safety and efficacy of the drug in humans in a manner that satisfies the agency's standards. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities, including the FDA, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety, potency and efficacy of our product candidates in humans. A failure of one or more clinical trials can occur at any stage of testing or at any time during the trial process. The outcome of preclinical testing and early clinical trials may not be predictive of the results of later clinical trials as to safety or efficacy, particularly if later clinical trials have a materially different trial design. For example, in our Phase 1 trial of SION-719 in healthy volunteers, SION-719 achieved exposure levels that, based on our preclinical cystic fibrosis human bronchial epithelial ("CFHBE") model, we believed had the potential to provide clinically meaningful benefit as an add-on to the standard of care. However, the PreciSION CF trial did not meet its key activity endpoint of sweat chloride reduction, resulting in our decision to discontinue development of SION-719 as an add-on to the standard of care. Our development of SION-451 + SION-2222 is also informed by our CFHBE model, and the results of the AscenSION CF trial may differ from those predicted by our preclinical and early clinical data.

The historical failure rate for product candidates in our industry is high, particularly in the earlier stages of development. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We have not completed all of the clinical trials required for the approval of any of our product candidates. We cannot assure you that any preclinical study or clinical trial that we are conducting, or may conduct in the future, will demonstrate consistent or adequate efficacy and safety to obtain regulatory approval to market our product candidates.

We may incur additional costs and experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

We may incur additional costs and experience delays in clinical trials for our product candidates, and we do not know whether future clinical trials, if any, will begin on time, need to be redesigned, enroll an adequate number of patients on time or be completed on schedule, if at all. We may experience numerous unforeseen events during or as a result of preclinical studies or clinical trials that could delay or prevent our ability to continue or complete clinical development, receive marketing approval or commercialize our product candidates, including:

- regulators or institutional review boards not authorizing us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- experiencing delays in reaching, or failing to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of our product candidates producing negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- failing to demonstrate statistical significance in clinical trials of our product candidates, which may impact the timing and design of late-stage clinical trials for such product candidates, or failing to demonstrate statistical significance in late-stage trials despite promising early stage results;
- the number of patients required for clinical trials of our product candidates being larger than we anticipate;
- enrollment in our clinical trials being slower or otherwise more challenging than we anticipate, for example, due to the availability of standard of care therapy or other therapies for CF, the reluctance of patients to discontinue standard of care therapy in order to participate in certain of our future clinical trials, including AscenSION CF, and the possibility of reduced interest from patients, physicians or clinical trial sites in our clinical trials following the results of our PreciSION CF trial;
- participants dropping out of our clinical trials, including due to requirements that may be burdensome to participants, such as frequent clinic visits, adverse events arising from transitioning from the standard of care to enroll in the trial or from dual combination treatments, or failing to return for post-treatment follow-up at a higher rate than we anticipate;
- our product candidates having undesirable side effects (including drug-drug interactions), unexpected toxicology findings, or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials;
- our third-party contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards requiring that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- future collaborators, if any, conducting clinical trials in ways they view as advantageous to them but that are suboptimal to us;
- the cost of clinical trials of our product candidates being greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary, including comparator drug, to conduct clinical trials of our product candidates being insufficient, inadequate or too costly; and
- budget reductions, hiring freezes, or similar actions at clinical trial sites and other institutions negatively impacting the ability of sites to conduct clinical trials or increasing the costs to us of conducting our trials.

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 favorable or if there are safety concerns, we may, among other things:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or

- have the product removed from the market after obtaining marketing approval.

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

We have not yet completed all testing of any product candidate in clinical trials. Preclinical, interim, topline and preliminary results from our preclinical studies or clinical trials are not necessarily predictive of the results or analyses of such results of later clinical trials. If we cannot replicate the positive results from any preclinical studies or clinical trials of our current or potential future product candidates that have positive results, or if we suffer any other significant setbacks in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our current or potential future product candidates.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical studies, Phase 1 and Phase 2a clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics, and to understand the side effects of product candidates at various doses and dosing schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later large-scale efficacy trials will be successful, nor does it predict final results. Our product candidates may fail to show the desired safety, tolerability, pharmacokinetic profile, and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials, particularly because our NBD1 stabilizer product candidates target the NBD1 domain of the cystic fibrosis transmembrane conductance regulator (“CFTR”) protein, a novel target which has not previously been tested in CF patients.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Such setbacks have occurred and may occur for many reasons, including: clinical sites and investigators may deviate from clinical trial protocols, whether due to lack of training or otherwise, and we may fail to detect any such deviations in a timely manner; patients may fail to adhere to any required clinical trial procedures, including post-treatment follow-up; our product candidates may fail to demonstrate effectiveness or safety in certain patient subpopulations or at all; or our clinical trials may not adequately represent the patient populations we intend to treat, whether due to limitations in our trial designs or otherwise. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development.

Similarly, from time to time, we may publish interim, topline or preliminary results from our preclinical studies and clinical trials, which are based on a preliminary analysis of then-available data. We make assumptions, estimations, calculations and conclusions as part of our preliminary analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. Interim results 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. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim, topline and preliminary data should be viewed with caution until the final data are available. Adverse differences between interim, topline or preliminary data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

For example, we have historically prioritized our pipeline by employing an *in vitro* electrophysiology assay to measure CFTR function in a CF model, known as the CFHBE model, which uses lung cells from CF patients and has been shown to be predictive of clinical outcomes for approved CFTR modulators. In preclinical studies using our CFHBE model, the combination of either SION-719 or SION-451 with one of our complementary modulators or the components of Trikafta showed significant improvement in *in vitro* CFTR activity and the potential for restoration of CFTR function to wild-type levels. However, our PreciSION CF Phase 2a trial evaluating SION-719 as an add-on to Trikafta in people with CF did not meet its key activity endpoint of sweat chloride reduction, and we have not yet replicated, and may never replicate, the results predicted by our CFHBE model in any clinical trials. While we have proposed hypotheses based on post hoc analyses to explain the PreciSION CF outcome, including potential confounding factors such as participant dosing non-adherence and biological interactions, these analyses are exploratory in nature and may not be validated in future trials. Our decision to advance SION-451 + SION-2222 into the AscenSION CF trial is informed in part by these post hoc findings, and if the underlying hypotheses prove incorrect, the AscenSION CF trial may also fail to demonstrate the anticipated clinical benefit. Further, our product candidates may fail to show the desired safety and efficacy at the dose ranges we have identified using our CFHBE model. Accordingly, positive results in our preclinical studies using our CFHBE model may not be predictive of the results in clinical trials and may fail to be replicated in clinical trials.

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 investors or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the interim, topline or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Targeting the NBD1 domain of the CFTR protein is novel, and the time, cost of development, and likelihood of successful product development is difficult to predict.

Our NBD1 stabilizer product candidates target the NBD1 domain of the CFTR protein, which is a novel target. Other companies have discontinued programs targeting NBD1 as they were unable to develop compounds that could successfully bind to NBD1. Given the novelty of our product candidates and the inherent risk in our plans to develop a combination therapy, we may not be able to successfully develop any products.

While products modulating the CFTR protein have been approved by the FDA and comparable foreign regulatory authorities, to date, no product that directly targets the NBD1 domain has been approved, and to our knowledge, no product candidate directly targeting the NBD1 domain is currently in development. As a result, we have limited evidence regarding the efficacy, safety and tolerability of our NBD1 stabilizers, and it is difficult to predict the developmental challenges we may encounter as our NBD1 stabilizers proceed through clinical trials, including SION-451 + SION-2222 in the AscenSION CF trial. It is also difficult for us to predict the time and cost of development of an NBD1 stabilizer-anchored combination therapy, whether any of our clinical trials will be successful, and whether our novel approach will result in the successful development and regulatory approval of any product candidates. Any development problems we experience in the future related to our NBD1 stabilizer product candidates or any of our complementary modulator candidates may cause significant delays or unanticipated costs, and such development problems may not be able to be solved. The novelty of our product candidates and our combination therapy approach may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. For example, the FDA could require additional studies that may be difficult or impossible to perform, or prohibitively costly. Any of these factors may prevent us from completing clinical trials that we may initiate, and obtaining regulatory approval of or commercializing any product candidates we may develop, on a timely or profitable basis, if at all.

Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious or unacceptable adverse side effects or unexpected toxicology findings may be

identified during the development of our product candidates, which could prevent or delay further clinical development, regulatory approvals and commercialization, impact the product's labeling, if approved, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.

Clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target indication. If our product candidates are associated with serious or significant adverse side effects in clinical trials or have adverse safety findings in nonclinical studies, we may need to abandon their development or limit development to narrower uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a benefit-risk perspective. The FDA or other comparable foreign regulatory authority or an institutional review board or ethics committee may also require that we suspend, discontinue or limit our clinical trials based on safety information, or that we conduct additional animal or human studies regarding the safety and efficacy of our product candidates, which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the indication, if approved. Many product candidates that initially showed promise in early-stage testing have later been found to cause adverse side effects that prevented further development of the product candidate. For example, although the dual combinations evaluated in our Phase 1 clinical trial of SION-451 were generally well tolerated in the dose ranges and regimens we plan to advance, we may observe new adverse events, or increases in the frequency or severity of previously observed adverse events, with longer durations of treatment, including during the planned 28-day dosing period in our AscenSION CF Phase 2 proof-of-concept trial evaluating SION-451 in combination with SION-2222. Safety and tolerability results from our completed clinical trials may not be predictive of those observed with longer durations of treatment.

Additionally, if one or more of our product candidates receives marketing approval, and we or others subsequently identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw, suspend or limit approvals of such product or seek an injunction against its manufacture or distribution;
- we may be required to recall a product;
- regulatory authorities may require additional warnings on the labels, such as a boxed warning or a contraindication;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product is distributed or administered, conduct additional clinical trials or change the labeling of a product or be required to conduct additional post-marketing studies or surveillance;
- we could be sued and held liable for harm caused to patients;
- sales of the product may decrease significantly or the product could become less competitive;
- we may not be able to achieve or maintain third-party payor coverage and adequate reimbursement; and
- our reputation and physician or patient acceptance of our products may suffer.

There can be no assurance that we will resolve any issues related to any product-related AEs to the satisfaction of the FDA or comparable foreign regulatory authorities in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of a particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

We may not be able to obtain orphan drug designation or exclusivity for our product candidates, and even if we do, we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We may seek orphan drug designation or exclusivity in the indications targeted by our current or future product candidates. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. For example, under the Orphan Drug

Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition. In order for the FDA to grant orphan drug designation to one of our product candidates, the agency must find that the product candidate is indicated for the treatment of a condition or disease that affects fewer than 200,000 individuals in the U.S. or that affects 200,000 or more individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making the product candidate available for the disease or condition will be recovered from sales of the product in the U.S. Orphan drug designation must be requested before submitting an NDA. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet the required standard, or that the data we have provided are not adequate to justify the scientific plausibility of our product candidate.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular drug for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same use or indication for seven years, except in limited circumstances such as if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated.

In addition, even after an orphan drug is approved, the FDA can subsequently approve a different product candidate for the same use or indication without any requirement to demonstrate potential for clinical superiority (efficacy, safety, or other major contribution to patient care) relative to a product that already has orphan designation or exclusivity. In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug or biologic and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

A similar regulatory scheme governs approval of orphan product candidates by the European Medicines Agency (“EMA”) in the European Union. The orphan product marketing exclusivity period in the European Union is ten years, which can be reduced to six years if at the end of the fifth year it is determined that a product no longer meets the criteria for orphan drug designation, including if the product is sufficiently profitable so that market exclusivity is no longer justified. The EMA may conclude that our product candidate(s) may not meet the criteria to justify orphan designation.

While we may in the future seek designations for our product candidates with the FDA and comparable foreign regulatory authorities that are intended to confer benefits such as a faster development process, an accelerated regulatory pathway or priority review, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

The FDA and comparable foreign regulatory authorities offer certain designations for product candidates that are designed to encourage the research and development of product candidates that are intended to address conditions with significant unmet medical need. These designations may confer benefits such as additional interaction with regulatory authorities, a potentially accelerated regulatory pathway and priority review of the marketing application(s). However, there can be no assurance that we will successfully obtain such designations for our product candidates. In addition, while such designations could expedite the development or approval process, they generally do not change the standards of product quality, safety or efficacy required to be demonstrated in support of approval. Even if we obtain such designations for our product candidates, there can be no assurance that we will realize their intended benefits.

For example, we may seek a Fast Track Designation for one or more of our product candidates. If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

We may seek Breakthrough Therapy Designation for any product candidate that we develop. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs 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 designated as breakthrough therapies by the FDA are also eligible for accelerated approval and priority review.

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. Further, the receipt of Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to conventional FDA procedures and does not assure approval by the FDA. In addition, even if any product candidate we develop qualifies for Breakthrough Therapy Designation, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

Even in the absence of obtaining Fast Track and/or Breakthrough Therapy Designations, a sponsor can seek priority review at the time of submitting a marketing application. The FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting adverse reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, or evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months from FDA's acceptance of the application for review. Priority review designation may be rescinded if a product no longer meets the qualifying criteria.

We may not be able to submit investigational new drug applications ("INDs") or IND amendments to commence clinical trials on the timelines we expect, and even if we are able to, the FDA may not permit us to proceed.

We may not be able to submit INDs for our product candidates on the timelines we expect. For example, we may experience manufacturing delays or other delays with IND-enabling studies. Moreover, we cannot be sure that submission of an IND will result in the FDA allowing related clinical trials to begin, or that, even if such trials do begin, issues will not arise that suspend or terminate such clinical trials. Additionally, even if such regulatory authorities agree with the design and implementation of the clinical trials set forth in an IND, we cannot guarantee that such regulatory authorities will not change their requirements in the future. These considerations also apply to new clinical trials we may submit as amendments to existing INDs.

Any product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them is approved.

As part of its decision to approve or grant marketing authorization for one or more of our product candidates, the FDA or other regulatory agencies may require us to perform certain post-marketing activities, such as completion of ongoing or planned studies, initiation of new studies or post-marketing clinical trials (including to assess safety risks), or additional analyses of existing data. Typically, we are required to provide annual updates on the progress of such required activities and to complete the activities by the assigned completion dates. Later discovery of previously unknown problems with our product candidates, including AEs of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of such products, withdrawal of the product from the market or voluntary or mandatory product recalls;

- restrictions on or revisions to the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine, including under a REMS program;
- fines, receipt of warning or untitled letters or suspension of clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or withdrawal of marketing approvals;
- product seizure or detention or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

Additionally, the FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. Although physicians may prescribe products for uses not described in the product's labeling, known as off-label uses, in their professional medical judgment, the FDA and comparable foreign regulatory agencies impose stringent restrictions on manufacturers' communications regarding off-label use, and if we market our products, if approved, in a manner inconsistent with their approved labeling, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice and other comparable foreign regulatory agencies. Violation of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws, state consumer protection laws and laws of other comparable foreign regulatory agencies.

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

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

Successful and timely completion of clinical trials will require that we identify and enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or comparable foreign regulatory authorities, or if a large number of patients withdraw. We cannot predict how successful we will be at enrolling subjects in our clinical trials. We anticipate conducting clinical trials that would require patients to discontinue standard of care therapy, including our upcoming AscenSION CF proof-of-concept trial, and we may experience challenges finding, enrolling and retaining CF patients who are willing to discontinue their current treatment regimens to participate in such trials. For example, AbbVie previously terminated part of a Phase 2 trial that was intended to evaluate multiple doses of SION-3067 in combination with a fixed dose of SION-2222 because this part was deemed not enrollable due to, among other reasons, the increasing availability of the standard of care for CF. Subject enrollment is affected by other factors including:

- the patient eligibility criteria as defined in the applicable protocol;
- the size of the patient population required for analysis of the trial's primary endpoints and the process for identifying patients;
- the actual and perceived risks and benefits of the product candidate in the trial;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- competing third-party clinical trials, including trials conducted by Vertex Pharmaceuticals Incorporated ("Vertex"), enrolling currently or in the future, and clinicians' and patients' perceptions as to the potential

advantages and risks of the third-party product candidate being studied in relation to other available therapies, including the standard of care, other therapies currently approved, or that may be approved in the future, for CF, or other investigational drugs in clinical development for CF;

- the willingness of patients to be enrolled in our clinical trials, including the possibility that patients, physicians or clinical trial sites will have reduced interest in our clinical trials following the results of our PreciSION CF trial;
- the success of efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- our ability to obtain and maintain informed consent;
- the risk that patients enrolled in our clinical trials will drop out of the trials prior to completion;
- the cost to, or lack of adequate compensation for, prospective patients; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

Furthermore, we expect to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance. Even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients. We cannot assure you that our assumptions used in determining expected clinical trial timelines are correct or that we will not experience delays or difficulties in enrollment, or be required by the FDA or comparable foreign regulatory authorities to increase our enrollment, which would result in the delay of completion of such trials beyond our expected timelines.

The results of clinical trials conducted at clinical trial sites outside the U.S. might not be accepted by the FDA, and data developed outside of a foreign jurisdiction similarly might not be accepted by such foreign regulatory authority.

We have conducted certain of our clinical trials in Australia, and we plan to conduct additional clinical trials outside of the U.S. in the future. Clinical trials conducted in Australia using “unapproved therapeutic goods,” or those that have not yet been evaluated by the Therapeutic Goods Association (“TGA”) for quality, safety and efficacy, must occur pursuant to either the Clinical Trial Notification Scheme or the Clinical Trial Approval Scheme. In each case, the trial is supervised by a Human Research Ethics Committee (“HREC”), an independent review committee set up under the guidelines of the Australian National Health and Medical Research Council that reviews, approves and provides continuing oversight of trial protocols and amendments, and of the methods and material to be used in obtaining and documenting informed consent of the trial subjects. Although the FDA or comparable foreign regulatory authorities may accept data from clinical trials conducted outside the relevant jurisdiction, acceptance of these data is subject to certain conditions. For example, the FDA requires that the clinical trial must be well-designed and conducted and performed by qualified investigators in accordance with ethical principles such as institutional review board or ethics committee approval and informed consent, the trial population must adequately represent the U.S. population and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. Further, the FDA may consider an on-site inspection to be necessary in which case they must be able to validate the data through such an inspection or other appropriate means. In addition, while these clinical trials are subject to the applicable local laws, acceptance of the data by the FDA will be dependent upon its determination that the trials were conducted consistent with all applicable U.S. laws and regulations. There can be no assurance that the FDA will accept data from trials conducted outside of the U.S. as adequate support of a marketing application. Additionally, recent policy proposals in the U.S., if enacted in the future, may make acceptance by the FDA or inclusion of foreign data in a U.S. marketing application more difficult or costly. Similarly, any data submitted to foreign regulatory authorities may not adhere to their standards and requirements for clinical trials and data from trials conducted outside of such jurisdiction may not be accepted.

If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted, which may increase costs or time required to complete the clinical trial.

Conducting clinical trials outside the U.S. also exposes us to additional risks, including risks associated with:

- additional foreign regulatory requirements, including with respect to data privacy and security, which may impose heightened data processing requirements and transfer restrictions;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- inconsistent standards for reporting and evaluating clinical data and AEs;
- varying standards or availability of CF care, resulting in data that may differ from patients who have received the U.S. standard of care therapy;
- any pandemic, epidemic or public health emergencies;
- diminished protection of intellectual property in some countries; and
- political instability, civil unrest, war or similar events that may jeopardize our ability to commence, conduct or complete a clinical trial and evaluate resulting data.

If any third-party manufacturer of our product candidates is unable to increase the scale of its production of our product candidates or increase the product yield of its manufacturing, then our manufacturing costs may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of our product candidates, our third-party manufacturers will be required to produce adequate quantities of our product candidates and meet required timelines. Further, as we seek to scale-up our manufacturing processes, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of our product candidates. The transition to larger scale production could prove difficult. In addition, if our third-party manufacturers are not able to optimize their manufacturing processes to increase the product yield for our product candidates, or if they are unable to produce increased amounts of our product candidates while maintaining the same quality, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operations.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

The manufacture of our product candidates is complex and requires significant expertise. As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, various aspects of the development program, such as manufacturing methods and formulation, may be altered in an effort to reduce costs or optimize processes and product characteristics, and such outcomes may not be achieved. Any of these changes could cause our product candidates to perform differently and affect the results of our current or future clinical trials. Such changes may also require additional testing, or notification to or approval by the FDA or another comparable regulatory authority. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue.

Due to the significant resources required for the development of our pipeline, and depending on our ability to access capital, we must prioritize the development of certain product candidates over others. We may expend our limited resources to pursue a particular product candidate or indication and fail to

capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we are focused on a single indication, CF, and we focus our research and development efforts on certain selected development programs and product candidates. We are currently primarily focused on the development of our proprietary dual combination program of SION-451 + SION-2222. As a result, we may forego or delay pursuit of opportunities with other product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the viability, commercial potential or target market for any of our current or future product candidates, our business, financial condition and results of operations could be materially and 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 a product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks Related to Our Dependence on Third Parties

We are dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates, if approved. If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business and we expect that we may need to enter into additional license agreements in the future. In particular, we have entered into license agreements with Sanofi and AbbVie, pursuant to which, among other things, we have secured exclusive licenses for certain intellectual property and know-how relating to CFTR modulator therapies to commercialize certain compounds, patents and proprietary information and inventions. Our existing license agreements impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license. Our business could suffer, for example, if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. In particular, we are substantially dependent on the success of our dual combination product candidates, SION-451 and SION-2222, and to the extent we are unable to develop other product candidates at the time of such termination, it would have a material adverse effect on our business, financial condition, results of operations and prospects, including, but not limited to, cessation of our operations. See the section titled “*Business—License and Collaboration Agreements*” in our Annual Report on Form 10-K for the year ended December 31, 2025 (the "Annual Report") for a description of our license agreements.

As we have done previously, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same intellectual property licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement intellectual property. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our product candidates and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed intellectual property in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

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

We currently rely on, and in the future intend to rely on, third parties to conduct a significant portion of our clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We currently engage CROs and other vendors to conduct our clinical trials, and similarly expect to engage CROs and other vendors for future clinical trials for our current and any future product candidates that we may progress to clinical development. We expect to continue to rely on third parties, including but not limited to clinical data management organizations, healthcare institutions operating as clinical sites and clinical investigators, to conduct those clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. Switching or adding CROs or other vendors involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO or vendor commences work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. We may encounter challenges or delays in our CRO/vendor relationships in the future which may cause a material adverse impact on our business, financial condition and prospects.

In addition, any third parties conducting our clinical trials will not be our employees, and, except for including contractual obligations and remedies for breach of such obligations in our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approvals for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly. In addition, 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 development activities that could harm our competitive position.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not directly control their businesses or related activities. Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCPs

for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register certain ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Further, upon inspection by a given regulatory authority, such regulatory authority may not agree with our determination that any of our clinical trials complies with GCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these requirements may require us to repeat clinical trials, which would delay the regulatory approval process.

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

We contract with third parties for the manufacture of our product candidates for clinical drug supply and expect to continue to do so for commercialization, if our product candidates are approved. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or obtain such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We do not have any cGMP manufacturing facilities and have no plans to develop our own clinical or commercial-scale manufacturing capabilities. We currently rely, and expect to continue to rely, on contract development and manufacturing organizations (“CDMOs”) for the cGMP manufacture of our product candidates and related raw materials for clinical development. In addition, we expect to rely on CDMOs for the commercial supply of any products for which we receive marketing approval. This reliance increases the risk that we will not have sufficient quantities of our product candidates or products, if approved, or obtain such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts. For example, any of these third parties may terminate their engagements with us at any time or may face production shortages or other supply interruptions or otherwise be unable to secure the requisite raw materials to support our planned clinical activities. In addition, the availability of CDMOs to manufacture our product candidates/drugs may depend in part on the CDMOs’ schedules for manufacturing other companies’ products or product candidates. If we need to modify our development plans or enter into alternative arrangements, which may not be readily available or available on acceptable terms, it could delay our product development activities and increase our expenses.

Our reliance on CDMOs for manufacturing activities reduces our control over these activities, but does not relieve us of our responsibility to ensure compliance with all required regulations. In particular, we do not have control over a supplier’s or manufacturer’s compliance with laws, regulations and applicable cGMP standards or similar regulatory requirements and other laws and regulations, such as those related to environmental health and safety matters. If our CDMOs cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or comparable foreign regulatory authorities, we may be unable to obtain regulatory approval of our potential future marketing applications. Although we conduct routine qualification audits and have quality agreements in place with our CDMOs, we have no direct operational control over their ability to maintain adequate quality control, quality assurance and qualified personnel. Our CDMOs may face manufacturing or quality control problems causing production and shipment delays, or CDMOs may fail to maintain compliance with the applicable cGMP requirements. The facilities used by our CDMOs are subject to continual review and periodic inspections by the FDA and comparable foreign regulatory authorities. If the FDA or a comparable foreign regulatory authority finds deficiencies with or does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, 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, if approved. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or drugs,

operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supply of our products.

Our use of foreign CROs and CDMOs in some jurisdictions may be or may become subject to U.S. legislation, sanctions, tariffs, trade restrictions and/or other regulatory requirements, which may increase the cost of and cause delays in the procurement or supply of materials for, or manufacture of, our product candidates or have an adverse effect on our ability to secure significant commitments from governments to purchase our potential therapies. For example, legislation signed into law in December 2025, known as the BIOSECURE Act, implements a prohibition on U.S. government contracts, grants, and loans from being used towards biotechnology equipment and services produced or provided by "biotechnology companies of concern." By December 18, 2026, the Director of the Office of Management and Budget ("OMB"), will publish a full list of biotechnology companies of concern based on recommendations from key federal Secretaries and Directors, including Defense, Justice, HHS, Commerce, National Intelligence, Homeland Security, State, and National Cyber. The Director of OMB will thereafter review and update that list at least annually, based on recommendations from those key federal Secretaries and Directors. We have an agreement with a Chinese biotechnology company, pursuant to which it may manufacture certain of our clinical trial materials from time to time. On June 8, 2026, the Department of Defense added this Chinese company to the Section 1260H list, which may result in the company being designated a biotechnology company of concern under the BIOSECURE Act once OMB publishes its list. The company challenged its designation in federal court, and in August 2026 the court preliminarily enjoined the Department of Defense from giving effect to the designation while the litigation is pending. The outcome of the litigation is uncertain. The BIOSECURE Act has the potential to severely restrict the ability of companies, including us, to work with certain Chinese biotechnology companies of concern without losing the ability to contract with, or otherwise receive funding from, the U.S. government. If the company with which we have an agreement is ultimately designated as a biotechnology company of concern, we may need to transition to alternative suppliers, which could be costly, time-consuming and disruptive to our clinical development programs. Additionally, our use of foreign CROs and CDMOs, including those located in China, may be subject to unanticipated changes in the geopolitical landscape that could have negative impacts on our business operations.

There have been, and may continue to be, significant changes to U.S. trade policies, sanctions, legislation, treaties, and tariffs, including, but not limited to, trade policies and tariffs affecting products from outside of the U.S. The extent and duration of increased tariffs and the resulting impact on general economic conditions and on our business are not known and depend on various factors, such as negotiations between the U.S. and affected countries, their respective responses, potential exemptions or exclusions, and availability and cost of alternative sources of supply. Supply chain disruptions and delays could also negatively impact our cost of materials and production processes. If we are unable to obtain necessary materials in sufficient quantity and in a timely manner, the development of our product candidates may be delayed, which could significantly harm our business.

Further, any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. If our current CDMOs cannot perform as agreed, we may be required to replace such manufacturers. We may incur added costs and delays in identifying and qualifying any such replacement. In addition, our current and anticipated dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We may seek to establish collaborations, license agreements and other similar arrangements with third parties for the development or commercialization of our product candidates. If we are not able to establish them on commercially reasonable terms, or if those arrangements are not successful, we may have to alter our development and commercialization plans.

The development and potential commercialization of our product candidates will require substantial additional funding. For some of our product candidates, we may seek to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the U.S. If we enter into any such additional arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our future collaborators dedicate to the development or

commercialization of our product candidates. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

We face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, the design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities outside the U.S., the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, and industry and market conditions generally. The collaborator may also consider alternative product candidates for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

Collaborations involving our product candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected, or at all;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated, including for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

We may not be able to negotiate future collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of any product candidate that we planned to collaborate on, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our

expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, any future collaborations that we enter into may not be successful. The success of our future collaboration arrangements will depend heavily on the efforts and activities of our future collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. If conflicts arise between any future collaborators and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. For example, our future collaborators could conduct multiple product development efforts and could develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop. In addition, collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party, and any such termination or expiration may adversely affect us financially or harm our business.

Risks Related to Our Intellectual Property

If we or our licensors are unable to obtain, maintain and enforce intellectual property rights relating to any of our product candidates, or if the scope of the protection obtained is not sufficiently broad, our competitors or other third parties could develop and commercialize products similar or identical to ours, our ability to successfully commercialize our product candidates may be adversely affected and we may not be able to compete effectively in our markets.

We rely upon a combination of patents, access to certain third-party trade secrets and confidentiality agreements to protect the intellectual property related to our product candidates. These legal measures afford only limited protection, and competitors or others may gain access to or use our intellectual property and proprietary information. Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the U.S. and in other countries with respect to our proprietary product candidates.

We cannot predict whether the patent applications we currently or may in the future pursue will issue as patents in any particular jurisdiction or will provide sufficient protection against competitors or other third parties. Currently, much of our patent portfolio, including the applications related to our NBD1 product candidates, are in various stages of the patent prosecution process. Nor can we predict the outcome of any challenge by our competitors to the validity or enforceability of any such patents. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a generic product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of the competitive advantage necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approval, the period of time during which we could market a product candidate under patent protection could be reduced (unless we obtain a patent term extension corresponding to such delays).

Our ability to obtain and maintain valid and enforceable patents depends on our inventions being patentable in light of the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to invent the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to make the inventions claimed in those owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patents and patent applications may not issue as patents and even if issued, may be challenged and invalidated or rendered unenforceable. Furthermore, even if a patent is granted, our competitors or other third parties may be able to circumvent the patent by developing similar or alternative products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Moreover, depending on the terms of any existing and future in-licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering intellectual property in-licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our and our licensors' pending and future patent applications may not result in patents

being issued which protect our product candidates or which effectively prevent others from commercializing competitive products.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the U.S. and abroad. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. We or our licensors may in the future become subject to a third-party pre-issuance submission of prior art, opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceeding and other similar proceedings challenging our patent rights or the patent rights of others in the U.S. Patent and Trademark Office (the "USPTO") or other foreign patent office. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products, or limit the duration of the patent protection of our product candidates.

In addition to the protection provided by our patent portfolio, we rely on access to certain third-party trade secret protection as well as confidentiality agreements to protect proprietary know-how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or intellectual property to enter into confidentiality agreements, certain employees may not have signed such agreements and we cannot provide any assurances that all such agreements have been duly executed, or that such trade secrets and other confidential proprietary information will not be disclosed. Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to the above-mentioned trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for intellectual property on which we do not have patent protection. If any trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that intellectual property or information to compete with us. If any trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We also seek to preserve the integrity and confidentiality of our proprietary data and third-party trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. However, our agreements and security measures may be breached, and we may not have adequate remedies for any breach. Also, if the steps taken to maintain trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover the trade secrets and our proprietary information. Further, the FDA may change its disclosure policies from time to time and make additional information publicly available on a routine basis. If we are unable to prevent material disclosure of the non-patented intellectual property related to our product candidates to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Patent terms may be inadequate to protect our competitive position on our products, if approved, for an adequate amount of time, and if we do not obtain protection under the Hatch-Waxman Amendments and similar non-U.S. legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, are limited. Even if additional patents covering our current or future product candidates are obtained, once the patent life has expired for a product, if approved, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are approved and commercialized. Because of these term limits, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours, if approved. If we do not have sufficient patent life to protect our product candidates, our business, financial condition, results of operations, and prospects may be adversely affected.

Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of any U.S. patents that may issue covering our product candidates may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product that is a new chemical entity as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request, and we cannot be certain what the length of the extension would be or if we will receive an extension at all. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from any applicable products could be reduced and could have a material adverse effect on our business.

If we fail to comply with our obligations in any current intellectual property licenses with third parties, or fail to obtain such licenses in the future, we could lose rights that are material to our business.

We have licensed third-party intellectual property that is material to our business, and may enter into additional license agreements in the future. We do not and will not own the patents or patent applications that underlie these licenses, and we may not control either the prosecution or the enforcement of the patents. Under such circumstances, we may be forced to rely upon our licensors to properly prosecute and file those patent applications and prevent infringement of those patents. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business.

If we or our licensors fail to maintain such patents, or if we or our licensors lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected.

Our rights to use the intellectual property and practice the inventions claimed in the licensed patents and patent applications are subject to our licensors abiding by the terms of those licenses and not terminating them. In addition, existing license agreements do, and future agreements may, impose diligence, development and commercialization timelines and milestone payment, royalty, insurance and other obligations, including rights of first negotiation (“ROFN”) for development of certain programs. For example, under our license agreement with AbbVie, AbbVie has a ROFN pursuant to which it would have an exclusive period to negotiate in good faith the terms of a commercial license transaction if we decide to pursue a license or sublicense to commercialize a licensed product under the AbbVie agreement prior to initiating Phase 3 clinical trials. This ROFN may delay or undermine our ability to enter into an otherwise beneficial transaction. If we fail to comply with our obligations, our licensors may have the right to terminate the licenses, in which event we might not be able to develop, manufacture or market any product that is covered by the intellectual property we in-license from such licensor and may face other penalties. If any of our licenses are terminated, we may lose our patent rights on a territory-by-territory basis, and such rights may be lost worldwide. Termination of any license agreement could reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property. Any of the foregoing outcomes could prevent us from commercializing relevant product candidates, which could have a material adverse effect on our operating results and overall financial condition.

In addition, disputes regarding obligations in licenses may require us to take expensive and time-consuming legal action to resolve, and, even if we are successful, may delay our ability to commercialize products and generate revenue. Further, if we are unable to resolve license issues that arise, we may lose rights to practice intellectual property that is required to make, use or sell products. We may require additional licenses in the future. Licenses to additional third-party intellectual property and materials that may be required

for our development programs may not be available on commercially reasonable terms, or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more-established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us.

In addition, disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including disputes concerning scope of rights granted under the license agreement and other interpretation-related issues; whether and the extent to which our product candidates and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we are unable to successfully obtain rights to required third-party intellectual property rights, we may have to abandon development of the relevant program or product candidate or expend time and resources re-designing the program or product candidate, which could have a material adverse effect on our business.

Intellectual property rights that we in-license in the future may also be granted through sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any future patents we obtain.

Our ability to obtain patents is highly uncertain because, to date, some legal principles remain unresolved, and there has not been a consistent policy regarding the breadth or interpretation of claims allowed in patents in the U.S. Furthermore, the specific content of patents and patent applications that are necessary to support and interpret patent claims is highly uncertain due to the complex nature of the relevant legal, scientific, and factual issues. Changes in either patent laws or interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

Patent reform legislation in the U.S. and other countries, including the Leahy-Smith America Invents Act (the “Leahy-Smith Act”) signed into law on September 16, 2011, and its implementation could increase the uncertainties around patent protection, costs, and the enforcement or defense of our patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. The Leahy-Smith Act included a number of significant changes to U.S. patent law. Such provisions affect the way patent applications are prosecuted, redefine prior art, and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. into a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business.

The Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations and financial condition. Further, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our or our licensors’ ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to

enforce our or our licensors' existing patents and patents that we or our licensors might obtain in the future. We cannot predict how future decisions by the courts, Congress or the USPTO may impact the value of our or our licensors' patents. Similarly, any adverse changes in the patent laws of other jurisdictions could have a material adverse effect on our business and financial condition. Changes in the laws and regulations governing patents in other jurisdictions could similarly have an adverse effect on our ability to obtain and effectively enforce our patent rights.

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

Competitors may infringe any intellectual property we may own or in-license, including our patents and trademarks. In addition, any intellectual property we may own or in-license may become the subject of a dispute, including those based on inventorship, priority, validity or unenforceability. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the U.S., counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent.

In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the intellectual property at issue on the grounds that our patent claims do not cover the intellectual property in question. Third parties may also raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of infringement, invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art. 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. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business.

Our defense of litigation or interference proceedings may fail and require us to cease using certain intellectual property or force us to take a license under the intellectual property rights of the prevailing party, if available. Even if successful, litigation or interference proceedings may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the U.S.

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

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain.

While no third parties to our knowledge have initiated legal proceedings against us to date, as our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties' patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including infringement, interference or derivation proceedings, post-grant review and *inter partes* review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize our product candidates or any future product candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such U.S. patent. Similarly, the burdens on us to invalidate patent claims in foreign jurisdiction may vary substantially and courts in those jurisdictions may not agree with us that the claims are invalid. The outcome of proceedings involving assertions of infringement, invalidity and unenforceability during patent litigation is unpredictable. Furthermore, if a patent holder believes that one of our product candidates infringes its patent, the patent holder may sue us even if we have received patent protection for our intellectual property. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit. Moreover, given the vast number of patents in our field of intellectual property, we cannot be certain that our current and future product candidates do not or will not infringe existing patents or that we will not infringe patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue commercializing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same intellectual property rights licensed to us. If we fail to obtain a required license, we may be unable to effectively market product candidates based on our intellectual property, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. Alternatively, we may need to redesign our products, which may be impossible or require substantial time and monetary expenditure. Under certain circumstances, we could be forced, including by court orders, to cease commercializing our product candidates. In addition, in any such proceeding or litigation, we could be found liable for substantial monetary damages, potentially including treble damages and attorneys' fees, if we are found to have willfully infringed the patent at issue. A finding of infringement that prevents us from commercializing our product candidates, requires us to redesign our products, or forces us to cease some of our business operations could materially harm our business.

The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

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

We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information, trade secrets or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

We may be subject to claims challenging the inventorship or ownership of our intellectual property, including any patents we obtain.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patent applications, any patents we obtain, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Although 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, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. 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, such as exclusive ownership of, or right to use, valuable intellectual property. If we no longer own intellectual property rights that are required to commercialize and protect our products, we may need to obtain a license to those rights, which may not be available on commercially reasonable terms, or at all. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Reliance on third parties requires us to share trade secrets, which increases the possibility that trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We rely on certain third-party trade secrets, technical know-how, proprietary information and other confidential information to protect our product candidates and as otherwise useful to our business. Monitoring unauthorized uses and disclosures of trade secrets and other confidential information is difficult, and we do not know whether the steps we have taken to protect our confidential intellectual property will be effective. We seek to protect our confidential information, in part, through confidentiality and non-disclosure agreements with our employees, consultants, collaborators, suppliers and other parties. These agreements typically restrict the ability of our employees, consultants, advisors and third-party contractors to use or disclose our proprietary information or publish data potentially relating to our proprietary information. Despite our efforts to protect trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other proprietary information by the parties to these agreements. There can be no assurance that these agreements will not be breached, including by disclosure of our confidential information. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and trade secret status could be lost as a result. We also 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 product candidates and processes. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators or others is inadvertently disclosed or subject to a breach or violation, we may be

liable to the owner of that confidential information. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary product candidates and processes will be effective.

To the extent that we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of our product candidates, we must, at times, share proprietary information with them. We may also conduct joint research and development programs that may require us to share potential trade secrets under the terms of our research and development partnerships or similar agreements. Despite the contractual provisions employed when working with third parties, the need to share confidential information increases the risk that such potential trade secrets become known by our competitors, are inadvertently incorporated into the product candidates of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and other confidential information, a competitor's discovery of such information or other unauthorized use or disclosure thereof could have an adverse effect on our business and results of operations.

Enforcing a claim that a third party illegally obtained and is using trade secrets or proprietary information is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets and the enforceability of confidentiality or similar types of agreements may vary from jurisdiction to jurisdiction.

We may enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our intellectual property in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as that in the U.S. or Europe. These products may compete with our product candidates, and our and our licensors' future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the U.S., but may issue as patents with claims of different scope or may even be refused in other jurisdictions. Furthermore, the requirements for patentability differ in certain jurisdictions and countries. Some countries do not grant claims directed to methods of treatment or have additional restrictions on the scope of method of treatment claims compared to the U.S. Accordingly, depending on the country, the scope of patent protection may vary for the same product candidate.

While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain protection efforts in all such markets. Additionally, the prosecution of patent applications in other jurisdictions is often a longer process and patents may be granted at a later date than in the U.S., potentially delaying our ability to assert such patents against competitors. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition in those jurisdictions.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the U.S. and Europe, and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property rights, which could make it difficult for us to stop the infringement of any patents we obtain or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put any patents we obtain at risk of being invalidated or

interpreted narrowly and our patent applications at risk of not issuing as patents, 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.

Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired.

In Europe, a new unitary patent system took effect on June 1, 2023, which may significantly impact European patents, including those granted before the introduction of the new system. Under the new system, applicants can, upon grant of a patent, opt for that patent to become a unitary patent which will be subject to the jurisdiction of a new unitary patent court ("UPC"). Patents granted before the implementation of the new system can be opted out of UPC jurisdiction, remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC may be challenged in a single UPC-based revocation proceeding that, if successful, could invalidate the patent in all countries who are signatories to the UPC. Further, because the UPC is a new court system and there is no precedent for the court's laws, there is increased uncertainty regarding the outcome of any patent litigation. We are unable to predict what impact the new patent regime may have on our ability to exclude competitors in the European market. In addition to changes in patent laws, geopolitical dynamics, such as Russia's incursion into Ukraine, may also impact our ability to obtain and enforce patents in particular jurisdictions. If we are unable to obtain and enforce patents as needed in particular markets, our ability to exclude competitors in those markets may be reduced.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the U.S. over the lifetime of our patents and/or applications and any patent rights we may obtain or license in the future. Furthermore, the USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents within prescribed time limits. If we or our licensors fail to maintain the patents and patent applications covering our product candidates or if we or our licensors otherwise allow our patents or patent applications to be abandoned or lapse, our competitors might be able to enter the market, which would hurt our competitive position and could impair our ability to successfully commercialize our product candidates in any indication for which they are approved.

Any trademarks we have obtained or may obtain may be infringed or otherwise violated, or successfully challenged. If our trademarks and trade names are not adequately protected, or if we are unable to obtain desired trademarks or trade names, then we may not be able to build brand name recognition in our markets of interest and our business may be adversely affected.

We expect to rely on trademarks as one means to distinguish our product candidates, if approved for marketing, from the drugs of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. During trademark registration proceedings in the U.S. and foreign jurisdictions, we may receive rejections. We are given an opportunity to respond to those rejections, but we may not be able to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties may oppose pending trademark registration applications or seek to cancel registered

trademarks. Any trademark applications we file may be rejected and registered trademarks may not be obtained, maintained or enforced. If we do not successfully register our trademarks, we may encounter difficulty in enforcing, or be unable to enforce, our trademark rights against third parties, which could adversely affect our business and our ability to effectively compete in the marketplace.

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

In addition, our unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on, misappropriating or violating other marks. In the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademark registrations may not survive such proceedings. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion.

Our competitors may also infringe or otherwise violate our trademarks and we may not have adequate resources to enforce our trademarks. We may not be able to protect our rights to our trademarks and trade names, which we need to build name recognition among potential collaborators or customers in our markets of interest. Any of the foregoing events may have a material adverse effect on our business.

Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, domain names or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

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

- others may be able to make products that are similar to or otherwise competitive with our product candidates but that are not covered by the claims of our current or future patents;
- an in-license necessary for the manufacture, use, sale, offer for sale or importation of one or more of our product candidates may be terminated by the licensor;
- we or future collaborators might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;
- we or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative product candidates or duplicate any of our product candidates without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;

- issued patents that we own or in-license may be held invalid or unenforceable as a result of legal challenges by our competitors;
- issued patents that we own or in-license may not provide coverage for all aspects of our product candidates in all countries;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership of our patents or patent applications may be challenged by third parties;
- we may not develop additional proprietary intellectual property that is patentable; and
- the patents of third parties may have an adverse effect on our business.

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

If we are unable to obtain licenses from third parties on commercially reasonable terms or fail to comply with our obligations under such agreements, our business could be harmed.

We currently have rights to intellectual property, through licenses from third parties, to identify and develop certain product candidates, and we may add additional licenses in the future as we expand our product candidate pipeline. Although we have succeeded in licensing intellectual property from third-party licensors, including AbbVie, in the past, we cannot assure our stockholders that we will be able to in-license or acquire the rights to any potential product candidates from third parties on acceptable terms or at all.

In addition, our license agreements may provide that our fields of use exclude particular fields. If we determine that rights to such fields are necessary to commercialize our product candidates or maintain our competitive advantage, we may need to obtain additional license rights in order to continue developing, manufacturing or marketing our product candidates. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties (potentially including our competitors) to receive licenses to a portion of the intellectual property that is subject to our existing licenses.

Various third parties practice in competitive areas and may have issued patents or patent applications that will issue as patents in the future, which could impede or preclude our ability to commercialize our product candidates. For any third-party patents that could be relevant to our product candidates, we rely in part on the “safe harbor” or research exemption under 35 U.S.C. § 271(e)(1), which exempts activities related to pursuing FDA approval for a drug product from patent infringement. However, while U.S. patent law provides such a “safe harbor” to our clinical product candidates under this provision, that exemption may expire when an NDA is submitted. Given the uncertainty of clinical trials, we cannot be certain of the timing of their completion and it is possible that we may submit an NDA for one of our future product candidates at a time when one or more relevant third-party patents is in force. It may therefore be necessary for us to use the patented or proprietary intellectual property of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. If we are unable to license such intellectual property, or if we are forced to license such intellectual property on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales or an obligation on our part to pay royalties and/or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same intellectual property licensed to us.

Additionally, we may collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution’s rights in intellectual property resulting from the collaboration. Even if we hold such an option, we may be unable to negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

In addition, the licensing or acquisition of third-party intellectual property rights is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the applicable product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates, or the methods for manufacturing them or to develop or license replacement intellectual property, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly.

Additionally, if we fail to comply with our obligations under license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market, or may be forced to cease developing, manufacturing or marketing, any product that is covered by these agreements or may face other penalties under such agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements, or current and future restrictions on our ability to freely assign or sublicense our rights under such agreements when it is in the interest of our business to do so, may result in our having to negotiate new or reinstated agreements with less favorable terms, cause us to lose our rights under these agreements, including our rights to important intellectual property or impede, or delay or prohibit the further development or commercialization of one or more potential product candidates that rely on such agreements.

Risks Related to Legal and Regulatory Compliance Matters

Our business operations and our relationships with healthcare providers, third-party payors, patients and other parties in the healthcare industry are subject, directly or indirectly, to significant regulation under a broad range of healthcare laws, including fraud and abuse laws. Any action against us for violation of such laws could harm our reputation and require significant resources for defense. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Pharmaceutical manufacturers, and the parties with which such manufacturers interact, are subject to extensive and complex regulation under a broad range of healthcare laws. Such laws, some of which will apply only if and when we have a marketed product, constrain our business operations, including the research and development, manufacturing, distribution, sales and promotion of our product candidates and products, if any are approved in the future, as well as educational and charitable activities. Arrangements with healthcare providers, third-party payors, patients and other parties in the healthcare industry, which may have a significant impact on our business, are regulated by fraud and abuse and other healthcare laws. For more information, see the section titled "*Business—Government Regulation—Other U.S. Healthcare Laws and Compliance Requirements*" in the Annual Report.

Healthcare laws regulating our business activities are broad and any exceptions may be narrow. Requirements may differ across jurisdictions. There may be limited guidance on the interpretation of the laws and their application to our specific activities. Interpretations of these laws by government enforcement agencies and courts are evolving. Efforts to ensure that our business operations will comply with applicable healthcare laws and regulations will involve substantial costs. We will need to develop and implement robust compliance policies and processes to seek to prevent and detect non-compliance and update such policies and processes as our operations and government expectations evolve, which will involve substantial and ongoing costs, and even with such policies and processes we cannot be certain to prevent non-compliance.

Given the broad scope, limited guidance and evolving government interpretations, our business activities may potentially be subject to challenge under these healthcare laws despite efforts to ensure compliance. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Such an action could also harm our reputation and adversely affect our business as a result. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

Even if we obtain regulatory approvals for our product candidates or any future product candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approvals for our product candidates or any future product candidates, such product candidates, once approved, will be subject to ongoing regulatory requirements applicable to manufacturing, labeling, packaging, storage, advertising, promoting, sampling, record-keeping and post-marketing activities, among other things. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP and GCP requirements for any clinical trials that we conduct post-approval. Manufacturers of approved products and their facilities are subject to continual review and periodic and unannounced inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. Any regulatory approvals that we receive for our product candidates or any future product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post-marketing testing, including additional trials and heightened surveillance to monitor the quality, safety and efficacy of the drug. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to promptly report any serious and unexpected adverse drug experiences and certain quality or production problems with our products to regulatory authorities along with other periodic reports.

Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to ensure compliance. We will also have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drug products are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we will not be allowed to promote our products for indications or uses for which they do not have approval, commonly known as off-label promotion. Physicians, on the other hand, may prescribe products for off-label uses. Although the FDA and other regulatory agencies do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies, including their sales force, with respect to off-label uses of products for which marketing approval has not been issued. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. The holder of an approved NDA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or manufacturing process. A company that is found to have improperly promoted off-label uses of their products may be subject to significant civil, criminal and administrative penalties.

In addition, drug manufacturers are subject to payment of annual fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with any product, if approved, such as adverse experiences of unanticipated severity or frequency, or problems with the facility where the product is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requesting revisions to the approved labeling to add new safety information, imposing of post-market studies or clinical trials to assess new safety risks or imposing distribution restrictions or other restrictions under a REMS program, requesting a recall or requiring withdrawal of the product from the market or suspension of manufacturing. If we fail to comply with

applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines, disgorgement of profits or revenue, warning letters or adverse publicity requirements;
- suspend or withdraw regulatory approvals;
- restrict product distribution or use, including full or partial holds on any ongoing or planned clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

Healthcare and other reform initiatives may have an adverse impact on our business and results of operations.

In the U.S. and some foreign jurisdictions, there have been and continue to be ongoing efforts to implement legislative and regulatory changes regarding the healthcare system. Such changes could prevent or delay marketing approval of any product candidates that we may develop; restrict or regulate post-approval activities; and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Although we cannot predict what healthcare reform efforts will be successful, such efforts may result in more rigorous coverage criteria, additional downward pressure on the price that we, or our future collaborators, may receive for any approved products, or in other consequences that may adversely affect our ability to achieve or maintain profitability. For more information, see the section titled "*Business—Government Regulation—Healthcare Reform*" in the Annual Report.

Among policy makers and third-party payors in the U.S. and elsewhere, there is significant and ongoing interest in implementing changes in the delivery and payment for healthcare services in order to contain healthcare costs, improve quality and/or expand access. In the U.S., the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives and changing policies and practices of the third-party payors. There has also been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices considering the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several congressional inquiries and proposed and enacted legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient support programs, or reform government program reimbursement methodologies for products. Individual states in the U.S. have also become increasingly active in implementing regulations designed to control pharmaceutical 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.

The U.S. has recently taken steps intended to reduce drug prices and establish and communicate most-favored-nation price targets to pharmaceutical manufacturers to bring prices for American patients in line with comparably developed nations, including recently proposed and finalized most-favored-nation Medicare reimbursement models issued by the Centers for Medicare and Medicaid Services ("CMS") through its Center for Medicare and Medicaid Innovation. In addition, several large pharmaceutical companies have already started to voluntarily reduce their pricing on select drugs indexed to lower prices around the globe. At the state level, legislatures have increasingly passed legislation and implemented regulations with similar goals, as well

as laws designed to control pharmaceutical and biotherapeutic product pricing, including restrictions on pricing or reimbursement at the state government level, limitations on discounts to patients, marketing cost disclosure and transparency measures, restrictions or other limitations on patient assistance, and, in some cases, policies to encourage importation from other countries (subject to federal approval) and bulk purchasing.

More specifically, in September 2026, CMS finalized the Global Benchmark for Efficient Drug Pricing ("GLOBE") Model, a mandatory Medicare Part B model that, beginning in 2027, would require manufacturers of certain included drugs and biologics to pay rebates to the extent Medicare Part B payment exceeds an international benchmark, and the proposed Guarding U.S. Medicare Against Rising Drug Costs ("GUARD") Model, a mandatory Medicare Part D model applying a similar international benchmark-based rebate approach that has not yet been finalized. The GLOBE final rule is expected to be challenged in litigation, and we cannot predict the outcome of any such challenge, whether or when GUARD will be finalized or in what form, or whether CMS will adopt similar models in the future. Although we do not currently market any products, these models, if they apply to us, may affect our development and investment decisions.

Adoption of new legislation at the federal or state level could affect demand for, or pricing of, any future products if approved for sale. We cannot, however, predict the ultimate content, timing or effect of any federal and state reform efforts. There is no assurance that federal or state healthcare reform will not adversely affect our future business and financial results. Additionally, implementation by third-party payors of policies and practices to limit coverage, manage utilization and reduce payment for drug products could adversely affect our ability to sell our products profitably. All these efforts may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

In addition, FDA regulations and guidance may be revised or reinterpreted by the FDA in ways that may significantly affect our business and our products. If executive actions impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. Any new regulations or guidance, or revisions or reinterpretations of existing regulations or guidance, may impose additional costs or lengthen FDA review times for our product candidates. We cannot determine how changes in regulations, statutes, policies or interpretations when and if issued, enacted or adopted, may affect our business in the future. Such changes could, among other things, require:

- significant changes to the design of planned clinical trials that impact their duration or cost;
- additional clinical trials to be conducted prior to obtaining approval;
- changes to manufacturing methods;
- recalls, replacements, or discontinuance of one or more of our products, if approved; and
- additional recordkeeping.

Such changes would likely require substantial time and impose significant costs, or could reduce the potential commercial value of our product candidates, and could materially harm our business and our financial results. In addition, delays in receipt of or failure to receive regulatory approvals for any of our product candidates would harm our business, financial condition, and results of operations. Further, we cannot predict the likelihood, nature, or extent of healthcare reform initiatives that may arise from future legislation or administrative action.

Risks Related to the Commercialization of Our Product Candidates

We face substantial competition. Our main competitor in the CF market holds substantial market share and has substantially greater resources than we do. We may not be able to compete successfully in this environment and, in particular, against a much larger competitor.

The biotechnology and pharmaceutical industries are characterized by rapid advances, intense competition and a strong emphasis on proprietary and novel products and product candidates. We face substantial competition with respect to our current product candidates and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from many different sources, including major pharmaceutical and biotechnology companies, academic institutions, governmental agencies, consortiums and public and private research institutions.

In particular, we expect to compete with Vertex, which has multiple approved products, as well as additional product candidates in development, for the treatment of CF that would compete with our product candidates, if approved. Vertex holds substantial market share in our product candidates' proposed markets, and has substantially greater name recognition, financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Our commercial opportunity could be reduced or eliminated if Vertex or another competitor develops and commercializes products that are safer or more effective, have fewer or less severe side effects, or are more convenient or are less expensive than any product candidate that we may develop. Vertex or another competitor may also obtain approval by FDA or comparable foreign regulatory authorities for its product candidates currently in development more rapidly than we may obtain approval for our product candidates. Competing products could present superior treatment alternatives and could render our product candidates obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Even if one or more of our product candidates achieves marketing approval, it may be priced at a significant premium over competitive products, or otherwise not achieve broad market access, resulting in reduced competitiveness. If we do not compete successfully, we may not generate or derive sufficient revenue from any product candidate for which we obtain marketing approval and may not become or remain profitable.

Smaller or early-stage companies could also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. This may include other small-molecule drug discovery companies using similar approaches or other types of therapies, such as gene therapy, gene editing and/or mRNA therapies. If successful, gene therapy, gene editing, and/or mRNA therapies could repair or replace the defective CFTR gene or produce functional CFTR protein and reduce or eliminate the need for CFTR modulators. 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 intellectual property complementary to, or that may be necessary for, our programs. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Even if any of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. There is currently a well-established standard of care for CF, Trikafta, with which physicians, CF patients and payors are very familiar and for which an established benefit-risk profile exists. In addition, in December 2024, the FDA approved Alyftrek, which targets the same mechanisms as Trikafta. Even if our product candidates are successful in registrational clinical trials, they may not be successful in displacing the standard of care if we are unable to demonstrate competitive efficacy, safety, ease of administration and/or cost-effectiveness. For example, physicians may be reluctant or unwilling to take their patients off their current medication and switch their treatment regimen to our product candidates, if approved, if the current medication is effective. Further, patients often acclimate to the treatment regimen that they are currently taking and may not want to switch unless recommended to do so by their physician for clinical reasons or required to do so due to lack of coverage and adequate reimbursement. Even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators and receive approval, concerns in the medical community related to the comparative efficacy or side effect profile of our products may hinder market acceptance and uptake.

Efforts to educate the medical community and third-party payors regarding the benefits of our product candidates, if approved, may require significant resources, including management time and financial resources, and may not be successful. If our product candidates do not achieve an adequate level of market acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy, safety and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects;

- our ability to offer our products at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- product labeling or product insert requirements of the FDA or comparable foreign regulatory authorities, including any limitations or warnings contained in a product's approved labeling, including any boxed warning;
- the product's acceptance into standard of care treatment algorithms by medical societies that could affect payor and physician uptake;
- the effectiveness of sales and marketing efforts, and the strength of sales, marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement for any product candidates, once approved;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- any restrictions on the use of our products together with other medications; and
- potential product liability claims and unfavorable publicity related to our products.

Any failure by one or more of our product candidates that obtains regulatory approvals to achieve market acceptance or commercial success would adversely affect our business prospects.

The success of our product candidates or any future product candidate will depend significantly on coverage and adequate reimbursement by third-party payors or the willingness of patients to pay for these products if not covered.

We believe that for any product candidates which may be approved, our success depends on obtaining and maintaining coverage and adequate reimbursement for such products for their respective approved indications, and the extent to which patients will be willing to pay out-of-pocket for such products in the absence of reimbursement for all or part of the cost. Accordingly, we will need to establish a coverage and reimbursement strategy for any approved product candidate. In the U.S. and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Government and private third-party payors decide which products they will cover and establish reimbursement levels. Coverage and reimbursement varies among third-party payors and new products face significant challenges in obtaining and maintaining coverage and adequate reimbursement, particularly if approved for indications with established treatments already on the market. For more information, see the section titled "*Business— Government Regulation—Coverage and Reimbursement*" in the Annual Report.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage for certain products, managing utilization of covered products and restricting the amount of reimbursement for covered products. Within the U.S., net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or requested by private payors in exchange for favorable coverage. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

There can be no assurance that our product candidates, even if they are approved for sale in the U.S. or in other countries, will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payors, or that coverage and an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably.

The market for our product candidates may be smaller than we estimate.

Our estimates of the potential market opportunity for our product candidates include several key assumptions, based on our industry knowledge, industry publications and third-party research reports. These assumptions include the number of patients who have CF, as well as the estimated reimbursement levels for each product candidate, if approved. While we believe our assumptions and the data underlying our estimates are reasonable, we have not independently verified the accuracy of the third-party data on which we have based our assumptions and estimates, and these assumptions and estimates may not be correct and the conditions supporting our assumptions or estimates may change at any time, including as a result of factors outside our control, thereby reducing the predictive accuracy of these underlying factors. Further, new studies may change the estimated incidence or prevalence of these diseases, and the potentially addressable patient population for our product candidates may not ultimately be amenable to treatment with our product candidates. If the actual market for any product candidates we may develop is smaller than we estimate, our revenues, if any, may be limited and it may be more difficult for us to achieve or maintain profitability.

Clinical trial and product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop, especially if our products are prescribed for off-label uses (even if we do not promote such uses). For example, we may be sued if our product candidates allegedly cause injury or are 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 candidate, negligence, strict liability and a breach of warranties. Claims may be brought against us by clinical trial participants, patients or others using, administering or selling products that may be approved in the future. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- termination of clinical trials;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- reduced resources of our management to pursue our business strategy;
- the inability to commercialize any products that we may develop; and
- a decline in our stock price.

Although we maintain clinical trial liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain marketing approval on any current or potential product candidates, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Business Operations, Employee Matters and Managing Our Growth

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

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. Following the Restructuring, our organization is significantly smaller and several of our executive officers have assumed expanded responsibilities. As a result, we are more dependent on a smaller number of key personnel, and the departure of any such individual could be more disruptive to our operations than it would have been prior to the Restructuring. Each of our executive officers may currently terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or employees. This lack of insurance means that we may not have adequate compensation for the loss of the services of these individuals.

The loss of the services of our executive officers or other key employees could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approvals of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Further, job candidates and existing employees often consider the value of the stock awards they receive in connection with their employment. If the perceived benefits of our stock awards decline, either because we are a public company or for other reasons, it may harm our ability to recruit and retain highly skilled employees. Our employees may be more likely to leave us if the shares they own have significantly appreciated in value relative to the original purchase prices of the shares, or if the exercise prices of the options that they hold are significantly below the market price of our common stock. Our stock price has declined significantly from its historical highs, which has diminished the retentive value of previously granted equity awards. In September 2026, we repriced certain outstanding stock options to address this concern, but there can be no assurance that the repricing will be sufficient to retain key employees or that further declines in our stock price will not necessitate additional retention measures.

The Restructuring undertaken to focus our resources and extend our cash runway may not achieve its intended outcomes.

In September 2026, we implemented the Restructuring, designed to focus our resources on advancing our dual combination program of SION-451 + SION-2222 and extend our operating runway. This restructuring plan included a reduction of our workforce by approximately 46% of our headcount. This reduction in force may result in unintended consequences and costs, such as the loss of institutional knowledge and expertise, attrition beyond the intended number of employees, decreased morale among our remaining employees, and the risk that we may not achieve the anticipated benefits of the reduction in force. In addition, while positions have been eliminated, we have retained employees focused on advancing the dual combination program, and we may be unsuccessful in distributing the duties and obligations of departed employees among our remaining employees.

The reduction in workforce could also make it difficult for us to pursue, or prevent us from pursuing, new opportunities and initiatives due to insufficient personnel, or require us to incur additional and unanticipated costs to hire new personnel to pursue such opportunities or initiatives. If our AscenSION CF trial or other development activities produce positive results, we may need to rapidly expand our workforce, and the Restructuring, the significant decline in our stock price and the competitive hiring environment in the biotechnology industry may make it more difficult or costly to attract qualified personnel on the timeline required.

We may further discover that, despite the implementation of the Restructuring, we may require additional capital to continue operating our business, and we may be unable to obtain such capital on acceptable terms, if at all. If we are unable to realize the anticipated benefits from the reduction in force, or if we experience significant adverse consequences from the reduction in force, our business, financial condition, and results of operations may be materially adversely affected.

If we advance our product candidates into later stages of development, we expect to expand our clinical development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

If we advance our product candidates into later stages of development, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our potential future growth, we must continue to implement and improve our managerial, operational and financial systems. Due to our limited financial resources and the limited experience of our management team in managing a company with such potential growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel as needed. The future expansion of our operations may lead to significant costs and may divert our management, business, and development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. Our future financial performance and our ability to compete effectively will depend, in part, on our ability to manage our growth effectively.

Our business could be affected by litigation, government investigations and enforcement actions.

We currently operate and plan to operate in a highly regulated industry and we could now or in the future be subject to litigation, government investigation and enforcement actions on a variety of matters in the U.S. or foreign jurisdictions, including, without limitation, intellectual property, regulatory, product liability, environmental, whistleblower, false claims, privacy, anti-kickback, anti-bribery, securities, commercial, employment and other claims and legal proceedings which may arise from conducting our business. Any determination that our operations or activities are not in compliance with existing laws or regulations could result in the imposition of fines, civil and criminal penalties, equitable remedies, including disgorgement, injunctive relief and/or other sanctions against us, and remediation of any such findings could have an adverse effect on our business operations.

Legal proceedings, government investigations and enforcement actions can be expensive and time-consuming. An adverse outcome resulting from any such proceedings, investigations or enforcement actions could result in significant damages awards, fines, penalties, exclusion from the federal healthcare programs, healthcare debarment, injunctive relief, product recalls, reputational damage and modifications of our business practices, which could have a material adverse effect on our business and results of operations. Even if such a proceeding, investigation or enforcement action is ultimately decided in our favor, the investigation and defense thereof could require substantial financial and management resources and cause reputational harm. For example, following the announcement of our PreciSION CF topline results in August 2026, the trading price of our common stock declined significantly. Securities litigation is often brought following a significant decline in the market price of a company's securities, and clinical-stage biopharmaceutical companies have been particularly susceptible to such litigation. Any such litigation, even if without merit, could result in substantial costs, divert management's attention and resources from our business, damage our reputation and adversely affect our ability to raise capital and pursue our business strategy.

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

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the

payment of user fees, and statutory, regulatory, and policy changes. In addition, government funding of the Securities and Exchange Commission (the "SEC") and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies, including substantial leadership, personnel and policy changes, may also slow the time necessary for new product candidates 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, including a 43-day period that began on October 1, 2025, due to budgetary issues and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, or if geopolitical or global health concerns 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 to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, government shutdowns or other disruptions at the FDA, SEC or other government agencies could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, suppliers and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates FDA or other comparable regulatory authority regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA or other comparable regulatory authority, manufacturing standards, foreign, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data.

In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud and abuse, such as the payment of kickbacks in return for business. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use or misrepresentation of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted both a code of business conduct and ethics and an insider trading policy, but it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. 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, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations. Any of these could adversely affect our ability to operate our business and our results of operations.

If our third-party manufacturers or suppliers do not comply with laws regulating the protection of the environment and health and human safety, our business could be affected adversely.

We and any CDMOs and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and

water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from the use of hazardous materials by us or by one of our third-party manufacturers, we could be held liable for any resulting damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended. We and our third-party manufacturers and suppliers cannot eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain general liability insurance as well as 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.

In addition, we and our third-party manufacturers and suppliers may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which may increase the cost of their services to us. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities for us or our third-party manufacturers and suppliers, or adversely impact our supply chain or reputation, which could in turn materially adversely affect our business, financial condition, results of operations and prospects.

Our insurance policies are expensive and only protect us from some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include property, general liability, employment directors' and officers' and employment practices insurance, however, we may not be able to maintain adequate levels of insurance coverage in the future. Further, an insurance carrier may seek to cancel or deny coverage after a claim has occurred.

We may engage in strategic transactions that could increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, subject us to other risks, adversely affect our liquidity, increase our expenses, present significant distractions to our management and harm our financial condition and results of operations.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases and strategic partnerships or out-licensing or in-licensing of intellectual property, product candidates or products. For example, we have entered into a license agreement with AbbVie that gives us exclusive worldwide rights to develop and commercialize three clinical-stage compounds. Additional potential transactions that we may consider in the future include a variety of business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. We may not be able to find suitable partners or acquisition candidates, and we may not be able to complete such transactions on favorable terms, if at all. Any future transactions could increase our near and long-term expenditures, result in potentially dilutive issuances of our equity securities, including our common stock, or the incurrence of debt, contingent liabilities, amortization expenses or acquired in-process research and development expenses, any of which could affect our financial condition, liquidity and results of operations. Future acquisitions may also require us to obtain additional financing, which may not be available on favorable terms or at all. These transactions may never be successful and may require significant time and attention of our management. In addition, the integration of any licensed assets that we may acquire rights to in the future may disrupt our existing business, may cause delays related to the integration of any licensed or acquired assets, and may be a complex, risky and costly endeavor for which we may never realize the full benefits. Furthermore, we may experience losses related to our entry into any licensing or partnerships, including as a result of failure to realize expected benefits or the materialization of unexpected liabilities or risks, which could have a material negative effect on our results of operations and financial condition. Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above, any additional transactions that we do complete could have a material adverse effect on our business, results of operations, financial condition and prospects.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations and the operations of our suppliers, CROs, CDMOs and clinical sites could be subject to earthquakes, power shortages, telecommunications or infrastructure failures, cybersecurity incidents, physical security breaches, water shortages, floods, hurricanes, typhoons, blizzards and other extreme weather conditions, fires, public health pandemics or epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. We rely or expect to rely on third-party manufacturers or suppliers to produce our product candidates and their components and on CROs and clinical sites to conduct our clinical trials, and do not currently have a redundant source of supply for all components of our product candidates. Our ability to obtain clinical or, if approved, commercial, supplies of our product candidates or any future product candidates could be disrupted if the operations of these suppliers were affected by a man-made or natural disaster or other business interruption, and our ability to commence, conduct or complete our clinical trials in a timely manner could be similarly adversely affected by any of the foregoing. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

Risks Related to Ownership of Our Common Stock

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

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

- the timing and success or failure of clinical trials for our product candidates or competing product candidates or any other change in the competitive landscape of our industry;
- our ability to successfully recruit and retain subjects for clinical trials and any delays caused by difficulties in such efforts;
- the timing and cost of, and level of investment in, research, development, regulatory approvals and commercialization activities relating to our product candidates, which may change from time to time;
- the cost of manufacturing our product candidates, 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;
- the level of demand and the indication for any approved products, which may vary significantly;
- the risk/benefit profile, cost, coverage, and reimbursement policies with respect to our product candidates, if approved, and existing and potential future drugs that compete with our product candidates;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- the timing and amount of any milestone, royalty or other payments payable by us or due to us under any collaboration, licensing or other similar agreement; and
- general market and economic conditions, including market conditions in the pharmaceutical and biotechnology sectors.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results 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 operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the 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 if we have met any previously publicly stated revenue or earnings guidance.

The trading price of the shares of our common stock may be volatile, and investors could lose all or part of their investment.

The market price for our stock has been highly volatile and may continue to be subject to wide fluctuations in response to various factors, some of which we cannot control. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of particular companies, including in connection with conflicts in various regions of the world, increasing inflation rates, interest rate changes, and changes in U.S. federal policy. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- the commencement, enrollment or results of our current or future clinical trials of our product candidates;
- adverse results from, delays in, suspension or termination of current or future clinical trials or preclinical studies of our product candidates, or any delay in advancing a clinical candidate;
- the success of competitive products gaining approvals or announcements by current and future competitors of their product development efforts;
- any delay in our regulatory filings for our product candidates or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings;
- adverse regulatory decisions, including the failure to authorize or approve the conduct of one or more clinical trials of our product candidates, the failure to receive regulatory approvals of our product candidates, or the failure of a regulatory authority to accept data from clinical trials conducted in other countries;
- the reporting of unfavorable preclinical or clinical results;
- our success or failure to identify, develop, acquire or license additional product candidates;
- the degree and rate of physician and market adoption of any of our current and future product candidates, if successfully developed and approved;
- manufacturing, supply or distribution delays or shortages, including our inability to obtain adequate supply of drug product, drug substance, raw materials or any commercially available product to be used in our clinical trials, at acceptable prices, or at all;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- unanticipated serious safety concerns related to the use of our product candidates or any other product candidate;
- our cash position, and any changes in financial estimates by us or by any equity research analysts who cover our stock;
- changes in our capital structure, such as future issuances of securities and the incurrence of additional debt;
- conditions or trends in our industry;
- investors' general perception of our company and our business;
- our ability to effectively manage our growth;
- overall performance of the equity markets;
- changes in the market valuations and stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;

- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, capital commitments or divestitures;
- third-party publications and discussions about our business on social media, forums and other websites;
- recruitment or departure of key personnel;
- trading volume of our common stock;
- sales of common stock by us or our stockholders in the future;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates;
- significant lawsuits, including patent or stockholder litigation;
- changes in the structure of healthcare payment systems;
- changes in accounting standards, policies, guidelines, interpretations or principles;
- regulatory or legal developments in the U.S. and foreign countries;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, geopolitical and stock market conditions; and
- other events or factors, many of which are beyond our control.

These and other market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the risks described in this section, or any of a broad range of other risks, could have a material adverse impact on the market price of our common stock. The price of our common stock may be disproportionately affected as investors may favor traditional profit-making industries and companies during times of market uncertainty and instability.

In the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. We face a heightened risk of such litigation following the significant decline in our stock price after the announcement of our PreciSION CF results in August 2026. Such litigation, if instituted, could result in substantial costs and divert management's attention and resources.

In addition, the trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of these analysts ceases coverage of us, fails to publish reports on us regularly or publish inaccurate or unfavorable research about our business, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

We may not be able to satisfy listing requirements of Nasdaq or obtain or maintain a listing of our common stock on Nasdaq.

Since our common stock is listed on Nasdaq, we must meet certain financial and liquidity criteria to maintain such listing. If we violate Nasdaq's listing requirements, our common stock may be delisted. If we fail to meet any of Nasdaq's listing standards, our common stock may be delisted. In addition, our board of directors may determine that the cost of maintaining our listing on a national securities exchange outweighs the benefits of such listing. A delisting of our common stock from Nasdaq may materially impair our stockholders' ability to buy and sell our common stock and could have an adverse effect on the market price of, and the efficiency of the trading market for, our common stock. The delisting of our common stock could significantly impair our ability to raise capital and the value of our stockholders' investment.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws may significantly reduce the value of our shares to a potential acquiror or make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change of control was considered favorable by our stockholders. For example, our board of directors has the authority to issue up to 10,000,000 shares of preferred stock and may fix the price, rights, preferences, privileges, and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change of control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors will be elected each year;
- stockholders are not entitled to remove directors other than by a two-thirds (2/3) vote and only for cause;
- stockholders are not permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change of control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in our stockholders' best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our executive officers, directors, principal stockholders, and their respective affiliates own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

Our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates beneficially own a significant percentage of our outstanding common stock. As a result, these persons, acting together, would be able to control all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than other stockholders. For example, because many of these stockholders purchased their shares at prices substantially below the current market price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders. The significant concentration of ownership may adversely affect the trading price of our common stock due to investors' perceptions that it may create conflicts of interest.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future.

We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future. Investors seeking cash dividends should not purchase our common stock. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

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

Our bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of, or a claim based on, fiduciary duty owed by any of our current or former directors, officers, and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws (including the interpretation, validity or enforceability thereof), or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery of the State of Delaware having personal jurisdiction over the indispensable parties named as defendants therein (the "Delaware Forum Provision"). The Delaware Forum Provision will not apply to any causes of action arising under the Securities Act of 1933, as amended (the "Securities Act") or the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our bylaws further provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the U.S. shall be the sole and exclusive forum for resolving any complaint asserting a cause or causes of action arising under the Securities Act (the "Federal Forum Provision"). In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision in our bylaws may impose additional litigation costs on stockholders in pursuing any such claims. Additionally, the forum selection clauses in our bylaws may limit our stockholders' ability to bring a claim in a forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. In addition, while the Delaware Supreme Court ruled in March 2020 that federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court were "facially valid" under Delaware law, there is uncertainty as to whether other courts will enforce our Federal Forum Provision. If the Federal Forum Provision is found to be unenforceable, we may incur additional costs associated with resolving such matters. The Federal Forum Provision may also impose additional litigation costs on stockholders who assert that the provision is not enforceable or invalid. The Court of Chancery of the State of Delaware and the federal district courts of the U.S. may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

General Risk Factors

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

In the ordinary course of our business, we and the third parties upon which we rely, process, collect, receive, store, use, transfer, protect, secure, dispose of, transmit, and share proprietary, confidential, and sensitive data, including personal data (such as health-related data), intellectual property and trade secrets. The secure processing, maintenance and transmission of such sensitive information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions.

Cyber-attacks, malicious internet-based activity, online and offline fraud, security breaches and other similar activities threaten the confidentiality, integrity, and availability of our sensitive information and information technology systems, and those of the third parties upon which we rely. Such threats are prevalent and continue

to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer “hackers,” threat actors, “hacktivists,” organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including, without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities.

These risks, as well as the number and frequency of cybersecurity events globally, may also be heightened during times of war or other major conflicts. We and the third parties upon which we rely are subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks (such as credential stuffing attacks), credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, electrical and telecommunications failures, earthquakes, fires, floods, and other similar threats. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, loss of sensitive data and income, reputational harm and diversion of funds.

We have a hybrid in-office/remote work environment, which, like other companies that have incorporated remote working, has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Future 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. Furthermore, 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.

We currently rely on third-party service providers and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, employee email, and other functions. We also currently rely on commercially available tools from third-party service providers to process and safeguard our sensitive information and business data. Our ability to monitor these third parties’ information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. 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, like others in our industry, have experienced and may continue to experience threats or system failures which could cause a security incident or other interruption that could result in unauthorized, unlawful or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties upon whom we rely. We have not yet conducted any internal audits or penetration tests on our information technology systems, nor have we enlisted any external parties to conduct security audits or penetration tests on our behalf. Such assessments, when conducted, could indicate vulnerabilities in our information technology systems which we may not be able to effectively remediate. If a security incident were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. We may expend significant resources or modify our business activities (including our clinical trial activities) to endeavor to protect against security incidents.

Certain data privacy and security obligations may require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive information. While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. Additionally, certain federal, state and foreign government requirements include obligations of companies to notify individuals of security breaches involving particular categories of personally identifiable information, which could result from breaches

experienced by us or the third parties upon whom we rely. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

We may not be able to detect and remediate vulnerabilities in our information technology systems because the threats and techniques used to exploit vulnerabilities change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. Such vulnerabilities could pose material risks to our business. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities. It is not possible to prevent all threats to our information technology systems and those of our third-party service providers, over which we exert less control, and any controls we implement to do so may prove to be ineffective.

If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may cause delays or disruptions in our clinical trials and development of product candidates, deter customers from using our products, and negatively impact our ability to grow and operate our business.

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. Further, although we maintain cyber liability insurance, we cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims. In addition to adverse impacts relating 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.

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

In the ordinary course of business, we and the third parties on which we rely process personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about clinical trial participants and sensitive third-party data. Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

The legislative and regulatory framework for the processing of personal data worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. In the U.S., there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure, transfer, security and processing of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act, the California Consumer Privacy Act (the "CCPA")), and other similar laws. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to HIPAA, which imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Depending on the facts and circumstances, we could be subject to civil and criminal penalties if we knowingly 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.

At the state level, numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses. For example, the CCPA establishes data privacy rights for individuals located in California and imposes certain requirements on how businesses can collect, use, and share personal information about such individuals. The California Privacy Rights Act (the "CPRA") significantly modified the CCPA and imposes additional obligations on companies covered by the legislation, including by expanding consumers' rights with respect to personal information, and established a state agency vested with the authority to enforce the CCPA. While these state laws exempt some data processed in the context of clinical trials, these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely. Furthermore, other states have proposed or enacted legislation that is focused on more narrow aspects of privacy. For example, a number of states have passed laws that are specifically focused upon health privacy, such as Washington's My Health My Data Act, which imposes an additional layer of protection regarding consumer health data and creates a private right of action. A smaller number of states have passed laws protecting biometric information. The effects of state and federal privacy laws are potentially significant and may require us to modify our data processing practices and policies and to incur substantial costs and potential liability in an effort to comply with them.

Additionally, through executive and legislative action, the federal government has taken steps to restrict data transactions involving certain sensitive data categories – including health data, genetic data, and biospecimens – with persons or entities affiliated with China and certain other countries of concern. For example, the Department of Justice's January 8, 2025, Rule on Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, prohibits transfers of certain types of data, including health data, genetic data, and biospecimens, to countries of concern, including China. Actual or alleged violations of these laws and regulations may be punishable by criminal and/or civil sanctions and may result in increased risk to our business of regulatory enforcement, litigation and reputational damage. These current and future data privacy laws and regulations may require us to modify our data collection or processing practices and policies, incur substantial costs and expenses in an effort to comply and increase our potential exposure to regulatory enforcement, reputational damage, and/or litigation.

Outside the U.S., an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, if we commence clinical trials in Europe, which we anticipate commencing if our product candidates advance to later stages of development, our processing of personal data in that context would become subject to the European Union's General Data Protection Regulation ("EU GDPR"), and/or the United Kingdom's so-called "UK GDPR" (together, the "GDPR"). The GDPR is wide-ranging in scope and imposes numerous requirements on companies that are subject to it, including relating to having a legal basis for processing personal data, relating to the processing of sensitive data (such as health data), obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, notification of data breaches, requiring data protection impact assessments for high risk processing and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union and the UK, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million (£17.5 million) or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the U.S. or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area ("EEA") and the United Kingdom ("UK") have significantly restricted the transfer of personal data to the U.S. and other countries whose privacy laws it considers inadequate. Other jurisdictions may adopt similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the U.S. 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 U.S. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the U.S., 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 U.S., 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 GDPR's cross-border data transfer limitations.

We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. If any privacy policies, marketing materials and other statements regarding data privacy and security that we publish are found to be deficient, lacking in transparency, deceptive, unfair or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, including the Federal Trade Commission, or other adverse consequences.

Laws and regulations related to privacy, data protection and security are continuing to evolve and are becoming increasingly stringent. Additionally, these laws may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions, creating uncertainty. Preparing for and complying with these obligations requires us to devote significant resources, which may necessitate changes to the ways in which we collect and process data, our information technologies, systems and to exercise greater oversight and control over third parties that process personal data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties we rely on fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we may face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims); additional reporting requirements and/or oversight; bans on processing personal data; orders to destroy or not use personal data; and imprisonment of company officials. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including, as relevant, clinical trials and development of product candidates); inability to process personal data or to operate or conduct clinical trials in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

Artificial intelligence presents risks and challenges that can impact our business, including by posing cybersecurity risks to our confidential information, proprietary information, and personal data.

We may use, and our vendors may incorporate, artificial intelligence ("AI"), both in our own development and implementation of AI and through the adoption of commercially available tools. The use of AI presents risks and challenges that could adversely affect our business and reputation, including cybersecurity, data privacy, IT, confidentiality, regulatory, legal, operational, competitive, reputational, intellectual property and other risks. For example, use of certain AI tools may increase the risk of unauthorized disclosure of confidential information, compromise of proprietary intellectual property, or inadvertent inclusion of third-party intellectual property or other protected material, which could result in disputes, claims of infringement, legal liabilities or financial losses. The rapidly evolving regulatory landscape surrounding AI also poses a risk, as new laws and regulations could impose additional compliance burdens, resulting in increased operational costs to comply with U.S. and non-U.S. laws concerning the use of AI, the nature of which cannot be determined at this time.

Additionally, our vendors may incorporate AI tools into their offerings, and the providers of these AI tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of AI, to engage in illegal activities involving the theft and misuse of personal information, confidential information

and intellectual property. The use of generative AI models in our internal or third-party systems may create new attack surfaces or methods for adversaries, which could impact us and our vendors. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

We are subject to governmental export and import controls, economic sanctions, anti-corruption laws and regulations of the U.S. and other jurisdictions. We can face criminal liability and other serious consequences for violations of these laws and regulations, which could harm our business.

We are subject to and required to comply with various export control, import and trade and economic sanctions laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations and sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls. These laws may prohibit or restrict our ability to transfer, sell or supply, our products to certain governments, persons, entities, countries, and territories, including those that are the target of comprehensive sanctions or an embargo.

We are also subject to anti-corruption and anti-bribery laws, including the U.S. Foreign Corrupt Practices Act of 1977, as amended (the "FCPA"), the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws, including the FCPA, generally prohibit companies and their employees, agents, CROs, contractors and other partners from offering, promising, giving, soliciting or authorizing others to give or receive anything of value, either directly or indirectly, to or from a non-U.S. government official in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. We can be held liable for the corrupt or other illegal activities of our employees, agents, CROs, contractors, and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violation 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.

If we fail to maintain an effective system of internal controls over financial reporting, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"), and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting.

We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for each year, as required by Section 404 of the Sarbanes-Oxley Act. This requires that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our initial public offering, we had never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner. In addition, when we lose our status as an "emerging growth company" and if we do not otherwise qualify as a "non-accelerated filer," our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting, which will require additional expense, resources and management commitment.

We may identify material weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls over financial reporting, we may not be able to produce timely and accurate consolidated financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

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

We are subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

We might not be able to utilize a significant portion of our net operating loss carryforwards.

We have generated, and expect to continue to generate, significant federal and state net operating loss (“NOL”) carryforwards. As of December 31, 2025, we had federal and state net operating loss carryforwards of $148.0 million and $155.4 million, respectively. Under current tax laws and regulations, these NOL carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the Tax Cuts and Jobs Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act, federal NOLs incurred in taxable years beginning after December 31, 2017 generally may be carried forward indefinitely, but the deductibility of such federal NOLs is limited to 80% of our taxable income for taxable years beginning after December 31, 2020. We cannot predict with certainty when, or whether, we will generate sufficient taxable income to use all of our NOLs.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "IRC"), and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% 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 NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We may have experienced ownership changes in the past and may experience ownership changes as a result of subsequent shifts in our stock ownership which may be out of our control. There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs by federal or state taxing authorities or other unforeseen reasons, portions of our existing NOLs could expire or otherwise be unavailable to reduce future income tax liabilities.

New tax laws or regulations, changes to existing tax laws or regulations or changes in their application to us may have a material adverse effect on our business, cash flows, financial condition or results of operations.

U.S. federal, state, local and foreign tax laws, regulations and administrative guidance are subject to change as a result of the legislative process and review and interpretation by the U.S. Internal Revenue Service, the U.S. Treasury Department and other taxing authorities. For example, budget reconciliation bill H.R. 1, referred to as the One Big Beautiful Bill Act ("H.R. 1") was signed into law on July 4, 2025, and made significant changes to U.S. federal tax law. Changes to tax laws (which changes may have retroactive application), including with respect to net operating losses and research and development tax credits, could adversely affect us or holders of our common stock.

For example, under H.R. 1, taxpayers are now permitted to immediately deduct domestic research and development expenses for taxable years beginning after December 31, 2024. Taxpayers may alternatively elect to capitalize and amortize such expenses. In addition, H.R. 1 provides that for taxable years beginning after December 31, 2021 and before January 1, 2025, certain eligible taxpayers generally may elect to retroactively

deduct expenses for domestic research and development expenses in such taxable years by filing amended tax returns for such taxable years, and all other taxpayers that are not eligible to make such an election and that amortized expenses for domestic research and development expenses in such taxable years generally may elect to accelerate and deduct the remaining unamortized amounts of such expenses (i) in the first taxable year beginning after December 31, 2024, or (ii) ratably over the two-taxable year period beginning with the first taxable year beginning after December 31, 2024. However, pursuant to Section 174 of the IRC, in taxable years beginning after December 31, 2021, foreign research and development expenses must continue to be capitalized and amortized.

In recent years, many changes to tax laws have been made and changes are likely to continue to occur in the future. Since early 2025, U.S. policy changes have been implemented at a rapid pace, and additional changes, including to tax laws, are likely. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations.

We are eligible to be treated as an "emerging growth company" and a "smaller reporting company" and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors. Beginning on January 1, 2027, we will no longer qualify as a smaller reporting company, which will increase our disclosure obligations and compliance costs.

Based on the market value of our common stock held by our non-affiliates as of June 30, 2026, we will no longer be a smaller reporting company as of January 1, 2027. However, under the SEC's transition rules, we will remain eligible to provide scaled disclosure according to exemptions available to smaller reporting companies until our Quarterly Report on Form 10-Q for the quarter ending March 31, 2027. Beginning with that report, we will be required to provide more extensive disclosure than we have provided historically, including expanded business description, management's discussion and analysis, market risk, related-party and corporate governance disclosures. Preparing this additional disclosure will require us to devote additional management time and to incur additional legal, accounting and other compliance costs. Notwithstanding our loss of smaller reporting company status, based on recent SEC guidance, we expect to continue to qualify as a non-accelerated filer through at least our fiscal year ending December 31, 2027, and as a non-accelerated filer we will remain exempt from the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act. Further, we expect to continue to qualify as an emerging growth company as described below.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"). For as long as we continue to be an emerging growth company, we may take advantage of certain exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- providing only two years of audited financial statements in addition to any required unaudited interim financial statements and correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the closing of our initial public offering, (b) in which we have total annual gross revenue of at least $1.235 billion or (c) in which we are deemed to be a large accelerated filer, which means, among other conditions, that the market value of our common stock that is held by non-affiliates exceeds $700

million as of the last business day of our most recently completed second fiscal quarter, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies, and we rely on this exemption.

We cannot predict if investors will find our common stock less attractive because we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We incur significant costs and demands upon management as a result of being a public company.

As a public company, we incur significant additional legal, accounting and other costs that we did not incur as a private company. We are now subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, Sarbanes-Oxley, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of Sarbanes-Oxley, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and certain corporate governance practices. Further, pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, the SEC has adopted additional rules and regulations in these areas, such as mandatory “say on pay” voting requirements that will apply to us when we cease to be an emerging growth company. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly as compared to a private company. If these requirements divert the attention of our management and personnel from other business concerns, they could have an adverse effect on our business. The increased costs will increase our net loss, and may require us to reduce costs in other areas of our business.

Unstable global economic and geopolitical conditions could adversely affect our business, financial condition, stock price, and results of operations.

The global economy and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, inflation, declines in economic growth, global supply chain disruptions, and uncertainty about economic stability. The global economy and financial markets may also be adversely affected by actual or anticipated changes in U.S. policies or regulatory environment, current or anticipated impact of military conflict, including the ongoing conflicts between Russia and Ukraine and in the Middle East, terrorism or other geopolitical events. Sanctions imposed by the U.S. and other countries in response to such conflicts may adversely impact the financial markets and the global economy, and the economic countermeasures by the affected countries or others could exacerbate market and economic instability.

There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. A severe or prolonged economic downturn could result in a variety of risks to our business, including weakened demand for any product candidates we may develop and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the equity and credit markets continue to deteriorate, it may make any necessary equity or debt financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay, scale back or discontinue the clinical development of one or more of our product candidates. In addition, there is a risk that our current or future service providers, manufacturers or other collaborators may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. We cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Changes in U.S. federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. For example, in 2025, the U.S. imposed broad-based tariffs on imports from almost all countries, including significant tariffs applicable to imports from China, where certain of our clinical trial materials are manufactured. In response to tariffs, other countries have implemented retaliatory tariffs on U.S. goods. While certain tariffs were subsequently suspended, modified, or temporarily reduced, their impact has already been seen, and we expect will continue to be seen, in global markets. Historically, tariffs have led to increased trade and political tensions between the U.S. and countries in the international community. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our financial condition or results of operations.

We may become involved in litigation that could divert management's attention and harm our business, and insurance coverage may not be sufficient to cover all costs and damages.

From time to time, we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, securities litigation, employment matters, security of patient and employee personal data, contractual relations with collaborators and licensors and intellectual property rights. We may be exposed to such litigation even if no wrongdoing on our part has occurred. Litigation to defend ourselves against claims by third parties, or to enforce any rights that we may have against third parties, could result in substantial costs and diversion of our resources, causing a material adverse effect on our business, financial condition, results of operations or cash flows.

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

(a) None.

(b) On February 6, 2025, the SEC declared effective our registration statement on Form S-1 (File No. 333-284352), as amended, filed in connection with our initial public offering (the "Registration Statement"). Pursuant to the Registration Statement, we issued and sold 12,176,467 shares of our common stock, which included 1,588,234 shares of common stock sold pursuant to the underwriters' full exercise of their option to purchase additional shares, at a price to the public of $18.00 per share. Goldman Sachs & Co. LLC, TD Securities (USA) LLC, Stifel, Nicolaus & Company, Incorporated, and Guggenheim Securities, LLC acted as representatives of the underwriters for the offering. We raised aggregate net proceeds of $199.6 million, after deducting underwriter discounts, commissions and other offering expenses.

We are holding a significant portion of the balance of the net proceeds in money market funds. There has been no material change in the planned use of the net proceeds from our initial public offering, as described in our final prospectus filed with the SEC on February 7, 2025 pursuant to Rule 424(b) under the Securities Act.

(c) None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

(a) None.

(b) None.

(c) During the three months ended September 30, 2026, none of our directors or executive officers adopted, modified, or terminated any contract, instruction or written plan for the purchase or sale of our securities that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) or any "non-Rule 10b5-1 trading arrangements" as defined in Item 408(c) of Regulation S-K.

Item 6. Exhibits

(a) Exhibits.

Exhibit Number	Description
3.1	Fifth Amended and Restated Certificate of Incorporation of Sionna Therapeutics, Inc. (Incorporated by reference to Exhibit 3.1 of the Registrant's Current Report on Form 8-K (File No. 001-42504) filed on February 10, 2025).
3.2	Amended and Restated Bylaws of Sionna Therapeutics, Inc. (Incorporated by reference to Exhibit 3.2 of the Registrant's Current Report on Form 8-K (File No. 001-42504) filed on February 10, 2025).
4.1	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.1 of the Registrant's Registration Statement on Form S-1 (File No. 333-284352) filed on January 17, 2025).
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*+	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded with the Inline XBRL document)

* Filed herewith.

\+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Quarterly Report and will not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

SIONNA THERAPEUTICS, INC.

Date: November 10, 2026	By:	/s/	Michael Cloonan
		Name:	Michael Cloonan, M.B.A.
		Title:	President and Chief Executive Officer
			(Principal Executive Officer)
Date: November 10, 2026	By:	/s/	Elena Ridloff
		Name:	Elena Ridloff, C.F.A.
		Title:	Chief Financial Officer and Chief Business Officer
			(Principal Financial Officer and Principal Accounting Officer)

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

I, Michael Cloonan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended September 30, 2026 of Sionna Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer 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 10, 2026

By: /s/ Michael Cloonan

Michael Cloonan

President and Chief Executive Officer
(Principal Executive Officer)

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

I, Elena Ridloff, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended September 30, 2026 of Sionna Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer 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 10, 2026

By: /s/ Elena Ridloff

Elena Ridloff

Chief Financial Officer and Chief Business Officer

(Principal Financial Officer and Principal Accounting Officer)

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

In connection with the Quarterly Report on Form 10-Q of Sionna Therapeutics, Inc. (the "Company") for the quarter ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned hereby certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, 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 10, 2026

By: /s/ Michael Cloonan

Michael Cloonan

President and Chief Executive Officer

(Principal Executive Officer)

Date: November 10, 2026

By: /s/ Elena Ridloff

Elena Ridloff

Chief Financial Officer and Chief Business Officer

(Principal Financial Officer and Principal Accounting Officer)