UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended 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: 001-41374

PEPGEN INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**85-3819886**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
321 Harrison Ave. 8th Floor Boston, Massachusetts	**02118**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (781) 797-0979

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.0001 per share	PEPG	Nasdaq Global Select 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, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

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

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

As of November 4, 2026, the registrant had 69,261,282 shares of common stock, $0.0001 par value per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or Form 10-Q, contains express or implied forward-looking statements that are based on our management's belief and assumptions and on information currently available to our management and which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this Form 10-Q include, but are not limited to, statements about:

- the initiation, timing, progress, results, and cost of our research and development programs and our current and future preclinical studies and clinical trials, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, and the period during which the results of our clinical trials will become available;
- our ability to efficiently develop our existing product candidate and discover new product candidates;
- our ability to successfully manufacture our investigational drug substances and drug product for preclinical use, for clinical trials and on a larger scale for commercial use, if our investigational drug candidate or any new product candidates are approved;
- our ability to obtain funding for our operations necessary to complete further development and commercialization of our existing product candidate or future product candidates;
- our ability to obtain and maintain regulatory approval of our existing product candidate or future product candidates;
- our ability to commercialize our product candidates, if approved;
- the pricing and reimbursement of our product candidates, if approved;
- the implementation of our business model, and strategic plans for our business and product candidates;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and our technology platform;
- estimates of our future expenses, revenues, capital requirements, and our needs for additional financing;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- our financial performance;
- the rate and degree of market acceptance of our product candidates;
- regulatory developments in the United States, or U.S., and foreign countries;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately, and our ability to identify and contract with alternative third-party suppliers and manufacturers in a timely manner and on reasonable terms, if needed;
- our ability to produce our products or product candidates with advantages in turnaround times or manufacturing cost;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key research and development or management personnel;
- the impact of laws and regulations;
- the impact of current or future litigation;
- developments relating to our competitors and our industry;
- the effects of any epidemics or pandemics, including mitigation efforts and economic effects, on any of the foregoing or other aspects of our business operations, including but not limited to our preclinical studies and clinical trials and any future studies or trials; and
- other risks and uncertainties, including those listed under the caption "*Risk Factors*."

In some cases, you can identify forward-looking statements by terminology such as "may," "will," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. These statements are only predictions, and are subject to change due to known and unknown risks, uncertainties, and other factors, which are, in some cases, beyond our control and which could materially affect results. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section titled "*Risk Factors*" and elsewhere in this Form 10-Q. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking statement is a guarantee of future performance. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. You should read this Form 10-Q and the documents that we reference in this Form 10-Q and have filed with the U.S. Securities and Exchange Commission, or the SEC, completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this Form 10-Q represent our views as of the date of this Form 10-Q. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should therefore not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Form 10-Q.

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 Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain.

This Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for our programs and product candidates. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from our own internal estimates and research as well as from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. While we are not aware of any misstatements regarding any third-party information presented in this Form 10-Q, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties and are subject to change based on various factors, including those discussed under the section titled "*Risk Factors*" and elsewhere in this Form 10-Q.

TRADEMARKS

This Form 10-Q contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to, including logos, artwork and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

SUMMARY OF MATERIAL RISKS ASSOCIATED WITH OUR BUSINESS

Our business is subject to numerous risks and uncertainties that you should be aware of in evaluating our business. These risks include, but are not limited to, the following:

- We are substantially dependent on the success of our lead product candidate, PGN-EDODM1. If we are unable to complete development of, obtain approval for and commercialize PGN-EDODM1 in a timely manner or at all, our business will be harmed.
- We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.
- We have never generated revenue from product sales and may never achieve or maintain profitability.
- We will need to raise substantial additional funding. If we are unable to raise substantial additional funding when needed, we could be forced to delay, scale back or discontinue our product development programs or future commercialization efforts.
- We are early in our development efforts, having conducted only two Phase 1 and three Phase 2 clinical trials since our inception and as a result it will be years before we commercialize a product candidate, if ever. If we are unable to advance our remaining clinical stage product candidate through clinical trials, obtain marketing approval and ultimately commercialize it, or experience significant delays in doing so, our business will be materially harmed.
- Drug development is a lengthy and expensive process, and preclinical and clinical testing is uncertain as to the outcome. We may encounter substantial delays in the commencement, enrollment or completion of our clinical trials and may never advance to clinical trials, or we may fail to demonstrate safety and effectiveness to the satisfaction of applicable regulatory authorities, which could prevent us from advancing or commercializing our product candidates on a timely basis, if at all.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, our ability to complete clinical trials may be adversely impacted.
- If any of our product candidates cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent the initiation or completion of clinical trials, regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.
- We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research, preclinical and clinical testing, and these third parties may not perform satisfactorily. If we need to replace one or more of these third parties, our development plans may be significantly delayed and we may expend more funds than currently planned.
- We currently depend on a small number of third-party suppliers to supply the product candidates that we are evaluating in our research and development programs. The loss of these or future third-party suppliers, or their inability to provide us with sufficient supply, could harm our business.
- We face significant competition, and if our competitors develop technologies or product candidates more rapidly than we do or their technologies or product candidates are more effective or have more favorable safety or tolerability profiles, our business and our ability to develop and successfully commercialize products may be adversely affected.
- We plan to seek approval from the FDA or comparable foreign regulatory authorities, where applicable, under the accelerated approval pathways. We may fail to obtain approval under such accelerated approval pathways. Moreover, these pathways may not lead to a faster development, regulatory review or approval process and do not increase the likelihood that our product candidates will receive marketing approval.
- If we are unable to obtain and maintain patent protection for our Enhanced Delivery Oligonucleotide platform, therapeutic development candidates or programs and/or other proprietary technologies we develop, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our therapeutic product candidates or programs and other proprietary technologies we may develop may be adversely affected.
- We expect to expand our headcount over time to support our 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.
- Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

- The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for holders of our common stock.

The summary risk factors described above should be read together with the text of the full risk factors below in the section titled "*Risk Factors*" in Part II, Item 1.A. and the other information set forth in this Form 10-Q, as well as in other documents that we file with the SEC. The risks summarized above or described in full below are not the only risks that we face. Additional risks and uncertainties not precisely 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.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PEPGEN INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(IN THOUSANDS)
(UNAUDITED)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 21,957	$ 60,514
Marketable securities	82,848	87,942
Prepaid expenses and other current assets	2,761	2,507
Total current assets	$ 107,566	$ 150,963
Property and equipment, net	1,102	1,903
Operating lease right-of-use asset	17,296	19,121
Restricted cash	1,548	1,548
Other assets	265	372
Total assets	$ 127,777	$ 173,907
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,906	$ 822
Accrued expenses	7,197	8,637
Operating lease liability	3,258	3,187
Total current liabilities	$ 12,361	$ 12,646
Operating lease liability, net of current portion	12,350	13,817
Total liabilities	$ 24,711	$ 26,463
Commitments and contingencies (Note 7)		
Stockholders' equity:		
Preferred stock	$ —	$ —
Common stock	7	7
Additional paid-in capital	519,305	508,544
Accumulated other comprehensive (loss) income	(162)	11
Accumulated deficit	(416,084)	(361,118)
Total stockholders' equity	$ 103,066	$ 147,444
Total liabilities and stockholders' equity	$ 127,777	$ 173,907

See accompanying notes to unaudited condensed consolidated financial statements.

PEPGEN INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE AMOUNTS)
(UNAUDITED)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 14,476	$ 13,423	$ 40,002	$ 57,192
General and administrative	5,907	5,231	18,262	16,715
Total operating expenses	$ 20,383	$ 18,654	$ 58,264	$ 73,907
Operating loss	$ (20,383)	$ (18,654)	$ (58,264)	$ (73,907)
Other income (expense):				
Interest income	1,005	634	3,348	2,598
Other expense, net	—	(6)	(6)	(6)
Total other income, net	1,005	628	3,342	2,592
Net loss before income tax	$ (19,378)	$ (18,026)	$ (54,922)	$ (71,315)
Income tax expense	(13)	—	(44)	—
Net loss	$ (19,391)	$ (18,026)	$ (54,966)	$ (71,315)
Net loss per share, basic and diluted	$ (0.28)	$ (0.52)	$ (0.79)	$ (2.14)
Weighted-average common shares outstanding, basic and diluted	69,260,353	34,362,876	69,185,230	33,268,264
Other comprehensive loss:				
Cumulative translation adjustment arising during the period	$ —	$ —	$ —	$ (1)
Unrealized (loss) gain on marketable securities	(45)	18	(173)	(47)
Comprehensive loss	$ (19,436)	$ (18,008)	$ (55,139)	$ (71,363)

See accompanying notes to unaudited condensed consolidated financial statements.

PEPGEN INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(IN THOUSANDS, EXCEPT SHARE AMOUNTS)
(UNAUDITED)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2025	68,874,944	$ 7	$ 508,544	$ 11	$ (361,118)	$ 147,444
Issuance of common stock from At-the-Market Sales Agreement, net of underwriters' fees	237,500	—	1,507	—	—	1,507
Vesting of restricted stock units	52,666	—	—	—	—	—
Exercise of stock options	2,848	—	7	—	—	7
Stock-based compensation expense	—	—	2,855	—	—	2,855
Unrealized loss on marketable securities	—	—	—	(70)	—	(70)
Net loss	—	—	—	—	(17,763)	(17,763)
Balance as of March 31, 2026	69,167,958	$ 7	$ 512,913	$ (59)	$ (378,881)	$ 133,980
Issuance of stock under the employee stock purchase plan	86,114	—	110	—	—	110
Vesting of restricted stock units	5,445	—	—	—	—	—
Stock-based compensation expense	—	—	3,313	—	—	3,313
Unrealized loss on marketable securities	—	—	—	(58)	—	(58)
Net loss	—	—	—	—	(17,812)	(17,812)
Balance as of June 30, 2026	69,259,517	$ 7	$ 516,336	$ (117)	$ (396,693)	$ 119,533
Exercise of stock options	1,538	—	4	—	—	4
Stock-based compensation expense	—	—	2,965	—	—	2,965
Unrealized loss on marketable securities	—	—	—	(45)	—	(45)
Net loss	—	—	—	—	(19,391)	(19,391)
Balance as of September 30, 2026	69,261,055	$ 7	$ 519,305	$ (162)	$ (416,084)	$ 103,066
Balance as of December 31, 2024	32,619,663	$ 3	$ 390,057	$ 23	$ (271,463)	$ 118,620
Vesting of restricted stock units	101,280	—	—	—	—	—
Stock-based compensation expense	—	—	3,432	—	—	3,432
Unrealized loss on marketable securities	—	—	—	(44)	—	(44)
Cumulative translation adjustment arising during the period	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(30,202)	(30,202)
Balance as of March 31, 2025	32,720,943	$ 3	$ 393,489	$ (22)	$ (301,665)	$ 91,805
Issuance of stock under the employee stock purchase plan	78,781	—	98	—	—	98
Stock-based compensation expense	—	—	2,363	—	—	2,363
Unrealized loss on marketable securities	—	—	—	(21)	—	(21)
Net loss	—	—	—	—	(23,087)	(23,087)
Balance as of June 30, 2025	32,799,724	$ 3	$ 395,950	$ (43)	$ (324,752)	$ 71,158
Issuance of stock in public offering, net of underwriters' fees and issuance costs	35,937,500	4	107,582	—	—	107,586
Exercise of stock options	10,000	—	27	—	—	27
Stock-based compensation expense	—	—	2,377	—	—	2,377
Unrealized gain on marketable securities	—	—	—	18	—	18
Net loss	—	—	—	—	(18,026)	(18,026)
Balance as of September 30, 2025	68,747,224	$ 7	$ 505,936	$ (25)	$ (342,778)	$ 163,140

See accompanying notes to unaudited condensed consolidated financial statements.

PEPGEN INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(IN THOUSANDS)
(UNAUDITED)

	Nine Months Ended September 30,	
	2026	2025
Cash flows from operating activities:		
Net loss	$ (54,966)	$ (71,315)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation	812	1,061
Stock-based compensation expense	9,133	8,172
Amortization and interest accretion related to operating lease	2,811	2,811
Amortization of premiums and accretion of discounts on marketable securities, net	(2,593)	(1,441)
Impairment of long-lived assets	—	701
Changes in operating assets and liabilities:		
Prepaids and other current and non-current assets	114	1,028
Accounts payable	1,104	(971)
Accrued expenses and other non-current liabilities	(1,440)	(3,523)
Operating lease liabilities, current and non-current	(2,382)	(2,313)
Net cash used in operating activities	$ (47,407)	$ (65,790)
Cash flows from investing activities:		
Purchases of property and equipment	(30)	(265)
Purchases of marketable securities	(100,487)	(6,726)
Maturities of marketable securities	108,000	58,000
Net cash provided by investing activities	7,483	51,009
Cash flows from financing activities:		
Issuance of common stock from At-the-Market Sales Agreement, net of underwriters' fees	1,511	—
Issuance of common stock in public offering, net of underwriters' fees	—	108,100
Payment of offering costs	(265)	(75)
Proceeds from employee equity plans	121	125
Net cash provided by financing activities	$ 1,367	$ 108,150
Effect of exchange rate changes on cash	—	(15)
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (38,557)	$ 93,354
Cash, cash equivalents and restricted cash at beginning of period	62,062	50,969
Cash, cash equivalents and restricted cash at end of period	$ 23,505	$ 144,323
Components of cash, cash equivalents and restricted cash		
Cash and cash equivalents	$ 21,957	$ 142,775
Restricted cash	1,548	1,548
Total cash, cash equivalents and restricted cash at end of period	$ 23,505	$ 144,323
Supplemental disclosure of cash flow information		
Cash paid for taxes	$ 49	$ 86
Deferred offering costs in accounts payable and accrued expenses	—	386

See accompanying notes to unaudited condensed consolidated financial statements.

PEPGEN INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(UNAUDITED)

1. Nature of Business and Basis of Presentation

PepGen Inc., hereinafter referred to as PepGen or the Company, is a clinical-stage biotechnology company advancing the next generation of oligonucleotide therapeutics with the goal of transforming the treatment of severe neuromuscular and neurologic diseases. The Company's principal offices are located in Boston, Massachusetts.

Liquidity

Since inception, the Company has not generated any revenue from product sales or other sources and has incurred significant operating losses and negative cash flows from operations. The Company's primary uses of cash, cash equivalents, and marketable securities to date have been funding research and development activities, business planning, establishing and maintaining the Company's intellectual property portfolio, hiring personnel, leasing premises and associated capital expenditures, raising capital, and providing general and administrative support for these operations. As of September 30, 2026, the Company had an accumulated deficit of $416.1 million. To date, the Company has funded operations primarily through private placements of convertible preferred stock, the sale of shares of common stock in its initial public offering, or IPO, the sale of shares of common stock under its At-the-Market Equity Offering Sales Agreement, or Sales Agreement, with Stifel, Nicolaus & Company, Incorporated, or Stifel, and through the sale of shares of common stock in follow-on public offerings in February 2024, referred to as the 2024 Offering, and in September 2025, referred to as the 2025 Offering. As of September 30, 2026, the Company had cash, cash equivalents, and marketable securities of $104.8 million.

The Company believes that its cash, cash equivalents, and marketable securities as of September 30, 2026, will be sufficient to fund its currently planned operations for at least the next 12 months from the issuance of these condensed consolidated financial statements.

As the Company continues to pursue its business plan to successfully develop and obtain regulatory approval for its product candidates, it expects to finance its operations through the sale of equity, debt financings or other capital resources, which could include income from collaborations, strategic partnerships or marketing, distribution, licensing or other strategic arrangements with third parties, or from grants. However, there can be no assurance that any additional financing or strategic transactions will be available to the Company on acceptable terms, if at all. If events or circumstances occur such that the Company does not obtain additional funding, it may need to delay, reduce or eliminate its product development or future commercialization efforts, which could have a material adverse effect on the Company's business, results of operations or financial condition.

Basis of Presentation and Consolidation

The accompanying condensed consolidated financial statements are unaudited and have been prepared in conformity with generally accepted accounting principles in the United States of America, or GAAP. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification and Accounting Standards Update of the Financial Accounting Standards Board, or FASB.

The condensed consolidated financial statements have been prepared on the same basis as the audited annual financial statements. Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. These condensed consolidated financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company's financial position as of September 30, 2026, and results of operations for the interim periods ended September 30, 2026 and September 30, 2025.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These condensed consolidated financial statements should be read in conjunction with the audited financial statements as of and for the years ended December 31, 2025 and 2024, and the notes thereto, included in the Company's Annual Report on Form 10-K, for the year ended December 31, 2025.

2. Summary of Significant Accounting Policies

The Company's significant accounting policies are disclosed in the audited financial statements for the year ended December 31, 2025 included in the Annual Report on Form 10-K for the year ended December 31, 2025. Since the date of those financial statements, there have been no changes to the Company's significant accounting policies.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard-setting bodies that the Company adopts as of the specified effective date. The Company qualifies as an "emerging growth company" as defined in the JOBS Act and has elected not to "opt out" of the extended transition related to complying with new or revised accounting standards, which means that when a standard is issued or revised and it has different application dates for public and non-public companies, the Company can adopt the new or revised standard at the time non-public companies adopt the new or revised standard and can do so until such time that the Company either (i) irrevocably elects to "opt out" of such extended transition period or (ii) no longer qualifies as an emerging growth company. The Company may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for non-public companies. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its consolidated financial statements and disclosures.

In November 2024, the FASB issued ASU No. 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40)*, or ASU 2024-03. ASU 2024-03 requires companies to disaggregate certain expenses presented on the face of the financial statements to enhance transparency and help investors better understand an entity's performance within the notes to the financial statements. The amendments in ASU 2024-03 are effective for fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The Company will not be required to adopt ASU 2024-03 until January 1, 2027. The Company is currently evaluating the potential impact of the adoption of ASU 2024-03 on its financial statement disclosures.

3. Fair Value Measurements

The following tables present information about the Company's financial assets that have been measured at fair value as of September 30, 2026 and December 31, 2025, and indicate the fair value of the hierarchy of the valuation inputs utilized to determine such fair value. In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities. All instruments held by the Company are Level 1. The following tables set forth marketable securities as of September 30, 2026 and December 31, 2025 (in thousands):

	As of September 30, 2026			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
U.S. Treasury notes	82,982	—	(134)	82,848
Total	$ 82,982	$ —	$ (134)	$ 82,848

	As of December 31, 2025			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
U.S. Treasury notes	87,903	39	—	87,942
Total	$ 87,903	$ 39	$ —	$ 87,942

The following tables set forth the fair value of the Company's financial assets measured at fair value on a recurring basis and indicate the level within the fair value hierarchy utilized to determine such values as of September 30, 2026 and December 31, 2025 (in thousands):

	As of September 30, 2026	
	Total	Level 1
Cash Equivalents:		
U.S. Treasury-backed money market funds	$ 19,042	$ 19,042
Marketable Securities:		
U.S. Treasury notes	82,848	82,848
Total	$ 101,890	$ 101,890

	As of December 31, 2025	
	Total	Level 1
Cash Equivalents:		
U.S. Treasury-backed money market funds	$ 58,147	$ 58,147
Marketable Securities:		
U.S. Treasury notes	$ 87,942	$ 87,942
Total	$ 146,089	$ 146,089

Money market funds are highly liquid investments that are valued based on quoted market prices in active markets, which represent a Level 1 measurement within the fair value hierarchy. These money market funds are classified on the consolidated balance sheets under cash and cash equivalents.

The following table summarizes the scheduled maturity for the Company's marketable securities for the period presented (in thousands):

	September 30, 2026
Maturing in one year or less	$ 82,848
Total	$ 82,848

4. Property and Equipment, Net

The cost and accumulated depreciation of property and equipment were as follows (in thousands):

	September 30, 2026	December 31, 2025
Lab equipment	$ 4,035	$ 4,035
Computer and office equipment	1,789	1,759
Construction in progress	—	20
Total property and equipment	5,824	5,814
Less: accumulated depreciation	(4,722)	(3,911)
Total property and equipment, net	$ 1,102	$ 1,903

Depreciation expense was $0.2 million and $0.3 million for the three months ended September 30, 2026 and September 30, 2025, respectively. Depreciation expense was $0.8 million for the nine months ended September 30, 2026 and $1.1 million for the nine months ended September 30, 2025.

During the second quarter of 2025, the Company also took $0.7 million in non-cash charges to research and development and general and administrative expense, associated with the impairment of unused lab equipment and computer and office equipment.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Employee-related expenses	$ 3,542	$ 4,663
Research and development expenses	2,225	2,993
Professional services	1,224	779
Other	206	202
Total accrued expenses	$ 7,197	$ 8,637

6. Related Party Transactions

Technology License Agreement with Oxford University Innovation Limited

In March 2018, the Company, Oxford University Innovation Limited, or OUI, and the Medical Research Council of United Kingdom Research and Innovation, or MRC, or collectively, the Licensors, entered into a license of technology agreement, which was subsequently amended in December 2018, further amended and restated in November 2020, and further amended in February 2022, referred to, as so amended, as the License Agreement. The Licensors and affiliates held shares of Series A-1 and Series A-2 preferred stock, Series B preferred stock and Class A common stock. The License Agreement provides the Company with an exclusive worldwide license to licensed data and technology owned by OUI and MRC in respect of cell-penetrating peptides for the treatment of Duchenne muscular dystrophy, spinal muscular atrophy, and other conditions. The License Agreement provides the Company with the rights to grant and authorize sublicenses to make, use, sell, and import products and otherwise exploit the patent rights.

As consideration for the license, the Company made an initial upfront payment in 2018, as well as a Restatement Completion Fee and a License Data Fee (each as defined in the License Agreement) in 2020 totaling $0.1 million.

The Company could be required to make milestone payments to the Licensors upon achievement of certain patent and commercial milestones related to the patents and commercialization of certain of the Company's product candidates. The aggregate potential milestone payments are $0.1 million. In 2024, the Company paid the Licensors a patent procurement milestone of £10,000, adjusted per the retail price index under the terms of the License Agreement. The Company also agreed to pay the Licensors low single digit royalties on net sales of any licensed products that are commercialized by the Company in excess of a threshold amount between £20 million and £30 million ($26.5 million and $39.7 million as of September 30, 2026), subject to certain adjustments. The term of the License Agreement continues until the later of (i) the date on which all the patents and patent applications covered thereunder have been abandoned or allowed to lapse or expired or been rejected or revoked or (ii) 20 years from the date of the original license agreement.

Upon completion of the IPO, the Company became obligated to pay OUI an exit fee between 0.5% to 2% of the value determined in an acquisition or initial public offering, not to exceed £5 million ($6.2 million as of the IPO date). The exit fee due to OUI, based on the proceeds of the IPO, was $1.4 million, which was paid during the second quarter of 2022 and included in research and development expense on the condensed consolidated statement of operations.

Oxford Science Enterprises, or OSE, which is an affiliate of OUI, owned 7.2% of the Company's outstanding common stock as of September 30, 2026.

RA Capital Management, L.P.

Entities affiliated with RA Capital Management, L.P., or RA Capital, purchased common stock in the Company's IPO in May 2022, the Company's 2024 Offering in February 2024, and the Company's 2025 Offering in September 2025. As of September 30, 2026, entities affiliated with RA Capital owned 29.0% of the Company's outstanding common stock.

Two members of the Company's board of directors, Dr. Joshua Resnick and Habib Dable, are affiliated with RA Capital. Pursuant to the governing legal documents of RA Capital, Dr. Resnick is obligated to transfer any cash compensation from the Company to RA Capital. Fees paid in cash for Dr. Resnick's service on the Company's board of directors were paid directly to RA Capital and are included within general and administrative expense on the consolidated statement of operations. The Company paid $10,000 and $11,000 for the three months ended September 30, 2026 and 2025, respectively, and $32,000 and $35,000 for the nine months ended September 30, 2026 and 2025, respectively, to RA Capital for Dr. Resnick's service on the board of directors.

7. Commitments and Contingencies

Legal proceedings

From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a liability for such matters when it is probable that future expenditures will be made and that such expenditures can be reasonably estimated.

On June 9, 2025, a putative shareholder class action captioned Johnny Karam v. PepGen Inc., et al. was filed in the United States District Court for the Eastern District of New York, naming the Company and its Chief Executive Officer and Chief Financial Officer as defendants. On November 18, 2025, the parties filed a stipulation of voluntary dismissal of the action. Separately, by a letter received by the Company on September 29, 2025, a putative shareholder of the Company made a purported shareholder demand based

on substantially the same allegations as those set forth in the above-mentioned putative class action suit. On December 1, 2025, the demanding shareholder withdrew the demand.

The Company is not party to any other litigation and does not have contingency reserves established for any litigation liabilities.

Other

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company agrees to indemnify, hold harmless, and to reimburse the indemnified party for losses suffered or incurred by the indemnified party, including in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third-party with respect to the Company's products. Further, the Company indemnifies its directors and officers who are, or were, serving at the Company's request in such capacities. The Company's maximum exposure under these arrangements is unknown as of September 30, 2026. The Company does not anticipate recognizing any significant losses relating to these arrangements. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements may be unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

8. Stockholders' Equity

Under the Third Amended and Restated Certificate of Incorporation, dated May 10, 2022, as amended on June 20, 2024, as currently in effect, the Company has the authority to issue a total of 500,000,000 shares of common stock (par value of $0.0001 per share) and 10,000,000 shares of undesignated preferred stock (par value of $0.0001 per share). Each share of common stock has the right to one vote. The holders of common stock are also entitled to receive dividends whenever funds are legally available and when declared by the board of directors, subject to the prior rights of holders of all classes of stock outstanding having priority rights as to dividends. The Company has never declared or paid cash dividends.

The Company has reserved shares of common stock for issuance, on an as-converted basis, as follows:

	September 30, 2026	December 31, 2025
Stock options	7,995,891	5,970,707
Restricted stock units	1,067,795	288,533
Authorized for future stock awards or option grants under the 2022 Plan	2,490,867	1,914,063
Authorized for future stock awards or option grants under the Inducement Plan	637,184	637,184
Authorized for future issuance under the ESPP	1,124,465	532,579
Total	13,316,202	9,343,066

9. Stock-Based Compensation

The Company maintains four equity compensation plans; the 2020 Stock Plan, or the 2020 Plan, the 2022 Stock Option and Incentive Plan, or the 2022 Plan, the 2022 Employee Stock Purchase Plan, or the ESPP, and the 2024 Inducement Plan, or the Inducement Plan. As of the Company's IPO in May 2022, the Company's board of directors determined that no further awards would be made under the 2020 Plan. The number of shares of common stock that may be issued under the 2022 Plan is subject to increase by the number of shares under any outstanding stock options forfeited and not exercised under the 2020 Plan. Additionally, the number of shares reserved for issuance under the 2022 Plan automatically increases on the first day of each fiscal year in an amount equal to the lower of (1) 5% of the shares of common stock outstanding on such date and (2) an amount determined by the Company's board of directors. The 2022 Plan allows the board of directors to grant incentive stock options, non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units, unrestricted stock awards, cash-based awards, and dividend equivalent rights to the Company's officers, employees, directors and other key persons. The number of shares reserved for issuance under the ESPP automatically increases on the first day of each fiscal year in an amount equal to the lesser of (i) one percent (1%) of the number of shares of common stock issued and outstanding on the immediately preceding December 31st, (ii) 678,000 shares of common stock or (iii) such number of shares of common stock as determined by the Administrator (as defined in the ESPP). In August 2024, the Company's board of directors adopted the Inducement Plan. The Inducement Plan provides for the grant of non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units, unrestricted stock awards, dividend equivalent rights and other stock-based awards with respect to an aggregate of 1,000,000 shares of common stock (subject to adjustment as provided in the Inducement Plan). Awards under the Inducement Plan may only be granted to new employees who were not previously employed by the Company or its affiliates in accordance with the requirements of Nasdaq Stock Market Rule 5635(c)(4).

As of September 30, 2026, 2,490,867 shares remained available for grant under the 2022 Plan, 1,124,465 shares remained available for issuance under the ESPP, and 637,184 shares remained available for issuance under the Inducement Plan.

Stock Option Repricing

On November 4, 2025, the Company's board of directors approved an option repricing, or the Repricing, effective as of November 4, 2025, or the Effective Date. The Repricing was undertaken in accordance with, and as permitted by, the Company's 2020 Plan, 2022 Plan, and Inducement Plan. Pursuant to the Repricing, all options granted pursuant to the 2020 Plan, 2022 Plan and Inducement Plan that are held by current employees, or Eligible Participants, were repriced, to the extent such options had an exercise price in excess of the 52-week high fair market value per share of the Company's common stock on the Nasdaq Global Select Market determined as of November 4, 2025, or Eligible Options.

The new exercise price for the Eligible Options is $4.53. However, in order to exercise the repriced options at the reduced exercise price, Eligible Participants are required to remain in service with the Company through the Retention Period (as defined herein). The "Retention Period" begins on the Effective Date and ends on the earliest of the following: (i) March 31, 2027; (ii) with respect to Eligible Options granted under the 2020 Plan, the occurrence of a Sale of the Company (as defined therein), or (iii) with respect to Eligible Options granted under the 2022 Plan and the Inducement Plan, the occurrence of a Sale Event (as defined therein).

As of the date of approval of the Repricing, the total number of shares underlying all Eligible Options was 3,557,903 shares. The Eligible Options previously had exercise prices ranging from $8.89 to $17.91 per share. There were no changes to the number of shares, the vesting schedule or the expiration date of the Eligible Options.

The effect of the option repricing resulted in a total incremental non-cash stock-based compensation expense of $3.4 million, which was calculated using the Black-Scholes option-pricing model, which is being recognized ratably through the 17-month Retention Period.

The Company recognized $0.5 million during the three months ended September 30, 2026, and $1.6 million during the nine months ended September 30, 2026 associated with the option repricing which is included in general and administrative and research and development expense in the consolidated statement of operations and comprehensive loss. There was no incremental stock-based compensation expense recognized for the three or nine months ended September 30, 2025.

Stock Option Activity

Stock option activity for the nine months ended September 30, 2026, was as follows:

	Stock Options		Weighted-Average Exercise Price
Outstanding as of December 31, 2025	5,970,707	$	4.48
Granted	2,771,316	$	5.61
Exercised	(4,386)	$	2.71
Canceled/Forfeited	(741,746)	$	4.59
Outstanding as of September 30, 2026	7,995,891	$	4.86

The weighted-average grant date fair value of options granted during the nine months ended September 30, 2026 was $4.63 per share. As of September 30, 2026, total unrecognized compensation cost related to stock options, inclusive of the repricing, was $17.3 million. This amount is expected to be recognized over a weighted average period of approximately 2.59 years.

Restricted Stock Units

A restricted stock unit, or RSU, represents the right to receive one share of common stock upon vesting of the RSU. In February 2024, the Company granted employees a one-time RSU award that vested in full on the one-year anniversary of the grant date, provided that the employee remained employed with the Company through the date of vesting. Certain employees, including employees who are executive officers of the Company, received a one-time RSU award that vests upon the achievement of certain performance-based clinical development milestones, or PSUs, provided that the employee remains employed with the Company through the date of vesting. Such awards could not vest in less than one year, regardless of when the performance milestone was achieved. This milestone was achieved and all PSUs vested on February 24, 2025. During the nine months ended September 30, 2025, the Company recognized $0.3 million in expense associated with the achievement of the performance milestone. No PSUs remain outstanding since March 31, 2025.

Beginning in 2025, the Company introduced RSU grants which generally vest over four years, provided the employee remains employed by the Company through the vesting dates. A summary of the Company's RSU activity and related information for the nine months ended September 30, 2026 is as follows:

	Service-based RSUs	Weighted-Average Grant Date Fair Value
Outstanding as of December 31, 2025	288,533	$ 3.08
Granted	991,113	5.95
Vested	(58,111)	2.69
Forfeited	(153,740)	5.34
Outstanding as of September 30, 2026	1,067,795	$ 5.44

The weighted average grant date fair value of the service-based RSUs granted during the nine months ended September 30, 2026 was $5.95. During the nine months ended September 30, 2026, 58,111 RSUs vested with a total fair value of $0.3 million. As of September 30, 2026, there was $4.9 million of unrecognized compensation costs related to unvested service-based RSUs, which are expected to be recognized over a weighted-average period of 3.29 years.

Stock-Based Compensation Expense

Stock-based compensation expense associated with stock options, RSUs, PSUs, and the Company's ESPP included in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 987	$ 930	$ 3,306	$ 3,449
General and administrative	1,978	1,447	5,827	4,723
Total stock-based compensation expense	$ 2,965	$ 2,377	$ 9,133	$ 8,172

10. Segment Reporting

ASU 2023-07 establishes standards for reporting information about operating segments on a basis consistent with the Company's internal organization structure as well as information about services categories, business segments and major customers in financial statements.

Segment reporting is prepared on the same basis that the chief executive officer, who is the Company's chief operating decision maker and manages the business, makes business decisions and assesses performance. The Company has one reportable segment and regularly reviews operating expenses and net loss on a consolidated basis. The Company operates in one location in Boston, Massachusetts focused on advancing oligonucleotide therapeutics with the goal of transforming the treatment of severe neuromuscular and neurologic diseases. The Company tracks external operating expenses on a program-by-program basis. Net loss is comprised of expenses related to the Company's two product candidates, one of which was discontinued in May 2025, and other personnel, facility, and general and administrative costs. Operating expenses and net loss are the basis the chief operating decision maker uses for the purpose of deciding how to allocate resources, specifically on a program basis as presented below.

The chief operating decision maker uses operating expenses and net loss to evaluate progress on research and development activities on the Company's two product candidates, one of which was discontinued in May 2025, as well as when making strategic decisions regarding the Company's research and development efforts. Operating expenses and loss are also used to monitor budget versus actual results.

The following table outlines the Company's functional operating expenses (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and Development Expenses				
PGN-EDODM1 direct research and development expenses	$ 7,724	$ 5,375	$ 19,503	$ 21,771
PGN-EDO51 direct research and development expenses	2	840	34	8,838
Platform and external research and development expenses	64	105	287	539
Personnel-related (including stock-based compensation)	4,978	4,955	14,827	18,267
Facility and related expenses	1,214	1,271	3,631	3,853
Other research and development expenses	494	877	1,720	3,924
Total research and development expense	14,476	13,423	40,002	57,192
General and Administrative Expenses				
Personnel-related (including stock-based compensation)	3,715	2,685	10,904	9,367
Facility and related expenses	488	519	1,495	1,511
Other general and administrative expenses	1,704	2,027	5,863	5,837
Total general and administrative expense	5,907	5,231	18,262	16,715
Loss from operations	(20,383)	(18,654)	(58,264)	(73,907)
Other income (expense):				
Interest income	1,005	634	3,348	2,598
Other expense, net	—	(6)	(6)	(6)
Total other income, net	1,005	628	3,342	2,592
Income tax expense	(13)	—	(44)	—
Net loss	$ (19,391)	$ (18,026)	$ (54,966)	$ (71,315)

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

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and the related notes included elsewhere in this Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Form 10-Q, including information with respect to our plans, strategies, objectives, expectations and intentions for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Form 10-Q, our actual results could differ materially from the results described in or implied by these forward-looking statements. Please also see the section titled "Special Note Regarding Forward-Looking Statements."

Overview

PepGen Inc., also referred to as "PepGen," "we," "our" or "us", is a clinical-stage biotechnology company advancing the next-generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases. Our proprietary Enhanced Delivery Oligonucleotide, or EDO, platform is founded on over a decade of research and development and leverages cell-penetrating peptides, or CPPs, to improve the uptake and activity of conjugated oligonucleotide therapeutics. Using these EDO peptides, our goal is to develop a research and development pipeline of oligonucleotide therapeutic candidates that are designed to target the root cause of serious diseases.

We are developing PGN-EDODM1 for the treatment of DM1 and are utilizing what we believe to be a unique mechanism of action and a different delivery approach compared to other approaches in more advanced stages of clinical development. We have conducted extensive preclinical studies of our product candidate, and these preclinical data form the basis of our clinical development plan for PGN-EDODM1.

Our clinical development program for PGN-EDODM1 includes three studies: (1) FREEDOM-DM1, or FREEDOM, a multinational, randomized, double-blind, placebo-controlled Phase 1 single ascending dose, or SAD, study which was conducted in Canada, the United Kingdom and the United States, (2) FREEDOM2-DM1, or FREEDOM2, a multinational, randomized, double blind, placebo-controlled Phase 2 multiple ascending dose, or MAD, study, which is being conducted in Canada, the United Kingdom, South Korea, Australia, and New Zealand, designed to assess PGN-EDODM1's safety and tolerability, splicing correction and functional outcome measures in DM1 patients, and (3) FREEDOM-OLE, which is an open label extension study open to participants who meet its eligibility criteria and have completed either the FREEDOM or FREEDOM2 studies. The FREEDOM2 study remains on partial clinical hold in the U.S. We are conducting, and remain actively engaged with FDA about, additional nonclinical mouse studies designed to generate data to respond to FDA feedback.

We recently reported topline results from the 10 mg/kg cohort in the FREEDOM2 study where PGN-EDODM1 was generally well-tolerated, with no serious adverse events (SAEs), all related treatment emergent adverse events (TEAEs) and non-related TEAEs reported being mild or moderate, and no signs of cumulative toxicity. At week 13, results from the 10 mg/kg cohort in the FREEDOM2 study demonstrated mean splicing correction of 46.5%. Mean splicing correction in the pooled placebo group (n=4) was 6.8% at week 13. Furthermore, in the 10 mg/kg cohort, certain functional endpoints included:

- Video hand opening time (vHOT), an objective measure of myotonia, improved 4.3-seconds at week 13 and 3.9-seconds at week 16; placebo improved 0.2 seconds at week 16. A dose dependent splicing correction of CLCN1, a muscle chloride channel believed to be the primary driver of myotonia in DM1, was also observed;
- Hand grip strength demonstrated a 6.3-point improvement in maximum predicted percent normal (PPN) score from baseline at week 16; placebo demonstrated a 0.9-point improvement in PPN at week 16;
- The 10-Meter Walk/Run Test (10mWRT) improved by 0.4 seconds while placebo improved 0.2 seconds, each at week 16.
- Patient Reported Outcomes measures were inconclusive at week 16; Patient Global Impression of Change (PGI-C) showed a favorable directional trend at 10 mg/kg versus placebo, and Myotonic Dystrophy Health Index (MDHI) total scores showed mixed results due to improvements in all groups, including the placebo arm, and differences in baseline measurements.

In the Phase 1 FREEDOM study, we enrolled adult participants to evaluate the safety and tolerability of PGN-EDODM1, as well as oligonucleotide muscle concentrations, splicing correction and functional outcome measures at day 28 and at week 16 following a single dose of PGN-EDODM1. We announced data from the three cohorts in the FREEDOM study (5 mg/kg, 10 mg/kg and 15 mg/kg) in 2025, highlighting that PGN-EDODM1 was generally well tolerated at all doses, with all drug-related adverse events mild or moderate in severity and a dose dependent mean splicing correction of 12.3%, 29.1% and 53.7% in the single 5 mg/kg, 10 mg/kg and 15 mg/kg cohorts, respectively. No meaningful improvements in any functional outcomes were observed in this single dose study.

Results from the 12.5 mg/kg cohort of FREEDOM2, which is fully enrolled, are expected in the first half of 2027. To date, 19 participants have enrolled in the open-label extension study with 100 doses given at 5 mg/kg and over 35 doses at 10 mg/kg. No

serious adverse events or dose-limiting toxicities have been reported in the open-label extension study, supporting a favorable tolerability profile, with one participant discontinuing treatment due to a hypersensitivity reaction. Data from FREEDOM2 and the FREEDOM-OLE studies are expected to support an end-of-Phase 2 meeting with the FDA in the second half of 2027 pending removal of the partial clinical hold in the United States.

The FDA has granted both orphan drug designation and Fast Track designation for PGN-EDODM1 for the treatment of DM1 and the EMA has also granted orphan medicinal product designation to PGN-EDODM1.

Initial Public Offering, ATM Program, Follow-on Offerings and Liquidity

In May 2022, we closed our initial public offering, or IPO, in which we sold an aggregate of 9,000,000 shares of common stock at a public offering price of $12.00 per share for gross proceeds of $108.0 million. In connection with the IPO, we granted the underwriters a 30-day option to purchase 1,350,000 additional shares of common stock, which they exercised in part to purchase 1,238,951 additional shares of common stock for gross proceeds of $14.9 million. We received approximately $122.9 million in gross proceeds and $110.2 million in net proceeds in the IPO, after deducting underwriters' fees and offering expenses.

Immediately prior to consummation of the IPO, all 12,546,805 outstanding shares of our redeemable convertible preferred stock, and 35,529 preferred stock warrants that were exercised on May 4, 2022, converted into 12,359,856 shares of our common stock.

On February 5, 2024, we issued and sold 1,000,000 shares of common stock at a purchase price of $10.00 per share under our at-the-market offering program, or ATM program, pursuant to an At-the-Market Equity Offering Sales Agreement, or Sales Agreement, with Stifel, Nicolaus & Company, Incorporated, or Stifel, resulting in net proceeds of $9.9 million. On February 9, 2024, we issued and sold 7,530,000 shares of common stock at a purchase price of $10.635 per share, which was the closing sale price of our common stock on the Nasdaq Global Select Market on February 6, 2024, in an underwritten follow-on offering, or the 2024 Offering. The 2024 Offering resulted in net proceeds of $76.4 million after deducting underwriters' fees of $3.7 million. Net proceeds from the ATM program and 2024 Offering, after deducting underwriters' fees and costs of the offerings, were $86.3 million. During the nine months ended September 30, 2026, the Company sold 237,500 shares of common stock under the Sales Agreement resulting in net proceeds of $1.5 million.

On September 26, 2025, we issued and sold 31,250,000 shares of common stock at a purchase price of $3.20 per share in an underwritten public offering, or the 2025 Offering. Pursuant to the underwriting agreement, we granted underwriters a 30-day option to purchase up to an additional 4,687,500 shares of common stock at a price of $3.20 per share which was exercised in full on September 25, 2025. The 2025 Offering resulted in net proceeds of $107.6 million after deducting underwriters' fees of $6.9 million and offering expenses of $0.5 million.

Since our inception, we have not generated any revenue from product sales or other sources and have incurred significant operating losses and negative cash flows from our operations. Our primary uses of cash to date have been to fund our research and development activities, business planning, establishing and maintaining our intellectual property portfolio, acquiring and developing product and technology rights, hiring personnel, leasing premises and associated capital expenditures, raising capital, and providing general and administrative support for these operations. To date, we have funded our operations primarily through private placements of our convertible preferred stock and proceeds from our IPO, the ATM program, the 2024 Offering, and the 2025 Offering.

We have incurred operating losses in each year since our inception. Our net losses were $55.0 million and $71.3 million for the nine months ended September 30, 2026 and September 30, 2025, respectively. As of September 30, 2026, we had cash, cash equivalents, and marketable securities of $104.8 million. As of September 30, 2026, we had an accumulated deficit of $416.1 million. Notwithstanding our decision to cease our research and development efforts in DMD, which we announced on May 28, 2025, we expect our expenses and operating losses will continue as we conduct our ongoing preclinical studies and current and planned clinical trials of PGN-EDODM1, continue our research and development activities, utilize third parties to manufacture our product candidates and related raw materials, hire additional personnel, protect our intellectual property and incur additional costs associated with being a public company, including audit, legal, regulatory, and tax-related services associated with maintaining compliance with an exchange listing and SEC requirements, director and officer insurance premiums, and investor relations costs. In addition, we have several development, regulatory and commercial milestone 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 preclinical studies, current and planned clinical trials, manufacturing campaigns and our expenditures on other research and development activities.

We believe that our existing cash, cash equivalents and marketable securities will be sufficient to fund our currently planned operations into 2028. We do not expect to generate any revenues from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which will not be for at least the next several years, if ever. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, until such time as we can generate significant revenue from sales of our product candidates, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potential collaborations, licenses and other similar arrangements. However, we may be

unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed would have a negative impact on our financial condition and could force us 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.

Components of Results of Operations

Operating Expenses

Research and Development

To date, our research and development expenses have primarily consisted of external and internal costs associated with our research and development activities, including our discovery and research efforts, the development of our proprietary EDO platform, and the preclinical and clinical development of our product candidates. Our research and development expenses include:

- external expenses, including expenses incurred under arrangements with third parties, such as clinical research organizations, or CROs, contract development manufacturing organizations, or CDMOs, consultants and our scientific advisors;
- personnel-related costs, including salaries, cash incentive compensation, payroll taxes, employee benefits, and stock-based compensation;
- costs for laboratory supplies and materials and reagents for chemical synthesis of product candidates; and
- facility costs, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies.

Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development activities are recorded as prepaid expenses until the goods or services are received.

The following table (in thousands) summarizes our research and development expenses for the three and nine months ended September 30, 2026 and September 30, 2025. The direct external development program expenses reflect external costs attributable to our clinical development candidates and preclinical candidates selected for further development. Our internal resources, personnel and infrastructure are not directly tied to any one research or drug discovery program and are deployed across multiple programs. As such, we do not track internal expenses on a program-specific basis.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
External expenses:				
PGN-EDODM1	$ 7,724	$ 5,375	$ 19,503	$ 21,771
PGN-EDO51	2	840	34	8,838
Other programs and unallocated expenses	64	105	287	539
Total external expense	7,790	6,320	19,824	31,148
Internal expenses:				
Personnel-related (including stock-based compensation)	4,978	4,955	14,827	18,267
Facilities and related costs	1,214	1,271	3,631	3,853
Other	494	877	1,720	3,924
Total research and development expenses	$ 14,476	$ 13,423	$ 40,002	$ 57,192

The Phase 1 FREEDOM clinical trial of our investigational drug candidate, PGN-EDODM1, has completed and we continue to conduct the ongoing Phase 2 FREEDOM2 and Open Label Extension clinical trials. On May 28, 2025, we announced that we decided to voluntarily discontinue development of PGN-EDO51 and have recently substantially completed the wind-down of DMD-related research and development activities. Research and development expenses for our lead program can be variable quarter-over-quarter due to the timing of manufacturing campaigns, which are accounted for under the percentage of completion method. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming. We may never succeed in achieving marketing approval for our remaining clinical stage product candidate or any new product candidates we develop.

The timelines and costs associated with research and development activities are uncertain and can vary significantly for our product candidate and development program due to the inherently unpredictable nature of preclinical and clinical development. We previously announced our decision to focus our development efforts on PGN-EDODM1, and to cease development of our

DMD-related programs, and may make further determinations as to which programs in our research and development pipeline to pursue and how much funding to direct to each program. We will need to raise substantial additional capital in the future.

Our future development costs may vary significantly based on factors such as:

- the status of clinical trials, the timing and costs, if any, associated with resolving clinical or partial clinical holds, animal and other preclinical studies and IND- or clinical trial application, or CTA-enabling studies;
- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- the duration of patient participation in the trials and follow-up;
- the cost and timing of manufacturing our product candidates and the cost and timing of our manufacturing campaigns;
- the efficacy and safety profile of our product candidates; and
- maintaining a continued acceptable safety profile of our products if any receive regulatory approval.

General and Administrative

General and administrative expenses consist primarily of personnel-related costs, including salaries, cash incentive compensation, payroll taxes, employee benefits, and stock-based compensation charges for those individuals in executive, finance, facility operations, and other administrative functions. Other significant costs include legal fees relating to intellectual property and corporate matters, professional fees for audit, accounting and consulting services, and insurance costs.

Other general and administrative expenses will remain consistent to support our public company operating expenses associated with audit, legal, regulatory, and tax-related services associated with maintaining compliance with our exchange listing and SEC requirements, director and officer insurance premiums, and investor relations.

Other Income (Expense), Net

Interest Income

Interest income consists of interest earned on our cash equivalents and marketable securities.

Other Income (expense)

Components of other income (expense) relate to realized and unrealized gains and losses on currency revaluation.

Income Taxes

We record tax expense for state taxes on interest income generated from the Company's cash equivalents and marketable securities. We have not recorded a U.S. provision for federal or state income taxes as we have no revenue and have incurred losses since inception.

Results of Operations

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

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

	Three Months Ended September 30,		Period-to-Period Change
	2026	2025	
Operating expenses:			
Research and development	$ 14,476	$ 13,423	$ 1,053
General and administrative	5,907	5,231	676
Total operating expenses	$ 20,383	$ 18,654	$ 1,729
Operating loss	$ (20,383)	$ (18,654)	$ (1,729)
Other income (expense):			
Interest income	1,005	634	371
Other expense, net	—	(6)	6
Total other income, net	1,005	628	$ 377
Net loss before income tax	$ (19,378)	$ (18,026)	$ (1,352)
Income tax expense	(13)	—	(13)
Net loss	$ (19,391)	$ (18,026)	$ (1,365)

Research and Development Expenses

Research and development expenses increased by $1.1 million from $13.4 million for the three months ended September 30, 2025, to $14.5 million for the three months ended September 30, 2026. This was primarily attributable to a $2.2 million increase in expense for preclinical research related to ongoing studies. This increase was additionally driven by a $0.7 million increase in consulting expense and a $0.3 million increase in clinical trial expense related to the ongoing FREEDOM2 study. These increases were partially offset by a $2.0 million decrease in manufacturing costs related to the timing of manufacturing campaigns.

General and Administrative Expenses

General and administrative expenses increased by $0.7 million from $5.2 million for the three months ended September 30, 2025, to $5.9 million for the three months ended September 30, 2026. The increase was primarily driven by an increase of $1.0 million in personnel-related costs, including $0.5 million in stock-based compensation expense. This increase was partially offset by a $0.4 million decrease in consulting and legal expenses.

Other Income (Expense), Net

Other income (expense), net was $1.0 million for the three months ended September 30, 2026 and $0.6 million for the three months ended September 30, 2025. Interest income is earned on the Company's cash equivalents and marketable securities.

Income Tax Expense

Income tax expense was $13,000 for the three months ended September 30, 2026 for state taxes on interest income generated from the Company's cash equivalents and marketable securities. Income tax expense was nil for the three months ended September 30, 2025.

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

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

	Nine Months Ended September 30,		Period-to-Period Change
	2026	2025	
Operating expenses:			
Research and development	$ 40,002	$ 57,192	$ (17,190)
General and administrative	18,262	16,715	1,547
Total operating expenses	$ 58,264	$ 73,907	$ (15,643)
Operating loss	$ (58,264)	$ (73,907)	$ 15,643
Other income (expense):			
Interest income	3,348	2,598	750
Other expense, net	(6)	(6)	-
Total other income, net	$ 3,342	$ 2,592	$ 750
Net loss before income tax	$ (54,922)	$ (71,315)	$ 16,393
Income tax expense	(44)	—	(44)
Net loss	$ (54,966)	$ (71,315)	$ 16,349

Research and Development Expenses

Research and development expenses decreased by $17.2 million from $57.2 million for the nine months ended September 30, 2025, to $40.0 million for the nine months ended September 30, 2026. This was primarily attributable to a $13.8 million decrease in manufacturing costs related to the timing of manufacturing campaigns, a $3.4 million decrease in personnel-related costs, and a $0.7 million decrease related to a non-cash charge taken during the prior year associated with the impairment of unused lab equipment. The decrease was additionally driven by a $2.3 million decrease in clinical trial expense as, prior to the discontinuation of our DMD program in late May 2025, we had two programs in clinical trials during a portion of the prior year, and there was a $0.7 million charge taken during the second quarter in the prior year for estimated wind-down costs for the two clinical trials for PGN-EDO51. These decreases are partially offset by a $2.5 million increase in expense for preclinical research related to ongoing studies and a $1.2 million increase in consulting expense.

General and Administrative Expenses

General and administrative expenses increased by $1.5 million from $16.7 million for the nine months ended September 30, 2025, to $18.3 million for the nine months ended September 30, 2026. The increase was primarily driven by an increase of $1.6 million in personnel-related costs, including $1.1 million in stock-based compensation expense.

Other Income (Expense), Net

Other income (expense), net was $3.3 million for the nine months ended September 30, 2026 and $2.6 million for the nine months ended September 30, 2025. Interest income is earned on the Company's cash equivalents and marketable securities.

Income Tax Expense

Income tax expense was $44,000 for the nine months ended September 30, 2026 for state taxes on interest income generated from the Company's cash equivalents and marketable securities. Income tax expense was nil for the nine months ended September 30, 2025.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception in January 2018, we have funded our operations primarily through the sale of our common stock and convertible preferred stock. We received aggregate gross proceeds of $163.9 million from these sales prior to our IPO. Additionally, in May 2022, we received gross proceeds from our IPO of $122.9 million.

On June 2, 2023, we filed a shelf registration statement on Form S-3 with the SEC, which covers the offering, issuance and sale of an amount up to $300.0 million in the aggregate of shares of our common stock, preferred stock, debt securities, warrants, and/or units or any combination thereof, which was declared effective on June 16, 2023.

On August 8, 2023, we filed a prospectus supplement and entered into the Sales Agreement with Stifel, as sales agent, which provides for the issuance and sale by us of up to $100.0 million of shares of common stock from time to time under the ATM program. On February 5, 2024, we issued and sold 1,000,000 shares of common stock at a purchase price of $10.00 per share under the ATM program, resulting in net proceeds of $9.9 million. During the nine months ended September 30, 2026, the Company sold 237,500 shares of common stock under the Sales Agreement resulting in net proceeds of $1.5 million.

On February 9, 2024, we issued and sold 7,530,000 shares of common stock in the 2024 Offering at a purchase price of $10.635 per share, resulting in net proceeds of $76.4 million after deducting underwriters' fees and offering costs of $3.7 million.

On June 28, 2024, we filed a second shelf registration statement on Form S-3 with the SEC, which covers the offering, issuance and sale of an amount up to $250.0 million in the aggregate of shares of our common stock, preferred stock, debt securities, warrants, and/or units or any combination thereof, which was declared effective on July 8, 2024.

On September 26, 2025, we issued and sold 31,250,000 shares of common stock at a purchase price of $3.20 per share in the 2025 Offering. Underwriters exercised their option to purchase an additional 4,687,500 shares of common stock at a price of $3.20 per share on September 25, 2025. The 2025 Offering resulted in net proceeds of $107.6 million after deducting underwriters' fees of $6.9 million and offering expenses of $0.5 million.

On May 18, 2026, we filed a shelf registration statement on Form S-3 with the SEC, which covers the offering, issuance and sale of an amount up to $400.0 million in the aggregate of shares of our common stock, preferred stock, debt securities, warrants, and/or units or any combination thereof, which was declared effective on May 21, 2026. Also on May 18, 2026, we filed a sales agreement prospectus as part of our shelf registration statement on Form S-3 and entered into the Sales Agreement with Stifel, as sales agent, which provides for the issuance and sale by us of up to $100.0 million of shares of common stock from time to time under the ATM program.

Future Funding Requirements

As of September 30, 2026, we had cash, cash equivalents, and marketable securities of $104.8 million. We believe that our existing cash, cash equivalents, and marketable securities will be sufficient to fund our currently planned operations into 2028. 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. We have based this estimate on assumptions that may prove to be wrong, and we could deplete our capital resources sooner than we expect. Additionally, the process of conducting preclinical studies and testing product candidates in clinical trials is costly, and the timing of progress and expenses in these studies and trials is uncertain.

Our future capital requirements will depend on many factors, including but not limited to:

- the scope, progress, costs and results of preclinical and clinical development for PGN-EDODM1, any additional product candidates we may develop and any new indications we may pursue;
- the scope, costs, timing and outcome of regulatory review of PGN-EDODM1, any additional product candidates we may develop and any new indications we may pursue;
- the cost and timing of manufacturing activities;
- the identification of additional research programs and product candidates;
- the costs and scope of the continued development of our EDO platform;
- the costs and timing of preparing, filing and prosecuting applications for patents, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including claims of infringement, misappropriation or other violations of third-party intellectual property;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate that receives marketing approval;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of our product candidates if marketing approval is received;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and

- the ongoing costs of operating as a public company.

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, and other similar 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 or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends. If we raise funds through collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. 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 through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market our drug candidates even if we would otherwise prefer to develop and market such drug candidates ourselves.

Cash Flows

The following table sets forth a summary of the net cash flow activity for the nine months ended September 30, 2026 and September 30, 2025 (in thousands):

	Nine Months Ended September 30,	
	2026	2025
Net cash (used in) provided by:		
Operating activities	$ (47,407)	$ (65,790)
Investing activities	7,483	51,009
Financing activities	1,367	108,150
Effect of exchange rate changes on cash	—	(15)
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (38,557)	$ 93,354

Operating Activities

For the nine months ended September 30, 2026, net cash used in operating activities was $47.4 million resulting from our net loss of $55.0 million and changes in our operating assets and liabilities of $2.6 million, partially offset by non-cash adjustments of $10.2 million. The net changes in our operating assets and liabilities were primarily due to a decrease in operating lease liabilities of $2.4 million. The non-cash adjustments included $9.1 million of stock-based compensation, $0.8 million of depreciation expense, $2.8 million of amortization and interest accretion on our operating lease, and $2.6 million of amortization of discounts on our marketable securities.

For the nine months ended September 30, 2025, net cash used in operating activities was $65.8 million resulting from our net loss of $71.3 million and changes in our operating assets and liabilities of $5.8 million, partially offset by non-cash adjustments of $11.3 million. The net changes in our operating assets and liabilities were primarily due to a decrease of $3.5 million in accrued expenses and a decrease of $2.3 million in operating lease liabilities. The non-cash adjustments included $8.2 million of stock-based compensation, $1.1 million of depreciation expense, $1.4 million of amortization of discounts on our marketable securities, and $2.8 million of amortization and interest accretion on our operating lease. Additionally, there was a non-cash adjustment of $0.7 million related to the impairment of unused lab equipment.

Investing Activities

Net cash provided by investing activities was $7.5 million for the nine months ended September 30, 2026 compared to net cash provided by investing activities of $51.0 million for the nine months ended September 30, 2025.

Net cash provided by investing activities for both periods was primarily associated with transactions involving our marketable securities.

Financing Activities

For the nine months ended September 30, 2026, net cash provided by financing activities was $1.4 million resulting primarily from proceeds from the sale of shares of common stock under the Sales Agreement.

For the nine months ended September 30, 2025, net cash provided by financing activities was $108.2 million resulting from $108.1 million in proceeds from the 2025 Offering and $0.1 million of proceeds from the purchase of shares under employee equity plans, partially offset by $0.1 million in the payment of offering costs.

Critical Accounting Policies and Estimates

There have been no material changes to our critical accounting policies and estimates from those described in our financial statements and the related notes and other financial information included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

As of September 30, 2026, we had $104.8 million in cash, cash equivalents, and marketable securities, consisting of cash in a readily available checking account and U.S. treasury-backed money market funds. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term maturities of our investments, we believe a hypothetical 100 basis point increase or decrease in interest rates during any of the periods presented would not have had a material impact on our financial results.

Concentration of Credit Risk

Financial instruments, which potentially subject us to concentrations of credit risk, consist of cash and money market accounts. As of September 30, 2026, our cash and money market accounts were held by three financial institutions in the U.S. At times, our deposits held in the U.S. may exceed the respective insured limits of the Federal Deposit Insurance Corporation.

Item 4. Controls and Procedures.

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Exchange Act) that are designed to ensure that information required to be disclosed in reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this report. In designing and evaluating our 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 their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2026, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business in accordance with the Exchange Act.

Changes in Internal Control over Financial Reporting

We are also required to maintain internal controls over financial reporting (as defined in Rules 13a-15(f) or 15d-15(f) under the Exchange Act) that are designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that (1) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the issuer, (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the issuer are being made only in accordance with authorizations of management and directors of the issuer, and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the issuer's assets that could have a material effect on the financial statements.

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

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be a party to litigation or subject to claims incident to the ordinary course of business. Although the results of litigation and claims cannot be predicted with certainty, we currently believe that the final outcome of these ordinary course matters will not have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors. As of September 30, 2026, we were not a party to any material legal proceedings.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully read and consider all of the risks described below, as well as the other information in this Form 10-Q, including our financial statements and the related notes and the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations," and in other documents we file with the SEC when evaluating our business. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. Unless otherwise indicated, references to our business being harmed in these risk factors will include harm to our business, reputation, financial condition, results of operations and future prospects. In such an event, the market price of our common stock could decline, and you may lose all or part of your investment. The risks described below are not intended to be exhaustive and are not the only risks that we face. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations and the market price of our common stock.

Risks Related to Our Financial Position and Need for Additional Capital

We are substantially dependent on the success of our lead product candidate, PGN-EDODM1. If we are unable to complete development of, obtain approval for and commercialize PGN-EDODM1 in a timely manner or at all, our business will be harmed.

We have no products approved for commercial sale and have not generated any revenue from product sales. Our ability to generate revenue from product sales and achieve profitability will depend on our ability, alone or with collaborators, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, PGN-EDODM1, our lead product candidate, and any future product candidates we may develop. As a result, we currently intend to devote a substantial portion of our resources and business efforts to the continued clinical development of PGN-EDODM1. The success of PGN-EDODM1, and any future product candidates we may develop, will depend on several factors, including the following:

- the successful completion of clinical trials of PGN-EDODM1 on a timely basis, including our ongoing FREEDOM2 clinical trial and any subsequent studies that may be required;
- successful outcome of discussions with the U.S. Food and Drug Administration, or the FDA, relating to the partial clinical hold placed on the FREEDOM2 study;
- the frequency and severity of any adverse events, or AEs, observed in PGN-EDODM1 clinical trials;
- maintaining and establishing relationships with contract research organizations, or CROs, and clinical trial sites for the clinical development of PGN-EDODM1 in the United States and internationally;
- the successful conduct and completion of clinical trials in compliance with applicable Good Clinical Practice, or GCP, and Good Laboratory Practice, or GLP, requirements;
- demonstration of efficacy, safety and tolerability profiles that are acceptable to the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or the EMA, or other comparable foreign regulatory authorities for marketing approval;
- the timely receipt of marketing approvals from applicable regulatory authorities;
- the scope and extent of any post-marketing approval commitments that may be required by regulatory authorities;
- the maintenance of existing, or establishment of new, supply arrangements with third-party suppliers and manufacturers to support the clinical development of PGN-EDODM1;

- the maintenance of existing, or establishment of new, scalable commercial manufacturing arrangements with third-party manufacturers to produce finished product suitable for commercial sale, if PGN-EDODM1 is approved;
- commercial acceptance by patients, physicians and the broader medical community;
- our ability to obtain coverage and adequate reimbursement from third-party payors, and patients' willingness to pay out-of-pocket in the absence of such coverage;
- our ability to compete effectively with other therapies, including those that may be further advanced in clinical development;
- our ability to negotiate favorable terms in any collaboration, licensing or other strategic arrangements and perform our obligations under such arrangements; and
- our ability to maintain, protect, enforce and defend our intellectual property portfolio in the United States and internationally.

We do not have complete control over many of these factors, including aspects of clinical development and the regulatory review process, the actions of regulatory authorities, potential challenges to our intellectual property rights, and the manufacturing, commercialization and distribution activities of third-party collaborators, if any. If we are not successful in addressing one or more of these factors in a timely manner or at all, we could experience significant delays or be unable to successfully commercialize PGN-EDODM1, which would materially harm our business, financial condition and prospects. If we do not receive marketing approval for PGN-EDODM1, we may not be able to continue our operations.

We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.

Since inception, we have incurred significant operating losses. Our net losses were $55.0 million and $71.3 million for the nine months ended September 30, 2026 and September 30, 2025, respectively. As of September 30, 2026, we had an accumulated deficit of $416.1 million. To date, we have financed our operations primarily with the proceeds raised from the sale of our convertible preferred stock in private placements and common stock in our IPO and our equity offerings in 2024 and 2025, described below. We have devoted substantially all of our financial resources and efforts to research and development activities, manufacturing, business planning, establishing and maintaining our intellectual property portfolio, acquiring and developing product and technology rights, hiring personnel, leasing premises and associated capital expenditures, raising capital, and providing general and administrative support for these operations. We are still in the early stages of development of our programs and currently only have one product candidate advancing into clinical development. We expect to continue to incur significant expenses and operating losses for the foreseeable future. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if and as we:

- continue to advance our PGN-EDODM1 program in DM1 through clinical development, including if enrollment for the FREEDOM2 study takes longer than anticipated or if we are unable to obtain clearance to initiate sites in additional jurisdictions (such as the US);
- address any new safety or efficacy concerns which may arise as we analyze interim, "top-line," or "preliminary" data from the ongoing FREEDOM2 clinical trial, or other planned or future clinical trials;
- advance any additional product candidates we identify through our research programs into IND- or CTA-enabling studies and clinical trials following regulatory clearance to commence clinical research;
- continue to develop and expand the capabilities of our proprietary EDO platform;
- establish manufacturing sources for our product candidates and secure supply chain capacity to provide sufficient quantities for preclinical and clinical development and commercial supply as well as other CMC activities which support registration, particularly as we scale for an anticipated Phase 3 pivotal trial and look to secure secondary source suppliers for our drug substance;
- seek marketing approvals for any product candidates that successfully complete pivotal clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional personnel, including clinical, regulatory and scientific employees and consultants;

- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

Even if we obtain regulatory approval of, and are successful in commercializing our product candidate or any future product candidates, we will continue to incur substantial research and development and other costs to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. 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.

We have never generated revenue from product sales and may never achieve or maintain profitability.

While we have initiated a Phase 1 and a Phase 2 clinical trial for PGN-EDODM1, we expect that it will be many years, if ever, before we have a product candidate ready for commercialization. All of our other research programs, which are currently paused, are nevertheless in the research or preclinical stage of development, and their risk of failure is therefore particularly high. To become and remain profitable, we must succeed in developing, obtaining the necessary regulatory approvals for and eventually commercializing a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including:

- obtaining marketing approval for our product candidates, most importantly PGN-EDODM1, including initiating, completing, and funding a large Phase 3 pivotal trial;
- manufacturing (or securing third-party manufacturers to manufacture), marketing and selling any products for which we may obtain regulatory approval;
- obtaining regulatory clearance to commence clinical trials and initiating and successfully completing such trials;
- identifying product candidates and completing preclinical development of our product candidates;
- achieving market acceptance of any products for which we obtain regulatory approval as a viable treatment option; and
- satisfying any post-marketing requirements.

We are only in the preliminary stages of some of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. Because of the numerous risks and uncertainties associated with product development, we are unable to accurately estimate or know the nature, timing or costs of the efforts that will be necessary to complete the preclinical and clinical development and commercialization of our product candidates or when, or if, we will be able to generate revenues or achieve profitability.

If we are successful in obtaining regulatory approval to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement, the nature of the competition in such market, and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable could impair our ability to raise capital, maintain our research and development efforts, expand our business or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will need to raise substantial additional funding. If we are unable to raise substantial additional funding when needed, we could be forced to delay, scale back or discontinue our product development programs or future commercialization efforts.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we identify, continue the research and development of, continue preclinical testing and initiate clinical trials of, arrange for the manufacturing of, and potentially seek marketing approval for our product candidates that successfully complete clinical testing. To date, we have completed a Phase 1 clinical trial for PGN-EDODM1, and are conducting a Phase 2 clinical trial as well as an open label

extension study for PGN-EDODM1. We will require significant additional funding to advance PGN-EDODM1 beyond the FREEDOM2 data readouts in 2026 to sustain our operations. We may be unable to raise additional funds, on favorable terms, if at all. If we are unable to raise such capital when needed, we will need to delay, reduce or terminate some or all planned activities to reduce costs and we may be required to initiate steps to cease operations and settle our liabilities. The failure to obtain sufficient additional funds on commercially acceptable terms to fund our operations and satisfy our obligations to creditors may have a material adverse effect on our business, results of operations and financial condition and jeopardize our ability to continue operations in the near-term.

Further, if we obtain marketing approval for PGN-EDODM1 or any other product candidate, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed, on attractive terms or at all, we may be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

As of September 30, 2026, we had cash, cash equivalents, and marketable securities of $104.8 million. Prior to our IPO in May 2022, we raised aggregate gross proceeds of $133.5 million from the private placement of convertible preferred stock. We raised aggregate gross proceeds of $122.9 million from our IPO. Subsequent to our IPO, in February 2024 we received net proceeds from our ATM program and 2024 Offering, after deducting underwriters' fees and costs of the offerings, of $86.3 million. In September 2025, we completed a follow-on public offering, or the 2025 Offering, which resulted in net proceeds of $107.6 million after deducting underwriters' fees and offering expenses. During the nine months ended September 30, 2026, we sold 237,500 shares of common stock under the Sales Agreement resulting in net proceeds of $1.5 million.

Our future capital requirements will depend on many factors, including:

- the scope, progress, including continued momentum of enrollment in our FREEDOM2 trial, costs and results of preclinical and clinical development for PGN-EDODM1, including the nature and size of further clinical trials for the development of PGN-EDODM1 such as a Phase 3 pivotal trial, and any additional product candidates we may develop or any new indications we may pursue;
- the scope, costs, timing and outcome of regulatory review of PGN-EDODM1 and any additional product candidates we may develop or any new indications we may pursue;
- the cost and timing of manufacturing activities, particularly as we scale for an anticipated Phase 3 pivotal trial and look to secure secondary source suppliers for our drug substance;
- the identification of additional research programs and product candidates;
- the costs and scope of the continued development of our EDO platform;
- the costs and timing of preparing, filing and prosecuting applications for patents, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including claims of infringement, misappropriation or other violations of third-party intellectual property;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate that receives marketing approval;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of our clinical stage product candidate if marketing approval is received;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

Developing our lead product candidate PGN-EDODM1, identifying other potential product candidates and conducting preclinical testing and clinical trials are time-consuming, expensive and uncertain processes requiring years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, may not be sufficient to sustain our operations. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our operations. We cannot be certain that additional funding will be available on acceptable terms, when needed or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts, when needed or on terms acceptable to us, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs, including PGN-EDODM1 or the commercialization of any such product candidate, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, and we may be required to initiate steps to cease operations, which could materially affect our business, financial condition and results of operations. We could be required to seek collaborators for product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2018, have no products approved for commercial sale and have not generated any revenue from product sales. To date, our operations have been limited to organizing and staffing our company, business planning, executing collaborations, raising capital, licensing, conducting research activities, conducting preclinical studies of our programs and clinical trials of our product candidates, manufacturing drug substance and drug product for our clinical programs, filing and prosecuting patent applications and providing general and administrative support for these operations. We have completed a Phase 1 clinical trial and are conducting a Phase 2 clinical trial as well as an open label extension for PGN-EDODM1. All of our other research programs are paused and, nevertheless, are in the research or preclinical stage of development, and their risk of failure is high. We have not yet demonstrated our ability to successfully complete clinical trials consistently, obtain marketing approvals, manufacture product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

Our limited operating history may make it difficult to evaluate our technology and industry and predict our future performance. Our limited history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by small, early-stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to continue to transition from a company with a research focus to a company capable of conducting development activities for multiple product candidates and then to a company supporting commercial activities. We may not be successful in such transitions. If we do not adequately address these risks and difficulties or successfully make such a transition, it could have a material adverse impact on our business.

Risks Related to Discovery, Development, Preclinical and Clinical Testing

We are early in our development efforts, having conducted only two Phase 1 and three Phase 2 clinical trials since our inception and as a result it will be years before we commercialize a product candidate, if ever. If we are unable to advance our product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts and have invested our research efforts to date in developing our EDO platform. Until May 2025, we had two product candidates in clinical trials —PGN-EDODM1 for DM1 and PGN-EDO51 for DMD. We have since voluntarily discontinued development of PGN-EDO51 and have substantially completed the wind-down of DMD-related research and development activities. We initiated a Phase 1 clinical trial, designated FREEDOM, for our product candidate, PGN-EDODM1, in December 2023 which was completed earlier this year. We are also conducting a Phase 2 clinical trial, FREEDOM2, in Canada, the U.K., South Korea, Australia and New Zealand, which has concluded dosing of the 5 mg/kg and 10 mg/kg cohorts with all eight patients having commenced dosing in the 12.5 mg/kg cohort.

We are primarily focusing our efforts on the clinical development of PGN-EDODM1 and, accordingly, have not completed IND- or CTA-enabling activities for any other product candidates or advanced any additional product candidates into clinical trials. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the

successful clinical development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Commencing clinical trials in the U.S. is subject to authorization by the FDA of an IND and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA requires us to complete additional preclinical studies, or we are required to satisfy other FDA or other regulator requests prior to commencing clinical trials, the start of our clinical trials may be delayed. For instance, in May 2023, we announced that FDA had placed a clinical hold on our planned Phase 1 FREEDOM clinical trial of PGN-EDODM1 in the U.S. We submitted a response to the FDA and in October 2023, we announced that the FDA had lifted the clinical hold, allowing us to initiate FREEDOM in the U.S. However, in March 2026, we announced that we received notice of a partial clinical hold from the FDA based on previously submitted preclinical pharmacology and toxicology studies, and we are conducting, and remain actively engaged with FDA about, additional nonclinical mouse studies designed to generate data to respond to FDA feedback. We may not be able to resolve the issues related to such partial clinical hold in a timely manner, or at all, which may negatively impact the PGN-EDODM1 program. Furthermore, in the future, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial, including our FREEDOM2 study of PGN-EDODM1, or disagree with or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to CTAs in other countries, including Canada and countries in Europe. Moreover, regulatory authorities in other countries could request that we pause dosing or further enrollment in one or more of our ongoing studies based in whole or in part on a clinical hold or partial clinical hold in the U.S.

Commercialization of our product candidates will require preclinical and clinical development; regulatory approval; manufacturing supply, capacity and expertise; a commercial organization; and significant marketing efforts. The success of our current and future product candidates will depend on many factors, including the following:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- regulatory clearance to initiate clinical trials under INDs, CTAs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- successful initiation, enrollment and completion of clinical trials, including under the FDA's Good Clinical Practice, or GCP, Good Laboratory Practice, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our clinical trials that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations to the satisfaction of the applicable regulatory authorities;
- receipt of marketing approvals from applicable regulatory authorities, including the completion of any required post-marketing studies or trials;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for our product candidates;
- commercial launch of our product candidates, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of our product candidates, including method of administration, if and when approved, by patients, the medical community and third-party payors;
- effective competition with other therapies;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of our product candidates following marketing approval, including acceptable results from any post-approval studies or clinical trials agreed to by us or required by FDA or other regulatory authorities; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payors.

Many of these factors are beyond our control and if we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates, which would materially harm our business. If we are unable to advance our product candidates to clinical development or successfully complete clinical trials, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

If we experience delays or difficulties in the enrollment of eligible patients in clinical trials, our ability to complete clinical trials may be adversely impacted.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success particularly with respect to PGN-EDODM1, which is currently our only clinical development program. We may not be able to identify, recruit and enroll a sufficient number of eligible patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- perceived risks and benefits of novel unproven approaches;
- size of the patient population, in particular for rare diseases such as the diseases on which we are initially focused, and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease or disorder under investigation;
- proximity and availability of clinical trial sites for prospective patients, including the clearance by FDA of clinical sites in the U.S. for the FREEDOM2 study;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- ability to recruit clinical trial investigators of appropriate competencies and experience;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment; and
- other factors outside of our control, such as the potential effects of any epidemics, pandemics or health crisis.

Our inability to enroll a sufficient number of eligible patients for our clinical trials, including our ongoing FREEDOM2 trial for PGN-EDODM1, 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 rely on and expect to continue 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 eligible patients for our clinical trials, we may have difficulty maintaining patients in our clinical trials. Many of the patients who end up receiving placebo may perceive that they are not receiving the product candidate being tested, and they may decide to withdraw from our clinical trials to pursue other alternative therapies rather than continue the trial with the perception that they are receiving placebo. If we have difficulty enrolling or maintaining a sufficient number of patients to conduct our clinical trials, we may need to delay, limit or terminate clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

Our approach to the discovery and development of product candidates based on our EDO platform is unproven, and we may not be successful in our efforts to identify, discover or develop potential product candidates.

The success of our business depends upon our ability to identify, develop and commercialize products based on our proprietary EDO platform. Our current product candidates that have been developed through our EDO platform are peptide-conjugated oligonucleotides designed to have a disease-modifying impact on degenerative neuromuscular diseases.

Our lead product candidate is currently in clinical-stage development, while other past product candidate programs were in the research or preclinical stage of development. Thus, our approach to treating muscle disease is unproven; for example past efficacy and safety results observed in the PGN-EDODM1 program may not result in a favorable safety profile or efficacy results as future clinical studies readout. In the future, our research programs may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates and our potential

product candidates may be shown to have harmful side effects in preclinical in vitro experiments or in vivo animal model studies, or in future clinical studies. In addition, our potential product candidates may not show promising signals of therapeutic effect in such experiments or studies, as was the case with PGN-EDO51, or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval. Further, because all of our development programs are based on our EDO platform, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs. In addition, we may be negatively impacted by the decision of other companies to discontinue development of products using technology similar to our technology. For example, Avidity Biosciences, Inc. (owned by Novartis) recently announced that its DM1 candidate, del-desiran, failed to show a statistically significant improvement over placebo with respect to its primary endpoint, VHOT, in a Phase 3 pivotal trial. Moreover, during 2024, Sarepta Therapeutics, Inc., or Sarepta, announced that it was discontinuing its SRP-5051 peptide-linked phosphorodiamidate morpholino, or PMO, development program, based on the risk-benefit of the program, including some patients experiencing hypomagnesemia even after treatment with SRP-5051 was discontinued, feedback from the FDA, and the evolving therapeutic landscape for DMD.

We advanced two product candidates, PGN-EDO51 and PGN-EDODM1, into the clinic, and completed a Phase 1 trial of PGN-EDO51 in healthy volunteers. However, the positive results we observed in our preclinical studies and in the completed Phase 1 trial of PGN-EDO51 were not repeated at the same level in our CONNECT1 Phase 2 clinical trial, and we decided to discontinue development of PGN-EDO51. Accordingly, the results observed in our preclinical and Phase 1 clinical studies of PGN-EDODM1 may not be repeated in our ongoing Phase 2 clinical study. Moreover, regulatory authorities may disagree with the interpretation of data from our trials.

Although we are continuing to focus the majority of our efforts on the advancement of our program in DM1, our EDO platform may fail to yield additional product candidates for clinical development for a number of reasons, including those discussed in these risk factors. In addition:

- we may not be able to assemble sufficient resources to acquire or discover product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may be covered by third parties' patents or other intellectual property rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate may be too complex and difficult to navigate successfully or economically.

If we are unable to identify and discover suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

Drug development is a lengthy and expensive process, and preclinical and clinical testing is uncertain as to the outcome. We may encounter substantial delays in the commencement, enrollment or completion of our clinical trials and may never advance to clinical trials, or we may fail to demonstrate safety and effectiveness to the satisfaction of applicable regulatory authorities, which could prevent us from advancing or commercializing our product candidates on a timely basis, if at all.

The risk of failure in developing product candidates is high. It is impossible to predict when or if any product candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development, obtain regulatory clearance to commence clinical trials, and then conduct extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans. To date, we have completed a Phase 1 clinical trial for PGN-EDODM1, and are conducting a Phase 2 clinical trial for PGN-EDODM1.

Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses, and earlier results, both preclinical and clinical, may not be indicative of future clinical trial results. For example, in the case of PGN-EDO51, our product candidate for DMD, based on the levels of dystrophin protein measured in the 10 mg/kg cohort of CONNECT1,

despite a favorable emerging safety profile, we decided to voluntarily discontinue development of PGN-EDO51 and have substantially completed the wind-down of DMD-related research and development activities, including our research efforts in DMD relating to exons 44, 45 and 53. Even if our clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance, varying interpretations of clinical data or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support clearance of our INDs, CTAs and other similar regulatory filings. We cannot be certain if the outcome of our preclinical studies and clinical trials will ultimately support further development of our product candidates, as was the case with PGN-EDO51 or future programs, or future regulatory approval and commercialization. Although we have completed a Phase 1 trial and are conducting a Phase 2 clinical trial of our lead product candidate, PGN-EDODM1, we cannot be certain of the outcome of our preclinical testing, and clinical studies for PGN-EDODM1 or any of our other product candidates and cannot predict whether the FDA, or comparable foreign regulatory authorities will accept our proposed clinical program for PGN-EDODM1, or whether the outcome of our preclinical testing and clinical studies will ultimately support the further development of PGN-EDODM1 or any other product candidates we may develop. Conducting preclinical testing is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity and novelty of the program, and often can be several years or more per program. In addition, the progress and timing of our preclinical studies, including pharmacology and toxicology studies, may be impacted by the limited supply of NHPs needed for such studies. As a result, we cannot be sure that we will be able to submit INDs, CTAs and other similar regulatory filings for our programs on the timelines we expect, if at all, and we cannot be sure that submission of such regulatory filings will result in the FDA, competent authorities of the EU member states, or comparable foreign regulatory authorities allowing clinical trials to begin, including in the case of our FREEDOM2 clinical study for PGN-EDODM1. For example, in March 2026, we announced that we received notice of a partial clinical hold from the FDA based on previously submitted preclinical pharmacology and toxicology studies. We are committed to working with the FDA to address their questions; however, we may not be able to resolve the issues related to such partial clinical hold in a timely manner, or at all, which may negatively impact the PGN-EDODM1 program. Furthermore, in May 2023, we announced that we received a clinical hold notice from the FDA regarding our IND application to initiate our Phase 1 FREEDOM study, and in June 2023, we provided an update on our plans with respect to this program. In October 2023, we announced that the FDA lifted the clinical hold on our Phase 1 FREEDOM study, allowing this study to proceed in the U.S.

Furthermore, product candidates are subject to continued preclinical safety studies, which may be conducted concurrently with our clinical testing. The outcomes of these safety studies may delay the launch of, or enrollment in clinical trials and could impact our ability to continue to conduct our clinical trials.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. The leadership changes at the FDA in the current administration may compound this uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in trial design, dose selection issues, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or IRB, or independent ethics committee approval, or the equivalent review groups for sites outside the U.S., at each clinical trial site;
- failure of the FDA to remove the partial clinical hold on PGN-EDODM1 or the imposition of further clinical holds by regulatory authorities as a result of serious adverse events, manufacturing concerns, or after an inspection of our clinical trial operations or trial sites;
- negative or inconclusive results observed in clinical trials, including failure to demonstrate statistical significance, which could lead us, or cause regulators to require us, to conduct additional clinical trials or abandon product development programs;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA’s GCPs or those of other regulatory authorities;
- failure by physicians to adhere to delivery protocols leading to variable results;

- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- failure of our third-party contractors to comply with regulatory requirements or to meet their contractual obligations to us in a timely manner, or at all;
- inability to recruit patients to participate in a clinical trial, including as a result of competition with other pharmaceutical and biotechnology companies and the patient population size for our product candidates;
- delays in having patients complete participation in a clinical trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events associated with a product candidate in development by another company, which are viewed to outweigh its potential benefits, and which may negatively impact the perception of our product due to a similarity in technology or approach;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the legal or regulatory regimes domestically or internationally related to patient rights and privacy;
- lack of adequate funding to continue the clinical trial; or
- lack of diminished revenue potential of the program(s) due to competition.

Clinical trials must be conducted in accordance with the FDA and other applicable regulatory authorities' legal requirements, regulations or guidelines, and are subject to oversight by these governmental agencies and IRBs or ethics committees at the medical institutions where the clinical trials are conducted. We could encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

In addition, disruptions caused by the effects of any epidemics or pandemics may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting or completing our planned and ongoing clinical trials.

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

Any inability to successfully complete preclinical studies and clinical trials, including in the case of the PGN-EDODM1 program, could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Any such changes could cause our product candidate to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using an altered process or formulation, and limit our ability to rely on data from clinical trials conducted with an earlier version of our product candidate. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Further, conducting clinical trials in foreign countries, as we plan to continue to do for our product candidates, including PGN-EDODM1, 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, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

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

- be delayed in obtaining marketing approval for product candidates, if 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 changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a Risk Evaluation and Mitigation Strategy, or REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

In particular, each of the conditions for which we plan to develop or are developing product candidates is a rare genetic disease with limited patient pools from which to draw for clinical trials. Further, because it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies or to try alternative therapies. Finally, we must compete with other companies with either approved therapies or investigational therapies in development for the conditions which we are developing product candidates for, which may further limit the pool of potential patients.

The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.

We are in the early stages of our programs and have completed a Phase 1 clinical trial and are conducting a Phase 2 clinical trial for our PGN-EDODM1 product candidate. We reported data from three cohorts of our Phase 1 trial, and from two cohorts of our Phase 2 trial of PGN-EDODM1, but we have not completed IND- or CTA-enabling activities or advanced any other product candidates (except PGN-EDO51) into clinical development. As a result, our belief in the capabilities of our platform is based on early research, preclinical studies, and early data from our early-stage studies and clinical trials. However, the results of preclinical studies may not be predictive of the results of later preclinical studies or clinical trials, and the results of any early-stage clinical trials may not be predictive of the results of later clinical trials. For example, since PGN-EDO51 did not achieve target dystrophin levels in the CONNECT1 trial, we made the decision to discontinue development of our DMD programs.

We have reported single-dose data for all of the planned cohorts (5, 10 and 15 mg/kg) in the Phase 1 FREEDOM study. While we observed robust dose-dependent mean splicing correction at the 5, 10 and 15 mg/kg dose level in the FREEDOM study, we observed limited change in functional outcomes in all cohorts of the Phase 1 FREEDOM study after a single dose as well as in the lowest dose (5 mg/kg) of the multi-dose Phase 2 FREEDOM2 study. We believe robust splicing correction with PGN-EDODM1 has the potential to lead to meaningful functional improvements with repeat dosing over time, as was the case in certain of the functional endpoints in the 10 mg/kg cohort of FREEDOM2; however, any such improvements may not be sustained or observed in future larger clinical studies or may not be considered clinically meaningful. Any determination in the future that PGN-EDODM1 may not yield sufficient functional improvement would harm our business, financial condition, results of operations and prospects.

Additionally, our planned clinical trials may utilize an “open-label” trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at

different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with any of our product candidates when studied in a controlled environment with a placebo or active control.

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

From time to time, we may publicly disclose preliminary or topline data from our preclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. The topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data and preliminary results should be viewed with caution until the final data are available. For example, based on preclinical, HV and early data in patients, we believed that with a longer treatment period and higher doses of PGN-EDO51, we could expect to see higher levels of exon skipped transcript potentially resulting in significant increases in dystrophin in our CONNECT1 Phase 2 trial. However, the data from the 10 mg/kg cohort of CONNECT1 demonstrated that PGN-EDO51 did not achieve target dystrophin levels, and we made the decision to discontinue development of our DMD programs.

From time to time, we may also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects. Further, disclosure of interim data could result in volatility in the price of our common stock.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is 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.

If PGN-EDODM1, or any of our future product candidates, cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent the initiation or completion of clinical trials, prejudice regulatory approval, limit the commercial potential of such candidate or result in significant negative consequences following any potential marketing approval.

Undesirable side effects caused by PGN-EDODM1, or future product candidates, could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities. For instance, in March 2026, we announced that we received notice of a partial clinical hold from the FDA based on previously submitted preclinical pharmacology and toxicology studies. Although other oligonucleotide therapeutics have received regulatory approval, ours is a novel approach to oligonucleotide therapy. As a result, there is uncertainty as to the safety profile of our product candidates compared to more well-established classes of therapies, or oligonucleotide therapeutics on their own. Moreover, there have been only a limited number of clinical trials involving the use of peptide conjugated oligonucleotide therapeutics or our proprietary technology used in our EDO platform.

For example, following the unblinding of the single-ascending dose portion of the FREEDOM trial, analysis of a kidney safety composite measure biomarker was conducted, and, at the 10 and 15 mg/kg dose levels, transient biomarkers associated with tubular insult were noted, as well as the observation of a DLT being identified at the 15 mg/kg dose level. Furthermore, we continued to

observe transient increases in albuminuria in certain patients in each of the 5 mg/kg and 10 mg/kg dose cohorts of the FREEDOM2 study. Although these laboratory findings resolved without medical treatment or intervention and were not associated with clinical symptoms, the clinical significance of these findings is not yet fully understood. As we continue to dose patients in the 12.5 mg/kg cohort of the FREEDOM2 study as well as in the Open Label Extension study, we are monitoring safety results, including continuing to note transient increases in certain kidney function biomarkers as well as moderate decreases in certain electrolyte measures, with a goal of determining the optimal dose for continued development including for a future pivotal trial. Accordingly, we may observe additional renal effects, including more pronounced or persistent laboratory abnormalities, clinically symptomatic renal events, or other safety signals as the study continues. Given the stage of clinical development of PGN-EDODM1, there can be no assurance that PGN-EDODM1 will not cause undesirable side effects in patients with further dosing and dose escalation, including, for example, safety events similar to those observed in our studies with PGN-EDO51. If such events occur, we may be required to modify, suspend, or terminate clinical trials, reduce dosing, implement additional monitoring, or conduct additional preclinical studies, which could delay development, increase costs, or adversely affect the commercial prospects of PGN-EDODM1.

Additionally, in our Phase 1 and Phase 2 clinical trials of PGN-EDO51, we observed mild, transient, reversible changes in kidney biomarkers that resolved without intervention in all but one participant who experienced a non-life threatening serious adverse event and transient mild to moderate hypomagnesemia that resolved without intervention or with low-dose oral magnesium supplementation. One participant experienced a reduction of his estimated glomerular filtration rate, or eGFR, that a subsequent nuclear scan indicated was in the normal range. There were no treatment-related SAEs, and all treatment-related adverse events were mild. Despite what we believed to be a favorable emerging safety profile for PGN-EDO51, in May 2025, following a review of the dystrophin data from the 10 mg/kg cohort, we made the voluntary decision to cease development of PGN-EDO51.

We may also observe additional safety or tolerability issues in ongoing or future clinical trials as we test our product candidates in larger populations, over longer durations, or with different dosing regimens. Historically, many third-party product candidates that initially showed promise in early-stage testing were later found to cause side effects that prevented further clinical development. It is possible that, if the use of our product candidates becomes more widespread following any regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. If such side effects become known later in development or upon approval, if any, such findings may harm our business, financial condition, results of operations and prospects significantly.

In addition, if our product candidates receive marketing approval, and we or others later identify undesirable side effects caused by treatment with such drug, a number of potentially significant negative consequences could result, including:

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

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

We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, 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 managerial resources, we are focusing the majority of our resources on one product candidate, PGN-EDODM1. As a result, we are delaying pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. 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 research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that 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. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Social media is increasingly being used to communicate about pharmaceutical companies' clinical development activities, and we intend to utilize appropriate social media in connection with our development efforts. Additionally, patients may use social media channels to comment on their experience in an ongoing blinded clinical trial or to report an alleged adverse event. If such disclosures occur in the future in connection with any of our sponsored clinical trials, there is a risk that trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product candidates. There is also a risk of inappropriate disclosure of sensitive or confidential information or negative or inaccurate posts or comments about us on any social networking website. In addition, we may encounter attacks on social media regarding our company, management or our product candidates, and fraudsters could and have attempted to illegally use our name on social media platforms to defraud the public. It is also possible for individuals or groups to target companies with disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide access to any of our product candidates under an expanded access policy, our reputation may be negatively affected and our business may be harmed. If any of these events were to occur or we fail to comply with applicable regulations, we could incur liability, face regulatory actions or incur other harm to our business.

Clinical trial and product liability lawsuits against us could divert our resources, could cause us to incur substantial liabilities and could limit commercialization of our product candidates.

We face an inherent risk of clinical trial and product liability exposure related to the testing of product candidates that have entered or eventually proceed to clinical trials, and we will face an even greater risk if we commercially sell any products that receive marketing approval. While we currently have only one product candidate in clinical development and none that have been approved for commercial sale, the future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we may incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our product candidates;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize our product candidates.

We have insurance coverage in place that we believe to be appropriate for our current phase of clinical development, but we may need to further increase this coverage for subsequent clinical trials, or if we commence commercialization of any product

candidates. 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. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

We have conducted, and may in the future conduct, certain clinical trials for our product candidates outside of the U.S. However, the FDA and comparable foreign regulatory authorities may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We intend to conduct clinical trials for our product candidates outside the U.S. such as FREEDOM and FREEDOM2 clinical trials, each of which had sites in multiple countries outside of the U.S. Currently, there are no U.S. sites opened in the FREEDOM2 due to the partial clinical hold of the FREEDOM2 trial in the U.S. Although the FDA may accept data from clinical trials conducted outside the U.S., acceptance of this data is subject to certain conditions imposed by the FDA. Where data from foreign clinical trials are intended to serve as the basis for marketing approval in the U.S., the FDA will not approve the application on the basis of foreign data alone unless those data are applicable to the U.S. population and U.S. medical practice; the studies were performed by clinical investigators of recognized competence; and the data are considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. If access to clinical trial sites or documentation is delayed or restricted, including for geopolitical reasons, the FDA may not accept such data in support of a marketing application which would adversely affect our development and approval plans. For studies that are conducted only at sites outside of the U.S. and not subject to an IND, the FDA generally does not provide advance comment on the clinical protocols for the studies, and therefore there is an additional potential risk that the FDA could determine that the study design or protocol for a non-U.S. clinical trial was inadequate, and require us to conduct additional clinical trials. In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. There can be no assurance the FDA will accept data from clinical trials conducted outside of the U.S. Recently, the FDA has 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. If the FDA does not accept data from our clinical trials of our product candidates, we would likely need to conduct additional clinical trials, which would be costly and time consuming and delay or permanently halt our development of our product candidates. Additionally, 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.

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

- additional foreign regulatory requirements;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- cultural differences in medical practice and clinical research;
- geopolitical risks; and
- diminished protection of intellectual property in some countries.

Risks Related to Our Dependence on Third Parties

We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily. If we need to replace one or more of these third parties, our development plans may be significantly delayed and we may expend more funds than currently planned.

We do not expect to independently conduct all aspects of our product manufacturing, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to many of these items, including CDMOs, for the manufacturing of any product candidates we test in preclinical or clinical development, as well as CROs for the conduct of our animal testing and research and for the conduct of our current and planned clinical trials. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities and significantly increase our expenses, which could adversely affect our business.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, we will remain responsible for ensuring that each of our IND- and CTA-enabling studies and clinical trials are conducted in accordance with the study plan and protocols. 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 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, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under conditions that comply with the FDA's current Good Manufacturing Practices, or cGMPs. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

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

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

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If the CROs and other third parties do not perform preclinical studies and clinical trials in a satisfactory manner, if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, or if they breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

If third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future IND, CTA and other similar regulatory filings and potential approval of our product candidates.

In addition, there are few CDMOs that have the capability to manufacture oligonucleotides and peptides, the key intermediates in the synthesis of the final active pharmaceutical ingredient, both of which are critical to the development and production of our product candidates. We are aware that one or more of our competitors are using either oligonucleotides, peptides, or both in their product candidates, and that they have engaged with many of these CDMOs, which may hinder our ability to also contract with those CDMOs. As a result, we may have difficulty finding and engaging sufficient third-party manufacturers to develop and manufacture our product candidates, which may affect our ability to conduct preclinical studies and clinical trials. Moreover, the BIOSECURE Act, which was enacted in December 2025 and is described in the risk factors below, could restrict U.S. government procurement from, and funding to entities that use the biotechnology equipment or services of, designated "biotechnology companies of concern," which could include certain of our vendors. If certain of these vendors are unable to continue to provide services or we are unable to contract with such vendors, this could further constrain our ability to find and engage third-party manufacturers to develop and manufacture our product candidates, which could adversely affect our business.

We currently depend on a small number of third-party suppliers to supply the product candidates that we are evaluating in our research and development programs. The loss of these or future third-party suppliers, or their inability to provide us with sufficient supply, could harm our business.

We do not own or operate manufacturing facilities and have no current plans to develop our own clinical or commercial-scale manufacturing capabilities. We rely on a small number of third-party suppliers for the manufacture and testing of the product candidates that we are evaluating in our research and development programs, including PGN-EDODM1. We expect to continue to depend on third-party suppliers for the manufacture of any product candidates we advance into preclinical and clinical development, as well as for commercial manufacture if those product candidates receive marketing approval. The facilities used by third-party manufacturers to manufacture our product candidates must be approved by the FDA, the EMA and any comparable foreign regulatory authority pursuant to inspections that will be conducted after we submit an NDA to the FDA or any comparable filing to the EMA or other foreign regulatory authority. We do not control the manufacturing process of, and are completely dependent on, third-party manufacturers for compliance with cGMP requirements for the manufacture of products. If these third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA, the EMA or any comparable foreign regulatory authority, we may incur delays in our clinical trials or regulatory submissions, and they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. FDA accepted our participation in its CMC Development and Readiness Pilot Program, or CDRP, a voluntary program intended to facilitate expedited CMC development of investigational drugs and biologics that have accelerated clinical development timelines by increasing communication with the FDA on CMC development; if we are unable to expedite CMC development through the pilot program, or if FDA modifies or terminates the pilot program with respect to PepGen, our current development plans and anticipated timelines could be adversely affected.

In addition, we have no control over the ability of third-party manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA, the EMA or any comparable foreign regulatory authority 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, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

We may also seek to eventually establish our own manufacturing facility for the long-term commercial supply of our product candidates for which receive regulatory approval, if any. If we determine to establish our own manufacturing facility and manufacture our products on our own, we will need to obtain the resources and expertise in order to build such manufacturing capabilities and to conduct such manufacturing operations. In addition, our conduct of such manufacturing operations will be subject to the extensive regulations and operational risks to which our third-party suppliers are subject. If we are not successful in building these capabilities or complying with the regulations or otherwise operating our manufacturing function, our commercial supply could be disrupted and our business could be materially harmed.

Our or a third party's failure to execute on our manufacturing requirements on commercially reasonable terms and in compliance with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or conduct preclinical studies or clinical trials of product candidates;
- delays in initiating or completing preclinical studies or clinical trials of product candidates or in submitting regulatory applications, or receiving marketing approvals, for product candidates;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease development or to recall batches of product candidates; and
- in the event of approval to market and commercialize any product, an inability to meet commercial demands for the product.

We are party to manufacturing agreements with a number of third-party manufacturers. We may be unable to maintain these agreements or establish any additional agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to maintain or establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third party;

- failure to manufacture according to our specifications;
- failure to manufacture according to our schedule or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

We compete with third parties for access to manufacturing facilities, in particular for the manufacture of oligonucleotides. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

We do not currently have arrangements in place for redundant supply or a second source for all required raw materials. If our existing or future third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in supply. An alternative manufacturer would need to be qualified and authorized pursuant to a submission to our approved NDA or NDA supplement which could result in further delay. Further, we will also need to verify, such as through analytical comparability or clinical bridging studies, that any new or modified manufacturing processes will produce our product candidate according to the specifications previously submitted to the FDA, the EMA or comparable foreign regulatory authorities. The delays associated with the verification of a new third-party manufacturer could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a third-party manufacturer may possess technology related to the manufacture of our product candidate that such third-party manufacturer owns independently. This would increase our reliance on such third-party manufacturer or require us to obtain a license from such third-party manufacturer in order to have another third-party manufacturer manufacture our product candidates. We may be unsuccessful in demonstrating the comparability of clinical supplies to those previously allowed into clinical development by the FDA, the EMA or comparable foreign regulatory authorities which could require the conduct of additional studies or clinical trials.

Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines. These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Finally, in December 2025, the National Defense Authorization Act for Fiscal Year 2026, or the NDAA, was enacted, which includes Section 851, commonly referred to as the "BIOSECURE Act." Once the BIOSECURE Act's prohibitions take effect, U.S. government agencies will not be permitted to procure biotechnology equipment or services from OMB-designated "biotechnology companies of concern", or BCCs. Additionally, government agencies will be prohibited from entering into contracts with entities that provide biotechnology equipment or services from OMB-designated BCCs in the performance of their contract(s) with the government, and the same restrictions apply to federal loan or grant funds for such equipment or services. We currently rely on third-party contract manufacturing organizations and other vendors located outside the United States, including WuXi AppTec which operates in China and manufactures our active pharmaceutical ingredient, or API, for PGN-EDODM1. While none of our vendors are currently designated as BCCs, on June 8, 2026, the Department of Defense added WuXi AppTec to the Section 1260H list of Chinese military companies. Under the BIOSECURE Act, a company on the 1260H list is designated a BCC if OMB determines that it is involved in the manufacturing, distribution, provision or procurement of biotechnology equipment or services, and OMB is required to publish its initial list of BCCs by December 18, 2026. WuXi AppTec challenged its 1260H designation in the U.S. District Court for the District of Columbia, and on August 7, 2026, the court preliminarily enjoined the Department of Defense from giving effect to the designation while the litigation proceeds. The injunction is preliminary, and OMB, which is not a party to the litigation, may designate WuXi AppTec as a BCC under the BIOSECURE Act's separate OMB designation pathway, which does not depend on the 1260H list. We therefore cannot predict whether or when OMB will designate WuXi AppTec as a BCC, or the outcome of the litigation. If Wuxi AppTec or one of our other current or future vendors with which we work is designated as a BCC, or if any of our collaborators, customers, investors, or future commercial partners become subject to BIOSECURE-related restrictions as a result of their relationships with such vendors, we could be required to terminate or restructure existing arrangements, transition manufacturing or other services to alternative suppliers, or delay or suspend development activities, subject to a grace period of 5 years during which time a company may be able to continue to work with such a restricted vendor, depending on timing and how BIOSECURE will be implemented. Although we currently have ongoing workstreams to secure a secondary source supplier of our API, any such transition could involve significant cost, operational complexity, regulatory risk, and delays, and alternative suppliers may not be available on acceptable terms or at all.

Our current and anticipated future dependence upon third parties for the manufacture of any product candidates we develop may adversely affect our development programs and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We are dependent on single-source suppliers for some of the components and materials used in our product candidates.

Many of the suppliers for components and materials used in our product candidates, including the manufacturer for active pharmaceutical ingredient and finished product for PGN-EDODM1, are single-sourced. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes, active pharmaceutical ingredients and finished goods could expose us to several risks, including disruptions in supply, price increases or late deliveries. There are, in general, relatively few alternative sources of supply for substitute components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sales. Establishing additional or replacement suppliers for these components, materials and processes could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any single-source supplier or service provider could lead to supply delays or interruptions which would damage our business, financial condition, results of operations and prospects.

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

We may enter into collaborations with third parties for the research, development and commercialization of certain of our product candidates. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We may seek third-party collaborators for the research, development and commercialization of certain of our product candidates. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or our product candidates pose numerous risks to us, including the following:

- collaborators would have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or 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;
- collaborators may be acquired by a third party having competitive products or different priorities, causing the emphasis on our product development or commercialization program under such collaboration to be delayed, diminished or terminated;
- collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the affected product candidates; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this Form 10-Q apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement 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 several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

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

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

Some of our future collaborators could also become our competitors. Our collaborators could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, fail to devote sufficient resources to the development and commercialization of products, or merge with or be acquired by a third party who may do any of these things. Any of these developments could harm our product development efforts.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a 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 design or results of clinical trials, the likelihood of approval by the FDA, the EMA or comparable foreign regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product

candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

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.

We may not be able to negotiate 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 the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the product candidate.

We are dependent on third-party vendors to provide certain licenses, products and services and our business and operations, including clinical trials, could be disrupted by any problems with our significant third-party vendors.

We engage a number of third-party suppliers and service providers to supply critical goods and services, such as contract research services, contract manufacturing services and information technology services. Disruptions to the business, financial stability or operations of these suppliers and service providers, including due to strikes, labor disputes or other disruptions to the workforce, for instance, if, as a result of a pandemic or health epidemic, employees are not able to come to work, or to their willingness and ability to produce or deliver such products or provide such services in a manner that satisfies the requirements put forth by the authorities, or in a manner that satisfies our own requirements, could affect our ability to develop and market our future product candidates on a timely basis. If these suppliers and service providers were unable or unwilling to continue to provide their products or services in the manner expected, or at all, we could encounter difficulty finding alternative suppliers. Even if we are able to secure appropriate alternative suppliers in a timely manner, costs for such products or services could increase significantly. Any of these events could adversely affect our results of operations and our business.

Risks Related to Regulatory Approval and Other Regulatory and Legal Compliance Matters

As an organization, we have only completed or initiated a small number of clinical trials and may be unable to do so for any of our other product candidates nor carry out or complete further studies for our lead candidate.

We have no experience as a company in conducting, completing and managing the full suite of clinical trials necessary to obtain regulatory approvals, including approval by the FDA, the European Commission or comparable foreign regulatory authorities, or in obtaining approval of any of our product candidates. We are early in our development efforts for our product candidates. We have initiated a Phase 1 and a Phase 2 clinical trial for PGN-EDODM1. We will need to successfully complete preclinical activities and additional clinical trials, in order to obtain FDA, European Commission or comparable foreign regulatory approval to market PGN-EDODM1, and any future product candidates.

Based on our initial observations of the preclinical and early clinical data with respect to PGN-EDODM1, we believe PGN-EDODM1 has the potential to lead to meaningful functional improvements with repeat dosing at 10 mg/kg or higher levels over time. However, our belief based on this data may be erroneous. There can be no assurance that our expectations of robust splicing correction and improved functional outcomes will be reflected in continued clinical evaluation of PGN-EDODM1 in DM1 patients, including in our ongoing FREEDOM2 study.

Although we completed a Phase 1 clinical trial for PGN-EDO51, completed a Phase 1 clinical trial for PGN-EDODM1, and have a Phase 2 clinical trial ongoing for PGN-EDODM1, we have not completed any additional clinical trials, and have limited experience as a company in preparing, submitting and prosecuting regulatory filings. In addition, we have had limited interactions with the FDA, the EMA and comparable foreign regulatory authorities and cannot be certain how many clinical trials of PGN-EDODM1, or any other product candidates will be required or how such trials should be designed. The FDA has previously provided feedback on our clinical trials for PGN-EDODM1. We may be unable to efficiently execute and complete necessary clinical trials for our product candidates in a way that leads to regulatory submission and approval of any of our product candidates, including potentially any accelerated approval. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, our current or planned clinical trials, could prevent us from or delay us in submitting NDAs for and commercializing our product candidates.

We plan to seek approval from the FDA or comparable foreign regulatory authorities, where applicable, under accelerated approval pathways. We may fail to obtain approval under such accelerated approval pathways. Moreover, these pathways may not lead to a faster development, regulatory review or approval process and do not increase the likelihood that our product candidates will receive marketing approval.

We plan to seek accelerated approval, if applicable, under the FDA's accelerated approval pathway for our PGN-EDODM1 product candidate and may have a similar strategy with future product candidates. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the disease and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA likely would require that we perform adequate and well-controlled post-marketing clinical trials to confirm the product's clinical benefit. These confirmatory trials must be completed with due diligence. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is permitted to require, as appropriate, that a post-approval confirmatory study or studies be underway (including commencing enrollment) prior to approval or within a specified time period after the date of accelerated approval was granted. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such studies, including progress toward enrollment targets, and the FDA must promptly post this information publicly. FDORA also gives the FDA increased authority to withdraw approval of a drug or biologic granted accelerated approval on an expedited basis if the sponsor fails to conduct such studies in a timely manner, send the necessary updates to the FDA, or if such post-approval studies fail to verify the drug's predicted clinical benefit. Under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory study or submit timely reports to the agency on their progress.

In addition, the FDA currently requires, unless otherwise informed by the agency, pre-approval of promotional materials for products receiving accelerated approval, which could adversely impact the timing of the commercial launch of the product. Thus, even if we seek to utilize the accelerated approval pathway, we may not be able to obtain accelerated approval and, even if we do, we may not experience a faster development, regulatory review or approval process for that product. In addition, receiving accelerated approval does not assure that the product's accelerated approval will eventually be converted to a full approval.

In the EU, under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use may perform an accelerated assessment of a marketing authorization application. Applicants requesting an accelerated assessment procedure must justify that the product candidate is expected to be of major public health interest, particularly from the point of view of therapeutic innovation.

Prior to seeking accelerated approval for PGN-EDODM1, we intend to seek feedback from the FDA or similar foreign regulatory authorities and will otherwise evaluate our ability to seek and receive accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA or similar application for accelerated approval or any other form of expedited development or review. Similarly, there can be no assurance that after subsequent FDA or similar foreign regulatory authorities feedback we will continue to pursue or apply for accelerated approval or any other form of expedited development or review, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or other expedited development or review, there can be no assurance that such submission or application will be accepted or that any expedited development or review will be granted on a timely basis, or at all, depending upon the adequacy of surrogate endpoint data, the strength of evidence supporting clinical benefit, safety finds, dose optimization and overall benefit-risk assessment which may be further compromised by the fact that there may be newly approved therapeutic products on the market addressing DM1 at the time of submission of our application. The FDA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development or review for PGN-EDODM1 or any other product candidates would result in a longer time period to commercialization of such product candidate, would increase the cost of development of such product candidate, and would harm our competitive position in the marketplace.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of our product candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, our product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the U.S., the EMA and comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product

candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction.

We have no experience as a company in submitting and supporting the applications necessary to gain marketing approvals and may need to rely on third parties to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and effectiveness. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the U.S. and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Of the large number of products in development, only a small percentage successfully complete the FDA, EMA or foreign regulatory approval processes and are commercialized. Even if our product candidates demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. In February 2026, the former FDA Commissioner publicly indicated that a single adequate and well-controlled pivotal clinical trial supported by confirmatory evidence would be the FDA's default standard moving forward for novel products, rather than two such trials; this statement was not a formal agency action. In June 2026, FDA issued revised draft guidance clarifying how sponsors can rely on one scientifically rigorous adequate and well-controlled clinical investigation with confirmatory evidence to satisfy the statutory substantial evidence of effectiveness standard. Additionally, in July 2026, in response to congressional inquiries, FDA indicated that informal statements, issued through journals or means other than formal notice and comment rule-making, are not reflective of official FDA policy and should not be interpreted as regulatory guidance. FDA retains broad discretion to require additional clinical data for any product candidate, including a second adequate and well-controlled clinical trial. The FDA, the EMA and comparable foreign regulatory authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Moreover, 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 FDA's regulations, policies, and decisions may become subject to increasing legal challenges, delays, and/or changes. If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

The FDA also has substantial discretion in the approval process. The number and types of preclinical studies and clinical trials that will be required for NDA approval varies depending on the product candidate, the disease or the condition that the product candidate is designed to treat and the regulations applicable to any particular product candidate. Despite the time and expense associated with preclinical studies and clinical trials, failure can occur at any stage.

Clinical trial failure may result from a multitude of factors including flaws in trial design, dose selection, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits, and failure in clinical trials can occur at any stage. Companies in the drug development industry frequently suffer setbacks in the advancement of clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Based upon negative or inconclusive results, we may decide, or regulators may require us, to conduct additional clinical trials or preclinical studies. In addition, data obtained from clinical trials is susceptible to varying interpretations, and regulators may not interpret our data as favorably as we do, which may further delay, limit or prevent marketing approval.

The FDA or any foreign regulatory authority could delay, limit or deny approval of a product candidate for many reasons, including because the FDA or such other regulatory authority:

- may disagree with the design or implementation of our trials;
- may not deem a product candidate to be safe or effective for its intended uses;
- determines that the product candidate does not have an acceptable benefit-risk profile;

- may not agree that the data collected from preclinical studies and clinical trials are acceptable or sufficient to support the submission of an NDA or other submission or to obtain regulatory approval, and may impose requirements for additional preclinical studies or clinical trials;
- may determine that adverse events experienced by participants in our clinical trials represent an unacceptable level of risk;
- may determine that the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- may not accept clinical data from trials that are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the U.S.;
- may disagree regarding the formulation, labeling and/or specifications;
- may not approve the manufacturing processes associated with a product candidate or may determine that a manufacturing facility does not have an acceptable compliance status;
- may change approval policies or adopt new regulations; or
- may not file a submission due to, among other reasons, the content or formatting of the submission.

Even if we eventually complete clinical testing and receive approval of an NDA or foreign marketing application for any product candidates, the FDA, EMA or applicable foreign regulatory authority may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-market clinical trials. The FDA, EMA or the applicable foreign regulatory authority also may approve or authorize for marketing a product candidate for a more limited indication or patient population that we originally request, and the FDA, EMA 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 of these restrictions or commitments could render an approved product not commercially viable, which would materially adversely impact our business and prospects.

Obtaining and maintaining marketing approval or commercialization of our product candidates in the U.S. does not mean that we will be successful in obtaining marketing approval of our product candidates in other jurisdictions. Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell our product candidates in the European Union, or EU, and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the U.S. generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the U.S., it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the U.S. on a timely basis, if at all. Approval by the FDA does not ensure approval by the EMA or regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the U.S. does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. Furthermore, now that the U.K. is no longer part of the EU, a separate authorization is needed to market medicinal products in the U.K. We may not be able to file for marketing approvals and may not receive the necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Any delay in obtaining, or an inability to obtain, any marketing approvals would prevent us from commercializing any product candidates in the U.K. and/or the EU and/or other foreign jurisdictions and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the U.K. and/or the EU and/or other foreign jurisdictions for our product candidates, which could significantly and materially harm our business.

We may seek one or more designations or expedited programs for one or more of our product candidates, but we might not receive such designations or be allowed to proceed on expedited program pathways, and even if we do and proceed on such expedited program pathways in the future, such designations or expedited programs may not lead to a faster development or regulatory

review or approval process, and each designation does not increase the likelihood that any of our product candidates will receive marketing approval in the U.S.

We have received fast-track designation for PGN-EDODM1 for the treatment of DM1 and may seek fast-track designation for any other product candidates we develop. If a drug is intended for the treatment of a serious or life-threatening condition and nonclinical or clinical data for the drug demonstrates the potential to address an unmet medical need for such a condition, the drug 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 for any of our other product candidates. Even with fast-track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast-track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast-track designation alone does not guarantee qualification for the FDA's priority review procedures.

We may seek a breakthrough therapy designation for some of our product candidates. 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 existing 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 may also be eligible for priority review and accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

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

We may pursue orphan drug designation for certain of our product candidates, and we may not be able to obtain such designation, or obtain or maintain the benefits of such designation including orphan drug exclusivity, and even if we do, that exclusivity may not prevent regulatory authorities from approving other competing products.

In September 2023, the FDA granted orphan drug designation to PGN-EDODM1 for the treatment of DM1. We may seek orphan drug designation for any other product candidates we develop; however, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. Orphan drug designation must be requested before submitting an NDA. A similar regulatory scheme governs orphan products in the EU.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. In addition, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same product for the same approved use or indication for seven years.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same approved use or indication. In addition, even after an orphan

drug is approved, the FDA can subsequently approve the same product for the same approved use or indication if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition. Further, even if we obtain orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products.

The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A marketing application for a product candidate with RPDD, if approved, may not meet the eligibility criteria for a Priority Review Voucher, or PRV, or the RPDD program may sunset before the FDA is able to consider eligibility for a voucher.

Designation of a drug as a product for a rare pediatric disease does not guarantee that a marketing application for such drug will meet the eligibility criteria for a rare pediatric disease PRV at the time the application is approved. Even if we receive RPDD from the FDA for one of our product candidates, we would need to request a rare pediatric disease PRV in the marketing application of that product candidate. The FDA may determine that any such marketing application, if approved, does not meet the eligibility criteria for a PRV. Under current law, after September 30, 2029, the FDA may not award any rare pediatric disease priority review vouchers, although the FDA's authority to do so could be extended by Congress in the future.

We may seek designation for our EDO platform as a designated platform technology, but we might not receive such designation, and even if we do, such designation may not lead to a faster regulatory review or approval process.

We may seek designation for our EDO platform as a designated platform technology. Under FDORA, a platform technology incorporated within or utilized by a drug or biological product is eligible for designation as a designated platform technology if (1) the platform technology is incorporated in, or utilized by, a drug approved under an NDA; (2) preliminary evidence submitted by the sponsor of the approved or licensed drug, or a sponsor that has been granted a right of reference to data submitted in the application for such drug, demonstrates that the platform technology has the potential to be incorporated in, or utilized by, more than one drug without an adverse effect on quality, manufacturing, or safety; and (3) data or information submitted by the applicable person indicates that incorporation or utilization of the platform technology has a reasonable likelihood to bring significant efficiencies to the drug development or manufacturing process and to the review process. A sponsor may request the FDA to designate a platform technology as a designated platform technology concurrently with, or at any time after, submission of an IND application for a drug that incorporates or utilizes the platform technology that is the subject of the request. If so designated, the FDA may expedite the development and review of any subsequent original NDA for a drug that uses or incorporates the platform technology. Even if we believe our EDO platform meets the criteria for such designation, the FDA may disagree and instead determine not to grant such designation. In addition, the receipt of such designation for a platform technology does not ensure that a drug will be developed more quickly or receive FDA approval. Moreover, the FDA may revoke a designation if the FDA determines that a designated platform technology no longer meets the criteria for such designation. For example, in July 2025, the FDA revoked Sarepta Therapeutics' platform technology designation for AAVrh74 given new safety information suggested the preliminary evidence on which the designation was based was insufficient to demonstrate that its platform technology had the potential to be incorporated in, or utilized by, more than one drug without an adverse effect on safety.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to post-market study requirements, marketing and labeling restrictions, and even recall or market withdrawal if unanticipated safety issues are discovered following approval. In addition, we may be subject to penalties or other enforcement action if we fail to comply with regulatory requirements.

The FDA, the EMA or a comparable foreign regulatory authority may not approve our current product candidate or any new product candidates derived from our platform. However, if the FDA, EMA or comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and record keeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, conformance with applicable product tracking and tracing requirements, establishment registration and listing, as well as continued compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval. Any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain

requirements for potentially costly post-marketing studies, and surveillance to monitor the safety and efficacy of the product. Additionally, sponsors of approved drugs and biologics must provide six months' notice to the FDA of any changes in marketing status, or for discontinuing or interrupting supply of certain drugs, including the withdrawal of a drug, and failure to do so could result in a letter citing such failure to comply and public posting of such letter and redacted company response which could damage the company's reputation. Later discovery of previously unknown problems with a product, including adverse events 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 the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on product distribution or use, or requirements to conduct post-marketing studies or clinical trials;
- fines, warning letters or other regulatory enforcement action;
- refusal by the FDA, the EMA or comparable foreign regulatory authorities to approve pending applications or supplements to approved applications filed by us;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

In addition, the FDA's, EMA's and other foreign regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Any product candidate for which we obtain marketing approval will be subject to restrictions, such as the laws and regulations prohibiting the promotion of off-label uses, or may need to be withdrawn 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 medicines, when and if any of them are approved.

The FDA, EMA and other foreign regulatory authorities 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. The FDA, EMA and other foreign regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use. In particular, a product may not be promoted for uses that are not approved by the FDA, EMA and other foreign regulatory authorities as reflected in the product's approved labeling. If we receive marketing approval for a product candidate, physicians may nevertheless prescribe it to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, 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. Violation of the Federal Food, Drug, and Cosmetic Act, or FDCA, 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 and state consumer protection laws. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. Further, FDA's Office of Prescription Drug Promotion ("OPDP") actively scrutinizes promotional communications, including digital and social media; any materials that are false, misleading or promote unapproved uses can lead to enforcement actions and could necessitate corrective communications. The government has also required companies to enter into consent decrees and/or imposed permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

In addition, later discovery of previously unknown problems with our medicines, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such medicines, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a medicine;

- restrictions on the distribution or use of a medicine;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of medicines;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our medicines;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

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.

Additionally, if our current product candidate or any new product candidates we develop receive marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to healthcare practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

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

We and our contract manufacturers are subject to significant regulation. The manufacturing facilities on which we rely may not continue to meet regulatory requirements, which could materially harm our business.

All entities involved in the preparation of product candidates for clinical trials or commercial sale, including any contract manufacturers, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP regulations. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of an NDA on a timely basis and must adhere to the FDA's cGMP regulations enforced through its facilities inspection program. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit any of our future manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable

regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product, or revocation of a pre-existing approval. Any such consequence would severely harm our business, financial condition and results of operations.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur significant costs.

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

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws, regulations and permitting requirements. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption. Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could in turn have a material adverse effect on our business, financial condition, results of operations and prospects.

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

The current U.S. administration is focused on reducing costs of the federal government generally, including significantly reducing the number of government employees. Without appropriate staffing levels or the appropriation of additional funding to federal agencies, our business operations related to our product development activities for the U.S. market could be impacted. The ability of the FDA to review and approve proposed clinical studies and new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, leadership, and policy changes, the FDA's ability to hire and retain leadership and key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other federal agencies, including substantial leadership departures, personnel cuts, and policy changes, may also slow the time necessary for proposed clinical studies to obtain clearance or new drugs to be reviewed and/or approved, which could harm our business. Changes and cuts in FDA staffing have been reported as resulting in delays in the FDA's

responsiveness or in its ability to review submissions or marketing applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all.

A prolonged government shutdown, such as took place in October 2025, or significant leadership, personnel, and/or policy changes, or other substantial modification in agency activities (including due to global health concerns or geopolitical factors) could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, government funding of other agencies on which our operations may rely, including those that fund research and development activities and clinical trials, is subject to the political process, which is inherently fluid and unpredictable. Disruptions at agencies that fund our research and development activities and our clinical trials, or changes to such agencies' budgets, may negatively impact our operations and ongoing clinical trials and may limit our ability to seek additional funding in the future. Future shutdowns or other disruptions could also affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary, and our ability to access the public markets.

With the current administration, there have been numerous legislative and regulatory changes, and there continues to be substantial uncertainty as to the extent and manner in which the Trump administration will continue to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates and any products for which we obtain approval. This uncertainty could present new challenges and/or opportunities as we navigate development and approval of our product candidates. Additionally, the administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment in which to pursue the development of new therapeutic candidates. Also, state governments may seek to address or react to changes at the federal level with changes to their regulatory frameworks in a manner that could impact our operations.

Our relationships with healthcare providers, physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates that we develop for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, market, sell and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal False Claims Act and Physician Payments Sunshine Act and regulations.

Additionally, we are subject to state and foreign equivalents of each of these healthcare laws and regulations, among others, some of which may be broader in scope and may apply regardless of the payor. Many U.S. states have adopted laws similar to the federal Anti-Kickback Statute and False Claims Act, and may apply to our business practices, including, but not limited to, research, distribution, sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental payors, including private insurers. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state and require the registration of pharmaceutical sales representatives. For additional information, see "*Business—Government Regulation—Healthcare Regulation—Other Healthcare Laws*" in our Annual Report on Form 10-K filed with the SEC for the year-ended December 31, 2025.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the EU and U.K. The provision of benefits or advantages to induce or reward improper performance generally is typically governed by the national anti-bribery laws of European Union Member States, and the Bribery Act 2010 in the U.K. Infringement of these laws could result in substantial fines and imprisonment. EU Directive 2001/83/EC, which is the EU Directive governing medicinal products for human use, further provides that, where medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages or benefits in kind may be supplied, offered or promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. This provision has been transposed into the Human Medicines Regulations 2012 and so remains applicable in the U.K. despite its departure from the EU.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional

organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The scope and enforcement of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. We have entered into consulting and scientific advisory board arrangements with physicians and other healthcare providers, including some who could influence the use of our product candidates, if approved. Compensation under some of these arrangements includes the provision of stock or stock options in addition to cash consideration. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including administrative, civil and criminal penalties, damages, fines, disgorgement, the exclusion from participation in federal and state healthcare programs, reputational harm, and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Further, defending against any such actions can be costly and time consuming, and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and individual imprisonment. If any of the above occur, our ability to operate our business and our results of operations could be adversely affected.

Healthcare legislative reform discourse and potential or enacted measures may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. In both the U.S. and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and in additional downward pressure on the price that we, or any collaborators, may receive for any approved products. For additional information, see "*Business—Government Regulation—Healthcare Regulation—Healthcare Reform and Legislative Updates*" in our Annual Report on Form 10-K filed with the SEC for the year-ended December 31, 2025.

In addition, the Trump Administration and Congress are pursuing a multi-pronged strategy to reduce U.S. drug prices through most-favored-nation ("MFN") pricing, which would tie U.S. drug prices to the lowest prices paid in comparable developed countries. This includes the May 12, 2025 Executive Order directing HHS to communicate MFN price targets to manufacturers, voluntary MFN pricing agreements entered into between the Administration and all 17 targeted manufacturers (which include commitments to Medicaid price parity, MFN pricing on new product launches, and participation in TrumpRx.gov in exchange for tariff relief and regulatory benefits), CMS's proposed mandatory GLOBE (Medicare Part B) and GUARD (Medicare Part D) payment models that would impose incremental rebate obligations on manufacturers of qualifying single-source drugs, and a voluntary Medicaid MFN model (GENEROUS). As finalized, GLOBE is narrower than proposed, including by excluding drugs that have orphan designation and whose approved indications are all for rare diseases or conditions, plasma-derived products and certain cell and gene therapies, and CMS has stated that it intends to waive mandatory participation for any calendar quarter in which a manufacturer participates in GENEROUS. On October 7, 2026, the Pharmaceutical Research and Manufacturers of America filed suit in the U.S. District Court for the District of Columbia challenging GLOBE as unlawful, beyond CMS's statutory authority and unconstitutional, and we cannot predict the outcome of that litigation or whether GLOBE will take effect on its current timeline. Legislative proposals, including the Most Favored Patient Act introduced in March 2026, seek to codify these initiatives into federal law. It is unclear whether and to what extent these initiatives will be implemented or survive legal challenge, and a similar MFN model finalized during the first Trump Administration was halted by federal courts and subsequently rescinded for procedural reasons; however, MFN pricing policy represents a significant and evolving uncertainty that could materially adversely affect our pricing strategy, revenue, and ability to commercialize our product candidates, if approved.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

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

We expect that other healthcare reform measures may be adopted in the future, which may result in additional reductions in Medicare and other healthcare funding, proposals to set pharmaceutical pricing based on a "most favored nation" approach, more rigorous coverage criteria, lower reimbursement, and new payment methodologies. This could lower the price that we receive for our products. Any denial in coverage or reduction in reimbursement from Medicare or other government-funded programs may result in a similar denial or reduction in payments from private payors, which may prevent us from being able to generate sufficient revenue, attain profitability or commercialize our products. It is not clear how other future potential changes to the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, each as amended, will change the reimbursement model and market outlook for our current and future product candidates.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants and partners, and in our clinical trials, our principal investigators. Misconduct by these parties could include intentional or unintentional failures to comply with FDA regulations or the regulations applicable in the EU and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA, the European Commission and other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the U.S. and require us to develop and implement costly compliance programs.

We are subject to numerous laws and regulations in each jurisdiction outside the U.S. in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing the provision of money or anything of value, directly or indirectly through parties, to any foreign official, official of a public international organization, or political party official or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the U.S. to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA and other anti-corruption laws potentially applicable to our business is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, compliance with the FCPA and other anti-corruption laws presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials.

Various U.S. export and sanctions laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., or the sharing with certain non-U.S. nationals, of certain products and technical data relating to those products. Furthermore, such export and sanctions laws include restrictions or prohibitions on the sale or supply of certain products and services to U.S. embargoed countries or sanctioned countries, governments, persons and entities. Our expansion outside of the U.S. has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the U.S., which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA and export and sanctions laws can result in significant civil and criminal penalties, imprisonment, the loss of export or import privileges, debarment, breach of contract and fraud litigation, reputational harm, and other consequences. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We are subject to stringent data protection, privacy, and security laws, regulations, standards and contractual obligations and actual or perceived failure to comply with such requirements could have a material adverse effect on our business, financial condition, results of operations or prospects.

We are subject to data privacy and protection laws, regulations, policies, standards and contractual obligations that impose certain requirements relating to the collection, transmission, storage and use of personal information. The legislative and regulatory landscape for data privacy and protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues. Actual or perceived failure to comply with laws and regulations governing personal information could result in government investigations and enforcement actions against us, fines, claims for damages by affected third parties, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of personal information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer or other processing of personal data, including personal health data, of individuals in the European Economic Area, or EEA, is subject to the EU General Data Protection Regulation, or EU GDPR, as well as national data protection laws in effect in the member states of the EEA, and similar processing of personal data regarding individuals in the U.K. is governed by the U.K. General Data Protection Regulation, or U.K. GDPR and the U.K. Data Protection Act 2018. In this document, "GDPR" refers to both the EU GDPR and the U.K. GDPR, unless specified otherwise. The GDPR imposes stringent requirements on companies that process personal data, including requirements relating to having a legal basis for processing personal data, stricter requirements relating to the processing of sensitive data (such as health data), where required by GDPR obtaining consent of the individuals to whom the personal data relates, establishing a legal basis for processing, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data that requires the adoption of administrative, physical and technical safeguards to protect such information, providing notification of data breaches to appropriate data protection authorities or data subjects, requiring data protection impact assessments for high risk processing and establishing means for data subjects to exercise rights in relation to their personal data and taking certain measures when engaging third-party processors. The GDPR's definition of personal data includes coded data. Failure to comply with the requirements of the

GDPR may result in warning letters, mandatory audits, orders to cease/change the use of data, and financial penalties, including fines, which can be up to 4% of global revenues or €20 million (£17.5 million for the U.K.), 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.

Among other requirements, the GDPR includes restrictions on cross-border transfers of personal data subject to the GDPR to countries outside the EEA/U.K. that have not been found to provide adequate protection to such personal data (third countries), unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, certification to the EU-U.S. Data Privacy Framework (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the framework) and the U.K. International Data Transfer Agreement/Addendum, or U.K. IDTA) has been put in place. Where relying on the SCCs /U.K. IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA/U.K. data protection regimes will require significant effort and cost and may result in us needing to make strategic considerations around where EEA/U.K. personal data is transferred and which service providers we can utilize for the processing of EEA/U.K. personal data. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the SCCs cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results. Switzerland has also adopted similar restrictions on transfer of personal data outside of its borders. Although the U.K. is regarded as a third country under the EU's GDPR, the European Commission has adopted an (adequacy decision) in favor of the U.K., a decision recognizing the U.K. as providing adequate protection under the EU GDPR and enabling data transfers from EU Member States to the U.K. without additional safeguards.

If we are unable to implement a valid solution for personal data transfers from the EEA, U.K. or Switzerland to the U.S. or other countries, we will face increased exposure to regulatory actions, substantial fines and injunctions against processing personal data in those jurisdictions. Inability to import personal data from the EEA, U.K. or Switzerland may also restrict our clinical trials activities in those jurisdictions; limit our ability to collaborate with contract research organizations as well as other service providers, contractors and other companies subject to data protection laws in those jurisdictions; and require us to increase our data processing capabilities in those jurisdictions at significant expense. Additionally, other countries outside of the EEA, U.K., and Switzerland have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business.

In December 2025, the European Commission adopted a decision determining that the U.K. continues to offer a level of data protection that is "essentially equivalent" to the EU standards, extending the validity of the U.K. adequacy decision for six years, until December 2031. This follows the U.K.'s adoption of the Data (Use and Access) Act 2025, or the DUAA, on June 19, 2025. While this renewal reduces immediate adequacy concerns, it is unclear how U.K. data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the U.K. will be regulated in the long term. Although the EU GDPR and the U.K. GDPR currently impose substantially similar obligations it is possible that over time the respective provisions, interpretations and enforcement of the EU GDPR and U.K. GDPR may further diverge in the future and create additional regulatory challenges and uncertainties. The lack of clarity on future U.K. laws and regulations and their interaction with EU laws and regulations could add legal risk, uncertainty, complexity and cost to our handling of U.K. and EEA personal data and our privacy and data security compliance programs, and could require us to implement different compliance measures for the U.K. and the EEA. The European Commission has also proposed reforms under the "Digital Omnibus" package which, if adopted, may further modify GDPR-related requirements, including by clarifying the scope of "personal data" and the treatment of coded or de-identified data, potentially requiring further adjustments to our European privacy compliance framework.

Given the breadth and depth of applicable data protection obligations, preparing for and complying with the GDPR and similar laws' requirements are rigorous and time intensive and require significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data. The GDPR, and other laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Similar privacy and data security requirements are either in place or underway in the U.S. There are numerous data protection laws that may be applicable to our activities, and a range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. Regulations promulgated pursuant to

the Health Insurance Portability and Accountability Act, as amended by the Health Information Technology for Economic and Clinical Health Act, and referred herein collectively as HIPAA, impose privacy, security and breach notification obligations on health plans, healthcare clearinghouses and certain healthcare providers, known as covered entities, as well as their business associates that perform certain services that involve creating, receiving, maintaining or transmitting individually identifiable health information for or on behalf of such covered entities, and their covered subcontractors. HIPAA establishes privacy and security standards that limit the use and disclosure of Protected Health Information, or PHI, and requires the implementation of administrative, physical and technological safeguards to protect the privacy of PHI and ensure the confidentiality, integrity and availability of electronic PHI. Most healthcare providers, including research institutions from which we obtain patient health information, are subject to HIPAA. We do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information.

The Federal Trade Commission, or FTC, and state Attorneys General are also aggressive in reviewing privacy and data security protections for consumers. According to the FTC, failing to take appropriate steps to keep consumers' personal information secure could constitute unfair or deceptive acts or practices in or affecting commerce in violation of Section 5(a) of the FTC Act. The FTC's current guidance for appropriately securing consumers' personal information is similar to what is required by the HIPAA security regulations, but this guidance may change in the future, resulting in increased complexity and the need to expend additional resources to ensure we are complying with the FTC Act.

New laws also are being considered or have been implemented at both the state and federal levels. For example, regulators and legislators in the U.S. are increasingly scrutinizing and restricting certain personal data transfers and transactions involving foreign countries. 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 data brokerage transactions involving certain sensitive personal data categories, including health data, genetic data, and biospecimens, to countries of concern, including China. The regulations also restrict certain investment agreements, employment agreements and vendor agreements involving such data and countries of concern, absent specified cybersecurity controls. Actual or alleged violations of these regulations may be punishable by criminal and/or civil sanctions and may result in exclusion from participation in federal and state programs.

As of January 2026, 20 states now have comprehensive privacy laws in effect. For example, the California Consumer Privacy Act of 2018, or the CCPA, which became effective on January 1, 2020, requires companies that process information of California consumers (as defined under the CCPA) to provide disclosures to such consumers about their data collection, use and sharing practices, provides Californian consumers with individual data privacy rights, imposes operational requirements for covered businesses, provides a private right of action for data breaches and creates a statutory damages framework. Although there are limited exemptions for PHI and clinical trial data under the CCPA, the CCPA and other similar laws could impact our business activities depending on how such laws are interpreted. Additionally as of January 1, 2023, the California Privacy Rights Act, or CPRA has significantly modified the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information and creating a state agency that is vested with authority to implement and enforce the CCPA.

Many other states have enacted or are considering comprehensive privacy legislation similar to the CCPA, and a broad range of legislative measures also have been introduced at state and federal level. Such legislation adds additional complexity, variation in requirements, restrictions and potential legal risk. The introduction of new comprehensive privacy laws across the country may require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. The existence of comprehensive privacy laws in different states in the country makes our compliance obligations more complex and costly and increases the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. There are also states that are specifically regulating health information. For example, Washington state passed a health privacy law that, as of June 30, 2024, regulates the collection and sharing of health information. This law also has a private right of action, which further increases the relevant compliance risk of collecting the health information of Washington residents. Connecticut and Nevada have also passed similar laws regulating consumer health data. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain specific types of information. For example, a small number of states, such as Illinois and Texas, have passed laws that regulate biometric data specifically. These various privacy and security laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. State laws are changing rapidly and there have been discussions in the U.S. Congress of new comprehensive federal data privacy law to which we could become subject, if enacted.

In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies,

procedures and systems, and may impede or restrict business activities, including clinical trial participant recruitment and advertising. Further, any failure by our third-party collaborators, service providers, contractors or consultants to comply with applicable law, regulations or contractual obligations related to data privacy or security could result in proceedings against us by governmental entities or others.

We may also publish privacy policies and other documentation regarding our collection, processing, use and disclosure of personal information and/or other confidential information. Although we endeavor to comply with our published policies and other documentation, we may at times fail to do so or may be perceived to have failed to do so. Despite our efforts, we may not be successful in achieving compliance if our employees or vendors fail to comply with our published policies and documentation. Such failures can subject us to potential international, local, state and federal action and litigation.

All of these evolving compliance and operational requirements impose significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. In addition, such requirements may require us to modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy notices, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Any failure or perceived failure by us to comply with any applicable federal, state or foreign laws and regulations relating to data privacy and security could result in damage to our reputation, as well as proceedings or litigation by governmental agencies or other third parties, including consumer class actions related to these laws and the overall protection of personal information. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business, financial condition, results of operations or prospects.

Further, the use of new and evolving technologies, such as artificial intelligence, in our business may result in spending material resources and presents risks and challenges that can impact our business including by posing security and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability.

Our use of new and evolving technologies, such as artificial intelligence, may present risks and challenges that can impact our business, including by posing cybersecurity and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability.

We may use and integrate artificial intelligence, or AI, into our business processes through implementation of AI and through the adoption of commercially available tools. Use of this technology could pose cybersecurity, data privacy, IT, intellectual property, regulatory, legal, operational, competitive, reputational and other risks and challenges that could affect our business. Specifically, risks related to accuracy, bias, artificial intelligence hallucinations, discrimination, harmful content, misinformation, fraud, scams, targeted attacks (including model poisoning or data poisoning), surveillance, data leakage, environmental harms, and other harms may flow from any use or deployment of AI technologies. If we enable or use solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability.

A growing number of legislators and regulators are adopting laws and regulations and have focused enforcement efforts on the adoption of AI, and use of such technologies in compliance with ethical standards and societal expectations. These developments may increase our compliance burden and costs in connection with use of AI and lead to legal liability if we fail to meet evolving legal standards or if use of such technologies results in harms or other causes of action we did not predict. For example, the EU's Artificial Intelligence Act ("AI Act") is now in effect and is expected to undergo amendments, as introduced in the EU's November 2025 Digital Omnibus. As enacted, the AI Act imposes significant obligations on providers and deployers of AI systems, and encourages providers and deployers of AI systems to account for EU ethical principles in their development and use of these systems. The scope of requirements depends on legal and risk determinations that rely on novel legal provisions that have not yet been interpreted by courts or regulators, and non-compliance can lead to significant fines.

In the U.S., the AI regulatory environment is complex and uncertain. Over the past year, states have advanced, and in some cases passed, dozens of laws focusing on AI governance and regulation, including on deployment of AI in healthcare settings. At the federal level, the Trump Administration has endorsed a federal moratorium on the enforcement of state AI laws, including through a December 11, 2025, executive order on "Ensuring a National Policy Framework for Artificial Intelligence." So far, these efforts have not been successful at curtailing state action on AI regulation, contributing to a complicated legislative patchwork, which may be litigated in state and federal courts. In addition, various federal regulators have issued guidance and focused enforcement efforts on the use of AI in regulated sectors. The U.S. Food and Drug Administration, for example, issued draft guidance on the use of AI in regulatory decision-making for drug and biological products that centers on the context of use while establishing a credibility

assessment framework for establishing and evaluating AI model outputs intended to support regulatory decision-making. If we develop or use AI systems governed by these laws or regulations, including as informed by regulatory guidance, we will need to meet various standards of data quality, transparency, monitoring and human oversight, and we would need to adhere to specific and potentially burdensome and costly ethical, accountability, and administrative requirements, with the potential for significant enforcement or litigation in the event of any perceived non-compliance.

The rapid evolution of AI will require the application of significant resources to design, develop, test and maintain such systems to help ensure that AI is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. The use of certain AI technologies can also give rise to intellectual property risks, including by disclosing or otherwise compromising our confidential or proprietary intellectual property, or by undermining our ability to assert or defend ownership rights in intellectual property created with the assistance of artificial intelligence tools.

Our vendors may in turn 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 artificial intelligence, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. In addition, 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. The integration of AI systems, by us or by our vendors, may increase cybersecurity risk. 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.

If any of our product candidates obtains regulatory approval and does not receive appropriate periods of non-patent exclusivity, competitors could enter the market with generic versions of such products more quickly than we expect, which may result in a material decline in sales of our products.

Under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments to the FDCA, a company may file an abbreviated new drug application, or ANDA, seeking approval of a generic version of an approved innovator product. Under the Hatch-Waxman Amendments, a company may also submit an NDA under section 505(b)(2) of the FDCA that references the FDA's prior approval of the innovator product. A 505(b)(2) NDA product may be for a new or improved version of the original innovator product. The Hatch-Waxman Amendments also provide for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA filing and review) of an abbreviated new drug application, or ANDA, or 505(b)(2) NDA.

In the U.S., once an NDA is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," or the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of ANDAs in the U.S. In support of an ANDA, a generic manufacturer generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration, and adequate labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning, in part, that it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Moreover, many states allow or require substitution of therapeutically equivalent generic drugs at the pharmacy level even if the branded drug is prescribed. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug may be lost to the generic product.

The FDA may not finally approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a NCE. For the purposes of this provision, an NCE is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a patent certification that a patent covering the listed drug is invalid unenforceable or will not be infringed by the generic product. In that case, the applicant may submit its application four years following approval of the listed drug and seek to launch its generic product even if we still have patent protection for our product unless an infringement suit is timely filed by the NDA or patent holder in which case the FDA cannot approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier.

Three-year exclusivity is given to a drug if it contains an active moiety that has previously been approved, and the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted

by or for the applicant and are essential to the approval of the NDA. This form of marketing exclusivity is known as New Clinical Investigation, or NCI, exclusivity. If our product candidates are approved with only NCI exclusivity, generic manufacturers may file their ANDAs any time following approval of our product candidates and seek to launch their generic products following the expiration of the three year market exclusivity period, even if we still have patent protection for our product unless an infringement suit is timely filed triggering a 30 month stay on approval of the generic product (subject to the disposition of the patent litigation).

While we believe that our product candidates may be NCEs in the U.S., the FDA may determine, however, that they are not eligible for NCE exclusivity but receive three years of NCI exclusivity instead, if and when FDA approves an NDA for the product. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to any patents exclusivity we may have. If an ANDA applicant certifies to the invalidity or non-infringement of listed patents and an infringement suit is timely filed by the NDA or patent holder, the FDA cannot finally approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier.

Accordingly, if any of our product candidates is approved, competitors could file ANDAs for generic versions of these products or 505(b)(2) NDAs that reference our product candidates. If there are patents listed for our product candidates in the Orange Book, any ANDA and 505(b)(2) NDA applicants would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. FDORA also requires that the FDA consider therapeutic equivalence determinations for certain Section 505(b)(2) drugs that are pharmaceutical equivalents to listed drugs relied upon in an application either at the time of approval or up to 180 days post-approval, upon request of the sponsor. These therapeutic equivalence determinations could have an adverse effect on our business. Because we remain early in the research and preclinical development of our product candidates, we cannot predict which, if any, patents in our current portfolio or patents we may obtain in the future will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents or the outcome of any such suit.

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

Risks Related to Commercialization

We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our product candidates from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently are pursuing the development of products for the treatment of many of the disorders for which we are conducting research programs. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render our product candidates obsolete or non-competitive. Our competitors also may obtain FDA, European Commission or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

We expect to face competition from existing products and product candidates in development for our programs. For our sole clinical-stage product candidate, PGN-EDODM1, there are currently no approved therapies to treat the underlying cause of the disease. Pipeline candidates currently in development to treat DM1 include several approaches that target DMPK RNA. These include DYNE-101, an antibody conjugated antisense oligonucleotide in Phase 2 clinical development by Dyne, with an ongoing registrational expansion cohort reportedly intended to support a potential submission for accelerated approval in the U.S. and a confirmatory Phase 3 clinical trial; AOC 1001, an antibody linked siRNA in Phase 3 clinical development by Avidity Biosciences, Inc., or Avidity, which

has been acquired by Novartis; VX-670, a peptide conjugated PMO in Phase 2 originally developed by Entrada Therapeutics, Inc., or Entrada, and Vertex; and ARO-DM1/SRP-1003, a conjugated siRNA in Phase 1/2 clinical development by Sarepta, originally developed by Arrowhead Pharmaceuticals, Inc., or Arrowhead. There are additional approaches under development such as ATX-01, a microRNA that modulates expression of MBNL1 by Arthex Biotech S.L., that is in Phase 1/2a clinical development. Another small molecule, tideglusib, which is a GSK3-ß inhibitor is in clinical development by AMO for the congenital phenotype of DM1.

Several gene editing and gene delivery treatments are in discovery or preclinical development by Vertex, Sanofi, and EditForce. In addition, Design Therapeutics, Inc. is developing an approach to prevent formation of CUG hairpins; and Expansion Therapeutics, Inc. and Arrakis Therapeutics are developing an approach utilizing the interaction of small molecules with RNA in preclinical development.

We will also compete more generally with other companies developing alternative scientific and technological approaches, including other companies working to develop conjugates with oligonucleotides for extra-hepatic delivery, including Alnylam Pharmaceuticals, Inc., Aro Biotherapeutics Co., Arrowhead, Avidity (which has been acquired by Novartis), Dicerna Pharmaceuticals, Inc. (acquired by Novo Nordisk), Dyne, Entrada, Ionis Pharmaceuticals, Inc., PYC Therapeutics Limited and Sarepta, as well as gene therapy and gene editing approaches.

Many of the companies against which we compete or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Accordingly, our competitors may be more successful than us in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approval for treatments and achieving widespread market acceptance, rendering our treatments obsolete or non-competitive.

Additionally, mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products, the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing products could present superior treatment alternatives, including by being more effective, safer, more convenient, less expensive or marketed and sold more effectively than any of our products, if approved. Competitive products or technological approaches may make any products we develop, or our EDO platform, obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products, if approved, could be adversely affected.

Even if one or more 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 progresses successfully through clinical development and receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Sales of medical products depend in part on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost-effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost-effective as compared with competing treatments. Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues 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 and safety of such product candidates as demonstrated in clinical trials;
- the potential advantages and limitations compared to alternative treatments;

- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments;
- the clinical indications for which the product is approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

If the market opportunities for any product candidates we develop are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of our programs are small, and the addressable patient population even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth.

We focus our research and product development on treatments for rare diseases. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research that we conducted, and may prove to be incorrect or contain errors. New studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

Our target patient populations are relatively small, and as a result, the pricing and reimbursement of our product candidates, if approved, is uncertain, but must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell product candidates will be adversely affected.

The estimates of market opportunity and forecasts of market growth included in this Form 10-Q if any, or our other public disclosures may prove to be inaccurate, and even if the markets in which we compete achieve the forecasted growth, our business may not grow at similar rates, or at all.

Market opportunity estimates and growth forecasts included in this Form 10-Q, if any, or our other public disclosures are subject to significant uncertainty and are based on assumptions and estimates which may not prove to be accurate. The estimates and forecasts included in this Form 10-Q or our other public disclosures relating to size and expected growth of our target market, if any, may prove to be inaccurate. Even if the markets in which we compete meet any size estimates and/or growth forecasts included in this Form 10-Q, our business may not grow at similar rates, or at all. Our growth is subject to many factors, including our success in implementing our business strategy, which is subject to many risks and uncertainties.

The pricing and third-party payor coverage and reimbursement status of newly approved products are uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our future product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

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. Our ability to successfully commercialize our product

candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. For additional information, see "*Business—Government Regulation—Healthcare Regulation—Coverage and Reimbursement*" in our Annual Report on Form 10-K filed with the SEC for the year-ended December 31, 2025.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the U.S., the decisions about coverage and reimbursement for new products under the Medicare program are made by the Centers for Medicare & Medicaid Services, or CMS. Private payors tend to follow CMS to a substantial degree. However, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the drug product. Further, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the EU may be more conservative than CMS. Factors payors consider in determining reimbursement are based on whether the product is:

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

Additionally, net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price, or ASP, and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs.

Outside the U.S., international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in the EU, the U.K., Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the U.S. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the U.S., the reimbursement for our product candidates may be reduced compared with the U.S. and may be insufficient to generate commercially reasonable revenues and profits.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing our product candidates if any are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we may build a sales and marketing infrastructure to market certain of our product candidates if they receive marketing approval. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts, or third-party efforts, to commercialize our products include:

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

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

Risks Related to Our Intellectual Property

If we or our licensors are unable to obtain, maintain, defend, and enforce patent and other intellectual property protection for any product candidates or technology, or if the scope of the patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize our product candidates or our technology may be adversely affected due to such competition.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the U.S. and other jurisdictions. We and our licensors have sought, and will seek, to protect our proprietary position by filing additional patent applications in the U.S. and abroad related to certain technologies and our platform that are important to our business. However, our patent portfolio is at a somewhat early stage; we do not own any patents and there are 41 issued patents, which we in-licensed from Oxford University Innovation Limited, or OUI, and the Medical Research Council of United Kingdom Research and Innovation, or MRC, and substantive examination of many of the currently pending patent applications we own or license has yet to begin or is in early stage prosecution. In addition, there can be no assurance as to whether or when our patent applications will issue as granted patents. Our ability to stop third parties from making, using, selling, marketing, offering to sell, importing and commercializing our product candidates and our technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property that cover our platform and technology. If we are unable to secure, maintain, defend and enforce patents and other intellectual property with respect to our product candidates and our technology, it would have a material adverse effect on our business, financial condition, results of operations and prospects.

Our pending Patent Cooperation Treaty, or PCT, patent applications are not eligible to become issued patents until, among other things, we file a national stage patent application within 30 to 32 months, depending on the jurisdiction, from such application's priority date in the jurisdictions in which we are seeking patent protection. Similarly, our pending provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of such

provisional patent application's filing date. If we do not timely file such national stage patent applications or non-provisional patent applications, we may lose our priority date with respect to such PCT or provisional patent applications, respectively, and any patent protection on the inventions disclosed in such PCT or provisional patent applications, respectively. While we and our licensors intend to timely file national stage and non-provisional patent applications relating to our PCT and provisional patent applications, respectively, we cannot predict whether any such patent applications will result in the issuance of patents. If we or our licensors do not successfully obtain issued patents, or, if the scope of any patent protection we or our licensors obtain is not sufficiently broad, we will be unable to prevent others from using our product candidates or our technology or from developing or commercializing technology and products similar or identical to ours or other competing products and technologies. Any failure to obtain or maintain patent protection with respect to our product candidates or our EDO platform would have a material adverse effect on our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, defend, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. We and our licensors may not be able to obtain, maintain, or defend patents and patent applications due to the subject matter claimed in such patents and patent applications being in the public domain or otherwise unpatentable. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. In addition, our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors or other third party partners may file patent applications and even obtain patents based on inventions that we shared with them under contractual terms of confidentiality and non-use. Consequently, we would not be able to prevent any third party from using any of our technology that is in the public domain to compete with our product candidates, or we may need to engage in litigation or other proceedings at the USPTO or foreign patent offices to preserve the exclusivity of our product candidates and platform technology.

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 patent rights are highly uncertain. Our pending and future owned and licensed patent applications may not result in patents being issued which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and products or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all, and even if such patent applications do issue as patents, they may not issue in a form, or with a scope of claims, that will provide us with any meaningful protection, prevent others from competing with us or otherwise provide us with any competitive advantage. In addition, the scope of claims of an issued patent can be reinterpreted after issuance, and changes in either the patent laws or interpretation of the patent laws in the U.S. and other jurisdictions may diminish the value of our patent rights or narrow the scope of our patent protection. Furthermore, our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

Third parties have developed technologies that may be related or competitive to our own technologies and product candidates and may have filed or may file patent applications, or may have obtained issued patents, claiming inventions that may overlap or conflict with those claimed in our owned or licensed patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our current and future product candidates and technology. Publications of discoveries in the scientific literature often lag 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 know for certain whether the inventors of our owned or licensed patents and patent applications were the first to make the inventions claimed in any owned or licensed patents or pending patent applications, or that we 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 patent applications may not issue as patents and even if issued, may be challenged and invalidated or ruled unenforceable.

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 other jurisdictions. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our owned or licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our owned or licensed pending patent applications. We may become involved in opposition, derivation, re-examination, inter partes review, post-grant review or interference proceedings and similar proceedings in foreign jurisdictions (for example, opposition proceedings) challenging our owned or licensed patent rights. In addition, a third party may claim that our owned or licensed patent rights are invalid or unenforceable in a litigation. An adverse result in any litigation or patent office proceeding could put one or more of our owned or licensed patents at risk of being invalidated, ruled unenforceable or interpreted narrowly and could allow third parties to commercialize products identical or similar to our product candidates and compete directly with us,

without payment to us. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges and proceedings may result in loss of patent rights, exclusivity 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 technology and products, or limit the duration of the patent protection of our technology and our product candidates. Such challenges and proceedings may also result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Moreover, there could be public announcements of the results of hearings, motions or other interim proceedings or developments related to such challenges and proceedings. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Furthermore, patents have a limited lifespan. In the U.S., the expiration of a patent is generally 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. Patent term adjustments and extensions may be available; however, the overall term of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent and other intellectual property rights may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our technology and our product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Our rights to develop and commercialize any product candidates are subject and may in the future be subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future intellectual property license agreements, are not able to obtain adequate rights, or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business.

We are and expect to continue to be reliant upon third-party licensors for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. For example, we rely on a license from OUI and MRC, to certain patent rights and know-how of OUI and MRC, or the OUI/MRC License. The OUI/MRC License imposes, and we expect that any future license agreement will impose, specified diligence, milestone payment, fee payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses.

Furthermore, our licensors have, or may in the future have, the right to terminate a license if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If our license agreements are terminated, we may lose our rights to develop and commercialize product candidates and technology, lose patent protection, experience significant delays in the development and commercialization of our product candidates and technology, incur liability for damages, hinder our ability to raise additional capital, and attract investors, create litigation distracting to management, which diverts capital resources, and allow third parties, including our competitors, to obtain license rights to the intellectual property previously licensed to us and assert against us a claim of infringement. If these in-licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. Even if these licenses are not terminated, we may not have rights to intellectual property owned or controlled by our licensors that relate to our product candidate or platform technology. Thus, our licensors may grant rights to such intellectual property, which is not licensed to us, to third parties, including our competitors, which could undermine the competitive position of our product candidate and platform technology and potentially adversely affect the pricing and desirability of our product candidate and platform technology. 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, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with our product candidates and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;

- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the OUI/MRC License is, and future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement. In addition, if disputes over intellectual property that we have licensed or any other dispute related to our license agreements prevent or impair our ability to maintain our license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

While the OUI/MRC License grants certain exclusive patent and technology rights to us, license agreements we have entered into, including the OUI/MRC License, and may enter into in the future, may provide for certain activities as being non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors with respect to the intellectual property licensed to us under such license agreements. Accordingly, these license agreements may not provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and our product candidates.

Moreover, some of our in-licensed patent and other intellectual property rights are, and may in the future be, subject to third party interests such as co-ownership. If we are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

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

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by one or more third parties. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. or foreign government and timely electing title to such inventions. A failure to meet these obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications.

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

Filing, prosecuting, maintaining, enforcing and defending patents and other intellectual property rights on our technology and our product candidates in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the U.S. could be less extensive than those in the U.S. Thus, we may not have applied for patent protection to protect our product candidate or platform technology in all countries that we may commercialize our products or

platform technology. Even if we have applied for such patent protection outside of the U.S., in some cases, we or our licensors may not be able to obtain patent or other intellectual property protection for certain technology and product candidates. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as federal and state laws in the U.S. For example, methods of human treatment that are patentable in the U.S. are not patentable in certain countries outside of the U.S. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering our product candidates and our technology in all jurisdictions outside the U.S. and, as a result, may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the U.S., or from selling or importing products made using our inventions in and into the U.S. or other jurisdictions. Third parties may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent or other intellectual property protection to develop their own products and, further, may export otherwise infringing, misappropriating or violating products to territories where we have patent or other intellectual property protection, but enforcement is not as strong as that in the U.S. These products may compete with our product candidates and our technology and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Additionally, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain jurisdictions, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our patent and other intellectual property rights or marketing of competing products in violation of our intellectual property rights generally. For example, an April 2019 report from the Office of the U.S. Trade Representative identified a number of countries, including China, Russia, Argentina, Chile and India, where challenges to the procurement and enforcement of patent rights have been reported. Proceedings to enforce our or our licensors' patent and other intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patent and other intellectual property rights at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors 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.

Many jurisdictions have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many jurisdictions limit the enforceability of patents against government agencies or government contractors. In these jurisdictions, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

We may be involved in lawsuits to protect or enforce our patents or other intellectual property or the intellectual property of our licensors, which could be expensive, time-consuming, and unsuccessful.

Competitors may infringe our patents or other intellectual property or the intellectual property of our licensors. To cease such infringement or unauthorized use, we may be required to file patent infringement claims, which can be expensive and time-consuming and divert the time and attention of our management and scientific personnel. Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. In addition, in an infringement proceeding or a declaratory judgment action, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceeding could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

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

Changes in either the patent laws or interpretation of patent laws in the U.S. and worldwide, including patent reform legislation such as the Leahy-Smith America Invents Act, or Leahy-Smith Act, could increase the uncertainties and costs surrounding the prosecution of any owned or in-licensed patent applications and the maintenance, enforcement or defense of any current in-licensed issued patents and issued patents we may own or in-license in the future. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the U.S., the first to invent the claimed invention was entitled to the patent, while outside the U.S., the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the U.S. transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our in-licensed issued patents and issued patents we may own or in-license in the future, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Since patent applications in the U.S. and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim unpatentable even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to review patentability of our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. 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. As one example, in the case Assoc. for Molecular Pathology v. Myriad Genetics, Inc., the U.S. Supreme Court held that certain claims to DNA molecules are not patentable simply because they have been isolated from surrounding material. Moreover, in 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. Accordingly, in view of the guidance memo, there can be no assurance that claims in our patent rights covering our product candidates or our technology will be held by the USPTO or equivalent foreign patent offices or by courts in the U.S. or in foreign jurisdictions to cover patentable subject matter. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

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 patent 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 owned or licensed patent rights. We rely on our outside counsel and other professionals or our licensing partners to correctly pay these fees due to the USPTO and non-U.S. government patent agencies. The USPTO and various non-U.S. government patent agencies also require

compliance with several procedural, documentary and other similar provisions during the patent application process. We rely on our outside counsel and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment, loss of priority or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to certain intellectual property through the OUI/MRC License. Because our programs may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these intellectual property rights. In addition, with respect to any patent or other intellectual property rights that we co-own with third parties, we may require exclusive licenses to such co-owners' interest in such patent or other intellectual property rights. However, we may be unable to secure such licenses or otherwise acquire or in-license any intellectual property rights related to compositions, methods of use, processes or other components from third parties that we identify as necessary for our product candidates and our technology on commercially reasonable terms, or at all. Even if we are able to in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. 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. 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.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license 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 third parties, potentially blocking our ability to pursue our research program and develop and commercialize our product candidates.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have licensed, 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 technology, 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 have a material adverse effect on our business, financial condition, results of operations and prospects.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or before administrative bodies in the U.S. or abroad.

Our owned and licensed patent rights may be subject to priority, validity, inventorship and enforceability disputes. If we or our licensors are unsuccessful in any of these proceedings, such patent rights may be narrowed, invalidated or held unenforceable, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or we may be required to cease the development, manufacture and commercialization of one or more of our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or one of our licensors initiate legal proceedings against a third party to enforce a patent covering our product candidates or our technology, the defendant could counterclaim that the patent covering the product candidate or technology is invalid or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, interference proceedings, derivation proceedings, post grant review, inter partes review and equivalent proceedings such as opposition, invalidation and revocation

proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our product candidates or our technology or prevent third parties from competing with our product candidates or our technology. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or technology. Such a loss of patent protection could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the U.S. are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, contractors and other parties who have access to such technology and processes. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breach or violate the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets and third parties could use our trade secrets to compete with our product candidates and our technology. Additionally, we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems; however, such systems and security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors or other third parties. Competitors or third parties could purchase our product candidates or our technology and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our intellectual property rights or develop their own competitive technologies that fall outside the scope of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our business, financial condition, results of operations and prospects could be materially and adversely affected.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators, if any, to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation in which third parties may assert infringement, misappropriation or other violation claims against us, alleging that our product candidates, manufacturing methods, formulations or administration methods are covered by their patents. Given the vast number of patents and other intellectual property in our field of technology, we cannot be certain or guarantee that we do not infringe, misappropriate or otherwise violate patents or other intellectual property. Other companies and institutions have filed, and continue to file, patent applications that may be related to our technology and, more broadly, to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that we may be subject to claims of infringement of the patent rights of third parties. If a

patent holder believes the manufacture, use, sale or importation of our product candidates or our technology infringes its patent, the patent holder may sue us even if we have licensed other patent rights for our technology.

We are aware of certain patents in the U.S. and other jurisdictions owned by third parties that claim subject matter that relates to our product candidates and the EDO platform. Such third parties may assert these patents against us in litigation in the U.S. or other jurisdictions. The outcome of any such litigation is uncertain and, even if we prevail, the costs of such litigation could have a material adverse effect on our financial position, result in disclosure of our trade secrets, distract key personnel from the continued development of our business, and adversely affect our ability to enter or maintain commercial relationships with collaborators, clients or customers. If we are unsuccessful in such litigation, we could be prevented from commercializing products, required to pay damages, including treble damages, or could be required to take licenses from such third parties which may not be available on commercially reasonable terms, if at all. The amount of potential damages could be significant and have a material adverse effect on our business.

It is also possible that we have failed to identify relevant third-party patents or applications. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before, or after, issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates or our technology and we may not be aware of such patents. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the U.S. may remain confidential until a patent issues. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and our technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our product candidates or the use of our product candidates.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our product candidates and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

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

Competitors may challenge the validity and enforceability of our patent rights or those of our licensing partners, infringe, misappropriate or otherwise violate our or our licensors' patent and other intellectual property rights, or we may be required to defend against claims of infringement, misappropriation or other violation. Litigation and other proceedings in connection with any of the foregoing claims can be unpredictable, expensive and time-consuming. Even if resolved in our favor, litigation or other proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific, technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase

our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors or other third parties may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace and could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or be required to obtain licenses to such intellectual property rights, which may not be available on commercially reasonable terms or at all. An inability to incorporate such intellectual property rights would harm our business and may prevent us from developing or successfully commercializing our product candidates. In addition, we may lose personnel as a result of such claims and any such litigation, or the threat thereof may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our product candidates and our technology, which would have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our scientific and management personnel.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Furthermore, individuals executing agreements with us may have pre-existing or competing obligations to a third party, such as an academic institution or government body, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we own may have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we or our licensors may in the future be subject to claims by former employees, consultants or other third parties asserting an ownership right in our owned or licensed patent rights. An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar technology and therapeutics, without payment to us, or could limit the duration of the patent protection covering our technology and our product candidates. Such challenges may also result in our inability to develop, manufacture or commercialize our technology and product candidates without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our owned or licensed patent rights are threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future technology and product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we do not obtain patent term extension for our product candidates, our business may be harmed.

Under the Hatch-Waxman Amendments to the FDCA, a company may file an ANDA seeking approval of a generic version of an approved innovator product. Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates and our technology, one or more of our U.S. patents that we license or may own in the future may be eligible for limited patent term extension under Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during 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 product, a method for using it or a method for manufacturing it may be extended. The application

for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or technology. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patent rights, trade secrets or other intellectual property. If we or our licensors 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 intellectual property that is important to our product candidates or our technology. 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.

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

Our current and future trademark applications in the U.S. and other foreign jurisdictions may not be allowed or may be subsequently opposed. Once filed and registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. As a means to enforce our trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings. This can be expensive and time-consuming, particularly for a company of our size. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

Intellectual property rights do not necessarily address all potential threats.

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. For example:

- others may be able to make products that are similar to our product candidates but that are not covered by intellectual property, including the claims of the patents, that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating, or otherwise violating our owned or licensed intellectual property rights;

- it is possible that our or our licensors' current or future pending patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by third parties;
- third parties might conduct research and development activities in jurisdictions where we do not have patent or other intellectual property rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

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

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor or other third party will discover our trade secrets or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our product candidates and to perform quality testing, and because we may need to collaborate with various third parties for the advancement of our product candidates and technology, we may be required to, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaboration agreements, services agreements, consulting agreements and other similar agreements prior to beginning research or disclosing any proprietary information to such third parties. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently or purposefully incorporated into the technology of themselves or others or are disclosed or used in violation of these agreements, such as in the filing of patent applications unbeknownst to us. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, some courts inside and outside the U.S. are less willing or unwilling to protect trade secrets. We may need to share our proprietary information, including trade secrets, with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors, and those affiliated with or controlled by state actors. Further, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third-party, we would have no right to prevent them from using that technology or information to compete with us. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Risks Related to Employee Matters, Managing Growth and Other Operational Matters

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

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

The loss of the services of our executive officers or other key employees could impede the achievement of our research, 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 approval 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 research and 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. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We may need to expand our headcount over time to support our 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.

As of September 30, 2026, we had 53 full-time employees. As our development progresses including commencing a pivotal Phase 3 clinical study and related NDA filing for PGN-EDODM1, we expect to experience growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any product candidate receives marketing approval, sales, marketing, distribution and coverage and reimbursement capabilities. To manage our potential future growth, we must continue to implement and improve our managerial, operational and financial systems, and continue to recruit and train necessary qualified personnel and longer-term, may need to expand our facilities. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we expect to pursue new platforms and product candidates in the future in several therapeutic areas and across a range of diseases. Successfully developing product candidates for, and fully understanding the regulatory and manufacturing pathways to, each of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

Our international activities subject us to various risks, and our failure to manage these risks could adversely affect our results of operations.

We face significant operational risks as a result of doing business internationally, such as:

- fluctuations in foreign currency exchange rates;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- potentially adverse and/or unexpected tax consequences, including penalties due to the challenge by tax authorities on our tax position;
- potential changes to the accounting standards, which may influence our financial situation and results;
- compliance with tax, employment, immigration and labor laws should we have any employees living or traveling abroad;
- becoming subject to the different, complex and changing laws, regulations and court systems of multiple jurisdictions and compliance with a wide variety of foreign laws, treaties;
- reduced protection of, or significant difficulties in enforcing, intellectual property rights, or increased risk of intellectual property disputes, in certain countries;
- difficulties in attracting and retaining qualified consultants, contractors, and personnel;

- restrictions imposed by any applicable local labor practices and laws on our business and operations, including unilateral cancellation or modification of contracts;
- rapid changes in global government, economic and political policies and conditions, political or civil unrest or instability, terrorism or epidemics and other similar outbreaks or events, and potential failure in confidence of our suppliers or customers due to such changes or events;
- geopolitical tensions that affect our activities, operations and/or operations of our contractors, consultants, collaborators, vendors or partners; and
- tariffs, trade protection measures, import or export licensing requirements, trade embargoes and other trade barriers.

We face risks associated with tariffs and other trade restrictions, which may have a material adverse impact on our results of operations and financial condition.

We face risks related to tariffs and other trade protection measures—including those that have been or may be imposed by the United States or other countries—as well as import or export licensing requirements, trade embargoes, sanctions (including those administered by the U.S. Department of the Treasury's Office of Foreign Assets Control), and other trade barriers (including further legislation or actions taken by the United States or other countries that restrict trade). These risks include protectionist or retaliatory measures that may limit or complicate the sourcing of raw materials, equipment, and other components critical to our research and development activities.

In July 2026, the United States imposed significant tariffs ranging from 10% to 12.5% on virtually all imports to the United States and has also imposed significantly higher rates targeting certain imports in select industries, including certain pharmaceuticals. U.S. tariffs on Chinese-origin products remain higher than tariffs on products from other countries. The United States is conducting, or has recently completed, investigations that could result in additional broad-based or sector-specific tariffs. In response, several countries have enacted retaliatory measures, and the situation remains unpredictable. Current or future tariffs will result in increased research and development expenses, including with respect to increased costs associated with active pharmaceutical ingredients (APIs), raw materials, laboratory equipment and research materials and other components used in the development and manufacturing of our product candidates which may be subject to such tariffs.

We may face increased costs and operational disruptions if existing or future tariffs are applied to materials or components used in the development and manufacture of our product candidates. These risks also extend to indirect effects, such as retaliatory tariffs imposed by other countries or additional non-tariff trade barriers. As a result, our research and development activities, manufacturing timelines, and overall financial condition could be materially adversely affected.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses, technologies or assets, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products or product candidates resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with collaborators as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities and other known liabilities;

- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

Our internal information technology systems, or those of our vendors, collaborators or other contractors or consultants, may fail or suffer from cyber security incidents or breaches, loss or leakage of data and other disruptions or compromise, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, or trigger contractual and legal obligations, potentially exposing us to liability, reputational harm or otherwise adversely affecting our business and financial results.

We are increasingly dependent upon information technology systems and infrastructure to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we, our vendors, collaborators and other contractors or consultants, do so in a secure manner to maintain the availability, security, confidentiality, privacy and integrity of such confidential information.

Despite the implementation of security measures, given the size and complexity of our internal information technology systems and those of our current and future vendors, collaborators and other contractors or consultants, and the increasing amounts of confidential information that we and our affiliated third parties maintain, such information technology systems are still vulnerable to damage or interruption from security incidents, data breaches, computer viruses, computer hackers, malicious code, ransomware, social engineering attacks (including phishing attacks), employee error, theft or misuse, denial-of-service attacks, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war, telecommunication and electrical failures or other compromise. The risk of a cybersecurity incident, data breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely for a portion of their time, which may create additional opportunities for cybercriminals to exploit vulnerabilities. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies. As such, we may experience cybersecurity incidents, breaches, or compromises that may remain undetected for an extended period. We may be unable to anticipate all types of cybersecurity threats, or implement preventive measures effective against all such cybersecurity threats. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection and to remove or obfuscate forensic evidence.

We and certain of our service providers are from time to time subject to cyberattacks and cybersecurity incidents, breaches, or compromises. While we do not believe that we have experienced any significant system failure, accident or cybersecurity breach or incident to date, if such an event were to occur, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary or confidential information or other disruptions. For example, the loss of clinical trial data from clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity incident or breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the incident or breach to counterparties, data subjects, regulators or others could be material. In addition, our remediation efforts may not be successful. Moreover, if the information technology systems of our vendors, collaborators and other contractors and consultants become subject to disruptions or cybersecurity incidents or breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information.

To the extent that any disruption, cybersecurity incident, data breach, or compromise were to result in a loss of, or damage to, our or our vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential

or proprietary information, we could incur liability, including litigation exposure and penalties and fines. Any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our customers or employees, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information. We could become the subject of regulatory action or investigation, and our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed. As a result of such an event, we may also be in breach of our contractual obligations. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

The financial exposure from the events referenced above could either not be insured against or not be fully covered through any insurance that we maintain. In addition, we cannot be sure that our existing insurance coverage will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages as a result of the events referenced above.

Our operations or those of the third parties upon whom we depend might be affected by the occurrence of a natural disaster, pandemic or other catastrophic event.

We depend on our employees, consultants, CDMOs and CROs, as well as regulatory agencies and other parties, for the continued operation of our business. While we maintain disaster recovery plans, they might not adequately protect us. Despite any precautions we take for natural disasters or other catastrophic events, these events, including terrorist attack, pandemics, hurricanes, fire, floods and ice and snowstorms, could result in significant disruptions to our research and development, preclinical studies, clinical trials, and, ultimately, commercialization of our products. Long-term disruptions in the infrastructure caused by events, such as natural disasters, the outbreak of war, the escalation of hostilities and acts of terrorism or other "acts of God," particularly involving cities in which we have offices, manufacturing or clinical trial sites, could adversely affect our businesses. Although we carry business interruption insurance policies and typically have provisions in our contracts that protect us in certain events, our coverage might not respond or be adequate to compensate us for all losses that may occur. Any natural disaster or catastrophic event affecting us, our CDMOs, CROs, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults, or non-performance by financial institutions or transactional counterparties, could adversely affect our current and projected business operations and our financial condition and results of operations.

Events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. Although we are not a borrower or party to any such instruments with any financial institution that has experienced such events, if we were to borrow money in the future and if any of our lenders or counterparties to any such instruments were to be placed into receivership, we may be unable to access such funds. In addition, if any of our customers, suppliers or other parties with whom we conduct business are unable to access funds pursuant to such instruments or lending arrangements with such a financial institution, such parties' ability to pay or perform their obligations to us or to enter into new commercial arrangements requiring additional payments to us or additional funding could be adversely affected. In this regard, counterparties to credit agreements and arrangements with banks in receivership or other financial difficulty, and third parties such as beneficiaries of letters of credit (among others), may experience direct impacts from the closure or reorganization of such financial institutions and uncertainty remains over liquidity concerns in the broader financial services industry. Similar impacts have occurred in the past, such as during the 2008-2010 financial crisis.

Inflation and rapid increases in interest rates have led to a decline in the trading value of previously issued government securities with interest rates below current market interest rates. Although the U.S. Department of Treasury, FDIC and Federal Reserve Board have announced a program to provide up to $25 billion of loans to financial institutions secured by certain government securities held by financial institutions to mitigate the risk of potential losses on the sale of such instruments, widespread demands for customer withdrawals or other liquidity needs of financial institutions for immediate liquidity may exceed the capacity of such program. Additionally, there is no guarantee that the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions in the future, or that they would do so in a timely fashion.

Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect us, the financial institutions with which we have financial arrangements directly, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry.

In addition, investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity, our current and/or projected business operations, and financial condition and results of operations.

The effects of a future pandemic, epidemic or outbreak of an infectious or highly contagious disease, may materially and adversely affect our business and financial results and could cause a disruption in the development of our product candidates.

Public health crises such as pandemics could adversely impact our business. For example, we and our CDMOs and CROs in the past experienced a reduction in the capacity to undertake research-scale production and to execute some preclinical studies, and we may face future similar disruptions that affect our ability to initiate and complete preclinical studies or clinical trials. We may also encounter disruptions in procuring items that are essential for our research and development activities, such as raw materials used in the manufacture of any product candidates, laboratory supplies used in our preclinical and clinical studies, or animals that are used for preclinical testing for which there are or may be shortages because of efforts to address a future health pandemic. The ultimate extent to which a future outbreak of highly infectious or contagious diseases, impacts our operations or those of our third-party partners, including our preclinical studies or clinical trial operations, will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the scope, severity and duration of an outbreak, actions taken to contain an outbreak or mitigate its impact, and the direct and indirect economic effects of an outbreak and containment measures, among other developments.

Risks Related to Ownership of Our Common Stock

The stock price of our common stock has been and may continue to be volatile or may decline regardless of our operating performance and prospects.

You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. An active or liquid market in our common stock may not develop or, if it does develop, it may not be sustainable, and the prices at which shares of our common stock trade in the market have fluctuated considerably and may fluctuate in the future considerably or decline or be quite volatile regardless of our operating performance and prospects. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price or the per share price you paid for your shares.
Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic collaborations or acquire companies or products by using our shares of common stock as consideration.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. In February 2024, we sold shares of our common stock under our ATM program and in the 2024 Offering, receiving aggregate net proceeds of $86.3 million after deducting underwriters' fees and costs of the offerings. In September 2025, we sold shares of our common stock in a public offering which resulted in net proceeds of $107.6 million after deducting underwriters' fees and estimated offering expenses. Any debt financing or preferred equity financing, if available, may involve, agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring

additional debt, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed or on terms acceptable to us, we may be required to delay, limit, reduce or eliminate some or all of our research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. In the event one or more analysts downgrade our stock price or change their opinion of our stock price, our stock price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our stock price or trading volume to decline.

The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for holders of our common stock.

The stock market in general, and the market for smaller biopharmaceutical companies in particular, have experienced extreme price volatility and volume fluctuations that have often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the price you paid for it. The market price for our common stock has also been volatile and fluctuated substantially, and it may be influenced by many factors, including:

- timing and results of, or developments in, clinical trials of our product candidates or those of our competitors or potential competitors;
- adverse regulatory rulemaking, guidance or decisions, including failure to receive marketing approvals for our product candidates;
- our success in commercializing any product candidates that may be approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the U.S. and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to our product candidates;
- the results of our efforts to discover, develop, acquire or in-license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to our financial condition or results, clinical outcomes, development timelines or recommendations by securities analysts;
- variations in our financial condition or results or the financial condition or results of companies that are perceived to be similar to us;
- sales of our common stock by us, our executive officers, directors or principal stockholders or others;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry, political and market conditions, high inflation and capital market disruptions, and government macroeconomic policies; and

- the other factors described in this "*Risk factors*" section.

Any of the factors listed above could materially adversely affect your investment in our common stock, and our common stock may trade at prices significantly below the price you paid for our stock, which could contribute to a loss of all or part of your investment. In such circumstances the trading price of our common stock may not recover and may experience a further decline.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation has often been instituted against that company. Any lawsuit to which we are a party in the future, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and maintain liability insurance coverages and may also result in the diversion of management's attention and resources.

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

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the 2008 global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn resulting from the effects of tariffs recently imposed by the United States, which have resulted in other countries imposing additional tariffs on imports from the U.S., including substantial tariffs on imports from the U.S. announced by China, and are likely to continue to result in more retaliatory tariffs and a reduction in global trade could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. In addition, the current geopolitical conflicts could disrupt or otherwise adversely impact our operations and those of third parties upon which we rely. Related sanctions, import/export controls or other actions that have been or may be initiated by nations, including the U.S. or the EU, including recent legislation – the BIOSECURE Act – in the U.S. relating to China and certain biotechnology companies of concern, or actions taken by Russia (e.g., potential cyberattacks, disruption of energy flows, etc.) could adversely affect our business and/or our supply chain, our CROs, CDMOs and other third parties with which we conduct business. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could impair our ability to achieve our growth strategy, could harm our financial performance and stock price and could require us to delay or abandon clinical development plans. 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, including increasing interest rates and high inflation, and financial market conditions could adversely impact our business.

Our executive officers, directors and principal shareholders, if they choose to act together, will continue to have the ability to significantly influence all matters submitted to stockholders for approval.

Based upon our common stock outstanding as of September 30, 2026, our executive officers, directors, greater than five percent shareholders and their affiliates beneficially own approximately 47.7% of our outstanding common stock. As a result, if these stockholders were to choose to act together, they would be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs, even though some of these persons or entities may have interests that are different than those of yours. For example, these stockholders, if they choose to act together, could significantly influence the election of directors and approval of any merger, consolidation or sale of substantially all of our assets.

This concentration of ownership may:

- delay, defer or prevent a merger, consolidation or sale of all or substantially all of our assets that may be desired by other stockholders;
- delay, defer or prevent a change in control transaction involving us that other stockholders may desire; or
- entrench our management and board of directors.

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

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

The sale of a significant number of shares of our common stock, or the perception that such sales could occur, could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock, impair our ability to raise capital through the sale of additional equity securities, and make it more difficult for you to sell your common stock at a time and price that you deem appropriate.

In addition, holders of an aggregate of 8,778,170 shares of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We have also filed or will file registration statements on Form S-8 to register all of the shares of common stock that we are able to issue under our equity compensation plans. Shares registered under these registration statements on Form S-8 can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates, vesting arrangements, exercise of options and any contractual restrictions that may apply to such shares.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or JOBS Act. We may remain an EGC until the end of the year that is the fifth anniversary of the closing of our IPO, although if the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have annual gross revenues of $1.235 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than $1.0 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- 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; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Even after we no longer qualify as an EGC, we may continue to qualify as a smaller reporting company, which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation. In addition, if we are a smaller reporting company with less than $100 million in annual revenue, we would not be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404. In reliance on these exemptions, we have taken advantage of reduced reporting obligations in this Form 10-Q.

We cannot predict whether investors will find our common stock less attractive if 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.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to take advantage of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the

new or revised standard and will do so until such time that we either irrevocably elect to "opt out" of such extended transition period or no longer qualify as an EGC. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

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

As a public company, and even more so after we are no longer an EGC or a smaller reporting company, we incur or will incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote and will need to continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased and may continue to increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements and have made and will make some activities more time-consuming and costly compared to when we were a private company. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified members of our board of directors.

We cannot predict or estimate the amount of additional costs we may incur as a public company or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Pursuant to Section 404, we were required to furnish a report by our management on our internal control over financial reporting beginning with the Form 10-K for the year ended December 31, 2023. However, while we remain an EGC or a smaller reporting company with less than $100 million in annual revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial and accounting personnel, engaging outside consultants and adopting a detailed work plan to assess and document the adequacy of internal control over financial reporting, continuing steps to improve control processes as appropriate, validating through testing that controls are functioning as documented and implementing a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements, particularly if such material weakness results in the necessity of a restatement of our historical financial statements.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could harm our business and have a negative effect on the trading price of our stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. However, for as long as we are an EGC under the JOBS Act or a smaller reporting company with less than $100 million in annual revenue, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404. We could be an EGC for up to five fiscal years after our IPO, which occurred on May 6, 2022. An independent assessment of the effectiveness of our internal control over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal control over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation, which could have a negative effect on the trading price of our stock.

Anti-takeover provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.

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

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66.7% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws.

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

Our second amended and restated bylaws designate the Court of Chancery of the State of Delaware and the federal district courts of the United States of America 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 and employees.

Our second amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders;

- any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; or
- any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine.

These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by 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 second amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America shall, to the fullest extent permitted by law, be the sole and exclusive forum for the resolution of any claims arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our second amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders 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. If a court were to find the exclusive forum provision contained in our second amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving such action in other jurisdictions, all of which could materially adversely affect our business, financial condition and operating results.

We may not be able to continue to satisfy listing requirements of the Nasdaq Global Select Market or maintain a listing of our common stock on the Nasdaq Global Select Market.

Our common stock is listed on the Nasdaq Global Select Market. We must meet certain financial and liquidity criteria to maintain such listing, including a minimum bid price of $1.00 per share. If we violate or fail to meet any of the Nasdaq Global Select Market'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 the Nasdaq Global Select Market 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 your investment.

General Risk Factors

Changes in tax laws or regulations or in their implementation or interpretation may adversely affect our business and financial condition.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business or financial condition. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. A number of other factors could materially adversely affect our business and financial condition including: tax policy initiatives and reforms under consideration (such as those related to the Organization for Economic Co-Operation and Development's, or OECD, Base Erosion and Profit Shifting, or BEPS, Project, the European Commission's state aid investigations and other initiatives), the practices of tax authorities in jurisdictions in which we operate; the resolution of issues arising from tax audits or examinations and any related interest or penalties. Such changes may include (but are not limited to) the taxation of operating income, investment income, dividends received or (in the specific context of withholding tax) dividends paid.

The OECD Pillar Two Model Rules established a minimum global effective tax rate of 15% on country-by-country basis. EU member states along with many other countries adopted or expected to adopt the OECD Pillar Two Model effective January 1, 2024 or thereafter. The OECD and other countries continue to publish guidelines and legislation which include transition and safe harbor rules. We continue to monitor new legislative changes and assess the global impact of the Pillar Two Model Rules. Based on our initial assessment, we anticipate Pillar Two top-up taxes to be immaterial.

The U.S. government may enact significant new changes to the taxation of business entities including, among others, an increase in the corporate income tax rate. Furthermore, the rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service, or IRS, and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

We are unable to predict what tax reform may be proposed or enacted in the future or what effect such changes would have on our business, but such changes, to the extent they are brought into tax legislation, regulations, policies or practices in jurisdictions in which we operate, could increase the estimated tax liability that we have expensed to date and paid or accrued on our balance sheets, and otherwise affect our financial position, future results of operations, cash flows in a particular period and overall or effective tax rates in the future in countries where we have operations, reduce post-tax returns to our shareholders and increase the complexity, burden and cost of tax compliance.

Tax authorities may disagree with our positions and conclusions regarding certain tax positions, or may apply existing rules in an unforeseen manner, resulting in unanticipated costs, taxes or non-realization of expected benefits.

A tax authority may disagree with tax positions that we take, which could result in increased tax liabilities. For example, His Majesty's Revenue & Customs, the IRS or another tax authority could challenge our allocation of income by tax jurisdiction and the amounts paid between our affiliated companies pursuant to our intercompany arrangements and transfer pricing policies, including amounts paid with respect to our intellectual property development. Similarly, a tax authority could assert that we are subject to tax in a jurisdiction where we believe we have not established a taxable connection, often referred to as a "permanent establishment" under international tax treaties, and such an assertion, if successful, could increase our expected tax liability in one or more jurisdictions.
A tax authority may take the position that material income tax liabilities, interest and penalties are payable by us, in which case we expect that we might contest such assessment. Contesting such an assessment may be lengthy and costly and if we were unsuccessful in disputing the assessment, the implications could increase our anticipated effective tax rate, where applicable.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be subject to limitations.

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses, or NOLs, or research and development tax credit carryforwards. As of December 31, 2025, we had federal NOLs of $177.3 million and state NOL carryforwards of $199.6 million. We did not generate U.K. NOLs in 2025 as our U.K. entity, PepGen Limited, was dissolved on December 10, 2024.

As a company that carries out extensive research and development activities, we sought to benefit from the U.K. research and development tax relief programs, being the Small and Medium-sized Enterprises R&D tax relief program, or SME Program, and, to the extent that our projects are grant funded or relate to work subcontracted to the company by third parties, the Research and Development Expenditure Credit program, or RDEC Program. Under the SME Program, we may be able to surrender the trading losses that arise from our qualifying research and development activities for a cash rebate of approximately 33.4% of the surrenderable losses. The majority of our research and development activities during 2021 were eligible for inclusion within these tax credit cash rebate claims. We may not be able to continue to claim payable research and development tax credits in the future if we cease to qualify as an SME, based on size criteria concerning employee headcount, turnover and gross assets or if we no longer conduct qualifying research and development activities through our wholly-owned subsidiary PepGen Limited. The U.K. Finance Act of 2021 introduced a cap on payable credit claims under the SME Program in excess of £20,000 with effect from April 2021 by reference to, broadly, three times the total PAYE and NICs liability of the company, subject to an exception which prevents the cap from applying. That exception requires the company to be creating, taking steps to create or managing intellectual property, as well as having qualifying research and development expenditure in respect of connected parties which does not exceed 15% of the total qualifying expenditure. If such exception does not apply, this could restrict the amount of credit that we are able to claim. For the nine months ended September 30, 2026, our research and development tax credits from the U.K. government were not material as the intellectual property was transferred from our wholly-owned U.K. subsidiary, PepGen Limited, to the parent company, PepGen Inc. in January 2022.

For U.S. federal income tax purposes, in general, under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year

period, is subject to limitations on its ability to utilize its pre-change NOLs and other pre-change tax attributes, including research and development tax credit carryforwards, to offset post-change income or taxes. We have not conducted a study to assess whether any such ownership changes have occurred. We may experience such ownership changes in the future. As a result, if, and to the extent that, we earn net taxable income, our ability to use our NOL carryforwards and research and development tax credit carryforwards to offset such taxable income may be subject to limitations.

Additionally, the use of the U.K. NOL carryforwards could be restricted, under Part 14 of the Corporation Tax Act 2010, if a "change in ownership" of either PepGen Inc. or PepGen Limited were to occur and certain other conditions are met. A "change in ownership" is defined, broadly, as the acquisition by one or more persons of more than half of the ordinary share capital of a company. The use of the U.K. NOL carryforwards could be restricted if, within a certain period of a change in ownership, there is a major change in the conduct of PepGen Limited's trade, PepGen Limited's trading activities become small or negligible, or if certain other conditions are met.

Any restructuring or change in the nature of our operations of our company may give rise to tax liabilities and/or restrictions in the amount and/or availability of tax attributes.

We have undergone, and may in the future undertake, changes in the nature or conduct of our operations. For example, pursuant to an asset transfer agreement effective as of January 1, 2022, we effected a novation of all intellectual property assets of our wholly-owned U.K. subsidiary PepGen Limited to PepGen Inc., which resulted in the recording of a tax charge of $3.7 million, including $0.7 million related to an uncertain tax position. PepGen Limited was dissolved on December 10, 2024 and we recognized a $0.7 million tax benefit associated with the resolution of the uncertain tax position. Any future actions regarding transfer of assets from our U.K. subsidiary or other potential international subsidiaries could give rise to tax liabilities for us and/or to the erosion of our tax attributes (such as NOLs).

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.

As a public company, we are exposed to the risk of securities class action litigation.

Historically, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years. For example, a putative class action complaint was filed on June 9, 2025 in the United States District Court for the Eastern District of New York, entitled Johnny Karam v. PepGen Inc., et al., which was subsequently voluntarily dismissed in November 2025. However, whether or not plaintiff's claims are successful, this type of litigation is often expensive and diverts management's attention and resources, which could adversely affect the operation of our business. If we are ultimately required to pay significant defense costs, damages or settlement amounts, such payments could adversely affect our operations.

We may be the target of similar litigation in the future. Any future litigation could result in substantial costs and divert our management's attention and resources, which could cause serious harm to our business, operating results and financial condition. We maintain liability insurance; however, if any costs or expenses associated with this or any other litigation exceed our insurance coverage, we may be forced to bear some or all of these costs and expenses directly, which could be substantial.

We may be exposed to significant foreign exchange risk.

We incur portions of our expenses, and may in the future derive revenues, in a variety of currencies. As a result, we are exposed to foreign currency exchange risk as our results of operations and cash flows are subject to fluctuations in foreign currency exchange rates. Fluctuations in currency exchange rates have had, and will continue to have, an impact on our results as expressed in U.S.

dollars. We currently do not engage in hedging transactions to protect against uncertainty in future exchange rates between particular foreign currencies and the euro. We cannot predict the impact of foreign currency fluctuations, and foreign currency fluctuations in the future may adversely affect our financial condition, results of operations and cash flows.

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

Unregistered Sales of Equity Securities

None.

Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

During the third quarter ended September 30, 2026, none of our directors or officers (as defined in Rule 16a-1(f) of the Exchange Act) adopted or terminated any contract, instruction or written plan for the purchase or sale of our securities intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act or any "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K).

Item 6. Exhibits.

Exhibit Number	Description
3.1*	Third Amended and Restated Certificate of Incorporation (Incorporated by reference to Exhibit 3.1 to the Company's Quarterly Report on Form 10-Q filed on June 16, 2022 (File No. 001-41374)).
3.2*	Certificate of Correction to Third Amended and Restated Certificate of Incorporation, filed with the Secretary of State of Delaware on November 7, 2022 (Incorporated by reference to Exhibit 3.2 to the Company's Quarterly Report on Form 10-Q filed on November 10, 2022 (File No. 001-41374)).
3.3*	Certificate of Amendment to the Third Amended and Restated Certificate of Incorporation of PepGen Inc. (Incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on June 20, 2024 (File No. 001-41374))
3.4*	Second Amended and Restated By-laws (Incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on March 15, 2024 (File No. 001-41374)).
4.1*	Amended and Restated Investors' Rights Agreement, dated July 30, 2021, among the Company and certain of its stockholders (Incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1, as amended (File No. 333-264335)).
4.2*	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.2 to the Company's Registration Statement on Form S-1, as amended (File No. 333-264335)).
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 Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2+	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Previously Filed

** Filed herewith

+ The certification furnished in Exhibits 32.1 and 32.2 hereto are deemed to accompany this Form 10-Q and will not be deemed "filed" for purposes of Section 18 of the Exchange Act. Such certification will not be deemed to be incorporated by reference into any filings under the Securities Act, or the Exchange Act, except to the extent that the Company specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

PEPGEN INC.

Date: November 10, 2026 By: /s/ James McArthur

James McArthur
Chief Executive Officer

Date: November 10, 2026 By: /s/ Noel Donnelly

Noel Donnelly
Chief Financial Officer

Exhibit 31.1

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

I, James McArthur, certify that:

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

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

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

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

Date: November 10, 2026

By: /s/ James McArthur

James McArthur

Chief Executive Officer

(Principal Executive Officer)

Exhibit 31.2

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

I, Noel Donnelly, certify that:

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

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

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

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

Date: November 10, 2026

By: /s/ Noel Donnelly

Noel Donnelly

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION 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 PepGen Inc. (the "Company") for the quarter ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company hereby certifies, pursuant to 18 U.S.C. §1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, to the best of my knowledge, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; 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/ James McArthur

James McArthur

Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION 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 PepGen Inc. (the "Company") for the quarter ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company hereby certifies, pursuant to 18 U.S.C. §1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, to the best of my knowledge, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; 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/ Noel Donnelly

Noel Donnelly

Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)