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, 2024

OR

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

For the transition period from _____ to _____

Commission File Number: 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 1, 2024, the registrant had 32,595,737 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 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 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 candidates 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 candidates are approved;
- our ability to obtain funding for our operations necessary to complete further development and commercialization of our product candidates;
- our ability to obtain and maintain regulatory approval of our 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;
- developments relating to our competitors and our industry;
- the effects of the COVID-19 pandemic, or any future 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 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 10-Q and the documents that we reference in this 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 10-Q represent our views as of the date of this 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 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 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 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 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 10-Q.

TRADEMARKS

This 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 have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.
- We will need to raise substantial additional funding. If we are unable to raise capital 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. We have only completed a Phase 1 clinical trial and initiated Phase 2 clinical trials for our lead product candidate, as well as initiated a Phase 1 clinical trial and more recently, a Phase 2 clinical trial of a second product candidate, 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.
- Our business is highly dependent on the clinical advancement of our programs and modalities and is especially dependent on the success of our lead product candidates, PGN-EDO51 and PGN-EDODM1. Delay or failure to advance programs or modalities, including PGN-EDO51 and PGN-EDODM1, could adversely impact our business.
- Preclinical and clinical development involves a lengthy and expensive process with an uncertain outcome, and the results of preclinical and clinical studies, including studies of PGN-EDO51, PGN-EDODM1 and PGN-EDO53, are not necessarily predictive of the results of later preclinical studies and any clinical trials of our product candidates. Our product candidates may not have favorable results in clinical trials, if any, or receive regulatory approval on a timely basis, if at all.
- Substantial delays in the commencement, enrollment or completion of our clinical trials and advancement of our clinical trials, or failure to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities could prevent us from commercializing product candidates we determine to develop on a timely basis, if at all.
- 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 have to replace one or more of these third parties, we may be unable to enter into new agreements in a timely manner or on reasonable terms, if at all.
- 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.
- If we are unable to obtain and maintain patent protection for our Enhanced Delivery Oligonucleotide, or EDO, 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 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 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)

	September 30, 2024 (unaudited)	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 42,289	$ 80,774
Marketable securities	96,568	29,633
Prepaid expenses and other current assets	3,512	2,271
Total current assets	$ 142,369	$ 112,678
Property and equipment, net	$ 3,901	$ 4,764
Operating lease right-of-use asset	21,990	23,620
Restricted cash	1,548	1,548
Other assets	426	442
Total assets	$ 170,234	$ 143,052
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,930	$ 1,005
Accrued expenses	7,809	13,522
Operating lease liability	3,071	3,004
Total current liabilities	$ 16,810	$ 17,531
Operating lease liability, net of current portion	15,969	17,100
Total liabilities	$ 32,779	$ 34,631
Commitments and contingencies (Note 7)		
Stockholders' equity (deficit)		
Preferred stock	$ —	$ —
Common stock	3	2
Additional paid-in capital	386,582	289,867
Accumulated other comprehensive income	91	34
Accumulated deficit	(249,221)	(181,482)
Total stockholders' equity	$ 137,455	$ 108,421
Total liabilities and stockholders' equity	$ 170,234	$ 143,052

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,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 17,722	$ 20,540	$ 57,517	$ 51,826
General and administrative	5,449	4,240	15,877	12,129
Total operating expenses	$ 23,171	$ 24,780	$ 73,394	$ 63,955
Operating loss	$ (23,171)	$ (24,780)	$ (73,394)	$ (63,955)
Other income (expense)				
Interest income	1,826	1,578	5,682	5,054
Other (expense) income, net	(39)	(88)	(27)	(230)
Total other income, net	1,787	1,490	5,655	4,824
Net loss before income tax	$ (21,384)	$ (23,290)	$ (67,739)	$ (59,131)
Income tax expense	—	—	—	—
Net loss	$ (21,384)	$ (23,290)	$ (67,739)	$ (59,131)
Net loss per share, basic and diluted	$ (0.66)	$ (0.98)	$ (2.17)	$ (2.49)
Weighted-average common shares outstanding, basic and diluted	32,581,542	23,790,430	31,240,622	23,788,950
Other comprehensive (loss) income:				
Cumulative translation adjustment arising during the period	$ (20)	$ (70)	$ (72)	$ 24
Unrealized gain on marketable securities	186	—	129	—
Comprehensive loss	$ (21,218)	$ (23,360)	$ (67,682)	$ (59,107)

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	Accumulated Other Comprehensive (Loss)	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income	Deficit	Equity (Deficit)
Balance as of December 31, 2023	23,823,241	$ 2	$ 289,867	$ 34	$ (181,482)	$ 108,421
Issuance of stock in public offering, net of underwriters' fees and issuance costs of $3,730	7,530,000	1	76,351	—	—	76,352
Issuance of common stock from At-the-Market Sales Agreement, net of underwriters' fees of $100	1,000,000	—	9,900	—	—	9,900
Exercise of stock options	36,194	—	395	—	—	395
Stock-based compensation expense	—	—	2,022	—	—	2,022
Unrealized loss on marketable securities	—	—	—	(20)	—	(20)
Cumulative translation adjustment arising during the period	—	—	—	(51)	—	(51)
Net loss	—	—	—	—	(18,020)	(18,020)
Balance as of March 31, 2024	32,389,435	3	378,535	(37)	(199,502)	178,999
Exercise of stock options	128,354	—	1,403	—	—	1,403
Issuance of stock under the employee stock purchase plan	32,614	—	149	—	—	149
Stock-based compensation expense	—	—	2,862	—	—	2,862
Unrealized loss on marketable securities	—	—	—	(37)	—	(37)
Cumulative translation adjustment arising during the period	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(28,335)	(28,335)
Balance as of June 30, 2024	32,550,403	3	382,949	(75)	(227,837)	155,040
Exercise of stock options	39,299	—	407	—	—	407
Stock-based compensation expense	—	—	3,226	—	—	3,226
Unrealized gain on marketable securities	—	—	—	186	—	186
Cumulative translation adjustment arising during the period	—	—	—	(20)	—	(20)
Net Loss	—	—	—	—	(21,384)	(21,384)
Balance as of September 30, 2024	32,589,702	$ 3	$ 386,582	$ 91	$ (249,221)	$ 137,455
Balance as of December 31, 2022	23,713,196	$ 2	$ 282,566	$ (81)	$ (102,856)	$ 179,631
Stock-based compensation expense	—	—	1,348	—	—	1,348
Exercise of stock options	68,709	—	130	—	—	130
Net loss	—	—	—	—	(16,319)	(16,319)
Cumulative translation adjustment arising during the period	—	—	—	53	—	53
Balance as of March 31, 2023	23,781,905	2	284,044	(28)	(119,175)	164,843
Stock-based compensation expense	—	—	1,837	—	—	1,837
Exercise of stock options	31,642	—	85	—	—	85
Net loss	—	—	—	—	(19,522)	(19,522)
Cumulative translation adjustment arising during the period	—	—	—	41	—	41
Balance as of June 30, 2023	23,813,547	2	285,966	13	(138,697)	147,284
Stock-based compensation expense	—	—	1,941	—	—	1,941
Cumulative translation adjustment arising during the period	—	—	—	(70)	—	(70)
Net loss	—	—	—	—	(23,290)	(23,290)
Balance as of September 30, 2023	23,813,547	$ 2	$ 287,907	$ (57)	$ (161,987)	$ 125,865

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,	
	2024	**2023**
Cash flows from operating activities:		
Net loss	$ (67,739)	$ (59,131)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation	1,125	830
Stock-based compensation expense	8,110	5,126
Amortization and interest accretion related to operating lease	2,811	(212)
Amortization and accretion of premium and discounts on marketable securities, net	(2,905)	—
Other non-cash adjustments	20	—
Changes in operating assets and liabilities:		
Prepaids and other current and non-current assets	(1,239)	757
Accounts payable	4,916	2,561
Accrued expenses and other non-current liabilities	(5,750)	1,985
Operating lease liabilities, current and non-current	(2,246)	(1,489)
Net cash used in operating activities	$ (62,897)	$ (49,573)
Cash flows from investing activities:		
Purchases of property and equipment	(269)	(2,415)
Purchases of marketable securities	(123,900)	—
Maturities of marketable securities	60,000	—
Net cash used in investing activities	(64,169)	(2,415)
Cash flows from financing activities:		
Issuance of common stock upon initial public offering, net of underwriters' fees	76,878	—
Payment of offering costs	(511)	(430)
Issuance of common stock from At-the-Market Sales Agreement, net of underwriters' fees	9,900	—
Proceeds from employee equity plans	2,354	215
Net cash provided by financing activities	$ 88,621	$ (215)
Effect of exchange rate changes on cash	(40)	64
Net decrease in cash, cash equivalents and restricted cash	$ (38,485)	$ (52,139)
Cash, cash equivalents and restricted cash at beginning of period	82,322	183,225
Cash, cash equivalents and restricted cash at end of period	$ 43,837	$ 131,086
Components of cash, cash equivalents and restricted cash		
Cash and cash equivalents	$ 42,289	$ 129,538
Restricted cash	1,548	1,548
Total cash, cash equivalents and restricted cash at end of period	$ 43,837	$ 131,086
Supplemental noncash investing and financing activities		
Property and equipment included in accounts payable and accrued expenses	$ 14	$ 123
Deferred offering costs in accounts payable and accrued expenses	—	12
Cash paid for taxes	71	—

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.

The Company was initially formed as PepGen Limited on January 25, 2018, in the United Kingdom, or the U.K. On November 9, 2020, PepGen Limited completed a corporate reorganization, or the Reorganization. As part of the Reorganization, PepGen Limited formed PepGen, a Delaware corporation with nominal assets and liabilities, for the purpose of consummating the Reorganization. In connection with the Reorganization, the existing stockholders of PepGen Limited exchanged each of its classes of shares of PepGen Limited for the same number and class of common stock of PepGen on a one-to-one basis. The newly issued stock of PepGen had substantially identical rights to the exchanged shares of PepGen Limited. As a result of the exchange, PepGen became the sole stockholder of PepGen Limited. Upon the completion of the Reorganization on November 23, 2020, the historical financial statements of PepGen Limited became the historical financial statements of PepGen as the Reorganization was deemed to be between entities under common control.

Liquidity and Capital Resources

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, 2024, the Company had an accumulated deficit of $249.2 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 a follow-on public offering, referred to as the Follow-on Offering. As of September 30, 2024, the Company had cash, cash equivalents, and marketable securities of $138.9 million. The Company believes that its cash, cash equivalents, and marketable securities as of September 30, 2024, will be sufficient to fund its currently planned operations for at least the next 12 months from the issuance of these unaudited condensed consolidated financial statements.

The Company filed a shelf registration statement on Form S-3 with the United States Securities and Exchange Commission, or SEC, which covers the offering, issuance and sale of an amount up to $300.0 million in the aggregate of shares of 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, the Company filed a prospectus supplement and entered into the Sales Agreement with Stifel, as sales agent, which provides for the issuance and sale by the Company of up to $100.0 million of shares of common stock from time to time in “at-the-market” offerings. The sales agent is entitled to receive a commission of up to 3.0% of gross proceeds from sales under the Sales Agreement. On February 5, 2024, the Company sold 1,000,000 shares of common stock at a price of $10.00 per share under the Sales Agreement, resulting in net proceeds of $9.9 million. On February 9, 2024, the Company sold 7,530,000 shares of common stock in the Follow-on Offering, pursuant to an underwriting agreement with Leerink Partners LLC dated as of February 6, 2024, at a price of $10.635 per share resulting in net proceeds of $76.4 million after deducting underwriting fees and offering costs of $3.7 million. Net proceeds from sales under the Sales Agreement and Follow-on Offering, after deducting underwriters' fees and costs, were $86.3 million. On June 28, 2024, the Company 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 common stock, preferred stock, debt securities, warrants, and/or units or any combination thereof, which was declared effective on July 8, 2024.

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.

Restricted cash on the condensed consolidated balance sheet as of December 31, 2023, previously included in other assets, has been presented as a separate line item. The condensed consolidated financial statements have otherwise 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, 2024, and results of operations for the interim periods ended September 30, 2024 and September 30, 2023.

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, 2023 and 2022, and the notes thereto, included in the Company's Annual Report on Form 10-K, filed with the SEC on March 6, 2024, or Form 10-K, as amended by the Form 10-K/A filed on March 29, 2024.

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, 2023 included in the Form 10-K. Since the date of those financial statements, there have been no changes to the Company's significant accounting policies.

Recently Issued Accounting Pronouncements Not Yet Adopted

In November 2023, the Financial Accounting Standards Board issued ASU 2023-07, *Segment Reporting (Topic 280)*. This update expands segment disclosure requirements, including new segment disclosure requirements for entities with a single reportable segment among other disclosure requirements. This update is effective for the Company in the consolidated financial statements for the year ending December 31, 2024, and interim periods beginning on January 1, 2025. The adoption of this standard only impacts disclosures and is not expected to have a material impact on the Company's consolidated financial statements.

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, 2024 and December 31, 2023, 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. Fair value determined by Level 2 inputs utilize observable inputs other than Level 1 prices, such as quoted prices for similar assets or liabilities, quoted market prices in markets that are not active or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities. Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability. The following tables set forth marketable securities as of September 30, 2024 and December 31, 2023 (in thousands):

	As of September 30, 2024			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
U.S. Treasury notes	$ 96,439	$ 144	$ (15)	$ 96,568
Total	$ 96,439	$ 144	$ (15)	$ 96,568

	As of December 31, 2023			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
U.S. Treasury notes	29,622	11	—	29,633
Total	$ 29,622	$ 11	$ —	$ 29,633

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, 2024 and December 31, 2023 (in thousands):

	As of September 30, 2024			
	Total	Level 1	Level 2	Level 3
Cash Equivalents:				
U.S. Treasury-backed money market funds	$ 35,474	$ 35,474	$ —	$ —
Marketable Securities:				
U.S. Treasury notes	96,568	96,568	—	—
Total	$ 132,042	$ 132,042	$ —	$ —

	As of December 31, 2023			
	Total	Level 1	Level 2	Level 3
Cash Equivalents:				
U.S. Treasury-backed money market funds	$ 64,397	$ 64,397	$ —	$ —
U.S. Treasury notes	$ 11,980	$ 11,980	$ —	$ —
Marketable Securities:				
U.S. Treasury notes	$ 29,633	$ 29,633	$ —	$ —
Total	$ 106,010	$ 106,010	$ —	$ —

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 unaudited condensed consolidated balance sheet under cash and cash equivalents.

4. Property and Equipment, Net

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

	September 30, 2024	December 31, 2023
Lab equipment	$ 4,989	$ 4,821
Computer and office equipment	1,557	1,446
Construction in process	45	69
Total property and equipment	6,591	6,336
Less: accumulated depreciation	(2,690)	(1,572)
Total property and equipment, net	$ 3,901	$ 4,764

Depreciation expense was $0.4 million and $0.3 million for the three months ended September 30, 2024 and September 30, 2023, respectively. Depreciation expense was $1.1 million and $0.8 million for the nine months ended September 30, 2024 and September 30, 2023, respectively.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Research and development expenses	$ 2,679	$ 9,521
Employee-related expenses	3,142	2,368
Professional services	1,113	715
Other	875	918
Total accrued expenses	$ 7,809	$ 13,522

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 world wide 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. 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 ($26.7 million and $40.1 million as of September 30, 2024), 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.

During the three and nine months ended September 30, 2024, one member of the Company's board of directors, Dr. Christopher Ashton, was employed by Oxford Science Enterprises, or OSE, which is an affiliate of OUI. On September 19, 2024, the Company announced that Dr. Ashton had resigned from the board of directors, effective as of September 30, 2024.

As of September 30, 2024, OSE owned 14.6% of the Company's outstanding common stock. Pursuant to the terms of Dr. Ashton's employment agreement with OSE, he was obligated to transfer any cash compensation from the Company to OSE. Fees paid in cash for Dr. Ashton's service on the Company's board of directors were paid directly to OSE and were included within general and administrative expense on the unaudited condensed consolidated statement of operations. The Company paid $13,125 and $11,875 for the three months ended September 30, 2024 and 2023, respectively and $36,875 and $35,625 for the nine months ended September 30, 2024 and 2023, respectively to OSE as compensation for Dr. Ashton's service on the board of directors.

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 and the Company's Follow-on Offering in February 2024. As of September 30, 2024, entities affiliated with RA Capital owned 32.8% 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 unaudited condensed consolidated statement of operations. The Company paid $12,000 and $10,750 for the three months ended September 30, 2024 and 2023, respectively and $33,500 and $32,250 for the nine months ended September 30, 2024 and 2023, 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.

The Company is not party to any 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, 2024. 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. No cash dividends were declared by the board of directors during the three or nine months ended September 30, 2024 and September 30, 2023.

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

	September 30, 2024	December 31, 2023
Stock options issued and outstanding	5,670,753	4,233,203
Unvested restricted stock and performance stock units	102,820	—
Authorized for future stock awards or option grants under the 2022 Plan	740,948	1,091,187
Authorized for future stock awards or option grants under the 2024 Inducement Plan	797,184	—
Stock options issued and outstanding under the employee stock purchase plan	421,924	216,306
Stock options issued and outstanding	7,733,629	5,540,696

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. As of September 30, 2024, 740,948 shares remained available for grant under the 2022 Plan and 421,924 shares remained available for issuance under the ESPP.

2024 Inducement Plan

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, 2024, 797,184 shares remained available for issuance under the Inducement Plan.

Stock Option Activity

Stock option activity for the nine months ended September 30, 2024, was as follows:

	Stock Options		Weighted-Average Exercise Price
Outstanding as of December 31, 2023	4,233,203	$	10.83
Granted	1,974,483	$	15.04
Exercised	(203,847)	$	10.82
Canceled/Forfeited	(333,086)	$	13.37
Outstanding as of September 30, 2024	5,670,753	$	12.15

The weighted-average grant date fair value of options granted during the nine months ended September 30, 2024 was $10.83 per share. The Company had 5,670,753 unvested stock options outstanding as of September 30, 2024. As of September 30, 2024, total unrecognized compensation cost related to stock options was $32.6 million. This amount is expected to be recognized over a weighted average period of approximately 2.67 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 vests fully on the one-year anniversary of the grant date, provided that the employee remains employed with the Company. 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. Such awards cannot vest in less than one year, regardless of when the performance milestone is achieved. The Company's chief executive officer did not receive any RSU or PSU awards. A summary of the Company's RSU and PSU activity and related information for the nine months ended September 30, 2024 is as follows:

	Time-Based RSUs	PSUs		Weighted-Average Grant Date Fair Value
Outstanding as of December 31, 2023	—	—	$	—
Granted	77,645	28,555		10.64
Vested	—	—		—
Forfeited	(3,380)	—		10.64
Issued and unvested as of September 30, 2024	74,265	28,555	$	10.64

The weighted average grant date fair value of the time-based RSUs and the PSUs granted during the nine months ended September 30, 2024 was $10.64. As of September 30, 2024, there was $0.3 million of unrecognized compensation costs related to unvested time-based RSUs, which are expected to be recognized over a weighted-average period of 0.4 years, and $0.3 million of unrecognized compensation costs related to unvested PSUs, which are dependent upon achievement of the aforementioned clinical development milestones.

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,	
	2024	2023	2024	2023
Research and development	$ 1,419	$ 785	$ 3,433	$ 2,167
General and administrative	1,807	1,156	4,677	2,959
Total stock-based compensation expense	$ 3,226	$ 1,941	$ 8,110	$ 5,126

10. Income Taxes

The Company recorded income tax expense of nil for the three months ended September 30, 2024 and 2023 and for the nine months ended September 30, 2024 and 2023.

On January 1, 2022, or the Transfer Date, the Company's wholly owned subsidiary, PepGen Limited, transferred all intellectual property, or IP, assets to the parent company, PepGen, in an arm's length transaction at fair value pursuant to an asset transfer agreement. The fair value of the IP assets is a non-recurring fair value measurement. The Company engaged valuation specialists to calculate the IP value, and the IP value was measured using the historical cost method. The historical cost method estimated the fair value of the IP assets using the historical cost base of the IP assets and the expected market return as of the Transfer Date. The significant assumption inherent in estimating the fair value using the historical cost method was the expected market return. The Company utilized a 40% expected market return, which a third-party investor may expect as a return on their investment, and which is based on studies of venture capital investment returns. The Company calculated the fair value of the IP assets by computing the present value of the historical cost base using the 40% expected market return. The assumptions used in the estimation of the IP assets represent level 3 inputs of the fair value hierarchy.

The transfer of the IP assets resulted in an estimated tax charge during 2022 to His Majesty's Revenue & Customs, or HMRC, after considering net operating loss carryforwards, of $4.4 million, inclusive of the $0.7 million uncertain tax position. The Company paid amounts owed to HMRC in September 2023.

The Company recognizes the impact of an uncertain income tax position taken on its income tax returns at the amount that is more likely than not to be sustained upon audit by the relevant taxing authority. An uncertain income tax position will be recognized if it has less than a 50% likelihood of being sustained. The tax positions are analyzed at least quarterly, and adjustments are made as events occur that warrant adjustments for those positions. As of September 30, 2024, the Company maintains the liability for the uncertain tax position related to the IP transfer of $0.7 million, which is accounted for in accrued expenses on the unaudited condensed consolidated balance sheet.

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 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this 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 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 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, we are generating a pipeline of oligonucleotide therapeutic candidates that are designed to target the root cause of serious diseases.

We are focused on addressing the underlying cause of Duchenne muscular dystrophy, or DMD, and myotonic dystrophy type 1, or DM1, that have high unmet need. Our current pipeline consists of two clinical stage programs, PGN-EDO51 for DMD patients who are amenable to exon 51 skipping and PGN-EDODM1 for DM1 patients, along with several additional preclinical stage programs. We anticipate expanding this pipeline over time to include other neuromuscular targets as well as potential opportunities in neurologic diseases.

We completed a first-in-human Phase 1 clinical trial in healthy volunteers, or HVs, with our lead product candidate, PGN-EDO51, in the third quarter of 2022. In the Phase 1 HV clinical trial, treatment with PGN-EDO51 resulted in the highest levels of exon skipping in humans following a single dose compared to publicly available data for a single dose of other DMD exon 51-skipping approaches that are approved or in clinical development.

Our clinical development program for PGN-EDO51 comprises two parallel Phase 2 studies of PGN-EDO51 in DMD patients whose mutations are amenable to an exon 51-skipping approach. The first study, CONNECT1-EDO51, or CONNECT1, is an ongoing open-label, multiple ascending dose, or MAD, Phase 2 study in boys and young men living with DMD being conducted in Canada. The CONNECT1 study was designed to provide proof-of-concept for our EDO platform and PGN-EDO51, as well as inform the design and conduct of our CONNECT2-EDO51, or CONNECT2, study. In July 2024, we reported initial clinical data from the CONNECT1 study from the 5 mg/kg starting dose cohort (n=3). PGN-EDO51 produced mean exon skipping in biceps tissue of 2.15% at week 13 compared to baseline, measured by RT-PCR. In addition, PGN-EDO51 achieved a mean muscle-adjusted dystrophin level of 1.49% of normal (0.70% increase from baseline) and a mean absolute dystrophin level (by Western blot analysis) of 0.61% of normal (0.26% increase from baseline), in each case after four doses, measured at week 13. As of October 31, 2024, all three participants in this cohort were continuing to be dosed with PGN-EDO51 at 5 mg/kg in the long-term extension, or LTE, phase of the clinical trial. As of October 31, 2024, a total of 10 doses had been administered at 10 mg/kg in the ongoing CONNECT1 study. Based on the totality of data in both the 5 mg/kg cohort and the ongoing 10 mg/kg cohort in the CONNECT1 trial as of October 31, 2024, we believe PGN-EDO51 has a favorable emerging safety profile. There have been no treatment-related serious adverse events, or SAEs, and all treatment-related adverse events have been mild or moderate. There was no sustained elevation in kidney biomarkers. There were also no changes in hepatic function and no cases of hypokalemia, anemia or thrombocytopenia. Asymptomatic hypomagnesemia has been observed in two participants in the 10 mg/kg cohort and treated with oral magnesium supplementation. As of October 31, 2024, all participants continue in the study as planned, with no discontinuations, dose modifications or dose interruptions.

Based on learnings from the 5 mg/kg cohort, we have amended the CONNECT1 study protocol. The changes include adjusting the timing for the final biopsy from Day 7 to Day 28, adjusting the Performance of Upper Limb, or PUL, test entry score from three to four for inclusion, and adjusting the eligible age group from eight years of age and older to six - 16 years of age, all subject to regulatory clearance. We have also expanded the 10 mg/kg cohort from three to four participants. With these adjustments, we expect to report results from the 10 mg/kg cohort by year-end 2025.

The second Phase 2 study of PGN-EDO51, CONNECT2, is a multinational, randomized, double-blind, placebo-controlled MAD study. The CONNECT2 study will evaluate multiple dose cohorts and trial participants will be administered PGN-EDO51 once every four weeks for six months. We will assess safety, tolerability, exon skipping, dystrophin expression and functional outcomes in this study. Based on the data from CONNECT1, including PGN-EDO51's favorable emerging safety profile to date, we are also working to optimize the design of the CONNECT2 trial. The CONNECT2 study, together with data from the CONNECT1 study, is intended to support a potential accelerated approval pathway for PGN-EDO51, subject to regulatory authority feedback.

In February 2024, we received clearance from the Medicines and Healthcare products Regulatory Agency, or the MHRA, to initiate CONNECT2 in the United Kingdom, or U.K. We plan to continue to engage with regulators in the European Union, or the EU,

regarding the CONNECT2 study and expect to file an investigational new drug, or IND, application and open the clinical trial in the U.S. by year-end, subject to regulatory clearance.

The U.S. Food and Drug Administration, or the FDA has granted both orphan drug designation and rare pediatric disease designation, or RPDD, for PGN-EDO51 for the treatment of DMD patients who are amenable to exon 51 skipping.

We are also 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. In May 2023, we announced that we received a clinical hold notice from the FDA, regarding our investigational new drug, or IND, application to initiate our first-in-human Phase 1 FREEDOM-DM1, or FREEDOM, study. In September 2023, we announced that Health Canada had cleared our Clinical Trial Application, or CTA, for the FREEDOM study in Canada. In October 2023, we announced that the FDA lifted the clinical hold on the FREEDOM study, allowing this study to proceed in the U.S. In December 2023, we announced that the MHRA had cleared our CTA for the FREEDOM study in the U.K. and we began dosing patients in this study. FREEDOM is a multinational, randomized, double-blind, placebo-controlled, single ascending dose, or SAD, study designed to assess PGN-EDODM1's safety and tolerability, splicing correction and functional outcome measures in DM1 patients. We expect to report data from both the 5 mg/kg and 10 mg/kg dose cohorts in this study by the end of the first quarter of 2025.

The safety data from the ongoing FREEDOM study has informed the design of the FREEDOM2-DM1, or FREEDOM2, Phase 2 randomized, double-blind, placebo-controlled MAD study of PGN-EDODM1 in DM1 patients. FREEDOM2 is designed to assess PGN-EDODM1's safety and tolerability, splicing correction and functional outcome measures in DM1 patients. Both Health Canada and the MHRA have cleared our CTA filings for the FREEDOM2 study, and we expect to initiate patient dosing in the fourth quarter of 2024. We also plan to open the FREEDOM2 study in the U.S., subject to regulatory clearance.

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

In addition to these lead candidates, we are developing EDO candidates for additional DMD sub-populations amenable to skipping of other exons, including exon 53, 45 and 44. We have previously reported robust exon 53-skipping levels following either a single dose or multiple doses in non-human primates, or NHPs, for our PGN-EDO53 program. We continue to evaluate PGN-EDO53 in CTA and/or IND-enabling preclinical studies.

Initial Public Offering, ATM Program, Follow-on Offering 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 of common stock shares 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 Follow-on Offering. The Follow-on Offering resulted in net proceeds of $76.4 million after deducting underwriters' fees of $3.7 million. Net proceeds from the ATM program and Follow-on Offering, after deducting underwriters' fees and costs of the offerings, were $86.3 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 and the Follow-on Offering.

We have incurred operating losses in each year since our inception. Our net losses were $67.7 million and $59.1 million for the nine months ended September 30, 2024 and September 30, 2023, respectively. As of September 30, 2024, we had cash, cash equivalents, and marketable securities of $138.9 million. As of September 30, 2024, we had an accumulated deficit of $249.2 million. We expect our expenses and operating losses will continue as we conduct our ongoing preclinical studies and current and planned

clinical trials, 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 and current and planned clinical trials and our expenditures on other research and development activities.

Based on our currently planned operations, we believe that our existing cash, cash equivalents and marketable securities, will be sufficient to fund our operations into 2026. 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.

Corporate Reorganization

We were initially formed as PepGen Limited on January 25, 2018, in the U.K. On November 9, 2020, PepGen Limited initiated a corporate reorganization, or the Reorganization. As part of the Reorganization, PepGen Limited formed PepGen, a Delaware corporation with nominal assets and liabilities, for the purpose of consummating the Reorganization. In connection with the Reorganization, the existing shareholders of PepGen Limited exchanged each of their classes of shares of PepGen Limited for the same number and class of common stock of PepGen on a one-to-one basis. The newly issued stock of PepGen had substantially identical rights to the exchanged shares of PepGen Limited. As a result of the exchange, PepGen became the sole shareholder of PepGen Limited. Upon the completion of the Reorganization on November 23, 2020, the historical financial statements of PepGen Limited became the historical financial statements of PepGen, as the Reorganization was deemed to be between entities under common control.

After the Reorganization was completed, PepGen Limited began the process of transferring operations, including financial management functions, to PepGen pursuant to an intercompany services agreement, effective as of April 2021, and certain assets, including a novation of all intellectual property assets, pursuant to an asset transfer agreement, effective as of January 1, 2022. After the transfer of intellectual property and other assets from PepGen Limited to PepGen, there were limited operations through the end of 2022 at PepGen Limited.

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, 2024 and September 30, 2023. 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,	
	2024	2023	2024	2023
External expenses:				
PGN-EDO51	$ 2,826	$ 7,790	$ 15,270	$ 16,146
PGN-EDODM1	5,131	5,154	15,979	12,588
PGN-EDO53	337	111	494	207
Other programs and unallocated expenses	517	588	1,278	3,700
Total external expense	8,811	13,643	33,021	32,641
Internal expenses:				
Personnel-related (including stock-based compensation)	6,332	4,629	17,126	12,001
Facilities and related costs	1,426	1,269	4,374	3,884
Other	1,153	999	2,996	3,300
Total research and development expenses	$ 17,722	$ 20,540	$ 57,517	$ 51,826

We expect to increase our research and development expenses in the remainder of 2024, as compared to the same period in 2023, as we conduct our ongoing Phase 2 clinical trials for PGN-EDO51 and our ongoing Phase 1 clinical trial of PGN-EDODM1, and potentially initiate dosing in our FREEDOM2 Phase 2 clinical trial for PGN-EDODM1. We also continue to increase our research and development headcount to support these programs. Research and development expenses for our lead programs 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 any of our product candidates.

The timelines and costs associated with research and development activities are uncertain and can vary significantly for each product candidate and development program due to the inherently unpredictable nature of preclinical and clinical development. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to preclinical and clinical results, regulatory developments, and ongoing assessments as to each program's commercial potential. 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, animal and other preclinical studies and IND- 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.

We anticipate that our general and administrative expenses will increase in 2024 as compared to 2023 primarily due to personnel costs, including stock-based compensation expense. 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 money market mutual funds and short-term U.S. treasury holdings.

Other income (expense)

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

Income Taxes

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. We did not record any tax liability for the three and nine months ended September 30, 2024 or September 30, 2023.

Results of Operations

Comparison of the Three Months Ended September 30, 2024 and September 30, 2023.

The following table summarizes our results of operations for the three months ended September 30, 2024 and September 30, 2023 (in thousands):

	Three Months Ended September 30,		Period-to-Period Change
	2024	2023	
Operating expenses:			
Research and development	$ 17,722	$ 20,540	$ (2,818)
General and administrative	5,449	4,240	1,209
Total operating expenses	$ 23,171	$ 24,780	$ (1,609)
Operating loss	$ (23,171)	$ (24,780)	$ 1,609
Other income (expense), net			
Interest income	1,826	1,578	248
Other (expense) income, net	(39)	(88)	49
Total other income (expense), net	1,787	1,490	$ 297
Net loss before income tax	$ (21,384)	$ (23,290)	$ 1,906
Income tax expense	—	—	—
Net loss	$ (21,384)	$ (23,290)	$ 1,906

Research and Development Expenses

Research and development expenses decreased by $2.8 million from $20.5 million for the three months ended September 30, 2023, to $17.7 million for the three months ended September 30, 2024. This was attributable to a $3.8 million decrease in manufacturing costs related to the timing of manufacturing campaigns, and a $2.7 million decrease in preclinical costs as our two lead programs are now in clinical trials. These decreases were partially offset by increased costs associated with the advancement of clinical trials for our two lead programs, including a $1.0 million increase in clinical trial costs, and a $1.7 million increase in personnel-related costs, including an increase of $0.6 million in stock-based compensation expense. There was also a $0.6 million increase in consulting expenses and a $0.2 million increase in facility and other office related expenses.

General and Administrative Expenses

General and administrative expenses increased by $1.2 million from $4.2 million for the three months ended September 30, 2023, to $5.4 million for the three months ended September 30, 2024. The increase was primarily driven by an increase of $1.1 million in personnel-related costs due to increased headcount, including $0.7 million in stock-based compensation expense.

Other Income (Expense), Net

Other income (expense), net was income of $1.8 million for the three months ended September 30, 2024 and $1.5 million for the three months ended September 30, 2023. Interest is earned through our cash deposits and U.S. Treasury-backed money market funds.

Income Tax Expense

Income tax expense was nil for the three months ended September 30, 2024 and September 30, 2023.

Comparison of the Nine Months Ended September 30, 2024 and September 30, 2023.

The following table summarizes our results of operations for the nine months ended September 30, 2024 and September 30, 2023 (in thousands):

	Nine Months Ended September 30,		Period-to-Period Change
	2024	2023	
Operating expenses:			
Research and development	$ 57,517	$ 51,826	$ 5,691
General and administrative	15,877	12,129	3,748
Total operating expenses	$ 73,394	$ 63,955	$ 9,439
Operating loss	$ (73,394)	$ (63,955)	$ (9,439)
Other income (expense), net			
Interest income	5,682	5,054	628
Other (expense) income, net	(27)	(230)	203
Total other income (expense), net	$ 5,655	$ 4,824	$ 831
Net loss before income tax	$ (67,739)	$ (59,131)	$ (8,608)
Income tax expense	—	—	—
Net loss	$ (67,739)	$ (59,131)	$ (8,608)

Research and Development Expenses

Research and development expenses increased by $5.7 million from $51.8 million for the nine months ended September 30, 2023, to $57.5 million for the nine months ended September 30, 2024. This increase was attributable to expenses related to the advancement of clinical trials for our two lead programs, including a $7.2 million increase in clinical trial costs, a $1.0 million increase in manufacturing costs, and a $5.1 million increase in personnel-related costs, including an increase of $1.3 million in stock-based compensation expense. Additionally, there was an increase of $0.4 million in facility and other office related expenses, including depreciation, and a $0.2 million increase in consulting expense. These increases were partially offset by a $8.6 million decrease in preclinical costs as our two lead programs are now in clinical trials.

General and Administrative Expenses

General and administrative expenses increased by $3.7 million from $12.1 million for the nine months ended September 30, 2023, to $15.9 million for the nine months ended September 30, 2024. The increase was driven primarily by an increase of $3.3 million in personnel-related costs due to increased headcount, including $1.7 million in stock-based compensation expense, and an increase of $0.6 million in facility and office related expenses related to rent expense for our operating lease and depreciation on office furniture. These increases were partially offset by a $0.2 million decrease in insurance expense.

Other Income (Expense), Net

Other income (expense), net was income of $5.7 million for the nine months ended September 30, 2024 and $4.8 million for the nine months ended September 30, 2023. Interest is earned through our cash deposits and U.S. Treasury-backed money market funds.

Income Tax Expense

Income tax expense was nil for the nine months ended September 30, 2024 and September 30, 2023.

Liquidity and Capital Resources

Sources of Liquidity

From our inception in January 2018 through September 30, 2024, 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.

On February 9, 2024, we issued and sold 7,530,000 shares of common stock in the Follow-on 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.

Future Funding Requirements

As of September 30, 2024, we had cash, cash equivalents, and marketable securities of $138.9 million. Based on our currently planned operations, we believe that our existing cash, cash equivalents, and marketable securities will be sufficient to fund our operations into 2026. However, our forecast of 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 our product candidates, any additional product candidates we may develop and any new indications we may pursue;

•the scope, costs, timing and outcome of regulatory review of our product candidates, 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 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, 2024 and September 30, 2023 (in thousands):

	Nine Months Ended September 30,	
	2024	2023
Net cash (used in) provided by:		
Operating activities	$ (62,897)	$ (49,573)
Investing activities	(64,169)	(2,415)
Financing activities	88,621	(215)
Effect of exchange rate changes on cash	(40)	64
Net decrease in cash, cash equivalents and restricted cash	$ (38,485)	$ (52,139)

Operating Activities

For the nine months ended September 30, 2024, net cash used in operating activities was $62.9 million resulting from our net loss of $67.7 million, and changes in our operating assets and liabilities of $4.3 million, partially offset by non-cash adjustments of $9.2 million. The net changes in our operating assets and liabilities were primarily due to an increase in accounts payable of $4.9 million related to manufacturing commitments. The change was further driven by a decrease in accrued expenses of $5.8 million, an increase in prepaids and other current assets of $1.2 million, and a decrease in operating lease liabilities of $2.2 million. The $5.8 million decrease in accrued expenses was primarily due to lower research and development accruals as a result of the timing of manufacturing campaigns. The non-cash adjustments included $8.1 million of stock-based compensation, $1.1 million of depreciation expense, $2.9 million of amortization of discounts on our marketable securities, and $2.8 million of amortization and interest accretion on our operating lease.

For the nine months ended September 30, 2023, net cash used in operating activities was $49.6 million resulting from our net loss of $59.1 million partially offset by non-cash adjustments of $5.7 million and changes in our operating assets and liabilities of $3.7 million. The net changes in our operating assets and liabilities were primarily due to an increase in accounts payable of $2.6 million, accrued expenses of $2.0 million, and prepaids and other assets of $0.7 million, and a decrease in operating lease liabilities of $1.5 million. The non-cash adjustments included $5.1 million of stock-based compensation, $0.8 million of depreciation expense, and $0.2 million of amortization and interest accretion on our operating lease.

Investing Activities

For the nine months ended September 30, 2024, net cash used in investing activities was $64.2 million resulting from $123.9 million in purchases of marketable securities partially offset by $60.0 million in maturities and sales of marketable securities.

For the nine months ended September 30, 2023, net cash used in investing activities was $2.4 million related to the purchase of property and equipment, primarily associated with building lab space at our new Boston headquarters.

Financing Activities

For the nine months ended September 30, 2024, net cash provided by financing activities was $88.6 million resulting from $76.9 million in proceeds from the Follow-on Offering, $9.9 million in proceeds from sales under the Sales Agreement, and $2.4 million from proceeds from the purchase of shares under employee equity plans, partially offset by $0.5 million in the payment of deferred offering costs.

Net cash provided by financing activities was $0.2 million during the nine months ended September 30, 2023 related to the payment of deferred offering costs related to the 2023 registration statement on Form S-3 partially offset by proceeds from the purchase of shares under employee equity plans.

Critical Accounting Polices 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, 2023, as amended by the Form 10K/A filed on March 29, 2024.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

As of September 30, 2024, we had $138.9 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, 2024, 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 Depository Insurance Corporation and Financial Services Compensation Scheme.

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, 2024, 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 control 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 control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2024 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, 2024, 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 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 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 $67.7 million and $59.1 million for the nine months ended September 30, 2024 and September 30, 2023, respectively. As of September 30, 2024, we had an accumulated deficit of $249.2 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 early 2024, described below. We have devoted substantially all of our financial resources and efforts to 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. We are still in the early stages of development of our programs and have only advanced two product candidates 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:

- complete preclinical activities for our programs in DMD and DM1 and continue to advance them through clinical development;
- 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;
- 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 clinical, regulatory and scientific personnel;
- 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, one or more of our 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 completed our Phase 1 clinical trial for PGN-EDO51 and initiated two Phase 2 clinical trials for PGN-EDO51, a Phase 1 clinical trial for PGN-EDODM1 and more recently, 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. 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:

•identifying product candidates and completing preclinical development of our product candidates;

•obtaining regulatory clearance to commence clinical trials and initiating and successfully completing such trials;

•obtaining marketing approval for our product candidates;

•manufacturing (or securing third-party manufacturers to manufacture), marketing and selling any products for which we may obtain regulatory approval;

•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 most 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, 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 capital 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 any product candidates that successfully completes clinical testing. To date, we have only completed a Phase 1 clinical trial for our first product candidate, PGN-EDO51 and initiated one additional Phase 1 clinical trial for PGN-EDODM1 and two Phase 2 clinical trials for PGN-EDO51 and one Phase 2 clinical trial for PGN-EDODM1. In addition, if we obtain marketing approval for any 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, 2024, we had cash, cash equivalents, and marketable securities of $138.9 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.

On February 5, 2024, we sold shares of common stock under our ATM program, resulting in net proceeds of $9.9 million. On February 9, 2024, we sold shares of common stock in our Follow-on Offering, resulting in net proceeds of $76.4 million after deducting underwriters' fees of $3.7 million. Net proceeds from the ATM program and Follow-on Offering, after deducting underwriters' fees and costs of the offerings, were $86.3 million.

Based on our currently planned operations, we believe that our existing cash, cash equivalents, marketable securities will be sufficient to fund our operations into 2026. However, we have based this estimate on assumptions that may prove to be wrong, and our operating plans may change as a result of many factors, including factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect and could be forced to seek additional funding sooner than planned.

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

•the scope, progress, costs and results of preclinical and clinical development for our product candidates 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 our product candidates and any additional product candidates we may develop or 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 costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain 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 or the commercialization of any product candidate, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, 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, filing and prosecuting patent applications and providing general and administrative support for these operations. One of our product candidates, PGN-EDO51, completed a Phase 1 clinical trial and we have initiated one additional Phase 1 clinical trial for PGN-EDODM1 and two Phase 2 clinical trials for PGN-EDO51 and more recently, a Phase 2 clinical trial for PGN-EDODM1. All of our other research programs are still 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 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. We have only completed a Phase 1 clinical trial for our lead product candidate and initiated several additional clinical trials, 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. We have a portfolio of research programs and we have two product candidates in clinical trials — PGN-EDO51 for DMD and PGN-EDODM1 for DM1. We have completed a Phase 1 clinical trial for our first product candidate, PGN-EDO51 in HVs. We have initiated our Phase 2 CONNECT1 trial for PGN-EDO51 in Canada, and began dosing patients in January of 2024. In July 2024, we reported initial data from the low dose cohort (5 mg/kg) in this trial. Based on this data, we have amended the CONNECT1 study protocol to implement several changes. We received clearance from the MHRA to initiate our multinational Phase 2 CONNECT2 trial for PGN-EDO51 in February 2024. Based on data from our CONNECT1 trial we are also optimizing the design of the CONNECT2 trial. We have initiated a Phase 1 clinical trial, designated FREEDOM, for our second product candidate, PGN-EDODM1, and began dosing patients in December 2023. We have also opened a Phase 2 clinical trial, FREEDOM2, in Canada and the U.K.

We are evaluating our third product candidate, PGN-EDO53 in preclinical studies, but have not completed IND- or CTA-enabling activities for PGN-EDO53 or any of our other product candidates or advanced any of our other 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 example, 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. Even after initiating FREEDOM in the U.S., the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial, including with respect to our CONNECT2 study of PGN-EDO51 or 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.

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 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, or GLP, 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.

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 candidates are currently in clinical-stage development, while our other product candidates are still in the research or preclinical stage of development and our approach to treating muscle disease is unproven. 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 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.

We have advanced our first two product candidates, PGN-EDO51 and PGN-EDODM1, into the clinic, and have completed a Phase 1 trial of PGN-EDO51 in HVs. However, the positive results we have observed in our preclinical studies and in the completed Phase 1 trial may not be repeated in future clinical trials, including in patients with DMD amenable to an exon-51 skipping approach, and regulatory authorities may disagree with the interpretation of data from our trials.

Although we are advancing our initial programs in DMD and 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;
- 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 only completed a Phase 1 clinical trial of PGN-EDO51 and initiated an additional Phase 1 clinical trial of PGN-EDODM1, two Phase 2 clinical trials of PGN-EDO51 and more recently, 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. Even if the 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 or future programs, or future regulatory approval and commercialization. Although we have completed a Phase 1 study of our lead product candidate, PGN-EDO51, initiated two Phase 2 studies of PGN-EDO51 and initiated a Phase 1 and a Phase 2 clinical trial of our second candidate, PGN-EDODM1, we cannot be certain of the completion or outcome of our preclinical testing and studies for our other product candidates and cannot predict whether the FDA, European Medicines Agency, or EMA, or comparable foreign regulatory authorities will accept our proposed clinical programs for PGN-EDO51 or PGN-EDODM1, or whether the outcome of our preclinical testing and studies will ultimately support the further development of our other product candidates, including PGN-EDO53. 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, EMA or comparable foreign regulatory authorities allowing clinical trials to begin, including in the case of our CONNECT2 clinical study for PGN-EDO51 and our FREEDOM2 clinical study for PGN-EDODM1. For example, 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. 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;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or 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 the COVID-19 pandemic, or any future 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 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. 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, 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 are rare genetic diseases 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 successfully completed a Phase 1 HV clinical trial in Canada for our lead product candidate, PGN-EDO51, have initiated a Phase 1 clinical trial for our PGN-EDODM1 product candidate and reported early data from the low dose cohort of our first Phase 2 trial of PGN-EDO51 in DMD patients, but we have not completed IND- or CTA-enabling activities for our other product candidates or advanced any other product candidates into clinical development. As a result, our belief in the capabilities of our platform is based on early research, preclinical studies, our completed Phase 1 clinical trial in HVs and early data from our Phase 2 study of PGN-EDO51 and our Phase 1 study of PGN-EDODM1. 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, we may not see the same levels of exon skipping, oligonucleotide delivery or dystrophin production in DMD patients as was observed in our preclinical studies or, with respect to exon skipping and oligonucleotide delivery, in our Phase 1 HV study of PGN-EDO51. While we saw high mean levels of exon skipping (2.15%) in all participants at the 5 mg/kg starting dose of our CONNECT1 Phase 2 study after four doses and three months of treatment, we did not achieve the level of dystrophin that we had anticipated. With a longer treatment period and higher doses of PGN-EDO51, we expect to see higher levels of exon skipped transcript potentially resulting in significant increases in dystrophin, but this may not be the case. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Our clinical trials may not ultimately be successful or support further clinical development of our product candidates. There is a high failure rate for product candidates proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving encouraging results in earlier studies. Any such setbacks in our clinical development could materially harm our business and results of operations.

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.

If we experience delays or difficulties in the enrollment of 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. We may not be able to identify, recruit and enroll a sufficient number of 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;

•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 the COVID-19 pandemic or a future pandemic or health crisis.

For example, DMD amenable to exon 51 skipping is a rare disease, and there are several therapies approved in certain territories or in clinical development, which can make enrollment in our CONNECT1 and CONNECT2 trials more challenging. Our inability to enroll a sufficient number of patients for our clinical trials, including our ongoing trials for PGN-EDO51 and 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 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.

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. For example, in September 2022, we announced results from our Phase 1 clinical trial of PGN-EDO51, in July 2024, we announced initial results from our CONNECT1 Phase 2 trial from the low dose (5 mg/kg) cohort as well as high level safety information for the ongoing 10 mg/kg dose cohort, and more recently, we provided additional safety information from the ongoing CONNECT1 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.

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 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 have completed dosing up to 15 mg/kg in a Phase 1 clinical trial of PGN-EDO51, in which PGN-EDO51 was generally well-tolerated at clinically active doses, and are dosing patients at 5 mg/kg and 10 mg/kg in an ongoing Phase 2 clinical trial of PGN-EDO51, in which we believe PGN-EDO51 has continued to demonstrate a favorable safety profile as of October 31, 2024, but there can be no assurance that our product candidates will not cause undesirable side effects in patients. For example, in preclinical toxicology studies of PGN-EDO51 in normal NHPs, we observed transient, clinical signs of hypotension in some animals treated at a dose level higher than that which we intend to evaluate in the clinic. In addition, in our Phase 1 clinical trial of PGN-EDO51, at 15 mg/kg, 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. While the trial was not halted by the safety review committee nor put on hold by Health Canada, under the protocol for this Phase 1 clinical trial, any non-life-threatening SAE was considered a dose-limiting toxicity. This participant was admitted to the hospital for less than 24 hours, received intravenous hydration, and then was re-admitted to the Phase 1 unit and completed the study. We also observed transient mild to moderate hypomagnesemia in two participants in the Phase 1 trial, which did not require intervention. Based on published data and other publicly available information, such adverse events are consistent with the types of events reported with this class of oligonucleotides in general. In the ongoing CONNECT1 Phase 2 study, based on the totality of data in both the 5 mg/kg cohort and the ongoing 10 mg/kg cohort as of October 31, 2024, we believe PGN-EDO51 has a favorable emerging safety profile. There have been no treatment-related SAEs and all treatment-related adverse events have been mild or moderate. There was no sustained elevation in kidney biomarkers. There were also no changes in hepatic function and no cases of hypokalemia, anemia or thrombocytopenia. Asymptomatic hypomagnesemia has been observed in two participants in the 10 mg/kg cohort and treated with oral magnesium supplementation. All participants continue in the study as planned, with no discontinuations, dose modifications or dose interruptions as of October 31, 2024.

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 and only one completed trial involving the proprietary technology used in our EDO platform.

Despite the outcome of our Phase 1 clinical trial of PGN-EDO51 and the early results from our Phase 2 CONNECT1 study, further results from our CONNECT1 or future results from our CONNECT2 Phase 2 clinical trials for PGN-EDO51 or studies of any other product candidate could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics than previously anticipated. If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, regulatory authorities may draw different conclusions, require additional testing to confirm these determinations, require more restrictive labeling or deny regulatory approval of the product candidate. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further clinical development of the product candidates.

It is possible that, as we test our product candidates in larger, longer and more extensive clinical trials, including with different dosing regimens, or as 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 focus on research programs and expect to focus on product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay 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. 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 will face an inherent risk of clinical trial and product liability exposure related to the testing of product candidates that 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 two product candidates 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 intend to 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 conducted our first clinical trial in Canada, and we intend to conduct one or more of our subsequent clinical trials for our product candidates outside the U.S., including our ongoing CONNECT1 Phase 2 trial in Canada, and our ongoing CONNECT2, FREEDOM and FREEDOM2 clinical trials, each of which has or will have sites in multiple countries outside of 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. 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, which could 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. If the FDA does not accept data from our clinical trials of our product candidates, it would likely result in the need for additional clinical trials, which would be costly and time consuming and delay or permanently halt our development of our product candidates.

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; 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.

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.

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 who 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, legislative proposals are pending that, if enacted, could negatively impact U.S. funding for certain biotechnology providers, including some of our vendors, that may have relationships with certain foreign governments or are viewed as posing a threat to national security. 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 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 of the product candidates that we are evaluating in our research programs. 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.

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 comparability or 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, legislative proposals are pending that, if enacted, could negatively impact U.S. funding for certain biotechnology providers, including some of our vendors, that may have relationships with certain foreign governments or are viewed as posing a threat to national security. This could result in our inability to continue to engage with one or more of our vendors or impair our ability to contract with new vendors, which could result in supply delays or shortages, clinical trial delays and the need to engage new vendors in a competitive marketplace.

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 may from time to time be dependent on single-source suppliers for some of the components and materials used in our product candidates.

Although we currently do not use any single-source supplier, we may from time to time depend on such suppliers for some of the components and materials used in our product candidates. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes and finished goods 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 sale. 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 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 the COVID-19 pandemic or a similar 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

Our lead product candidates are in clinical development, while all of our other product candidates are still in preclinical development. As an organization, we have only completed one clinical trial and initiated one other Phase 1 clinical trial and three Phase 2 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.

Although we are currently in clinical development for two product candidates, 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 EMA 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, and we have successfully completed a Phase 1 clinical trial and initiated Phase 2 trials for our lead product candidate, PGN-EDO51, and initiated a Phase 1 and a Phase 2 clinical trial for our second product candidate, PGN-EDODM1. We will need to successfully complete IND- or CTA-enabling activities, early-stage, later-stage and pivotal clinical trials, in order to obtain FDA, EMA or comparable foreign regulatory approval to market PGN-EDO51, PGN-EDODM1, and PGN-EDO53 and any future product candidates.

Carrying out clinical trials and the submission of a successful NDA is a complicated process. We completed our first Phase 1 clinical trial for PGN-EDO51 in the third quarter of 2022, and we began dosing patients in CONNECT1 in Canada in January 2024. We received clearance from the MHRA in February 2024 to initiate CONNECT2 in the U.K. in boys and young men living with DMD.

Based on the observed high tissue concentrations of oligonucleotide and exon skipping in muscle in our Phase 1 trial of PGN-EDO51 in HVs, as well as the early data from the low dose (5 mg/kg) cohort in our CONNECT1 Phase 2 trial of PGN-EDO51, we believe that these results could signal the potential for the accumulation of exon 51 skipped transcript and higher production of dystrophin protein in muscle tissue with repeated doses of PGN-EDO51 in DMD patients over a longer treatment period. However, our belief based on this data may be erroneous. There can be no assurance that our expectations of higher exon skipping and dystrophin production will be reflected in continued Phase 2 clinical evaluation of PGN-EDO51 in DMD patients.

Although we completed a Phase 1 clinical trial for our lead product candidate and have several additional clinical trials ongoing, 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-EDO51, PGN-EDODM1, PGN-EDO53 or any other product candidates will be required or how such trials should be designed. For example, the FDA has approved at least four drugs based on their minimal dystrophin production, and it is our belief that we may be able to pursue an accelerated approval pathway for PGN-EDO51 on that same basis, assuming we continue to see increases in exon skipping and dystrophin production with higher doses

and a longer treatment period. The FDA has previously provided feedback on our clinical trials for PGN-EDO51, as well as for PGN-EDODM1, and we have addressed their feedback in our clinical trial designs. To date, we have not received FDA feedback on any potential accelerated approval pathway for PGN-EDO51. We may be unable to successfully and efficiently execute and complete necessary clinical trials 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.

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. 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. For example, we expect that the FDA will require a post-marketing confirmatory trial of PGN-EDO51, if it is approved under the accelerated approval regulations requiring applicants to demonstrate clinical benefit in post-approval studies. 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 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, since the withdrawal of the U.K. from the EU, a separate authorization is needed to market medicinal products in Great Britain. 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 attempt 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.

We may in the future seek accelerated approval, where applicable, under the FDA's accelerated approval pathway. 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 irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. 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 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. We have reported encouraging early results from the first cohort in our Phase 2 CONNECT1 study of PGN-EDO51 and believe that with a longer treatment period and higher doses of PGN-EDO51, we may see higher levels of exon skipped transcript potentially resulting in significant increases in dystrophin. If we continue to receive positive results from our CONNECT1 study and receive positive results from the CONNECT2 study for PGN-EDO51 that show an acceptable safety and tolerability profile; a clinically meaningful increase in dystrophin levels, a surrogate endpoint, in the biceps of DMD patients; and robust exon skipping levels in the same tissue; we intend to pursue this accelerated approval pathway subject to discussions with the FDA. However, our Phase 2 clinical trials may fail to produce such data, and we may be unable to pursue the accelerated approval pathway as planned. To date we have not received FDA feedback on any potential accelerated approval pathway for PGN-EDO51.

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 any of our product candidates, 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 for our product candidates, 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. 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 our product candidate would result in a longer time period to commercialization of such product candidate, if any, could increase the cost of development of such product candidate, and could harm our competitive position in the marketplace.

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 some of our other product candidates. 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 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 an application 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 and in March 2024, the FDA granted orphan drug designation to PGN-EDO51 for the treatment of DMD patients whose mutations are amenable to an exon 51-skipping approach. We intend to seek orphan drug designation for some of our other product candidates; 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 therapeutic 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 condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition 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. While we have received RPDD from the FDA for PGN-EDO51 for the treatment of DMD patients who are amenable to exon 51 skipping, we would need to request a rare pediatric disease PRV in the marketing application of PGN-EDO51. The FDA may determine that any such marketing application, if approved, does not meet the eligibility criteria for a PRV. The authority for the FDA to award rare pediatric disease PRVs is currently limited to drugs that have received RPDD prior to December 20, 2024, and the FDA may only award rare pediatric disease PRVs for drugs that are approved by September 30, 2026. If a marketing application for PGN-EDO51 is not approved prior to September 30, 2026 for any reason, regardless of whether it meets the criteria for a rare pediatric disease PRV, it will not be eligible for a PRV, unless the authority for FDA to award rare pediatric disease PRVs is further extended through federal lawmaking.

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.

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 any of our 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 recordkeeping 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, under FDORA, sponsors of approved drugs and biologics must provide six months' notice to the FDA of any changes in marketing status, such as the withdrawal of a drug, and failure to do so could result in the FDA placing the product on a list of discontinued products, which would revoke the product's ability to be marketed. 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. 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 any of our product candidates 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 or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

During the COVID-19 public health emergency, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. If a prolonged government shutdown occurs, or if global health concerns prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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. For more information, see "*Business – Healthcare Regulation – Other Healthcare Laws*" in our Annual Report on Form 10-K filed with the SEC for the year-ended December 31, 2023.

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.

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. 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 more information, see "*Business – 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, 2023.

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, 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, collectively known as the ACA, 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 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 U.K. GDPR is currently largely in line with the EU GDPR, but the data protection regimes may diverge more in the future. 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. However, the U.K. adequacy decision will automatically expire in June 2025 unless the European Commission re-assesses and renews or extends that decision and remains under review by the Commission during this period. The U.K. government has confirmed that personal data transfers from the U.K. to the EEA remain free flowing. The U.K. Government announced that it plans to introduce a Digital Information and Smart Data Bill, or U.K. Bill, into the U.K. legislative process. If passed, the final version of the U.K. Bill may have the effect of further altering the similarities between the U.K. and EEA data protection regime and threaten the U.K. Adequacy Decision from the European Commission. This may lead to additional compliance costs and could increase our overall risk. 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.

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.

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. The Federal Trade Commission and state Attorneys General are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered or have been implemented at both the state and federal levels. 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 new individual data privacy rights, imposes new 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 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. The CPRA also created a new state agency that will be vested with authority to implement and enforce the CCPA.

Many other states have enacted or are considering similar legislation, and a broad range of legislative measures also have been introduced at the federal level. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, 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 would make our compliance obligations more complex and costly and may increase 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 recently 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 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 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 is discussion in the U.S. Congress of a new comprehensive federal data privacy law to which we may likely become subject, if enacted.

Further, regulations promulgated pursuant to HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and referred herein collectively as HIPAA, imposes 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.

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. 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 if they are found to be deceptive, unfair, or misrepresentative of our actual practices.

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.

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 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 new chemical entity, or 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 market and sell products or 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, EMA 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 each of our programs. Currently, patients with DMD are treated with corticosteroids to manage the inflammatory component of the disease. EMFLAZA® (deflazacort) is an FDA-approved corticosteroid marketed by PTC Therapeutics, Inc., or PTC. Individuals with DMD also use prednisone or prednisolone off-label. In addition, there are several FDA-approved exon skipping drugs: EXONDYS 51® (eteplirsen) and VYONDYS 53® (golodirsen), which are naked PMOs approved for the treatment of DMD patients amenable to exon 51 and exon 53 skipping, respectively, and are marketed by Sarepta Therapeutics, Inc., or Sarepta; and VILTEPSO® (viltolarsen), a naked PMO approved for the treatment of DMD patients amenable to exon 53 skipping, which is marketed in the U.S. by NS Pharma, Inc. Companies focused on developing treatments for DMD that target dystrophin production, as our DMD program does, include Dyne Therapeutics, Inc., or Dyne, with DYNE-251, an antibody-conjugated PMO that targets exon 51 skipping in a Phase 1/2 clinical trial;

BioMarin Pharmaceutical Inc., with BMN-351, a phosphorothioate oligonucleotide that targets exon 51 skipping currently in preclinical development; and Wave Life Sciences Ltd. with WVE-N531, a stereopure oligonucleotide in a Phase 1/2 clinical trial for patients amenable to exon 53 skipping. In November 2024, Sarepta announced it was discontinuing development of SRP-5051, a peptide-linked PMO for patients amenable to exon 51 skipping.

In addition, several companies are developing gene therapies to treat DMD. These include ELEVIDYS® (SRP-9001) which was approved in June 2023 for treatment of ambulatory pediatric patients aged 4 through 5 years with DMD with a confirmed mutation in the DMD gene. Sarepta filed an efficacy supplement to its biologics license application to expand the label of ELEVIDYS to encompass “treatment of DMD patients with a confirmed mutation in the DMD gene.” In June 2024, the FDA granted approval of an expansion to the labeled indication for ELEVIDYS to include individuals with DMD with a confirmed mutation in the DMD gene who are at least 4 years of age, granting traditional approval for ambulatory patients and accelerated approval for non-ambulatory patients. Continued approval for non-ambulatory DMD patients may be contingent upon verification of clinical benefit in a confirmatory trial. In addition, several other companies are developing investigational gene therapies to treat DMD, including Sarepta’s Galgt2 gene therapy program, and Solid Biosciences Inc.’s SGT-003 and REGENXBIO Inc.’s RGX-202, currently in clinical development. Gene editing treatments that are in preclinical development are also being pursued by Vertex Pharmaceuticals, Inc., or Vertex, and Sarepta. In June 2024, Pfizer announced that its experimental gene therapy for DMD failed to meet the primary and secondary goals of its Phase 3 trial and in July 2024 announced that it has stopped development for this therapy. We are also aware of several companies targeting non-dystrophin mechanisms for the treatment of DMD, including Edgewise Therapeutics, Inc. with EDG-5506, a muscle stabilizer that is currently in clinical development and Duvyzat (givinostat), an oral histone deacetylase, or HDAC inhibitor, that reduces fibrosis in patients with DMD and was approved by the FDA in March of 2024 for the treatment of DMD in patients six years of age and older, and is under review by the EMA.

For DM1, 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 AOC 1001, an antibody linked siRNA in Phase 1/2 clinical development with a global Phase 3 study planned for initiation in the second quarter of 2024 by Avidity Biosciences, Inc., or Avidity; DYNE-101, an antibody conjugated antisense oligonucleotide in Phase 1/2 clinical development by Dyne; and VX-670, a peptide conjugated PMO in Phase 1 by Entrada Therapeutics, Inc., or Entrada, and Vertex. There are additional approaches under development such as ATX-01, a microRNA that modulates expression of MBNL1 by Arthex Biotech S.L., that recently received IND clearance. Another small molecule, tideglusib, which is a GSK3-ß inhibitor is in clinical development by AMO Pharma Ltd. for the congenital phenotype of DM1, recently failed to meet primary endpoint in a pivotal study.

Several gene editing treatments are in preclinical development by Vertex; an artificial site-specific RNA endonuclease gene therapy is being developed by Enzerna Biosciences Inc.; Design Therapeutics, Inc. is developing an approach to prevent formation of CUG hairpins; Expansion Therapeutics, Inc. is developing an approach utilizing the interaction of small molecules with RNA in preclinical development; and therapeutics based on biomolecular condensate biology in preclinical development by Dewpoint Therapeutics, Inc. and Pfizer.

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 Pharmaceuticals, Inc., Avidity, Dicerna Pharmaceuticals, Inc. (acquired by Novo Nordisk), Dyne, Entrada, Ionis Pharmaceuticals, Inc., NeuBase Therapeutics, 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 10-Q 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 10-Q 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 10-Q relating to size and expected growth of our target market may prove to be inaccurate. Even if the markets in which we compete meet the size estimates and growth forecasts included in this 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 more information, see "*Business – Healthcare Regulation – Coverage and Reimbursement*" in our Annual Report on Form 10-K filed with the SEC for the year-ended December 31, 2023.

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 to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to educate adequate numbers of physicians on the benefits of any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors;
- the inability to price our products 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 and defend 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 an early stage; except for one issued patent and seven applications currently under examination, which we in-licensed from Oxford University Innovation Limited, or OUI, and the Medical Research Council of United Kingdom Research and Innovation, or MRC, substantive examination of the currently pending patent applications we own or license has yet to begin. 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. 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. 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.

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 product 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 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, and incur liability for damages. 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. 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 may enter into in the future may be 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. 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 outside the U.S. 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. 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 will be 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. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. 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 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 or could be required to take licenses from such third parties which may not be available on commercially reasonable terms, if at all.

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 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 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, 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 Drug Price Competition and Patent Term Restoration Act of 1984, or 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 the 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 incorporated into the technology of others or are disclosed or used in violation of these agreements. 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 offer letters 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 expect to expand our headcount 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, 2024, we had 76 full-time employees. As our development progresses, we expect to experience significant 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 anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, 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 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.

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 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, 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. As a result of the effects of a pandemic, such as the COVID-19 pandemic or other health crisis, 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, 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 its 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 the COVID-19 pandemic, or 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, including the COVID-19 pandemic or similar outbreaks, could adversely impact our business. For example, in connection with COVID-19, we and our CDMOs and CROs have 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. 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 ongoing efforts to address COVID-19 or a future health pandemic. The ultimate extent to which COVID-19, or a future outbreak of other 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 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 Follow-on Offering, receiving aggregate net proceeds of $86.3 million after deducting underwriters' fees and costs of the offerings. 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 may be influenced by many factors, including:

- timing and results of, or developments in, preclinical studies and 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, including conditions resulting from the effects of the COVID-19 pandemic, 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, 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 the COVID-19 pandemic or future pandemic 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 military conflict between Russia and Ukraine, armed conflict in Israel and

the Gaza Strip and military action in other parts of the Middle East 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 pending legislative proposals 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, 2024, our executive officers, directors, greater than five percent shareholders and their affiliates beneficially own approximately 47.4% 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 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 are required to furnish a report by our management on our internal control over financial reporting beginning with this 10-Q. 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. 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, 2023, we had federal and state NOL carryforwards of $23.4 million. We did not generate U.K. NOLs in 2023 or the first quarter of 2024, and do not anticipate any going forward.

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, 2024, 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 pre-change 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. Any future actions regarding transfer of assets from our U.K. subsidiary or other 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 may be at an increased 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. If we were to be sued, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

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.

Use of Proceeds from our Public Offering of Common Stock

On May 10, 2022, we closed our IPO in which we sold an aggregate of 9,000,000 shares 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. On May 16, 2022, the underwriters exercised the option in part and we issued 1,238,951 shares of our common stock for gross proceeds of $14.9 million. From the IPO and option exercise by the underwriters, we received approximately $122.9 million in gross proceeds and $110.2 million in net proceeds, after deducting underwriting discounts and estimated offering expenses payable by us. None of the underwriting discounts and commissions or offering expenses were incurred or paid, directly or indirectly, to any of our directors or officers or their associates or to persons owning 10% or more of our common stock or to any of our affiliates.

The offer and sale of all of the shares of our common stock in our IPO were registered under the Securities Act pursuant to a registration statement on Form S-1, as amended (File No. 333- 264335), which was declared effective by the SEC on May 5, 2022. BofA Securities, Inc., SVB Securities LLC, Stifel, Nicolaus & Company, Incorporated and Wedbush Securities Inc. acted as underwriters for the IPO.

Unregistered Sales 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, 2024, 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).
10.1**	PepGen Inc. 2024 Inducement Plan and forms of agreements thereunder
10.2**	Employment Agreement, dated August 19, 2024, between Paul D. Streck, MD, MBA and the Company
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 7, 2024	By:	/s/ James McArthur **James McArthur** **Chief Executive Officer**
Date: November 7, 2024	By:	/s/ Noel Donnelly **Noel Donnelly** **Chief Financial Officer**

Exhibit 10.1

PEPGEN INC.

2024 INDUCEMENT PLAN

SECTION 1. GENERAL PURPOSE OF THE PLAN; DEFINITIONS

The name of the plan is the PepGen Inc. 2024 Inducement Plan (the "Plan"). The purpose of the Plan is to enable PepGen Inc. (the "Company") to grant equity awards to induce highly-qualified prospective officers and employees who are not currently employed by the Company and its Affiliates to accept employment and provide them with a proprietary interest in the Company. It is anticipated that providing such persons with a direct stake in the Company's welfare will assure a closer identification of their interests with those of the Company and its stockholders, thereby stimulating their efforts on the Company's behalf and strengthening their desire to remain with the Company. The Company intends that the Plan be reserved for persons to whom the Company may issue securities without stockholder approval as an inducement pursuant to Rule 5635(c)(4) of the Marketplace Rules of the NASDAQ Stock Market, Inc.

The following terms shall be defined as set forth below:

"Act" means the U.S. Securities Act of 1933, as amended, and the rules and regulations thereunder.

"Administrator" means either the Board or the compensation committee of the Board or a similar committee performing the functions of the compensation committee and which is comprised of not less than two Non-Employee Directors who are independent.

"*Affiliate*" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

"Award" or *"Awards,"* except where referring to a particular category of grant under the Plan, shall include Non-Qualified Stock Options, Stock Appreciation Rights, Restricted Stock Awards, Restricted Stock Units, Unrestricted Stock Awards, and Dividend Equivalent Rights.

"Award Certificate" means a written or electronic document setting forth the terms and provisions applicable to an Award granted under the Plan. Each Award Certificate is subject to the terms and conditions of the Plan.

"Board" means the Board of Directors of the Company.

"Code" means the U.S. Internal Revenue Code of 1986, as amended, and any successor Code, and related rules, regulations and interpretations.

"Consultant" means a consultant or adviser who provides *bona fide* services to the Company or an Affiliate as an independent contractor and who qualifies as a consultant or advisor under Instruction A.1.(a)(1) of Form S-8 under the Act.

"Dividend Equivalent Right" means an Award entitling the grantee to receive credits based on cash dividends that would have been paid on the shares of Stock specified in the Dividend Equivalent Right (or other award to which it relates) if such shares had been issued to and held by the grantee.

"Effective Date" means the date on which the Plan is approved by the Board as set forth in Section 19.

"Exchange Act" means the Securities Exchange Act of 1934, as amended, and the rules and regulations thereunder.

"Fair Market Value" of the Stock on any given date means the fair market value of the Stock determined in good faith by the Administrator; provided, however, that if the Stock is listed on the National Association of Securities Dealers Automated Quotation System ("NASDAQ"), NASDAQ Global Market, The New York Stock Exchange or another national securities exchange or traded on any established market, the determination shall be made by reference to market quotations. If there are no market quotations for such date, the determination shall be made by reference to the last date preceding such date for which there are market quotations.

"Non-Employee Director" means a member of the Board who is not also an employee of the Company or any Subsidiary.

"Non-Qualified Stock Option" means any Stock Option that is not an "incentive stock option" under Section 422 of the Code.

"Option" or *"Stock Option"* means any option to purchase shares of Stock granted pursuant to Section 5.

"Restricted Shares" means the shares of Stock underlying a Restricted Stock Award that remain subject to a risk of forfeiture or the Company's right of repurchase.

"Restricted Stock Award" means an Award of Restricted Shares subject to such restrictions and conditions as the Administrator may determine at the time of grant.

"Restricted Stock Units" means an Award of stock units subject to such restrictions and conditions as the Administrator may determine at the time of grant.

"Sale Event" means the occurrence of any of the following events (i) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (ii) a merger, reorganization or consolidation pursuant to which the holders of the Company's outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or other

equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction, (iii) the sale of all of the Stock of the Company to an unrelated person, entity or group thereof acting in concert, or (iv) any other transaction in which the owners of the Company's outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

"*Sale Price*" means the value as determined by the Administrator of the consideration payable, or otherwise to be received by stockholders, per share of Stock pursuant to a Sale Event.

"Section 409A" means Section 409A of the Code and the regulations and other guidance promulgated thereunder.

"Service Relationship" means any relationship as an employee, Non-Employee Director or Consultant of the Company or any Affiliate. Unless as otherwise set forth in the Award Certificate, a Service Relationship shall be deemed to continue without interruption in the event a grantee's status changes from full-time employee to part-time employee or a grantee's status changes from employee to Consultant or Non-Employee Director or vice versa, provided that there is no interruption or other termination of Service Relationship in connection with the grantee's change in capacity.

"Stock" means the Common Stock, par value $0.0001 per share, of the Company, subject to adjustments pursuant to Section 3.

"Stock Appreciation Right" means an Award entitling the recipient to receive shares of Stock (or cash, to the extent explicitly provided for in the applicable Award Certificate) having a value equal to the excess of the Fair Market Value of the Stock on the date of exercise over the exercise price of the Stock Appreciation Right multiplied by the number of shares of Stock with respect to which the Stock Appreciation Right shall have been exercised.

"Subsidiary" means any corporation or other entity (other than the Company) in which the Company has at least a 50 percent interest, either directly or indirectly.

"Unrestricted Stock Award" means an Award of shares of Stock free of any restrictions.

SECTION 2. ADMINISTRATION OF PLAN; ADMINISTRATOR AUTHORITY TO SELECT GRANTEES AND DETERMINE AWARDS

(a)Administration of Plan. The Plan shall be administered by the Administrator.

(b)Powers of Administrator. The Administrator shall have the power and authority to grant Awards consistent with the terms of the Plan, including the power and authority:

(i)to select the individuals to whom Awards may from time to time be granted;

(ii)to determine the time or times of grant, and the extent, if any, of Non-Qualified Stock Options, Stock Appreciation Rights, Restricted Stock Awards, Restricted Stock Units, Unrestricted Stock Awards, and Dividend Equivalent Rights, or any combination of the foregoing, granted to any one or more grantees;

(iii)to determine the number of shares of Stock to be covered by any Award;

(iv)to determine and modify from time to time the terms and conditions, including restrictions, not inconsistent with the terms of the Plan, of any Award, which terms and conditions may differ among individual Awards and grantees, and to approve the forms of Award Certificates;

(v)to accelerate at any time the exercisability or vesting of all or any portion of any Award;

(vi) subject to the provisions of Section 5(c) or Section 6(d), as applicable, to extend at any time the period in which Stock Options and Stock Appreciation Rights may be exercised; and

(vii)at any time to adopt, alter and repeal such rules, guidelines and practices for administration of the Plan and for its own acts and proceedings as it shall deem advisable, including rules and regulations relating to sub-plans established for the purpose of satisfying applicable foreign laws or for qualifying for favorable tax treatment under applicable foreign laws; to interpret the terms and provisions of the Plan and any Award (including related written instruments); to make all determinations it deems advisable for the administration of the Plan; to decide all disputes arising in connection with the Plan; and to otherwise supervise the administration of the Plan.

All decisions and interpretations of the Administrator shall be binding on all persons, including the Company and Plan grantees.

(c)Reserved.

(d)Award Certificate. Awards under the Plan shall be evidenced by Award Certificates that set forth the terms, conditions and limitations for each Award which may include, without limitation, the term of an Award and the provisions applicable in the event Service Relationship terminates.

(e)Indemnification. Neither the Board nor the Administrator, nor any member of either or any delegate thereof, shall be liable for any act, omission, interpretation, construction or determination made in good faith in connection with the Plan, and the members of the Board and the Administrator (and any delegate thereof) shall be entitled in all cases to indemnification and reimbursement by the Company in respect of any claim, loss, damage or expense (including, without limitation, reasonable attorneys' fees) arising or resulting therefrom to the fullest extent permitted by law and/or under the Company's articles or bylaws or any directors' and officers' liability insurance coverage which may be in effect from time to time and/or any indemnification agreement between such individual and the Company.

(f)Non-U.S. Award Recipients. Notwithstanding any provision of the Plan to the contrary, in order to comply with the laws in other countries in which the Company and its Affiliates operate or have employees or other individuals eligible for Awards, the Administrator, in its sole discretion, shall have the power and authority to: (i) determine which Affiliates shall be covered by the Plan; (ii) determine which individuals outside the United States are eligible to participate in the Plan; (iii) modify the terms and conditions of any Award granted to individuals outside the United States to comply with applicable laws; (iv) establish subplans and modify exercise procedures and other terms and procedures, to the extent the Administrator determines such actions to be necessary or advisable (and such subplans and/or modifications shall be incorporated into and made part of this Plan); provided, however, that no such subplans and/or modifications shall increase the share limitations contained in Section 3(a) hereof; and (v) take any action, before or after an Award is made, that the Administrator determines to be necessary or advisable to obtain approval or comply with any local governmental regulatory exemptions or approvals. Notwithstanding the foregoing, the Administrator may not take any actions hereunder, and no Awards shall be granted, that would violate the Exchange Act or any other applicable United States securities law, the Code, or any other applicable United States governing statute or law.

SECTION 3. STOCK ISSUABLE UNDER THE PLAN; MERGERS; SUBSTITUTION

(a)Stock Issuable. The maximum number of shares of Stock reserved and available for issuance under the Plan shall be 1,000,000, subject to adjustment as provided in this Section 3. For purposes of this limitation, the shares of Stock underlying any Awards under the Plan that are forfeited, canceled, held back upon exercise of an Option or settlement of an Award to cover the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of Stock or otherwise terminated (other than by exercise) shall be added back to the shares of Stock available for issuance under the Plan. In the event the Company repurchases shares of Stock on the open market, such shares shall not be added to the shares of Stock available for issuance under the Plan. Subject to such overall limitations, shares of Stock may be issued up to such maximum number pursuant to any type or types of Award. The shares available for issuance under the Plan may be authorized but unissued shares of Stock or shares of Stock reacquired by the Company. Awards that may be settled solely in cash shall not be counted against the share reserve.

(b)Changes in Stock. Subject to Section 3(c) hereof, if, as a result of any reorganization, recapitalization, reclassification, stock dividend, extraordinary cash dividend, stock split, reverse stock split or other similar change in the Company's capital stock, the outstanding shares of Stock are increased or decreased or are exchanged for a different number or kind of shares or other securities of the Company, or additional shares or new or different shares or other securities of the Company or other non-cash assets are distributed with respect to such shares of Stock or other securities, or, if, as a result of any merger or consolidation, sale of all or substantially all of the assets of the Company, the outstanding shares of Stock are converted into or exchanged for securities of the Company or any successor entity (or a parent or subsidiary thereof), the Administrator shall make an appropriate or proportionate adjustment in (i) the maximum number of shares reserved for issuance under the Plan, (ii) the number and kind of shares or other securities subject to any then outstanding Awards under the Plan, (iii) the

repurchase price, if any, per share subject to each outstanding Restricted Stock Award, and (iv) the exercise price for each share subject to any then outstanding Stock Options and Stock Appreciation Rights under the Plan, without changing the aggregate exercise price (i.e., the exercise price multiplied by the number of shares subject to Stock Options and Stock Appreciation Rights) as to which such Stock Options and Stock Appreciation Rights remain exercisable. The Administrator shall also make equitable or proportionate adjustments in the number of shares subject to outstanding Awards and the exercise price and the terms of outstanding Awards to take into consideration cash dividends paid other than in the ordinary course or any other extraordinary corporate event. The adjustment by the Administrator shall be final, binding and conclusive. No fractional shares of Stock shall be issued under the Plan resulting from any such adjustment, but the Administrator in its discretion may make a cash payment in lieu of fractional shares.

(c)Mergers and Other Transactions. In the case of and subject to the consummation of a Sale Event, the parties thereto may cause the assumption or continuation of Awards theretofore granted by the successor entity, or the substitution of such Awards with new Awards of the successor entity or parent thereof, with appropriate adjustment as to the number and kind of shares and, if appropriate, the per share exercise prices, as such parties shall agree. To the extent that the parties to such Sale Event do not provide for the assumption, continuation or substitution of Awards, upon the effective time of the Sale Event, the Plan and all outstanding Awards granted hereunder shall terminate. In such case, except as may be otherwise provided in the relevant Award Certificate, all Awards with time-based vesting, conditions or restrictions shall become fully vested and exercisable or nonforfeitable as of the effective time of the Sale Event, and all Awards with conditions and restrictions relating to the attainment of performance goals may become vested and exercisable or nonforfeitable in connection with a Sale Event in the Administrator's discretion or to the extent specified in the relevant Award Certificate. In the event of such termination, (i) the Company shall have the option (in its sole discretion) to make or provide for a payment, in cash or in kind, to the grantees holding Options and Stock Appreciation Rights, in exchange for the cancellation thereof, in an amount equal to the difference between (A) the Sale Price multiplied by the number of shares of Stock subject to outstanding Options and Stock Appreciation Rights (to the extent then exercisable at prices not in excess of the Sale Price) and (B) the aggregate exercise price of all such outstanding Options and Stock Appreciation Rights (provided that, in the case of an Option or Stock Appreciation Right with an exercise price equal to or greater than the Sale Price, such Option or Stock Appreciation Right shall be cancelled for no consideration); or (ii) each grantee shall be permitted, within a specified period of time prior to the consummation of the Sale Event as determined by the Administrator, to exercise all outstanding Options and Stock Appreciation Rights (to the extent then exercisable) held by such grantee. The Company shall also have the option (in its sole discretion) to make or provide for a payment, in cash or in kind, to the grantees holding other Awards in an amount equal to the Sale Price multiplied by the number of vested shares of Stock under such Awards.

SECTION 4. ELIGIBILITY

Grantees under the Plan will be such employees of the Company and its Affiliates as to whom the Company may issue securities without stockholder approval in accordance with Rule

5635(c)(4) of the Marketplace Rules of the NASDAQ Stock Market, Inc as selected from time to time by the Administrator in its sole discretion; provided that Awards may not be granted to employees, who are providing services only to any "parent" of the Company, as such term is defined in Rule 405 of the Act, unless (i) the stock underlying the Awards is treated as "service recipient stock" under Section 409A or (ii) the Company, in consultation with its legal counsel, has determined that such Awards are exempt from or otherwise comply with Section 409A.

SECTION 5. STOCK OPTIONS

(a)Award of Stock Options. The Administrator may grant Stock Options under the Plan. Any Stock Option granted under the Plan shall be a Non-Qualified Stock Option, and shall be in such form as the Administrator may from time to time approve.

Stock Options granted pursuant to this Section 5 shall be subject to the following terms and conditions and shall contain such additional terms and conditions, not inconsistent with the terms of the Plan, as the Administrator shall deem desirable.

(b)Exercise Price. The exercise price per share for the Stock covered by a Stock Option granted pursuant to this Section 5 shall be determined by the Administrator at the time of grant but shall not be less than 100 percent of the Fair Market Value on the date of grant. Notwithstanding the foregoing, Stock Options may be granted with an exercise price per share that is less than 100 percent of the Fair Market Value on the date of grant (i) to individuals who are not subject to U.S. income tax on the date of grant, or (ii) if the Stock Option is otherwise exempt from or compliant with Section 409A.

(c)Option Term. The term of each Stock Option shall be fixed by the Administrator, but no Stock Option shall be exercisable more than ten years after the date the Stock Option is granted.

(d)Exercisability; Rights of a Stockholder. Stock Options shall become exercisable at such time or times, whether or not in installments, as shall be determined by the Administrator at or after the grant date. The Administrator may at any time accelerate the exercisability of all or any portion of any Stock Option. An optionee shall have the rights of a stockholder only as to shares acquired upon the exercise of a Stock Option and not as to unexercised Stock Options.

(e)Method of Exercise. Stock Options may be exercised in whole or in part, by giving written or electronic notice of exercise to the Company, specifying the number of shares to be purchased. Payment of the purchase price may be made by one or more of the following methods except to the extent otherwise provided in the Award Certificate:

(i)In cash, by certified or bank check or other instrument acceptable to the Administrator;

(ii)Through the delivery (or attestation to the ownership following such procedures as the Company may prescribe) of shares of Stock that are not then subject to restrictions under any Company plan. Such surrendered shares shall be valued at Fair Market Value on the exercise date;

(iii)By the optionee delivering to the Company a properly executed exercise notice together with irrevocable instructions to a broker to promptly deliver to the Company cash or a check payable and acceptable to the Company for the purchase price; provided that in the event the optionee chooses to pay the purchase price as so provided, the optionee and the broker shall comply with such procedures and enter into such agreements of indemnity and other agreements as the Company shall prescribe as a condition of such payment procedure; or

(iv)By a “net exercise” arrangement pursuant to which the Company will reduce the number of shares of Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price.

Payment instruments will be received subject to collection. The transfer to the optionee on the records of the Company or of the transfer agent of the shares of Stock to be purchased pursuant to the exercise of a Stock Option will be contingent upon receipt from the optionee (or a purchaser acting in his stead in accordance with the provisions of the Stock Option) by the Company of the full purchase price for such shares and the fulfillment of any other requirements contained in the Award Certificate or applicable provisions of laws (including the satisfaction of any taxes that the Company or an Affiliate is obligated to withhold with respect to the optionee). In the event an optionee chooses to pay the purchase price by previously-owned shares of Stock through the attestation method, the number of shares of Stock transferred to the optionee upon the exercise of the Stock Option shall be net of the number of attested shares. In the event that the Company establishes, for itself or using the services of a third party, an automated system for the exercise of Stock Options, such as a system using an internet website or interactive voice response, then the paperless exercise of Stock Options may be permitted through the use of such an automated system.

SECTION 6. STOCK APPRECIATION RIGHTS

(a)Award of Stock Appreciation Rights. The Administrator may grant Stock Appreciation Rights under the Plan. A Stock Appreciation Right is an Award entitling the recipient to receive shares of Stock (or cash, to the extent explicitly provided for in the applicable Award Certificate) having a value equal to the excess of the Fair Market Value of a share of Stock on the date of exercise over the exercise price of the Stock Appreciation Right multiplied by the number of shares of Stock with respect to which the Stock Appreciation Right shall have been exercised.

(b)Exercise Price of Stock Appreciation Rights. The exercise price of a Stock Appreciation Right shall not be less than 100 percent of the Fair Market Value of the Stock on the date of grant. Notwithstanding the foregoing, Stock Appreciation Rights may be granted with an exercise price per share that is less than 100 percent of the Fair Market Value on the date of grant (i) to individuals who are not subject to U.S. income tax on the date of grant, or (ii) if the Stock Appreciation Right is otherwise exempt from or compliant with Section 409A.

(c)Grant and Exercise of Stock Appreciation Rights. Stock Appreciation Rights may be granted by the Administrator independently of any Stock Option granted pursuant to Section 5 of the Plan.

(d)Terms and Conditions of Stock Appreciation Rights. Stock Appreciation Rights shall be subject to such terms and conditions as shall be determined on the date of grant by the Administrator. The term of a Stock Appreciation Right may not exceed ten years. The terms and conditions of each such Award shall be determined by the Administrator, and such terms and conditions may differ among individual Awards and grantees.

SECTION 7. RESTRICTED STOCK AWARDS

(a)Nature of Restricted Stock Awards. The Administrator may grant Restricted Stock Awards under the Plan. A Restricted Stock Award is any Award of Restricted Shares subject to such restrictions and conditions as the Administrator may determine at the time of grant. Conditions may be based on continuing employment (or other Service Relationship) and/or achievement of pre-established performance goals and objectives.

(b)Rights as a Stockholder. Upon the grant of the Restricted Stock Award and payment of any applicable purchase price, a grantee shall have the rights of a stockholder with respect to the voting of the Restricted Shares and receipt of dividends; provided that if the lapse of restrictions with respect to the Restricted Stock Award is tied to the attainment of performance goals, any dividends paid by the Company during the performance period shall accrue and shall not be paid to the grantee until and to the extent the performance goals are met with respect to the Restricted Stock Award. Unless the Administrator shall otherwise determine, (i) uncertificated Restricted Shares shall be accompanied by a notation on the records of the Company or the transfer agent to the effect that they are subject to forfeiture until such Restricted Shares are vested as provided in Section 7(d) below, and (ii) certificated Restricted Shares shall remain in the possession of the Company until such Restricted Shares are vested as provided in Section 7(d) below, and the grantee shall be required, as a condition of the grant, to deliver to the Company such instruments of transfer as the Administrator may prescribe.

(c)Restrictions. Restricted Shares may not be sold, assigned, transferred, pledged or otherwise encumbered or disposed of except as specifically provided herein or in the Restricted Stock Award Certificate. Except as may otherwise be provided by the Administrator either in the Award Certificate or, subject to Section 16 below, in writing after the Award is issued, if a grantee's employment (or other Service Relationship) with the Company and its Affiliates terminates for any reason, any Restricted Shares that have not vested at the time of termination shall automatically and without any requirement of notice to such grantee from or other action by or on behalf of, the Company be deemed to have been reacquired by the Company at their original purchase price (if any) from such grantee or such grantee's legal representative simultaneously with such termination of employment (or other Service Relationship), and thereafter shall cease to represent any ownership of the Company by the grantee or rights of the grantee as a stockholder. Following such deemed reacquisition of Restricted Shares that are represented by physical certificates, a grantee shall surrender such certificates to the Company upon request without consideration.

(d)Vesting of Restricted Shares. The Administrator at the time of grant shall specify the date or dates and/or the attainment of pre-established performance goals, objectives and other conditions on which the non-transferability of the Restricted Shares and the Company's right of

repurchase or forfeiture shall lapse. Subsequent to such date or dates and/or the attainment of such pre-established performance goals, objectives and other conditions, the shares on which all restrictions have lapsed shall no longer be Restricted Shares and shall be deemed "vested."

SECTION 8. RESTRICTED STOCK UNITS

(a)Nature of Restricted Stock Units. The Administrator may grant Restricted Stock Units under the Plan. A Restricted Stock Unit is an Award of stock units that may be settled in shares of Stock (or cash, to the extent explicitly provided for in the Award Certificate) upon the satisfaction of such restrictions and conditions at the time of grant. Conditions may be based on continuing employment (or other Service Relationship) and/or achievement of pre-established performance goals and objectives. The terms and conditions of each such Award shall be determined by the Administrator, and such terms and conditions may differ among individual Awards and grantees. Except in the case of Restricted Stock Units with a deferred settlement date that complies with Section 409A, at the end of the vesting period, the Restricted Stock Units, to the extent vested, shall be settled in the form of shares of Stock (or cash, to the extent explicitly provided for in the Award Certificate). Restricted Stock Units with deferred settlement dates are subject to Section 409A and shall contain such additional terms and conditions as the Administrator shall determine in its sole discretion in order to comply with the requirements of Section 409A.

(b)Reserved.

(c)Rights as a Stockholder. A grantee shall have the rights as a stockholder only as to shares of Stock acquired by the grantee upon settlement of Restricted Stock Units; provided, however, that the grantee may be credited with Dividend Equivalent Rights with respect to the stock units underlying his or her Restricted Stock Units, subject to the provisions of Section 11 and such terms and conditions as the Administrator may determine.

(d)Termination. Except as may otherwise be provided by the Administrator either in the Award Certificate or, subject to Section 16 below, in writing after the Award is issued, a grantee's right in all Restricted Stock Units that have not vested shall automatically terminate upon the grantee's termination of employment (or cessation of Service Relationship) with the Company and its Affiliates for any reason.

SECTION 9. UNRESTRICTED STOCK AWARDS

Grant or Sale of Unrestricted Stock. The Administrator may grant (or sell at par value or such higher purchase price determined by the Administrator) an Unrestricted Stock Award under the Plan. An Unrestricted Stock Award is an Award pursuant to which the grantee may receive shares of Stock free of any restrictions under the Plan. Unrestricted Stock Awards may be granted in respect of past services or other valid consideration, or in lieu of cash compensation due to such grantee.

SECTION 10. RESERVED

SECTION 11. DIVIDEND EQUIVALENT RIGHTS

(a)Dividend Equivalent Rights. The Administrator may grant Dividend Equivalent Rights under the Plan. A Dividend Equivalent Right is an Award entitling the grantee to receive credits based on cash dividends that would have been paid on the shares of Stock specified in the Dividend Equivalent Right (or other Award to which it relates) if such shares had been issued to the grantee. A Dividend Equivalent Right may be granted hereunder to any grantee as a component of an award of Restricted Stock Units or as a freestanding award. The terms and conditions of Dividend Equivalent Rights shall be specified in the Award Certificate. Dividend equivalents credited to the holder of a Dividend Equivalent Right may be paid currently or may be deemed to be reinvested in additional shares of Stock, which may thereafter accrue additional equivalents. Any such reinvestment shall be at Fair Market Value on the date of reinvestment or such other price as may then apply under a dividend reinvestment plan sponsored by the Company, if any. Dividend Equivalent Rights may be settled in cash or shares of Stock or a combination thereof, in a single installment or installments. A Dividend Equivalent Right granted as a component of an Award of Restricted Stock Units shall provide that such Dividend Equivalent Right shall be settled only upon settlement or payment of, or lapse of restrictions on, such other Award, and that such Dividend Equivalent Right shall expire or be forfeited or annulled under the same conditions as such other Award.

(b)Termination. Except as may otherwise be provided by the Administrator either in the Award Certificate or, subject to Section 16 below, in writing after the Award is issued, a grantee's rights in all Dividend Equivalent Rights shall automatically terminate upon the grantee's termination of employment (or cessation of Service Relationship) with the Company and its Affiliates for any reason.

SECTION 12. TRANSFERABILITY OF AWARDS

(a)Transferability. Except as provided in Section 12(b) below or otherwise determined by the Administrator, during a grantee's lifetime, his or her Awards shall be exercisable only by the grantee, or by the grantee's legal representative or guardian in the event of the grantee's incapacity. No Awards shall be sold, assigned, transferred or otherwise encumbered or disposed of by a grantee other than by will or by the laws of descent and distribution or pursuant to a domestic relations order. No Awards shall be subject, in whole or in part, to attachment, execution, or levy of any kind, and any purported transfer in violation hereof shall be null and void.

(b)Administrator Action. Notwithstanding Section 12(a), the Administrator, in its discretion, may provide either in the Award Certificate regarding a given Award or by subsequent written approval that the grantee may transfer his or her Non-Qualified Stock Options to his or her immediate family members, to trusts for the benefit of such family members, or to partnerships in which such family members are the only partners, provided that the transferee agrees in writing with the Company to be bound by all of the terms and conditions of this Plan and the applicable Award Certificate. In no event may an Award be transferred by a grantee for value.

(c)Family Member. For purposes of Section 12(b), “family member” shall mean a grantee’s child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, any person sharing the grantee’s household (other than a tenant of the grantee), a trust in which these persons (or the grantee) have more than 50 percent of the beneficial interest, a foundation in which these persons (or the grantee) control the management of assets, and any other entity in which these persons (or the grantee) own more than 50 percent of the voting interests.

(d)Designation of Beneficiary. To the extent permitted by the Company and valid under applicable law, each grantee to whom an Award has been made under the Plan may designate a beneficiary or beneficiaries to exercise any Award or receive any payment under any Award payable on or after the grantee’s death. Any such designation shall be on a form provided for that purpose by the Administrator and shall not be effective until received by the Administrator. If no beneficiary has been designated by a deceased grantee, or if the designated beneficiaries have predeceased the grantee, the beneficiary shall be the grantee’s estate or legal heirs.

SECTION 13. TAX WITHHOLDING

(a)Payment by Grantee. Each grantee shall, no later than the date as of which the value of an Award or of any Stock or other amounts received thereunder first becomes includable in the gross income of the grantee for income tax purposes, pay to the Company or any applicable Affiliate, or make arrangements satisfactory to the Administrator regarding payment of, any U.S. and non-U.S. federal, state, or local taxes of any kind required by law to be withheld by the Company or any applicable Affiliate with respect to such income. The Company and its Affiliates shall, to the extent permitted by law, have the right to deduct any such taxes from any payment of any kind otherwise due to the grantee or to satisfy any applicable withholding obligations by any other method of withholding that the Company and its Affiliates deem appropriate. The Company’s obligation to deliver evidence of book entry (or stock certificates) to any grantee is subject to and conditioned on tax withholding obligations being satisfied by the grantee.

(b)Payment in Stock. The Administrator may cause any tax withholding obligation of the Company or any applicable Affiliate to be satisfied, in whole or in part, by the Company withholding from shares of Stock to be issued pursuant to any Award a number of shares with an aggregate Fair Market Value (as of the date the withholding is effected) that would satisfy the withholding amount due; provided, however, that the amount withheld does not exceed the maximum statutory rate or such lesser amount as is necessary to avoid liability accounting treatment. For purposes of share withholding, the Fair Market Value of withheld shares shall be determined in the same manner as the value of Stock includible in income of the grantees. The Administrator may also require the Company’s tax withholding obligation to be satisfied, in whole or in part, by an arrangement whereby a certain number of shares of Stock issued pursuant to any Award are immediately sold and proceeds from such sale are remitted to the Company in an amount that would satisfy the withholding amount due.

SECTION 14. SECTION 409A AWARDS

Awards are intended to be exempt from Section 409A to the greatest extent possible and to otherwise comply with Section 409A. The Plan and all Awards shall be interpreted in accordance with such intent. To the extent that any Award is determined to constitute "nonqualified deferred compensation" within the meaning of Section 409A (a "409A Award"), the Award shall be subject to such additional rules and requirements as specified by the Administrator from time to time in order to comply with Section 409A. In this regard, if any amount under a 409A Award is payable upon a "separation from service" (within the meaning of Section 409A) to a grantee who is then considered a "specified employee" (within the meaning of Section 409A), then no such payment shall be made prior to the date that is the earlier of (i) six months and one day after the grantee's separation from service, or (ii) the grantee's death, but only to the extent such delay is necessary to prevent such payment from being subject to interest, penalties and/or additional tax imposed pursuant to Section 409A. Further, the settlement of any 409A Award may not be accelerated except to the extent permitted by Section 409A. The Company makes no representation that any or all of the payments or benefits described in the Plan will be exempt from or comply with Section 409A of the Code and makes no undertaking to preclude Section 409A of the Code from applying to any such payment. The grantee shall be solely responsible for the payment of any taxes and penalties incurred under Section 409A.

SECTION 15. TERMINATION OF SERVICE RELATIONSHIP, TRANSFER, LEAVE OF ABSENCE, ETC.

(a)Termination of Service Relationship. If the grantee's Service Relationship is with an Affiliate and such Affiliate ceases to be an Affiliate, the grantee shall be deemed to have terminated his or her Service Relationship for purposes of the Plan.

(b)For purposes of the Plan, the following events shall not be deemed a termination of a Service Relationship:

(i)a transfer to the employment of the Company from an Affiliate or from the Company to an Affiliate, or from one Affiliate to another; or

(ii)an approved leave of absence for military service or sickness, or for any other purpose approved by the Company, if the employee's right to re-employment is guaranteed either by a statute or by contract or under the policy pursuant to which the leave of absence was granted or if the Administrator otherwise so provides in writing.

SECTION 16. AMENDMENTS AND TERMINATION

The Board may, at any time, amend or discontinue the Plan and the Administrator may, at any time, amend or cancel any outstanding Award for the purpose of satisfying changes in law or for any other lawful purpose, but no such action shall materially and adversely affect rights under any outstanding Award without the holder's consent. The Administrator is specifically authorized to exercise its discretion to reduce the exercise price of outstanding Stock Options or Stock Appreciation Rights or effect the repricing of such Awards through cancellation and

re-grants. Nothing in this Section 16 shall limit the Administrator's authority to take any action permitted pursuant to Section 3(b) or 3(c).

SECTION 17. STATUS OF PLAN

With respect to the portion of any Award that has not been exercised and any payments in cash, Stock or other consideration not received by a grantee, a grantee shall have no rights greater than those of a general creditor of the Company unless the Administrator shall otherwise expressly determine in connection with any Award or Awards. In its sole discretion, the Administrator may authorize the creation of trusts or other arrangements to meet the Company's obligations to deliver Stock or make payments with respect to Awards hereunder, provided that the existence of such trusts or other arrangements is consistent with the foregoing sentence.

SECTION 18. GENERAL PROVISIONS

(a)No Distribution. The Administrator may require each person acquiring Stock pursuant to an Award to represent to and agree with the Company in writing that such person is acquiring the shares without a view to distribution thereof.

(b)Issuance of Stock. To the extent certificated, stock certificates to grantees under this Plan shall be deemed delivered for all purposes when the Company or a stock transfer agent of the Company shall have mailed such certificates in the United States mail, addressed to the grantee, at the grantee's last known address on file with the Company. Uncertificated Stock shall be deemed delivered for all purposes when the Company or a Stock transfer agent of the Company shall have given to the grantee by electronic mail (with proof of receipt) or by United States mail, addressed to the grantee, at the grantee's last known address on file with the Company, notice of issuance and recorded the issuance in its records (which may include electronic "book entry" records). Notwithstanding anything herein to the contrary, the Company shall not be required to issue or deliver any evidence of book entry or certificates evidencing shares of Stock pursuant to the exercise or settlement of any Award, unless and until the Administrator has determined, with advice of counsel (to the extent the Administrator deems such advice necessary or advisable), that the issuance and delivery is in compliance with all applicable laws, regulations of governmental authorities and, if applicable, the requirements of any exchange on which the shares of Stock are listed, quoted or traded. Any Stock issued pursuant to the Plan shall be subject to any stop-transfer orders and other restrictions as the Administrator deems necessary or advisable to comply with federal, state or foreign jurisdiction, securities or other laws, rules and quotation system on which the Stock is listed, quoted or traded. The Administrator may place legends on any Stock certificate or notations on any book entry to reference restrictions applicable to the Stock. In addition to the terms and conditions provided herein, the Administrator may require that an individual make such reasonable covenants, agreements, and representations as the Administrator, in its discretion, deems necessary or advisable in order to comply with any such laws, regulations, or requirements. The Administrator shall have the right to require any individual to comply with any timing or other restrictions with respect to the settlement or exercise of any Award, including a window-period limitation, as may be imposed in the discretion of the Administrator.

(c)No Fractional Shares. No fractional shares of Stock shall be issued or delivered pursuant to the Plan or any Award, and the Administrator shall determine whether cash, other securities or other property shall be paid or transferred in lieu of any fractional shares, or whether such fractional shares or any rights thereto shall be canceled, terminated or otherwise eliminated.

(d)Stockholder Rights. Until Stock is deemed delivered in accordance with Section 18(b), no right to vote or receive dividends or any other rights of a stockholder will exist with respect to shares of Stock to be issued in connection with an Award, notwithstanding the exercise of a Stock Option or any other action by the grantee with respect to an Award.

(e)Other Compensation Arrangements; No Employment Rights. Nothing contained in this Plan shall prevent the Board from adopting other or additional compensation arrangements, including trusts, and such arrangements may be either generally applicable or applicable only in specific cases. The adoption of this Plan and the grant of Awards do not confer upon any employee any right to continued employment with the Company or any Subsidiary.

(f)Trading Policy Restrictions. Option exercises and other Awards under the Plan shall be subject to the Company's insider trading policies and procedures, as in effect from time to time.

(g)Clawback Policy. A participant's rights with respect to any Award hereunder shall in all events be subject to reduction, cancellation, forfeiture or recoupment to the extent necessary to comply with (i) any right that the Company may have under any Company clawback, forfeiture or recoupment policy, as in effect from time to time or other agreement or arrangement with a grantee, or (ii) applicable law.

SECTION 19. EFFECTIVE DATE OF PLAN

This Plan shall become effective immediately upon approval by the Board.

SECTION 20. GOVERNING LAW

This Plan and all Awards and actions taken thereunder shall be governed by, and construed in accordance with, the General Corporation Law of the State of Delaware as to matters within the scope thereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Delaware, applied without regard to conflict of law principles.

SECTION 21. UNFUNDED PLAN

This Plan shall be unfunded. Although bookkeeping accounts may be established with respect to participants who are granted Awards under this Plan, any such accounts will be used merely as a bookkeeping convenience. The Company shall not be required to segregate any assets which may at any time be represented by Awards, nor shall this Plan be construed as

providing for such segregation, nor shall the Company or the Administrator be deemed to be a trustee of stock or cash to be awarded under the Plan.

DATE APPROVED BY BOARD OF DIRECTORS: August 15, 2024

NON-QUALIFIED STOCK OPTION AGREEMENT
UNDER THE PEPGEN INC.
2024 INDUCEMENT PLAN

Name of Optionee: _

No. of Option Shares: _

Option Exercise Price per Share: $_
[FMV on Grant Date]

Grant Date: _

Expiration Date: _
[No more than 10 years]

Pursuant to the PepGen Inc. 2024 Inducement Plan (the "Plan"), PepGen Inc. (the "Company") hereby grants to the Optionee named above an option (the "Stock Option") to purchase on or prior to the Expiration Date specified above all or part of the number of shares of Common Stock, par value $0.0001 per share (the "Stock"), of the Company specified above at the Option Exercise Price per Share specified above subject to the terms and conditions set forth herein and in the Plan. This Stock Option is not intended to be an "incentive stock option" under Section 422 of the Internal Revenue Code of 1986, as amended. This Stock Option is not intended to be an "incentive stock option" under Section 422 of the Internal Revenue Code of 1986, as amended.

1. Exercisability Schedule. No portion of this Stock Option may be exercised until such portion shall have become exercisable. Except as set forth below, and subject to the discretion of the Administrator (as defined in Section 1 of the Plan) to accelerate the exercisability schedule hereunder, this Stock Option shall be exercisable with respect to the following number of Option Shares on the dates indicated so long as the Optionee remains an employee of the Company or a Subsidiary on such dates:

Incremental Number of Option Shares Exercisable	Exercisability Date
_____ (___%)	_____
_____ (___%)	_____
_____ (___%)	_____
_____ (___%)	_____
_____ (___%)	_____

Once exercisable, this Stock Option shall continue to be exercisable at any time or times prior to the close of business on the Expiration Date, subject to the provisions hereof and of the Plan.

2. Manner of Exercise.

(a) The Optionee may exercise this Stock Option only in the following manner: from time to time on or prior to the Expiration Date of this Stock Option, the Optionee may give written notice to the Administrator of his or her election to purchase some or all of the Option Shares purchasable at the time of such notice. This notice shall specify the number of Option Shares to be purchased.

Payment of the purchase price for the Option Shares may be made by one or more of the following methods: (i) in cash, by certified or bank check or other instrument acceptable to the Administrator; (ii) through the delivery (or

attestation to the ownership) of shares of Stock that have been purchased by the Optionee on the open market or that are beneficially owned by the Optionee and are not then subject to any restrictions under any Company plan and that otherwise satisfy any holding periods as may be required by the Administrator; (iii) by the Optionee delivering to the Company a properly executed exercise notice together with irrevocable instructions to a broker to promptly deliver to the Company cash or a check payable and acceptable to the Company to pay the option purchase price, provided that in the event the Optionee chooses to pay the option purchase price as so provided, the Optionee and the broker shall comply with such procedures and enter into such agreements of indemnity and other agreements as the Administrator shall prescribe as a condition of such payment procedure; (iv) by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; or (v) a combination of (i), (ii), (iii) and (iv) above. Payment instruments will be received subject to collection.

The transfer to the Optionee on the records of the Company or of the transfer agent of the Option Shares will be contingent upon (i) the Company's receipt from the Optionee of the full purchase price for the Option Shares, as set forth above, (ii) the fulfillment of any other requirements contained herein or in the Plan or in any other agreement or provision of laws, and (iii) the receipt by the Company of any agreement, statement or other evidence that the Company may require to satisfy itself that the issuance of Stock to be purchased pursuant to the exercise of Stock Options under the Plan and any subsequent resale of the shares of Stock will be in compliance with applicable laws and regulations. In the event the Optionee chooses to pay the purchase price by previously-owned shares of Stock through the attestation method, the number of shares of Stock transferred to the Optionee upon the exercise of the Stock Option shall be net of the shares of Stock attested to.

(b) The shares of Stock purchased upon exercise of this Stock Option shall be transferred to the Optionee on the records of the Company or of the transfer agent upon compliance to the satisfaction of the Administrator with all requirements under applicable laws or regulations in connection with such transfer and with the requirements hereof and of the Plan. The determination of the Administrator as to such compliance shall be final and binding on the Optionee. The Optionee shall not be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Stock subject to this Stock Option unless and until this Stock Option shall have been exercised pursuant to the terms hereof, the Company or the transfer agent shall have transferred the shares to the Optionee, and the Optionee's name shall have been entered as the stockholder of record on the books of the Company. Thereupon, the Optionee shall have full voting, dividend and other ownership rights with respect to such shares of Stock.

(c) The minimum number of shares with respect to which this Stock Option may be exercised at any one time shall be 100 shares, unless the number of shares with respect to which this Stock Option is being exercised is the total number of shares subject to exercise under this Stock Option at the time.

(d) Notwithstanding any other provision hereof or of the Plan, no portion of this Stock Option shall be exercisable after the Expiration Date hereof.

3. Termination of Employment. If the Optionee's employment with the Company or a Subsidiary (as defined in the Plan) terminates, the period within which to exercise the Stock Option may be subject to earlier termination as set forth below.

(a)Termination Due to Death. If the Optionee's employment with the Company or a Subsidiary terminates by reason of the Optionee's death, any portion of this Stock Option outstanding on such date, to the extent exercisable on the date of death, may thereafter be exercised by the Optionee's legal representative or legatee for a period of 12 months from the date of death or until the Expiration Date, if earlier. Any portion of this Stock Option that is not exercisable on the date of death shall terminate immediately and be of no further force or effect.

(b)Termination Due to Disability. If the Optionee's employment with the Company or a Subsidiary terminates by reason of the Optionee's disability (as determined by the Administrator), any portion of this Stock Option outstanding on such date, to the extent exercisable on the date of such termination, may thereafter be exercised by the Optionee for a period of 12 months from the date of disability or until the Expiration Date, if earlier. Any portion of this Stock Option that is not exercisable on the date of disability shall terminate immediately and be of no further force or effect.

(c)Termination for Cause. If the Optionee's employment with the Company or a Subsidiary terminates for Cause, any portion of this Stock Option outstanding on such date shall terminate immediately and be of no further force and effect. For purposes hereof, "Cause" shall mean, unless otherwise provided in an employment or other service agreement between the Company and the Optionee, a determination by the Administrator that the Optionee shall be dismissed as a result of (i) any material breach by the Optionee of any agreement between the Optionee and the Company; (ii) the conviction of, indictment for or plea of nolo contendere by the Optionee to a felony or a crime involving moral turpitude; or (iii) any material misconduct or willful and deliberate non-performance (other than by reason of disability) by the Optionee of the Optionee's duties to the Company.

(d)Other Termination. If the Optionee's employment with the Company or a Subsidiary terminates for any reason other than the Optionee's death, the Optionee's disability or Cause, and unless otherwise determined by the Administrator, any portion of this Stock Option outstanding on such date may be exercised, to the extent exercisable on the date of termination, for a period of three months from the date of termination or until the Expiration Date, if earlier.

Any portion of this Stock Option that is not exercisable on the date of termination shall terminate immediately and be of no further force or effect.

The Administrator's determination of the reason for termination of the Optionee's employment with the Company or a Subsidiary shall be conclusive and binding on the Optionee and his or her representatives or legatees.

4. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Stock Option shall be subject to and governed by all the terms and conditions of the Plan, including the powers of the Administrator set forth in Section 2(b) of the Plan. Capitalized terms in this Agreement shall have the meaning specified in the Plan, unless a different meaning is specified herein.

5. Transferability. This Agreement is personal to the Optionee, is non-assignable and is not transferable in any manner, by operation of law or otherwise, other than by will or the laws of descent and distribution. This Stock Option is exercisable, during the Optionee's lifetime, only by the Optionee, and thereafter, only by the Optionee's legal representative or legatee.

6. Tax Withholding. The Optionee shall, not later than the date as of which the exercise of this Stock Option becomes a taxable event for Federal income tax purposes, pay to the Company or make arrangements satisfactory to the Administrator for payment of any Federal, state, and local taxes required by law to be withheld on account of such taxable event. The Company shall have the authority to cause the required tax withholding obligation to be satisfied, in whole or in part, by (i) withholding from shares of Stock to be issued to the Optionee a number of shares of Stock with an aggregate Fair Market Value that would satisfy the withholding amount due; or (ii) causing its transfer agent to sell from the number of shares of Stock to be issued to the Optionee, the number of shares of Stock necessary to satisfy the Federal, state and local taxes required by law to be withheld from the Optionee on account of such transfer.

7. No Obligation to Continue Employment. Neither the Company nor any Subsidiary is obligated by or as a result of the Plan or this Agreement to continue the Optionee's employment with the Company or a Subsidiary and neither the Plan nor this Agreement shall interfere in any way with the right of the Company or any Subsidiary to terminate the Optionee's employment with the Company or a Subsidiary at any time.

8. Integration. This Agreement constitutes the entire agreement between the parties with respect to this Stock Option and supersedes all prior agreements and discussions between the parties concerning such subject matter.

9. Data Privacy Consent. In order to administer the Plan and this Agreement and to implement or structure future equity grants, the Company, its subsidiaries and affiliates and certain agents thereof (together, the "Relevant Companies") may process any and all personal or professional data, including but not limited to Social Security or other identification number, home address and telephone number, date of birth and other information that is necessary or desirable for the administration of the Plan and/or this Agreement (the "Relevant Information"). By

entering into this Agreement, the Optionee (i) authorizes the Company to collect, process, register and transfer to the Relevant Companies all Relevant Information; (ii) waives any privacy rights the Optionee may have with respect to the Relevant Information; (iii) authorizes the Relevant Companies to store and transmit such information in electronic form; and (iv) authorizes the transfer of the Relevant Information to any jurisdiction in which the Relevant Companies consider appropriate. The Optionee shall have access to, and the right to change, the Relevant Information. Relevant Information will only be used in accordance with applicable law.

10.Notices. Notices hereunder shall be mailed or delivered to the Company at its principal place of business and shall be mailed or delivered to the Optionee at the address on file with the Company or, in either case, at such other address as one party may subsequently furnish to the other party in writing.

PEPGEN INC.

By:
Title:

The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned. Electronic acceptance of this Agreement pursuant to the Company's instructions to the Optionee (including through an online acceptance process) is acceptable.

Dated:

Optionee's Signature

Optionee's name and address:

RESTRICTED STOCK UNIT AWARD AGREEMENT
UNDER THE PEPGEN INC.
2024 INDUCEMENT PLAN

Name of Grantee: _____

No. of Restricted Stock Units:

Grant Date:

Pursuant to the PepGen Inc. 2024 Inducement Plan (the "Plan"), PepGen Inc. (the "Company") hereby grants an award of the number of Restricted Stock Units listed above (an "Award") to the Grantee named above. Each Restricted Stock Unit shall relate to one share of Common Stock, par value $0.0001 per share (the "Stock"), of the Company. This Award has been granted as an inducement pursuant to Rule 5635(c)(4) of the Marketplace Rules of NASDAQ Stock Market, Inc.

1. Restrictions on Transfer of Award. This Award may not be sold, transferred, pledged, assigned or otherwise encumbered or disposed of by the Grantee, and any shares of Stock issuable with respect to the Award may not be sold, transferred, pledged, assigned or otherwise encumbered or disposed of until (i) the Restricted Stock Units have vested as provided in Paragraph 2 of this Agreement and (ii) shares of Stock have been issued to the Grantee in accordance with the terms of the Plan and this Agreement.

2. Vesting of Restricted Stock Units. The restrictions and conditions of Paragraph 1 of this Agreement shall lapse on the Vesting Date or Dates specified in the following schedule so long as the Grantee remains an employee of the Company or a Subsidiary on such Vesting Dates. If a series of Vesting Dates is specified, then the restrictions and conditions in Paragraph 1 shall lapse only with respect to the number of Restricted Stock Units specified as vested on such date.

Incremental Number of Restricted Stock Units Vested	Vesting Date
_____ (__%)	
_____ (__%)	
_____ (__%)	
_____ (__%)	

The Administrator may at any time accelerate the vesting schedule specified in this Paragraph 2.

3. Termination of Employment. If the Grantee's employment with the Company or a Subsidiary terminates for any reason (including death or disability) prior to the satisfaction of the vesting conditions set forth in Paragraph 2 above, any Restricted Stock Units that have not vested as of such date shall automatically and without notice terminate and be forfeited, and neither the Grantee nor any of his or her successors, heirs, assigns, or personal representatives will thereafter have any further rights or interests in such unvested Restricted Stock Units.

4. Issuance of Shares of Stock. As soon as practicable following each Vesting Date (but in no event later than two and one-half months after the end of the year in which the Vesting Date occurs), the Company shall issue to the Grantee the number of shares of Stock equal to the aggregate number of Restricted Stock Units that have vested pursuant to Paragraph 2 of this Agreement on such date and the Grantee shall thereafter have all the rights of a stockholder of the Company with respect to such shares.

5. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Agreement shall be subject to and governed by all the terms and conditions of the Plan, including the powers of the Administrator set forth in Section 2(b) of the Plan. Capitalized terms in this Agreement shall have the meaning specified in the Plan, unless a different meaning is specified herein.

6. Tax Withholding. The Grantee shall, not later than the date as of which the receipt of this Award becomes a taxable event for Federal income tax purposes, pay to the Company or make arrangements satisfactory to the Administrator for payment of any Federal, state, and local taxes required by law to be withheld on account of such taxable event. The Company shall have the authority to cause the required tax withholding obligation to be satisfied, in whole or in part, by (i) withholding from shares of Stock to be issued to the Grantee a number of shares of Stock with an aggregate Fair Market Value that would satisfy the withholding amount due; or (ii) causing its transfer agent to sell from the number of shares of Stock to be issued to the Grantee, the number of shares of Stock necessary to satisfy the Federal, state and local taxes required by law to be withheld from the Grantee on account of such transfer.

7. Section 409A of the Code. This Agreement shall be interpreted in such a manner that all provisions relating to the settlement of the Award are exempt from the requirements of Section 409A of the Code as "short-term deferrals" as described in Section 409A of the Code.

8. No Obligation to Continue Employment. Neither the Company nor any Subsidiary is obligated by or as a result of the Plan or this Agreement to continue the Grantee's employment with the Company or a Subsidiary and neither the Plan nor this Agreement shall interfere in any way with the right of the Company or any Subsidiary to terminate the Grantee's employment with the Company or a Subsidiary at any time.

9. Integration. This Agreement constitutes the entire agreement between the parties with respect to this Award and supersedes all prior agreements and discussions between the parties concerning such subject matter.

10.Data Privacy Consent. In order to administer the Plan and this Agreement and to implement or structure future equity grants, the Company, its subsidiaries and affiliates and certain agents thereof (together, the "Relevant Companies") may process any and all personal or professional data, including but not limited to Social Security or other identification number, home address and telephone number, date of birth and other information that is necessary or desirable for the administration of the Plan and/or this Agreement (the "Relevant Information"). By entering into this Agreement, the Grantee (i) authorizes the Company to collect, process, register and transfer to the Relevant Companies all Relevant Information; (ii) waives any privacy rights the Grantee may have with respect to the Relevant Information; (iii) authorizes the Relevant Companies to store and transmit such information in electronic form; and (iv) authorizes the transfer of the Relevant Information to any jurisdiction in which the Relevant Companies consider appropriate. The Grantee shall have access to, and the right to change, the Relevant Information. Relevant Information will only be used in accordance with applicable law.

11.Notices. Notices hereunder shall be mailed or delivered to the Company at its principal place of business and shall be mailed or delivered to the Grantee at the address on file with the Company or, in either case, at such other address as one party may subsequently furnish to the other party in writing.

PEPGEN INC.

By:

Title:

The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned. Electronic acceptance of this Agreement pursuant to the Company's instructions to the Grantee (including through an online acceptance process) is acceptable.

Dated:

Grantee's Signature

Grantee's name and address:

EMPLOYMENT AGREEMENT

This Employment Agreement ("Agreement") is made between PepGen Inc. (the "Company"), and Paul D. Streck, MD, MBA (the "Employee") and is effective as of August 19, 2024 (the "Effective Date").

WHEREAS, the Company has completed a satisfactory background check and desires to employ you, and you desire to be employed by the Company on the terms and conditions contained herein.

NOW, THEREFORE, in consideration of the mutual covenants and agreements herein contained and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties agree as follows:

1.Employment.

(a)Term. The Company shall employ you and you shall be employed by the Company pursuant to this Agreement commencing as of the Effective Date and continuing until such employment is terminated in accordance with the provisions hereof (the "Term"). Your employment with the Company will be "at will," meaning that your employment may be terminated by the Company or you at any time and for any reason subject to the terms of this Agreement.

(b)Position and Duties. You shall serve as the Executive Vice President, Head of Research and Development of the Company and shall have such powers and duties as may from time to time be prescribed by the Chief Executive Officer (the "CEO"). You will perform your services remotely and report to the office in Massachusetts on a periodic basis (anticipated to be 2-3 days every other week) unless reasonably requested to report to the office in Massachusetts more frequently. You shall devote your full working time and efforts to the business and affairs of the Company and will not engage in any other business activities during your employment by the Company; provided that, subject to the Company's prior written approval, including any required approval by the Board of Directors (the "Board") or any Committee thereof, which will not unreasonably be delayed or withheld, you may act as a director or advisor to a non-competitive entity so long as such activities do not, individually or in the aggregate, interfere with the performance of your duties or constitute a conflict of interest.

2.Compensation and Related Matters.

(a)Base Salary. Your initial base salary shall be paid at the rate of $525,000 per year. Your base salary shall be subject to periodic review by the Board or the Compensation Committee of the Board (the "Compensation Committee"). The base salary in effect at any given time is referred to herein as "Base Salary." The Base Salary shall be payable in a manner that is consistent with the Company's usual payroll practices.

(b)**Sign-On Bonus**. The Company will pay you a one-time Sign On Bonus of sixty thousand dollars ($60,000), payable within three (3) months of the Start Date. You agree that if you voluntarily resign from employment within twelve (12) months following the Start

Date, you will repay the Company the gross amount of the Sign-On Bonus within ten (10) days following the date of termination.

(c)Annual Discretionary Incentive Compensation. You shall be eligible to receive annual cash incentive compensation as determined by the Board or the Compensation Committee. Your target annual incentive compensation shall be forty percent (40%) of your Base Salary and such percentage may be increased but not decreased. The target annual incentive compensation in effect at any given time is referred to herein as "Target Bonus." The actual amount of your annual incentive compensation, if any, shall be determined in the sole discretion of the Board or the Compensation Committee. Except as otherwise provided in the severance section herein, to earn incentive compensation, you must be employed by the Company on the day such incentive compensation is paid which shall be no later than March 15 of the year following the relevant performance year.

(d)Expenses. You shall be eligible for reimbursement for all reasonable and documented expenses incurred by you during the Term in performing services hereunder, in accordance with the policies and procedures then in effect and established by the Company for its executive officers, including, without limitation, travel expenses when Employee reports to the Company office in Boston, MA, pursuant to such an arrangement as the Company deems reasonable.

(e)Other Benefits. You shall be eligible to participate in or receive benefits under the Company's employee benefit plans in effect from time to time, subject to the terms of such plans, which terms are subject to change by the Company.

(f)Paid Time Off. You shall be eligible to take paid time off in accordance with the Company's applicable paid time off policy for executives, as may be in effect from time to time.

(g)Stock Option. The Company will recommend to the Board or the Compensation Committee that Employee be granted, effective as of the Effective Date, (i) a stock option to purchase 40,000 shares of the Company's common stock, par value $0.0001 per share ("Common Stock") (the "New Hire ISO"), and (ii) as a material inducement to Employee's entering into this Agreement and commencing an employment relationship with the Company, an option to purchase 202,816 shares of the Company's Common Stock (the "Inducement NSO" and together with the New Hire ISO, the "New Hire Equity Awards"). The New Hire Equity Awards will be granted at an exercise price per share that is no less than the Fair Market Value (as defined in the Company's 2022 Stock Option and Incentive Plan and 2024 Inducement Plan, as the case may be) per share of the Company's Common Stock as of the date of grant and will have the following terms:

(i)New Hire ISO: Subject to the terms of the Company's 2022 Stock Option and Incentive Plan and the applicable award agreement evidencing the New Hire ISO, the shares underlying the New Hire ISO shall vest as follows: 25% upon the first anniversary of the Effective Date, and the balance vesting monthly over the following thirty-six (36) months), subject to Employee's continued employment with the Company as of each such vesting date (except as set forth below in Section 6(a)(ii)). The New Hire

ISO shall be intended to qualify as an "incentive stock option" under Section 422 Code (defined below) to the maximum extent permitted by applicable law;

(ii)Inducement NSO: Subject to the terms of the Company's 2024 Inducement Plan and the applicable award agreement evidencing the Inducement NSO, the shares underlying the Inducement NSO shall vest as follows: 25% upon the first anniversary of the Effective Date, and the balance vesting monthly over the following thirty-six (36) months), subject to Employee's continued employment with the Company as of each such vesting date (except as set forth below in Section 6(a)(ii)); and

(iii)Beginning in the calendar year 2025, and subject to the approval and discretion of the Board (or a duly authorized Committee thereof) and subject to Employee's continued employment with the Company on the date of grant, Employee shall be eligible to receive additional equity awards on an annual basis, subject to the approval of the Board or the Compensation Committee. The equity award approved in calendar year 2025, if any, shall not be subject to pro-ration due to a partial year of service in 2024.

(iv)The Company agrees to use commercially reasonable efforts to file a Registration Statement on Form S-8 to register the shares of Common Stock underlying the Inducement NSO prior to the vesting of any portion of such award.

3.Termination. Your employment hereunder may be terminated without any breach of this Agreement under the following circumstances:

(a)Death. Your employment hereunder shall terminate upon death.

(b)Disability. The Company may terminate your employment if you are disabled and unable to perform or expected to be unable to perform the essential functions of your then existing position or positions under this Agreement with or without reasonable accommodation for a period of 180 days (which need not be consecutive) in any 12-month period. If any question shall arise as to whether during any period you are disabled so as to be unable to perform the essential functions of your then existing position or positions with or without reasonable accommodation, you may, and at the request of the Company shall, submit to the Company a certification in reasonable detail by a physician selected by the Company to whom you or your guardian has no reasonable objection as to whether you are disabled or how long such disability is expected to continue, and such certification shall for the purposes of this Agreement be conclusive of the issue. You shall cooperate with any reasonable request of the physician in connection with such certification. If such question shall arise and you shall fail to submit such certification, the Company's determination of such issue shall be binding on you. Nothing in this Section 3(b) shall be construed to waive your rights, if any, under existing law including, without limitation, the Family and Medical Leave Act of 1993, 29 U.S.C. §2601 *et seq*. and the Americans with Disabilities Act, 42 U.S.C. §12101 *et seq.*

(c)Termination by Company for Cause. The Company may terminate your employment hereunder for Cause. For purposes of this Agreement, "Cause" shall mean any of the following:

(i)conduct by you constituting a material act of misconduct in connection with the performance of your duties, including, without limitation, (A) willful failure or refusal to perform material responsibilities that have been requested by the CEO; (B) dishonesty to the CEO with respect to any material matter; or (C) misappropriation of funds or property of the Company or any of its subsidiaries or affiliates other than the occasional, customary and *de minimis* use of Company property for personal purposes;

(ii)the commission by you of acts satisfying the elements of (A) any felony or (B) a misdemeanor involving moral turpitude, deceit, dishonesty or fraud;

(iii)any misconduct by you, regardless of whether or not in the course of your employment, that would reasonably be expected to result in material injury or reputational harm to the Company or any of its subsidiaries or affiliates if you were to continue to be employed in the same position;

(iv)continued non-performance by you of your duties hereunder (other than by reason of your physical or mental illness, incapacity or disability) which has continued for more than 30 days following written notice of such non-performance from the CEO;

(v)a breach by you of any of the provisions contained in Section 8 of this Agreement or the Restrictive Covenants Agreement (as defined below);

(vi)a material violation by you of any of the Company's written employment policies; or

(vii)your failure to cooperate with a bona fide internal investigation or an investigation by regulatory or law enforcement authorities, after being instructed by the Company to cooperate, or the willful destruction or failure to preserve documents or other materials known to be relevant to such investigation or the inducement of others to fail to cooperate or to produce documents or other materials in connection with such investigation.

(d)Termination by the Company without Cause. The Company may terminate your employment hereunder at any time without Cause. A termination due to your death or disability under Section 3(a) or (b) shall not constitute a termination without Cause.

(e)Termination by You. You may terminate employment hereunder at any time for any reason, including but not limited to, Good Reason. For purposes of this Agreement, "Good Reason" shall mean that you have completed all steps of the Good Reason Process (hereinafter defined) following the occurrence of any of the following events without your consent (each, a "Good Reason Condition"):

(i)a material diminution in your responsibilities and duties that occurs within the 12 months following a Sales Event; ;

(ii) a material diminution in your Base Salary except for across-the-board salary reductions based on the Company's financial performance similarly affecting all or substantially all senior management employees of the Company;

(iii) a material change in the physical Company office location at which the Company requires you to provide services to the Company, such that there is an increase of at least forty (40) miles of driving distance between the Company's prior office location and its new location (and to avoid doubt, a home or remote office does not constitute a Company office location for these purposes);

(iv) a material breach by the Company of the compensation provisions of this Agreement.

The "Good Reason Process" consists of the following steps:

(v) you reasonably determine in good faith that a Good Reason Condition has occurred;

(vi) you notify the Company in writing of the first occurrence of the Good Reason Condition within 60 days of the first occurrence of such condition;

(vii) you cooperate in good faith with the Company's efforts, for a period of not less than 30 days following such notice (the "Cure Period"), to remedy the Good Reason Condition;

(viii) notwithstanding such efforts, the Good Reason Condition continues to exist; and

(ix) you terminate employment within 60 days after the end of the Cure Period.

If the Company cures the Good Reason Condition during the Cure Period, Good Reason shall be deemed not to have occurred.

If your employment with the Company is terminated for any reason, the Company shall pay or provide to you (or your authorized representative or estate) (i) any Base Salary earned through the Date of Termination; and (ii) any unpaid expense reimbursements (subject to, and in accordance with this Agreement) (collectively, the "Accrued Obligations").

4. Notice and Date of Termination; Resignations Upon Termination.

(a) Notice of Termination. Except for termination as specified in Section 3(a), any termination of your employment by the Company or any such termination by you shall be communicated by written Notice of Termination to the other party hereto. For purposes of this Agreement, a "Notice of Termination" shall mean a notice which shall indicate the specific termination provision in this Agreement relied upon.

(b)Date of Termination. "Date of Termination" shall mean: (i) if your employment is terminated by death, the date of death; (ii) if your employment is terminated on account of disability under Section 3(b) or by the Company for Cause under Section 3(c), the date on which Notice of Termination is given; (iii) if your employment is terminated by the Company without Cause under Section 3(d), the date on which a Notice of Termination is given or the date otherwise specified by the Company in the Notice of Termination; (iv) if your employment is terminated by you under Section 3(e) other than for Good Reason, 14 days after the date on which a Notice of Termination is given, and (v) if your employment is terminated by you under Section 3(e) for Good Reason, the date on which a Notice of Termination is given after the end of the Cure Period. Notwithstanding the foregoing, in the event that you give a Notice of Termination to the Company, the Company may unilaterally accelerate the Date of Termination and such acceleration shall not result in a termination by the Company for purposes of this Agreement.

(c)Resignations From Other Positions Upon Termination. In connection with your termination of employment for any reason, you shall be deemed to have resigned from all officer and board member positions that you hold with the Company or any of its subsidiaries and affiliates. You shall execute any documents in reasonable form as may be requested to confirm or effectuate any such resignations.

5.Severance Pay and Benefits Upon Termination by the Company without Cause or by You for Good Reason Outside the Sale Event Period. If your employment is terminated by the Company without Cause as provided in Section 3(d), or you terminate employment for Good Reason as provided in Section 3(e), in either case outside of the Sale Event Period (as defined below), then, in addition to the Accrued Obligations, and subject to (i) you signing a separation agreement and release in a form and manner provided by the Company, which shall include, without limitation, a general release of claims against the Company and all related persons and entities, and a reaffirmation of all of your Continuing Obligations (as defined below**),** and shall provide that if you breach any of the Continuing Obligations, all payments of the Severance Amount shall immediately cease (the "Separation Agreement and Release"), and (ii) the Separation Agreement and Release becoming irrevocable, all within 60 days after the Date of Termination (or such shorter period as set forth in the Separation Agreement and Release), which shall include a seven (7) business day revocation period:

(a)the Company shall pay you an amount equal to nine (9) months of your Base Salary (the "Severance Amount"); provided in the event you are entitled to any payments pursuant to the Restrictive Covenants Agreement, the Severance Amount received in any calendar year will be reduced by the amount you are paid in the same such calendar year pursuant to the Restrictive Covenants Agreement (the "Restrictive Covenants Agreement Setoff"); and

(b)subject to your copayment of premium amounts at the applicable active employees' rate and your proper election to receive benefits under the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended ("COBRA"), the Company shall pay to the group health plan provider, the COBRA provider or you a monthly payment equal to the monthly employer contribution that the Company would have made to provide health insurance to you if

you had remained employed by the Company until the earliest of (A) the twelve (12) month anniversary of the Date of Termination; (B) your eligibility for group medical plan benefits under any other employer's group medical plan; or (C) the cessation of your continuation rights under COBRA; provided, however, if the Company determines that it cannot pay such amounts to the group health plan provider or the COBRA provider (if applicable) without potentially violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), then the Company shall convert such payments to payroll payments directly to you for the time period specified above. Such payments shall be subject to tax-related deductions and withholdings and paid on the Company's regular payroll dates.

(c)the Company shall pay you a prorated portion of your Target Bonus for the year in which the Date of Termination occurs (the "Termination Year") (which portion shall be prorated by multiplying the Target Bonus amount by x/12, with x representing the number of completed full calendar months in the Termination Year prior to the Date of Termination), which portion the Company shall pay during the year following the Termination Year, on or around the same time the Company pays bonuses to other employees with respect to the Termination Year.

The amounts payable under Section 5 (a) and (b), to the extent taxable, shall be paid out in substantially equal installments in accordance with the Company's payroll practice over nine (9) months commencing within 60 days after the Date of Termination; provided, however, that if the 60-day period begins in one calendar year and ends in a second calendar year, the Severance Amount, to the extent it qualifies as "non-qualified deferred compensation" within the meaning of Section 409A of the Internal Revenue Code of 1986, as amended (the "Code"), shall begin to be paid in the second calendar year by the last day of such 60-day period; provided, further, that the initial payment shall include a catch-up payment to cover amounts retroactive to the day immediately following the Date of Termination. Each payment pursuant to this Agreement is intended to constitute a separate payment for purposes of Treasury Regulation Section 1.409A-2(b)(2).

6.Severance Pay and Benefits Upon Termination by the Company without Cause or by the You for Good Reason within the Sale Event Period. The provisions of this Section 6 shall apply in lieu of, and expressly supersede, the provisions of Section 5 if (i) your employment is terminated either (a) by the Company without Cause as provided in Section 3(d), or (b) by you for Good Reason as provided in Section 3(e), and (ii) the Date of Termination is within 12 months after the occurrence of the first event constituting a Sale Event (such period, the "Sale Event Period"). These provisions shall terminate and be of no further force or effect after a Sale Event Period.

(a)If your employment is terminated by the Company without Cause as provided in Section 3(d) or you terminate employment for Good Reason as provided in Section 3(e) and in either case the Date of Termination occurs during the Sale Event Period, then, in addition to the Accrued Obligations, and subject to the signing of the Separation Agreement and Release by you and the Separation Agreement and Release becoming fully effective, all within the time frame set forth in the Separation Agreement and Release but in no event more than 60 days after the Date of Termination:

(i) the Company shall pay you a lump sum in cash in an amount equal to (A) 1.0 time your then current Base Salary (or your Base Salary in effect immediately prior to the Sale Event, if higher) plus (B) your Target Bonus for the Termination Year ((A) and (B), the "Sale Event Payment"); provided the Sale Event Payment shall be reduced by the amount of the Restrictive Covenants Agreement Setoff, if applicable; and

(ii) notwithstanding anything to the contrary in any applicable option agreement or other stock-based award agreement, all time-based stock options and other stock-based awards subject to time-based vesting held by you (the "Time-Based Equity Awards") shall immediately accelerate and become fully exercisable or nonforfeitable as of the later of (i) the Date of Termination or (ii) the effective date of the Separation Agreement and Release (the "Accelerated Vesting Date"); *provided* that any termination or forfeiture of the unvested portion of such Time-Based Equity Awards that would otherwise occur on the Date of Termination in the absence of this Agreement will be delayed until the effective date of the Separation Agreement and Release and will only occur if the vesting pursuant to this subsection does not occur due to the absence of the Separation Agreement and Release becoming fully effective within the time period set forth therein. Notwithstanding the foregoing, no additional vesting of the Time-Based Equity Awards shall occur during the period between your Date of Termination and the Accelerated Vesting Date; and

(iii) subject to your copayment of premium amounts at the applicable active employees' rate and your proper election to receive benefits under COBRA, the Company shall pay to the group health plan provider, the COBRA provider or you a monthly payment equal to the monthly employer contribution that the Company would have made to provide health insurance to you if you had remained employed by the Company until the earliest of (A) the eighteen (18) month anniversary of the Date of Termination; (B) your eligibility for group medical plan benefits under any other employer's group medical plan; or (C) the cessation of your continuation rights under COBRA; provided, however, if the Company determines that it cannot pay such amounts to the group health plan provider or the COBRA provider (if applicable) without potentially violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), then the Company shall convert such payments to payroll payments directly to you for the time period specified above. Such payments shall be subject to tax-related deductions and withholdings and paid on the Company's regular payroll dates.

The amounts payable under this Section 6(a), to the extent taxable, shall be paid or commence to be paid within 60 days after the Date of Termination; provided, however, that if the 60-day period begins in one calendar year and ends in a second calendar year, such payments to the extent they qualify as "non-qualified deferred compensation" within the meaning of Section 409A of the Code, shall be paid or commence to be paid in the second calendar year by the last day of such 60-day period.

(b) Additional Limitation.

(i)Anything in this Agreement to the contrary notwithstanding, in the event that the amount of any compensation, payment or distribution by the Company to or for the benefit of you, whether paid or payable or distributed or distributable pursuant to the terms of this Agreement or otherwise, calculated in a manner consistent with Section 280G of the Code, and the applicable regulations thereunder (the "Aggregate Payments"), would be subject to the excise tax imposed by Section 4999 of the Code, then the Aggregate Payments shall be reduced (but not below zero) so that the sum of all of the Aggregate Payments shall be $1.00 less than the amount at which you became the subject to the excise tax imposed by Section 4999 of the Code; provided that such reduction shall only occur if it would result in you receiving a higher After Tax Amount (as defined below) than you would receive if the Aggregate Payments were not subject to such reduction. In such event, the Aggregate Payments shall be reduced in the following order, in each case, in reverse chronological order beginning with the Aggregate Payments that are to be paid the furthest in time from consummation of the transaction that is subject to Section 280G of the Code: (1) cash payments not subject to Section 409A of the Code; (2) cash payments subject to Section 409A of the Code; (3) equity-based payments and acceleration; and (4) non-cash forms of benefits; provided that in the case of all the foregoing Aggregate Payments all amounts or payments that are not subject to calculation under Treas. Reg. §1.280G-1, Q&A-24(b) or (c) shall be reduced before any amounts that are subject to calculation under Treas. Reg. §1.280G-1, Q&A-24(b) or (c).

(ii)For purposes of this Section 6(b), the "After Tax Amount" means the amount of the Aggregate Payments less all federal, state, and local income, excise and employment taxes imposed on you as a result of your receipt of the Aggregate Payments. For purposes of determining the After Tax Amount, you shall be deemed to pay federal income taxes at the highest marginal rate of federal income taxation applicable to individuals for the calendar year in which the determination is to be made, and state and local income taxes at the highest marginal rates of individual taxation in each applicable state and locality, net of the maximum reduction in federal income taxes which could be obtained from deduction of such state and local taxes.

(iii)The determination as to whether a reduction in the Aggregate Payments shall be made pursuant to Section 6(b)(i) shall be made by a nationally recognized accounting firm selected by the Company (the "Accounting Firm"), which shall provide detailed supporting calculations both to the Company and you within 15 business days of the Date of Termination, if applicable, or at such earlier time as is reasonably requested by the Company or you. Any determination by the Accounting Firm shall be binding upon the Company and you.

(c)Sale Event. "Sale Event" is defined in the Equity Documents, which definition is reproduced here for reference: the occurrence of any of the following events: (i) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (ii) a merger, reorganization or consolidation pursuant to which the holders of the Company's outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or

other equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction, (iii) the sale of all of the Stock (as defined in the Equity Documents) of the Company to an unrelated person, entity or group thereof acting in concert, or (iv) any other transaction in which the owners of the Company's outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

7.Section 409A.

(a)Anything in this Agreement to the contrary notwithstanding, if at the time of your separation from service within the meaning of Section 409A of the Code, the Company determines that you are a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that you become entitled to under this Agreement or otherwise on account of your separation from service would be considered deferred compensation otherwise subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (A) six months and one day after your separation from service, or (B) your death. If any such delayed cash payment is otherwise payable on an installment basis, the first payment shall include a catch-up payment covering amounts that would otherwise have been paid during the six-month period but for the application of this provision, and the balance of the installments shall be payable in accordance with their original schedule.

(b)All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by you during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year (except for any lifetime or other aggregate limitation applicable to medical expenses). Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

(c)To the extent that any payment or benefit described in this Agreement constitutes "non-qualified deferred compensation" under Section 409A of the Code, and to the extent that such payment or benefit is payable upon your termination of employment, then such payments or benefits shall be payable only upon your "separation from service." The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A-1(h).

(d)The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. Each payment pursuant to this Agreement or the Restrictive Covenants Agreement is intended to constitute a separate payment for purposes of Treasury Regulation Section 1.409A-2(b)(2). The parties agree that this

Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

(e)The Company makes no representation or warranty and shall have no liability to you or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A of the Code but do not satisfy an exemption from, or the conditions of, such Section.

8.Obligations.

(a)Restrictive Covenants Agreement. As a condition of your employment, including your opportunity to receive the compensation and benefits provided in this Agreement, you are required to enter into the Employee Confidentiality, Assignment, Nonsolicitation and Noncompetition Agreement, attached hereto as Exhibit A (the "Restrictive Covenants Agreement"). You acknowledge and agree that you received the Restrictive Covenants Agreement with this Agreement and at least ten (10) business days before the date the Restrictive Covenants Agreement is to become effective. For purposes of this Agreement, the obligations in this Section 8, those that arise in the Restrictive Covenants Agreement and any other agreement between you and the Company relating to confidentiality, assignment of inventions, or other restrictive covenants shall collectively be referred to as the "Continuing Obligations."

(b)Third-Party Agreements and Rights. You hereby confirm that you are not bound by the terms of any agreement with any previous employer or other party which restricts in any way your use or disclosure of information, other than confidentiality restrictions (if any), or your engagement in any business. You represent to the Company that your execution of this Agreement, your employment with the Company and the performance of your proposed duties for the Company will not violate any obligations you may have to any such previous employer or other party. In your work for the Company, you will not disclose or make use of any information in violation of any agreements with or rights of any such previous employer or other party, and you will not bring to the premises of the Company any copies or other tangible embodiments of non-public information belonging to or obtained from any such previous employment or other party.

(c)Litigation and Regulatory Cooperation. During and after your employment, you shall cooperate fully with the Company in (i) the defense or prosecution of any claims or actions now in existence or which may be brought in the future against or on behalf of the Company which relate to events or occurrences that transpired while you were employed by the Company, and (ii) the investigation, whether internal or external, of any matters about which the Company believes you may have knowledge or information. Your full cooperation in connection with such claims, actions or investigations shall include, but not be limited to, being available to meet with counsel to answer questions or to prepare for discovery or trial and to act as a witness on behalf of the Company at mutually convenient times and with due regard to your professional and personal commitments. During and after your employment with due regard to your professional and personal commitments, you also shall cooperate fully with the Company in connection with any investigation or review of any federal, state or local regulatory authority as any such investigation or review relates to events or occurrences that transpired while you were

employed by the Company. The Company shall reimburse you for any reasonable out-of-pocket expenses incurred in connection with your performance of obligations pursuant to this Section 8(c).

(d)Relief. You agree that it would be difficult to measure any damages caused to the Company which might result from any breach by you of the Continuing Obligations, and that in any event money damages would be an inadequate remedy for any such breach. Accordingly**,** you agree that if you breach, or propose to breach, any portion of the Continuing Obligations, the Company shall be entitled, in addition to all other remedies that it may have, to an injunction or other appropriate equitable relief to restrain any such breach without showing or proving any actual damage to the Company.

(e)Protected Disclosures and Other Protected Action. Nothing in this Agreement shall be interpreted or applied to prohibit you from making any good faith report to any governmental agency or other governmental entity (a "Government Agency") concerning any act or omission that you reasonably believe constitutes a possible violation of federal or state law or making other disclosures that are protected under the anti-retaliation or whistleblower provisions of applicable federal or state law or regulation. In addition, nothing contained in this Agreement limits your ability to communicate with any Government Agency or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including your ability to provide documents or other information, without notice to the Company. In addition, for the avoidance of doubt, pursuant to the federal Defend Trade Secrets Act of 2016, you shall not be held criminally or civilly liable under any federal or state trade secret law or under this Agreement or the Restrictive Covenants Agreement for the disclosure of a trade secret that (a) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (b) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal.

9.Consent to Jurisdiction. The parties hereby consent to the jurisdiction of the state and federal courts of Massachusetts. Accordingly, with respect to any such court action, you (a) submit to the exclusive personal jurisdiction of such courts; (b) consent to service of process; and (c) waive any other requirement (whether imposed by statute, rule of court, or otherwise) with respect to personal jurisdiction or service of process.

10.Waiver of Jury Trial. You and the Company irrevocably and unconditionally waives all right to trial by jury in any Proceeding (whether based on contract, tort or otherwise) arising out of or relating to this Agreement or YOUR employment by the Company or any affiliate of the Company, INCLUDING WITHOUT LIMITATION YOUrs or the Company's performance under, or the enforcement of, this Agreement.

11.Integration. This Agreement, the Equity Documents and the Continuing Obligations constitute the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior agreements between the parties concerning such subject matter. In signing this Agreement, you agree that you are not relying on any prior or contemporaneous promise, representation, communication or agreement by or with the Company or any Company agent, director or representative, in each case except as is expressly set forth herein.

12.Withholding; Tax Effect. All payments made by the Company to you under this Agreement shall be subject to, and net of any tax or other amounts required or permitted to be withheld by the Company under applicable law. Nothing in this Agreement shall be construed to require the Company to make any payments to compensate you for any adverse tax effect associated with any payments or benefits or for any deduction or withholding from any payment or benefit.

13.Assignment. The Company may assign its rights and obligations under this Agreement (including the Continuing Obligations) without your consent to any affiliate or to any person or entity with whom the Company shall hereafter effect a reorganization, consolidate with, or merge into or to whom it transfers all or substantially all of its properties or assets or to any other person or entity so long as such successor expressly assumes this Agreement. This Agreement shall inure to the benefit of and be binding upon you and the Company, and each of yours and the Company's respective successors, executors, administrators, heirs and permitted assigns.

14.Enforceability. If any portion or provision of this Agreement (including, without limitation, any portion or provision of any section of this Agreement) shall to any extent be declared illegal or unenforceable by a court of competent jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law.

15.Survival. The provisions of this Agreement shall survive the termination of this Agreement and/or the termination of your employment to the extent necessary to effectuate the terms contained herein.

16.Waiver. No waiver of any provision hereof shall be effective unless made in writing and signed by the waiving party. The failure of any party to require the performance of any term or obligation of this Agreement, or the waiver by any party of any breach of this Agreement, shall not prevent any subsequent enforcement of such term or obligation or be deemed a waiver of any subsequent breach.

17.Notices. Any notices, requests, demands and other communications provided for by this Agreement shall be sufficient if in writing and delivered in person or sent by a nationally recognized overnight courier service or by registered or certified mail, postage prepaid, return receipt requested, to you at the last address you has filed in writing with the Company or, in the case of the Company, at its main offices, attention of the Board.

18.Amendment. This Agreement may be amended or modified only by a written instrument signed by you and by a duly authorized representative of the Company.

19.No Other Severance Rights. Section 5 and Section 6 of this Agreement are mutually exclusive and in no event shall you be entitled to payments or benefits pursuant to both Section 5 and Section 6 of this Agreement. You are not entitled to or eligible for severance or

change in control benefits under any other agreement with, or policy or practice of, the Company.

20.Governing Law. This is a Massachusetts contract and shall be construed under and be governed in all respects by the laws of the Massachusetts, without giving effect to the conflict of laws principles thereof.

21.Counterparts. This Agreement may be executed in any number of counterparts, each of which when so executed and delivered shall be taken to be an original; but such counterparts shall together constitute one and the same document.

IN WITNESS WHEREOF, the parties have executed this Agreement effective on the Effective Date.

PEPGEN INC.

Date: August 19, 2024

By: /s/ James McArthur
James McArthur
Chief Executive Officer
(Principal Executive Officer)

EXECUTIVE

Date: August 19, 2024

By: /s/ Paul Streck
Paul Streck

Exhibit A

Restrictive Covenants Agreement

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 7, 2024

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 7, 2024

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, 2024, 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 his 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 7, 2024

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, 2024, 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 his 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 7, 2024

By: /s/ Noel Donnelly

Noel Donnelly

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