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, 2025

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: 001-39509

Dyne Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**36-4883909**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
1560 Trapelo Road Waltham, Massachusetts	**02451**
(Address of principal executive offices)	**(Zip Code)**

(781) 786-8230

(Registrant's telephone number, including area code)

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

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.0001 par value per share	DYN	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 October 31, 2025, the registrant had 142,824,687 shares of common stock, $0.0001 par value per share, outstanding.

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We own or have rights to trademarks, service marks and trade names that we use in connection with the operation of our business, including our corporate name, logos and website names. The service marks and trademarks that we own include the marks Dyne Therapeutics® and FORCE™. Other trademarks, service marks and trade names appearing in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, some of the trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q are listed without the ® and ™ symbols, but we will assert, to the fullest extent under applicable law, our rights to our trademarks, service marks and trade names.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or this Quarterly Report, contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act and Section 21E of the Securities Exchange Act of 1934, as amended, that involve substantial risk and uncertainties. All statements other than statements of historical fact, contained in this Quarterly Report, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue" "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," "would," or the negative of these words or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

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

- the initiation, timing, design, progress and results of our research and development programs, preclinical studies and clinical trials;
- the anticipated timing of the submission and clearance of investigational new drug applications, or INDs, and comparable foreign applications for any product candidates we may develop;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for any product candidates we may develop;
- our estimates regarding expenses, future revenue, capital requirements, need for additional financing and the period over which we believe our cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses, debt service obligations and capital expenditure requirements;
- our ability to comply with restrictive covenants under our loan agreement with Hercules Capital, Inc., or Hercules;
- our ability to satisfy interest and principal payments under our loan agreement with Hercules;
- our plans to develop and, if approved, subsequently commercialize any product candidates we may develop;
- the potential advantages of our FORCE platform;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and our expectations regarding our ability to obtain and maintain intellectual property protection;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- the impact of government laws and regulations;
- our competitive position and expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available; and
- our ability to establish and maintain collaborations or obtain additional funding.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in this Quarterly Report, particularly in Item 1A. "Risk Factors" in this Quarterly Report, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a competitive and rapidly changing 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, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments we may make or enter into.

You should read this Quarterly Report and the documents that we have filed or incorporated by reference as exhibits to this Quarterly Report with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report are made as of the date of this Quarterly Report, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that, if realized, could materially affect our business, prospects, operating results and financial condition. These risks are discussed more fully in the "Risk Factors" section of this Quarterly Report. These risks include, but are not limited to, the following:

- we will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts;
- our product candidates are in varying stages of preclinical and clinical development, and we have not completed clinical development of any product candidate. We do not expect to complete the commercialization of any product candidate at least until 2027, if ever. If we are unable to advance 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 may encounter substantial delays in commencement, enrollment or completion of our clinical trials and the data from the clinical trials of our product candidates may fail to demonstrate sufficient safety and efficacy to warrant further development or satisfy the applicable regulatory authorities, which could prevent us from commercializing any product candidates we determine to develop on a timely basis, if at all;
- our approach to the discovery and development of product candidates based on our FORCE platform is unproven, and we may not be successful in our efforts to develop our product candidates;
- the outcome of preclinical studies and initial data from earlier-stage clinical trials may not be predictive of final results of clinical trials or future clinical trials and data from trials in one indication may not be predictive of results of clinical trials in other indications;
- if our product candidates cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent us from conducting clinical trials or seeking or obtaining regulatory approval, limit the commercial potential of our product candidates or result in significant negative consequences to the extent such effects or adverse properties are observed following any marketing approval;
- our loan agreement with Hercules contains restrictive and financial covenants that may limit our operating flexibility and our failure to comply with the covenants or other terms of the loan agreement, including as a result of events beyond our control, could result in a default under the loan agreement that could materially and adversely affect our business;
- 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;
- we face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do;
- our rights to develop and commercialize certain of our product candidates are subject or 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 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; and
- 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.

PART I—FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (Unaudited)

Dyne Therapeutics, Inc.
Condensed Consolidated Balance Sheets (Unaudited)
(in thousands, except share and per share data)

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 573,610	$ 435,449
Marketable securities	218,277	206,819
Prepaid expenses and other current assets	14,376	17,011
Total current assets	806,263	659,279
Property and equipment, net	23,972	5,398
Right-of-use assets	21,818	24,615
Restricted cash and other assets	15,006	1,942
Total assets	$ 867,059	$ 691,234
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	12,061	6,562
Accrued expenses and other current liabilities	42,749	30,846
Lease liabilities	5,039	4,850
Total current liabilities	59,849	42,258
Long-term debt, net	99,147	—
Lease liabilities, net of current portion	16,280	19,138
Total liabilities	175,276	61,396
Stockholders' equity		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized at September 30, 2025 and December 31, 2024	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized at September 30, 2025 and December 31, 2024; 142,613,248 and 102,318,629 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	14	10
Additional paid-in capital	1,975,460	1,579,750
Accumulated other comprehensive loss	496	6
Accumulated deficit	(1,284,187)	(949,928)
Total stockholders' equity	691,783	629,838
Total liabilities and stockholders' equity	$ 867,059	$ 691,234

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

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss (Unaudited)
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 97,219	$ 92,800	$ 302,902	$ 199,601
General and administrative	16,673	12,859	49,153	47,177
Total operating expenses	113,892	105,659	352,055	246,778
Loss from operations	(113,892)	(105,569)	(352,055)	(246,778)
Other income (expense):				
Interest income	8,476	9,276	22,202	19,330
Interest expense	(2,904)	—	(2,998)	—
Other income (expense), net	279	(742)	(1,408)	(428)
Total other income (expense), net	5,851	8,534	17,796	18,902
Net loss	$ (108,041)	$ (97,125)	$ (334,259)	$ (227,876)
Net loss per share—basic and diluted	$ (0.76)	$ (0.96)	$ (2.74)	$ (2.49)
Weighted average common shares outstanding, basic and diluted	141,810,881	100,882,042	121,980,400	91,511,621
Comprehensive loss:				
Net loss	$ (108,041)	$ (97,125)	$ (334,259)	$ (227,876)
Other comprehensive loss:				
Unrealized gains on marketable securities, net	244	718	490	478
Comprehensive loss	$ (107,797)	$ (96,407)	$ (333,769)	$ (227,398)

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

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity (Unaudited)
(in thousands, except share data and issuance costs)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
Balance at January 1, 2025	102,318,629	$ 10	$ 1,579,750	$ 6	$ (949,928)	$ 629,838
Issuance of common stock in at-the-market offering, net of issuance costs of $4.4 million	10,660,159	1	140,652	—	—	140,653
Exercise of stock options	89,835	—	588	—	—	588
Stock-based compensation	—	—	13,020	—	—	13,020
Vesting of restricted stock units	565,159	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	235	—	235
Net loss	—	—	—	—	(115,361)	(115,361)
Balance at March 31, 2025	113,633,782	$ 11	$ 1,734,010	$ 241	$ (1,065,289)	$ 668,973
Exercise of stock options	488,457	—	2,795	—	—	2,795
Stock-based compensation	—	—	10,523	—	—	10,523
Vesting of restricted stock units	216,371	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	11	—	11
Net loss	—	—	—	—	(110,857)	(110,857)
Balance at June 30, 2025	114,338,610	$ 11	$ 1,747,328	$ 252	$ (1,176,146)	$ 571,445
Issuance of common stock in public offering, net of issuance costs of $14.1 million	27,878,788	3	215,800	—	—	215,803
Exercise of stock options	151,492	—	1,250	—	—	1,250
Stock-based compensation	—	—	11,082	—	—	11,082
Vesting of restricted stock units	244,358	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	244	—	244
Net loss	—	—	—	—	(108,041)	(108,041)
Balance at September 30, 2025	142,613,248	$ 14	$ 1,975,460	$ 496	$ (1,284,187)	$ 691,783

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
Balance at January 1, 2024	61,468,743	$ 6	$ 723,796	$ —	$ (632,510)	$ 91,292
Issuance of common stock in public offering, net of issuance costs of $21.3 million	19,722,500	2	323,849	—	—	323,851
Issuance of common stock in at-the-market offering, net of issuance costs of $3.3 million	3,800,465	1	97,870	—	—	97,871
Exercise of stock options	1,858,791	—	10,916	—	—	10,916
Stock-based compensation	—	—	19,906	—	—	19,906
Vesting of restricted stock units	239,150	—	—	—	—	—
Unrealized losses on marketable securities	—	—	—	(132)	—	(132)
Net loss	—	—	—	—	(65,649)	(65,649)
Balance at March 31, 2024	87,089,649	$ 9	$ 1,176,337	$ (132)	$ (698,159)	$ 478,055
Issuance of common stock in public offering, net of issuance costs of $23.0 million	12,075,000	1	351,328	—	—	351,329
Exercise of stock options	564,904	—	4,283	—	—	4,283
Stock-based compensation	—	—	6,736	—	—	6,736
Vesting of restricted stock units	389,965	—	—	—	—	—
Unrealized losses on marketable securities	—	—	—	(108)	—	(108)
Net loss	—	—	—	—	(65,102)	(65,102)
Balance at June 30, 2024	100,119,518	$ 10	$ 1,538,684	$ (240)	$ (763,261)	$ 775,193
Exercise of stock options	1,388,613	—	18,009	—	—	18,009
Stock-based compensation	—	—	8,745	—	—	8,745
Vesting of restricted stock units	245,247	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	718	—	718
Net loss	—	—	—	—	(97,125)	(97,125)
Balance at September 30, 2024	101,753,378	$ 10	$ 1,565,438	$ 478	$ (860,386)	$ 705,540

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

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows (Unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2025	2024
Cash flows from operating activities:		
Net loss	$ (334,259)	$ (227,876)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	34,625	35,387
Depreciation and amortization expense	1,528	1,223
Non-cash lease expense	128	434
Non-cash interest expense	62	—
Accretion of premium on marketable securities	(1,447)	(2,768)
Loss (gain) on sale of marketable securities	(14)	(26)
Loss (gain) on disposal of property and equipment	95	(2)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	3,184	(6,442)
Other non-current assets	(12,991)	—
Accounts payable and other liabilities	17,714	(7,964)
Net cash used in operating activities	(291,375)	(208,034)
Cash flows from investing activities:		
Purchases of property and equipment	(1,436)	(1,301)
Advance payment for long-lead equipment	(18,790)	—
Purchases of marketable securities	(153,823)	(264,729)
Maturities of marketable securities	135,847	76,215
Sales of marketable securities	8,469	1,749
Net cash used in investing activities	(29,733)	(188,066)
Cash flows from financing activities:		
Proceeds from issuance of common stock in public offering, net of issuance costs	215,803	675,180
Proceeds from issuance of common stock in at-the-market offering, net of issuance costs	140,653	97,871
Proceeds from exercise of stock options	4,633	33,208
Proceeds from issuance of long-term debt, net of issuance costs paid	98,802	—
Payment of debt issuance costs	(549)	—
Net cash provided by financing activities	459,342	806,259
Net increase (decrease) in cash, cash equivalents and restricted cash	138,234	410,159
Cash, cash equivalents and restricted cash, beginning of period	437,391	123,941
Cash, cash equivalents and restricted cash, end of period	$ 575,625	$ 534,100
Supplemental disclosures of cash flow information:		
Purchase of property and equipment in accounts payable	$ —	$ 48
Cash paid for interest	1,824	—

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

Dyne Therapeutics, Inc.
Notes to Condensed Consolidated Financial Statements (Unaudited)

1. Nature of Business and Basis of Presentation

Dyne Therapeutics, Inc. (the "Company") is a clinical-stage neuromuscular disease company focused on delivering functional improvement for people living with genetically driven neuromuscular diseases. The Company was incorporated in Delaware on December 1, 2017 and has a principal place of business in Waltham, Massachusetts.

The Company is subject to risks and uncertainties common to clinical-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for its product candidates, fluctuations in operating results, compliance with government regulations, the ability to establish clinical- and commercial-scale manufacturing processes and the ability to secure additional capital to fund operations. Product candidates and programs currently under development require significant research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful and product candidates receive regulatory approval, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The accompanying condensed consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. Since inception, the Company has funded its operations with proceeds from the sales of equity securities, and its initial borrowing under its Loan and Security Agreement (the "Loan Agreement") with Hercules Capital, Inc. ("Hercules"). The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents, and marketable securities will be sufficient to fund its operating expenses, debt service obligations and capital expenditure requirements for at least 12 months from the issuance of these condensed consolidated financial statements.

To continue its development efforts, the Company will need to obtain substantial additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements in order to fund its research and development and ongoing operating expenses. The Company may not be able to obtain financing on acceptable terms, when needed or at all, and the Company may not be able to enter into collaborations, strategic alliances or licensing arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. Any collaborations, strategic alliances or licensing arrangements may require the Company to relinquish rights to certain of its technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to the Company. If the Company is unable to obtain funding, the Company could be forced to delay, limit, reduce or eliminate some or all of its research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates, which could adversely affect its business prospects. Although management will continue to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations when needed or at all.

2. Summary of Significant Accounting Policies

The accompanying condensed consolidated financial statements reflect the operations of the Company and the Company's wholly-owned subsidiary, Dyne Therapeutics Securities Corporation. Intercompany balances and transactions have been eliminated in consolidation. The accompanying condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles ("GAAP") in the United States of America. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

The financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). The unaudited interim financial statements have been prepared on the same basis as audited annual financial statements, except certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. In the opinion of management, the interim financial information reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair representation

of the results for the reported periods. Accordingly, these financial statements should be read in conjunction with the financial statements and notes thereto included in the Company's annual report on Form 10-K filed with the SEC on February 27, 2025. The results for the three and nine months ended September 30, 2025 are not necessarily indicative of results to be expected for the year ending December 31, 2025, any other interim periods, or any future year or period.

Fair value measurements

Certain assets and liabilities are carried at fair value. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Unadjusted quoted prices in active markets that are accessible to the reporting entity at the measurement date for identical assets and liabilities.
- Level 2—Inputs other than quoted prices in active markets for identical assets and liabilities that are observable either directly or indirectly for substantially the full term of the asset or liability. Level 2 inputs include the following:
  - quoted prices for similar assets and liabilities in active markets;
  - quoted prices for identical or similar assets or liabilities in markets that are not active;
  - observable inputs other than quoted prices that are used in the valuation of the asset or liabilities (e.g., interest rate and yield curve quotes at commonly quoted intervals); and
  - inputs that are derived principally from or corroborated by observable market data by correlation or other means.
- Level 3—Unobservable inputs for the assets or liability (i.e., supported by little or no market activity). Level 3 inputs include management's own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

Net loss per share

Basic net loss per share is computed by dividing the net loss by the weighted average number of shares of common stock outstanding for the period. Diluted net loss is computed by adjusting net loss to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share is computed by dividing the diluted net loss by the weighted average number of shares of common stock outstanding for the period, including potential dilutive common shares assuming the dilutive effect of common stock equivalents.

The following potentially dilutive common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the computation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	September 30,	
	2025	**2024**
Options to purchase common stock	9,649,402	7,743,732
Unvested restricted stock units	2,591,337	2,718,092
Total	12,240,739	10,461,824

Recently Issued Accounting Pronouncements Not Yet Adopted

In November 2024, the FASB issued ASU 2024-03, *Income Statement-Reporting Comprehensive Income-Expense Disaggregation Disclosures (Topic 220-40)* ("ASU 2024-03"). The amendments in this update require new disclosures to disaggregate prescribed natural expenses underlying any income statement caption. ASU 2024-03 is effective for annual periods in fiscal years beginning after December 15, 2026, and interim periods thereafter. Early adoption is permitted. ASU 2024-03 applies on a prospective basis for periods beginning after the effective date. However, retrospective application to any or all prior periods presented is permitted. The Company is currently assessing the impact ASU 2024-03 will have on the condensed consolidated financial statements and disclosures.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740)* ("ASU 2023-09"). The amendments in this update require enhanced annual income tax disclosures for the effective tax rate reconciliation and income taxes paid. The amendments are effective for public business entities for annual periods beginning after December 15, 2024 and for annual periods beginning after December 15, 2025 for all other entities. Early adoption is permitted for annual financial statements that have not yet been issued or made available for issuance. ASU 2023-09 applies on a prospective basis to annual financial statements for periods beginning after the effective date. However, retrospective application in all prior periods presented is permitted. The Company is assessing what impact ASU 2023-09 will have on the condensed consolidated financial statements and disclosures.

In July 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted. The OBBBA makes permanent key elements of the Tax Cuts and Jobs Act of 2017, including 100% bonus depreciation, domestic research cost expensing and the business interest expense limitation, among other tax changes. The new legislation has multiple effective dates, with certain provisions effective in 2025 and others in the future. In accordance with ASC 740, *Accounting for Income Taxes*, the impacts of the OBBBA did not affect the Company's U.S. deferred tax assets or liabilities, as the Company continues to maintain a full valuation allowance against those balances.

3. Cash, Cash Equivalents and Restricted Cash

Cash includes cash in readily available checking accounts and cash equivalents include money market funds that invest in U.S. treasury securities and all highly liquid investments maturing within 90 days from the date of purchase.

Amounts included in restricted cash represent amounts pledged as collateral for letters of credit required for security deposits on the Company's leased facilities.

Cash, cash equivalents and restricted cash consisted of the following:

(in thousands)	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 573,610	$ 435,449
Restricted cash	2,015	1,942
Total	$ 575,625	$ 437,391

4. Fair Value Measurements

The following tables set forth marketable securities for the periods presented:

	As of September 30, 2025			
(in thousands)	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
Commercial paper	9,244	9	—	9,253
Corporate debt securities	169,435	367	(4)	169,798
Certificates of deposit	15,720	10	—	15,730
U.S. treasury notes	23,382	114	—	23,496
Total	$ 217,781	$ 500	$ (4)	$ 218,277

	As of December 31, 2024			
(in thousands)	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
Commercial paper	14,203	8	—	14,211
Corporate debt securities	162,078	131	(114)	162,095
Certificates of deposit	9,369	4	(1)	9,372
U.S. treasury notes	21,163	7	(29)	21,141
Total	$ 206,813	$ 150	$ (144)	$ 206,819

The following tables set forth by level, within the fair value hierarchy (see Note 2), the assets carried at fair value on a recurring basis for the periods presented:

	Fair Value Measurements as of September 30, 2025			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents				
Money market funds	$ 179,870	$ —	$ —	$ 179,870
Marketable securities				
Commercial paper	—	9,253	—	9,253
Corporate debt securities	—	169,798	—	169,798
Certificates of deposit	—	15,730	—	15,730
U.S. treasury notes	23,496	—	—	23,496
Total	$ 203,366	$ 194,781	$ —	$ 398,147

	Fair Value Measurements as of December 31, 2024			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents				
Money market funds	$ 3,535	$ —	$ —	$ 3,535
Marketable securities				
Commercial Paper	—	14,211	—	14,211
Corporate debt securities	—	162,095	—	162,095
Certificates of deposit	—	9,372	—	9,372
U.S. treasury notes	21,141	—	—	21,141
Total	$ 24,676	$ 185,678	$ —	$ 210,354

The fair value of U.S. government treasury notes and money market funds were determined by the Company based on quoted market prices, which represent a Level 1 measurement within the fair value hierarchy. Commercial paper, certificates of deposit, and corporate debt securities were valued by the Company using quoted prices in active markets for similar securities, which represent a Level 2 measurement within the fair value hierarchy.

There were no transfers between Level 1, Level 2, or Level 3 during the periods presented.

The following table summarizes the scheduled maturity for the Company's marketable securities for the periods presented:

(in thousands)	**September 30, 2025**
Maturing in one year or less	$ 147,397
Maturing after one year through two years	70,880
Maturing after two years	—
Total	$ 218,277

Financial instruments not recorded at fair value

The carrying values of cash, cash equivalents, accounts payable and accrued expenses that are reported on the balance sheets approximate their fair value due to the short-term nature of these assets and liabilities.

5. Property and Equipment

Property and equipment consisted of the following:

(in thousands)	September 30, 2025	December 31, 2024
Laboratory equipment	$ 10,897	$ 9,197
Office and computer equipment	2,486	2,355
Construction in process	18,790	619
Property and equipment—at cost	32,173	12,171
Less accumulated depreciation and amortization	(8,201)	(6,773)
Property and equipment—net	$ 23,972	$ 5,398

As of September 30, 2025, the Company was deemed to be the owner, for accounting purposes, of $18.8 million of long-lead equipment to be utilized for future manufacturing at a contract manufacturing organization, or CMO, facility.

Depreciation expense for the three and nine months ended September 30, 2025 was $0.5 million and $1.5 million, respectively. Depreciation expense for the three and nine months ended September 30, 2024 was $0.4 million and $1.2 million, respectively.

6. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	September 30, 2025	December 31, 2024
Payroll and benefits	$ 13,178	$ 13,095
Consulting services	1,115	1,608
Legal services	709	742
Research and development	26,918	15,401
Interest	829	—
Total	$ 42,749	$ 30,846

7. Long-Term Debt

On June 27, 2025 (the “Closing Date”), the Company entered into the Loan Agreement with Hercules, in its capacity as administrative agent and collateral agent (the “Agent”) and as a lender, and certain other financial institutions that from time to time become parties to the Loan Agreement as lenders (collectively, the “Lenders”). The Loan Agreement provides for term loans in an aggregate principal amount of up to $275.0 million under multiple tranches (the “Term Loans”), available as follows: (i) an initial term loan tranche funded on the Closing Date in aggregate principal amount of $100.0 million (the “Initial Tranche”); (ii) subject to the achievement of specified clinical, regulatory and commercial milestones, three additional term loan tranches totaling up to $115.0 million; and (iii) subject to approval by the Lenders’ investment committee in their discretion, a final term loan tranche of up to $60.0 million.

All unpaid principal and accrued and unpaid interest with respect to the Term Loans is due and payable in full on July 1, 2030 (the “Maturity Date”). The outstanding principal balance of the Term Loans bears interest at a floating interest rate per annum equal to the Wall Street Journal prime rate, subject to a floor of 7.50%, plus 2.45%. Accrued interest on the outstanding Term Loans is payable monthly. The Company may make payments of interest only until July 1, 2028, which interest only period may be extended until the Maturity Date upon the achievement of specified clinical, regulatory and commercial milestones. At the end of the interest only period, the Company is required to begin repayment of the outstanding principal of the Term Loans in equal monthly installments (or, in a single installment, if the interest-only period has been extended to the Maturity Date).

As collateral for the obligations under the Loan Agreement, the Company has granted to the Agent, for the benefit of the Lenders, a first-priority security interest in substantially all of its property, inclusive of intellectual property, subject to customary permitted liens and other exceptions set forth in the Loan Agreement.

The Loan Agreement contains customary representations and warranties, events of default and affirmative and negative

covenants, including a minimum cash covenant (the "Minimum Cash Covenant") requiring the Company to maintain specified levels of cash in accounts subject to a control agreement in favor of the Agent ("Qualified Cash") during the period commencing on April 1, 2026 (or January 1, 2027 if the Company has raised at least $350.0 million of cumulative net cash proceeds between June 4, 2025 and December 15, 2026 from the issuance of equity or indebtedness or payments under certain royalty or license transactions). The Minimum Cash Covenant will initially be set at 60% of the then outstanding principal balance of the Term Loans, is subject to adjustment and will not be tested at any time when the Company's market capitalization is greater than $1.65 billion. The Company is also required to maintain minimum net product revenue from the sale of z-basivarsen (also known as DYNE-101) and z-rostudirsen (also known as DYNE-251) starting nine months after U.S. Food and Drug Administration approval of z-basivarsen or z-rostudirsen (the "Minimum Revenue Covenant") if the outstanding principal balance of the Term Loans exceed $100.0 million. The Minimum Revenue Covenant will not be tested for any month to the extent that for each day during such month either (i) Qualified Cash is at least 100% of the Company's outstanding obligations under the Loan Agreement or (ii) the Company's market capitalization is greater than $1.65 billion and Qualified Cash is at least 50% of the Company's outstanding obligations under the Loan Agreement. Certain negative covenants under the Loan Agreement limit the ability of the Company, among other things, to incur future debt, grant liens, make investments, make acquisitions, distribute dividends and sell assets, subject in each case to certain exceptions.

Upon the occurrence of an event of default, including the failure by the Company to comply with the covenants under the Loan Agreement or the occurrence of a material adverse effect on the business, operations, properties, assets or financial condition of the Company, in each case subject to certain exceptions, and subject to any specified cure periods, all amounts owed by the Company under the Loan Agreement may be declared immediately due and payable by the Agent, and the Agent may foreclose on collateral.

The Loan Agreement requires the Company to pay closing fees, prepayment penalties and an end-of-term charge equal to 5.5% of the amount of Term Loans borrowed, which amount is due at the earlier of prepayment or the Maturity Date. A prepayment penalty applies to any prepayment of the Term Loans prior to the Maturity Date equal to (i) 2.0% of the principal amount prepaid if the prepayment occurs on or prior to the first anniversary of the Closing Date, (ii) 1.5% of the principal amount prepaid if the prepayment occurs after the first anniversary and on or prior to the second anniversary of the Closing Date, and (iii) 0.75% of the principal amount prepaid if the prepayment occurs after the second anniversary through the day before the Maturity Date.

The Company recorded debt discount and debt issuance costs of $1.2 million upon closing of the Loan Agreement. The Company is accruing the end-of-term charge on its condensed consolidated balance sheet.

Unamortized debt discount and issuance costs were recorded as a reduction of the carrying amount on the Term Loans and are amortized as interest expense using the effective-interest method. In addition, debt issuance costs of $0.5 million were recorded in restricted cash and other assets as of September 30, 2025.

As of September 30, 2025, the carrying value of the Term Loans approximates its fair value.

The obligations under the Term Loans as of September 30, 2025 consisted of the following (in thousands):

		September 30, 2025
Principal term loan balance	$	100,000
Unamortized debt discount and issuance costs		(1,136)
Accrued end of term fee		283
Long-term debt, net	$	99,147

The annual principal payments due under the Term Loans as of September 30, 2025 were as follows:

Year ending December 31,		(in thousands)
2025	$	—
2026		—
2027		—
2028		22,075
2029		47,650
2030		30,275
Total	$	100,000

The table of future principal payments excludes the end-of-term charge of 5.5% of the principal amount of the Term Loans borrowed, which is due upon the maturity of the Term Loans.

8. Stockholders' Equity

Common Stock

In November 2021, the Company filed a universal shelf registration statement on Form S-3 (the "2021 Shelf Registration Statement"), to register for sale from time to time up to $400.0 million of common stock, preferred stock, debt securities, warrants and/or units in one or more offerings. Further, in November 2021, the Company entered into an Open Market Sale Agreement (the "Sales Agreement") with Jefferies LLC ("Jefferies") as the Company's sales agent, and included in the 2021 Shelf Registration Statement a prospectus relating to the Sales Agreement pursuant to which, from time to time, the Company could offer and sell shares of its common stock having an aggregate offering price of up to $150.0 million in an "at-the-market" offering program. On January 4, 2024, the Company notified Jefferies that it was suspending and terminating the prospectus filed under the 2021 Shelf Registration Statement relating to the Sales Agreement for the Company's "at-the-market" offering program.

On March 5, 2024, the Company filed a universal shelf registration statement on Form S-3, (the “2024 Shelf Registration Statement”), and included a prospectus relating to the Sales Agreement. Under the 2024 Shelf Registration Statement, the Company may offer and sell debt securities, common stock, preferred stock, units and/or warrants from time to time at an indeterminate aggregate offering price in one or more offerings. In November 2024, the Company filed a prospectus supplement relating to the Sales Agreement, pursuant to which, in accordance with the Sales Agreement, the Company may offer and sell shares of its common stock having an aggregate offering price of up to $300.0 million in an “at-the-market” offering.

During the nine months ended September 30, 2025, the Company issued and sold an aggregate of 10,660,159 shares of common stock pursuant to the Sales Agreement for aggregate net proceeds of $140.6 million, after deducting commissions and offering expenses payable by the Company. The Company sold such shares at a weighted average price of $13.60 per share.

Atlas Venture and related affiliated entities were beneficial owners of approximately 6.4% of the Company's outstanding common stock as of September 30, 2025. The chairman of the Company's board of directors is a partner at Atlas Venture. On January 27, 2025, the Company issued and sold 1,111,111 shares of common stock through its at-the-market offering program that Atlas Venture and related affiliated entities purchased at a purchase price of $13.50 per share for aggregate gross proceeds of $15.0 million. The shares were purchased at the prevailing market price.

In July 2025, the Company completed a follow-on public offering, pursuant to which the Company issued and sold 27,878,788 shares of the Company's common stock. The Company received net proceeds of $215.8 million, after deducting underwriting discounts and commissions and offering expenses paid by the Company.

2020 Stock Incentive Plan

In August 2020, the Company’s board of directors adopted and the Company’s stockholders approved the 2020 Stock Incentive Plan, (the "2020 Plan"), which became effective on September 16, 2020. The 2020 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units ("RSUs") and other stock-based awards to employees, directors, consultants and advisors of the Company. The 2020 Plan is administered by the Company’s board of directors or by a committee appointed by the board of directors. Upon the effectiveness of the 2020 Plan, the Company ceased granting awards under the 2018 Stock Incentive Plan. The number of shares initially reserved for issuance under the 2020 Plan was 4,884,233. The number of shares of common stock reserved for issuance under the 2020 Plan may automatically increase on the first day of each fiscal year, beginning

with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including the fiscal year commencing on, January 1, 2030, 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. On January 1, 2025, 5,115,931 shares were added to the shares reserved for issuance under the 2020 Plan in the fifth of these annual increases.

As of September 30, 2025, 6,009,631 shares remained available for future issuance under the 2020 Plan.

2020 Employee Stock Purchase Plan

In August 2020, the Company's board of directors adopted and the Company's stockholders approved the 2020 Employee Stock Purchase Plan (the "2020 ESPP"), which became effective September 16, 2020. The 2020 ESPP is administered by the Company's board of directors or by a committee appointed by the board of directors. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 488,414 shares of common stock. The number of shares of common stock reserved for issuance under the 2020 ESPP may automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including the fiscal year commencing on, January 1, 2030, in an amount equal to the least of (1) 1,953,656 shares of common stock, (2) 1% of the shares of common stock outstanding on such date, and (3) an amount determined by the board of directors.

As of September 30, 2025, no offering periods have commenced under the 2020 ESPP and 488,414 shares remained available for issuance.

2024 Inducement Stock Incentive Plan

In March 2024, the Company's board of directors adopted the 2024 Inducement Stock Incentive Plan (the "2024 Inducement Plan"). The 2024 Inducement Plan provides for the grant of nonstatutory stock options, stock appreciation rights, restricted stock, RSUs and other stock-based awards with respect to an aggregate of 900,000 shares of Common Stock (subject to adjustment as provided in the 2024 Inducement Plan). Awards under the 2024 Inducement Plan may only be granted to new employees who were not previously an employee or director of the Company or are commencing employment with the Company following a bona fide period of non-employment, in either case, as an inducement material to the individual's entering into employment with the Company and in accordance with the requirements of Nasdaq Stock Market Rule 5635(c)(4). In February 2025, the Company's board of directors approved an additional 1,000,000 shares for issuance under the 2024 Inducement Plan. In April 2025, the Company granted inducement equity awards to three new employees as a material inducement to acceptance of their respective employment consisting of stock options to purchase up to an aggregate of 330,200 shares of the Company's common stock and RSUs with respect to an aggregate of 101,900 shares of the Company's common stock. During the three months ended September 30, 2025, the Company did not grant any inducement equity awards under the 2024 Inducement Plan.

As of September 30, 2025, 440,447 shares remained available for future issuance under the 2024 Inducement Plan.

Stock option valuation

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The fair value is determined based upon the quoted price of the Company's common stock. The Company lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected

dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The assumptions that the Company used to determine the grant-date fair value of options granted were as follows:

	Nine Months Ended September 30,	
	2025	2024
Expected volatility	67%	66%
Risk-free interest rate	3.71% — 4.52%	3.46% — 4.65%
Expected term (in years)	6	6
Expected dividend yield	—	—

Stock option activity

A summary of the Company's stock option activity and related information for the nine months ended September 30, 2025 is as follows:

(in thousands, except share and per share data)	Options	Weighted Average Exercise Price	Weighted Average Remaining Life (in years)	Aggregate Intrinsic Value
Outstanding as of January 1, 2025	8,681,473	$ 19.47	7.3	$ 61,021
Granted	3,149,353	10.44		
Exercised	(729,784)	6.35		
Canceled	(1,451,640)	18.71		
Outstanding as of September 30, 2025	9,649,402	$ 17.86	8.2	$ 14,497
Options exercisable as of September 30, 2025	3,528,987	$ 17.11	6.7	$ 6,051
Options vested or expected to vest as of September 30, 2025	9,649,402	$ 17.86	8.2	$ 14,497

The intrinsic value of options exercised for the nine months ended September 30, 2025 and 2024 totaled $4.3 million and $83.3 million, respectively. The weighted-average grant date fair value of the options granted during the nine months ended September 30, 2025 and 2024 was $6.78 per share and $19.65 per share, respectively. As of September 30, 2025 there was $66.0 million of unrecognized compensation expense, which the Company expects to recognize over a weighted-average period of 2.9 years.

Effective January 2025, the Company entered into a consulting agreement with its former chief medical officer resulting in the modification of previously issued stock option awards. The modification resulted in $0.9 million of additional stock-based compensation expense, all of which was recognized during the three months ended March 31, 2025. The expense was recorded within research and development expenses on the condensed consolidated statements of operations and comprehensive loss. Based on the terms of the consulting agreement, options to purchase 58,659 shares of common stock were forfeited in January 2025.

Effective April 2025, the Company entered into a consulting agreement with its former senior vice president of finance and administration resulting in the modification of previously issued stock option awards. The modification resulted in $0.1 million of additional stock-based compensation expense, all of which was recognized during the three months ended June 30, 2025. The expense was recorded within general and administrative expenses on the condensed consolidated statements of operations and comprehensive loss. Based on the terms of the consulting agreement, options to purchase 88,508 shares of common stock were forfeited in April 2025.

In July 2025, the Company granted 591,685 stock options, vesting over a three-year term from the grant date and contingent upon the achievement of certain market conditions. The fair value of these market-based stock options was estimated using a Monte Carlo valuation method, incorporating various option pricing inputs. Ultimately, over the three-year vesting term, these market-based stock options will result in an additional stock-based compensation expense of $2.9 million, with $0.3 million recognized during the quarterly period ended September 30, 2025.

Restricted stock units

An RSU represents the right to receive one share of common stock upon vesting of the RSU. The Company grants RSUs with service conditions that vest in four equal annual installments provided that the employee remains employed with the Company, RSUs with service conditions that vest in sixteen equal quarterly installments provided that the employee

remains employed with the Company and RSUs with performance-based vesting conditions. As of September 30, 2025, there are no RSUs with performance-based vesting conditions outstanding. The fair value of each RSU is based on the closing price of the Company's common stock on the date of grant. A summary of the Company's RSU activity and related information for the nine months ended September 30, 2025 is as follows:

	Number of Shares Underlying RSUs	Weighted Average Grant Date Fair Value
Issued and unvested as of January 1, 2025	3,722,024	$ 20.49
Granted	603,600	10.86
Vested	(1,025,888)	17.50
Forfeited	(708,399)	17.12
Issued and unvested as of September 30, 2025	2,591,337	$ 20.35

The fair value of RSUs vested during the nine months ended September 30, 2025 and 2024 totaled $12.8 million and $27.1 million, respectively. The weighted average grant date fair value of RSUs granted during the nine months ended September 30, 2024 was $34.32 per share. As of September 30, 2025, there was $48.3 million of unrecognized compensation costs related to unvested RSUs, which are expected to be recognized over a weighted-average period of 2.8 years.

Effective January 2025, the Company entered into a consulting agreement with its former chief medical officer resulting in the modification of previously issued RSU awards. The modification resulted in the recognition of $0.3 million of additional stock-based compensation expense, all of which was recognized during the three months ended March 31, 2025. The expense was recorded within research and development expenses on the condensed consolidated statements of operations and comprehensive loss. Based on the terms of the consulting agreement, 76,662 RSUs were forfeited in January 2025.

Effective April 2025, the Company entered into a consulting agreement with its former senior vice president of finance and administration resulting in the modification of previously issued RSU awards. The modification resulted in the recognition of $0.2 million of additional stock-based compensation expense, all of which was recognized during the three months ended June 30, 2025. The expense was recorded within general and administrative expenses on the condensed consolidated statements of operations and comprehensive loss. Based on the terms of the consulting agreement, 65,350 RSUs were forfeited in April 2025.

The Company recorded stock-based compensation expense in the following expense categories of its condensed consolidated statements of operations and comprehensive loss:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2025	2024	2025	2024
Research and development	$ 6,258	$ 4,450	$ 20,095	$ 12,252
General and administrative	4,824	4,295	14,530	23,135
Total	$ 11,082	$ 8,745	$ 34,625	$ 35,387

9. Segment Reporting

The Company's segment determination is based on the financial results that are regularly evaluated by the Company's chief operating decision maker ("CODM") to determine resource allocation and assess performance. The Company's CODM is its chief executive officer. The Company has one operating and one reportable segment. Net long-lived assets are all physically located in the United States and the total assets of the segment equal the total assets presented on the condensed consolidated balance sheet. The primary financial measure by which the CODM evaluates the business is net loss. The CODM uses net loss to monitor budget versus actual results to assess performance of the segment. A summary of the significant segment expenses reported to the CODM are shown below for the three and nine months ended September 30, 2025 and 2024:

(in thousands)	Three Months Ended September 30, 2025	2024	Nine Months Ended September 30, 2025	2024
Operating Expenses				
Z-basivarsen (DM1)	$ 37,853	$ 30,922	$ 117,964	$ 53,047
Z-rostudirsen (DMD)	22,666	29,021	73,314	62,677
Platform and external research and development	5,769	10,439	21,399	20,622
Personnel related	22,517	15,620	62,730	44,986
Stock-based compensation	11,082	8,745	34,625	35,387
Facility-related and other	9,448	5,988	26,703	17,345
Professional and consulting fees	4,557	4,924	15,320	12,714
Interest income	(8,476)	(9,276)	(22,202)	(19,330)
Interest expense	2,904	—	2,998	—
Other expense (income), net	(279)	742	1,408	428
Net loss	$ 108,041	$ 97,125	$ 334,259	$ 227,876

10. Subsequent Events

On October 31, 2025, the Company entered into a master manufacturing services agreement with a CMO which secures capacity at the CMO's manufacturing facilities for certain of the Company's product candidate components. The agreement obligates the Company to compensate the CMO for producing certain of the Company's product candidate components pursuant to a mutually agreed rolling forecast. Upon execution, the Company has committed to compensate the CMO at least $25.5 million in fees through March 2027. In specified termination circumstances, the agreement requires the Company to pay the CMO for services completed, the cost of the CMO's raw materials that cannot be repurposed, capital equipment and certain manufacturing activities previously committed to.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q, or this Quarterly Report, and our audited condensed consolidated financial statements and related notes for the year ended December 31, 2024, included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on February 27, 2025. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business, 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 Quarterly Report, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage company focused on delivering functional improvement for people living with genetically driven neuromuscular diseases. Leveraging the modularity of our FORCE platform, we are developing targeted therapeutics that are designed to overcome limitations in delivery to muscle tissue and the central nervous system, or CNS. Our proprietary FORCE platform therapeutics consist of a payload that we rationally design to target the genetic basis of the disease we are seeking to treat, a linker and an antigen-binding fragment, or Fab, that we attach to the payload using the linker. With our FORCE platform, we have the flexibility to deploy different classes of payloads (such as oligonucleotides and enzymes) with specific mechanisms of action that modify target functions. We leverage this modularity to focus on neuromuscular diseases with high unmet need, with etiologic targets and with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways.

Using our FORCE platform, we are assembling a broad portfolio of product candidates, including product candidates being developed for myotonic dystrophy type 1, or DM1, Duchenne muscular dystrophy, or DMD, facioscapulohumeral dystrophy, or FSHD, and Pompe disease. In addition, we plan to expand our portfolio through development efforts focused on rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases, including some with larger patient populations, and diseases involving the CNS. We have identified product candidates for each of our DM1, DMD, FSHD and Pompe programs that are in varying stages of preclinical and clinical development.

Our product candidate zeleciment basivarsen, or z-basivarsen (also known as DYNE-101) is being evaluated in the ACHIEVE trial, a global Phase 1/2 clinical trial consisting of a 24-week multiple ascending dose, or MAD, randomized, placebo-controlled period, a 24-week open-label extension, or OLE, a 96-week long-term extension, and a registrational expansion cohort. The primary endpoints of the MAD portion of the trial were safety and tolerability; with secondary endpoints of pharmacokinetics and pharmacodynamics, including change from baseline in splicing as measured by the composite alternative splicing index, or CASI-22, video hand opening time, or vHOT, as well as multiple other measures of muscle strength and function and patient-reported outcomes, including the Myotonic Dystrophy Health Index. In the MAD portion of the ACHIEVE trial, patients were randomized to receive z-basivarsen or placebo intravenously every four weeks or every eight weeks for 24 weeks, depending on cohort. Patient cohorts were dosed from 1.8 mg/kg to 6.8 mg/kg (approximate ASO dose). Following the MAD placebo-controlled period, patients transition to z-basivarsen treatment in the OLE portion of the trial and in the long-term extension. In January 2025, we announced the completion of the MAD portion of the trial.

We participated in a Type C meeting with the U.S. Food and Drug Administration, or FDA, in May 2025 and discussed the path to regulatory approval, including U.S. Accelerated Approval, for z-basivarsen in DM1. As a result of the meeting, we submitted a revised protocol for review by the FDA in June 2025 for the registrational expansion cohort of the ACHIEVE trial. The primary endpoint in the revised protocol for the registrational expansion cohort is change from baseline in middle finger myotonia as measured by vHOT at 6 months compared to placebo, which is intended to serve as an intermediate clinical endpoint for U.S. Accelerated Approval. Secondary endpoints include change from baseline in splicing as measured by CASI-22, muscle strength as assessed by Quantitative Muscle Testing, or QMT, performance on both the 10-Meter Walk/Run Test, or 10MWR, and 5 Times Sit to Stand Test, or 5xSTS, and the Myotonic Dystrophy Health Index, or MDHI, patient reported outcome measure, all at 6 months compared to placebo. We intend that the data from the registrational expansion cohort and the already enrolled patients in the MAD and ongoing long-term extension portions of the ACHIEVE trial will support a potential submission for U.S. Accelerated Approval.

The registrational expansion cohort is expected to enroll 60 patients at the 6.8 mg/kg once every eight weeks, or Q8W, dose cohort. The registrational expansion cohort has been initiated and full enrollment is planned for early in the second quarter of 2026, with data from this cohort planned for the first quarter of 2027 and potential submission for U.S.

Accelerated Approval planned for early in the third quarter of 2027. We began the U.S. site activation process in June 2025 and in September 2025, U.S. site activation began. In October 2025, U.S. patient enrollment and dosing were initiated. As of October 31, 2025, we have enrolled approximately half of the registrational expansion cohort. We continue to advance preparations for a Phase 3 clinical trial of z-basivarsen and plan to initiate the trial in the first quarter of 2026. We continue to pursue approval pathways for z-basivarsen outside of the U.S. We anticipate a potential launch of z-basivarsen in the first quarter of 2028, assuming we receive favorable data, priority review is granted, and FDA approval is received on the anticipated timeline.

In June 2025, the FDA granted Breakthrough Therapy Designation to z-basivarsen for the treatment of DM1.

Additionally, in June 2025, we reported positive long-term efficacy and safety data from adult DM1 patients enrolled in the MAD portion of the ACHIEVE trial, including data from the 6.8 mg/kg Q8W cohort (n=6) at up to 12 months. At the 6.8 mg/kg Q8W dose, z-basivarsen demonstrated robust and sustained improvement in myotonia as measured by vHOT as well as sustained improvements across multiple other endpoints. We believe these data support improvement in vHOT as an early indicator of clinical benefit with z-basivarsen in DM1 and its potential as an intermediate clinical endpoint for U.S. Accelerated Approval. Treatment with z-basivarsen led to an improvement in vHOT of 3.3 seconds as compared to placebo at 6 months, and new data demonstrated that mean improvements at 6 months were sustained at 12 months for vHOT, 10MWR, 5xSTS, MDHI, and QMT, which demonstrated a 10% improvement in strength at 6 months, increasing to 20% at 12 months relative to baseline. In October 2025, we reported additional analyses from the same 6.8 mg/kg Q8W cohort (n=6) data at 12 months. This included new data showing improvement from baseline in the 9-Hole Peg Test, or 9HPT, a measure of upper limb function focused on manual dexterity and coordination; QMT scores across both upper and lower extremities showing robust and sustained improvement; additional MDHI subscales of mobility, ability to do activities and upper extremity function showing robust and sustained improvement and the Patient Global Impression of Change, or PGI-C, and Clinician Global Impression of Change, or CGI-C, scales showing improvement in overall disease burden.

In June 2025, we also reported updated safety and tolerability data from the 56 patients enrolled through the 6.8 mg/kg Q8W cohort of the ACHIEVE trial. As of the data cutoff date of April 23, 2025, z-basivarsen demonstrated a favorable safety profile. Additionally, the majority of treatment-emergent adverse events were mild or moderate, and no related serious treatment-emergent adverse events were identified. In addition, no participants demonstrated persistent related anemia, and no clinically meaningful changes were observed in kidney or liver parameters. Also, no participants demonstrated kidney injury. Liver enzyme elevations were observed in a minority of participants, with no impact on liver function. Interpretation of the elevated liver enzyme levels was complicated by underlying liver disease and elevated baseline values up to approximately 2.5 times greater than the upper limit of normal.

Our product candidate zeleciment rostudirsen, or z-rostudirsen (also known as DYNE-251) is currently being evaluated in the DELIVER trial, a Phase 1/2 global clinical trial consisting of a 24-week MAD randomized, placebo-controlled period, a 24-week OLE, and a 192-week long-term extension, and a registrational expansion cohort. The trial, which is designed to be registrational, enrolled ambulant and non-ambulant males with DMD who are ages 4 to 16 and have mutations amenable to exon 51 skipping. The primary endpoints of the MAD portion were safety, tolerability and change from baseline in dystrophin levels as measured by Western blot. Additional endpoints in the MAD portion of the trial included pharmacokinetics and change from baseline in exon 51 skipping levels, muscle tissue percent dystrophin positive fibers, multiple assessments of muscle function, including North Star Ambulatory Assessment score, Stride Velocity 95th Centile and certain timed functional tests.

In the MAD portion of the DELIVER trial, patients were randomized to receive z-rostudirsen or placebo every four or eight weeks intravenously for 24 weeks, depending on cohort. Patients cohorts were dosed from 0.7 mg/kg to 20 mg/kg (approximate phosphorodiamidate morpholino oligomer, or PMO dose). Following the placebo-controlled period, patients transitioned to z-rostudirsen treatment in the open-label portion of the trial and in the long-term extension.

In September 2024, we announced the completion of the MAD portion of the trial, and in November 2024, we announced the initiation of the 20 mg/kg Q4W (approximate PMO dose) registrational expansion cohort to support submission for Accelerated Approval in the U.S.

In March 2025, we reported positive long-term safety and efficacy data from the MAD portion of the DELIVER trial. The efficacy assessment of the DELIVER trial reported in March 2025 was based on 12-month biomarker and functional data from six male patients enrolled in the 20 mg/kg (approximate PMO dose) cohort who were randomized to receive z-rostudirsen or placebo once every four weeks, or Q4W, and 18-month functional data from six male patients in the 10

mg/kg (approximate PMO dose) cohort who were randomized to receive z-rostudirsen or placebo Q4W. During the open label extension period, all participants in the 10 mg/kg cohort were dose escalated to 20 mg/kg Q4W regimen after month six. Z-rostudirsen demonstrated dose-dependent exon skipping and dystrophin expression and meaningful and sustained improvements from baseline in multiple functional endpoints in both cohorts. In the 20 mg/kg Q4W cohort, z-rostudirsen demonstrated best-in-class levels of dystrophin expression, exon skipping and percent dystrophin.

In March 2025, we also reported updated safety and tolerability data based on 54 participants enrolled in the DELIVER trial. As of the data cutoff date of February 7, 2025, z-rostudirsen demonstrated a favorable safety profile and the majority of treatment emergent adverse events were mild or moderate and no related serious treatment emergent adverse events were identified other than in two participants at the 40 mg/kg Q4W dose level which events were potentially related to study drug. One participant experienced symptoms which suggested hemolytic uremic syndrome, potentially caused by an infection. The second participant had a history of unexplained hemolytic anemia and developed fever and tonsillitis after joining the DELIVER trial. Both participants have recovered.

We completed enrollment of the 20 mg/kg Q4W (approximate PMO dose) registrational expansion cohort of 32 participants as part of the DELIVER trial in March 2025 and are planning for data from this cohort in December 2025 and potential submission for U.S. Accelerated Approval in the second quarter of 2026. We continue to pursue accelerated approval in the U.S. based on dystrophin as a surrogate endpoint. We continue to pursue approval pathways for z-rostudirsen outside of the U.S.

In August 2025, the FDA granted Breakthrough Therapy Designation to z-rostudirsen for the treatment of DMD, amenable to exon 51 skipping. Additionally, in September 2025, the Ministry of Health, Labour and Welfare in Japan granted Orphan Drug designation for z-rostudirsen in individuals with DMD who have mutations in the *DMD* gene that are amenable to exon 51 skipping.

In June 2024 and June 2025, we announced new preclinical data for DYNE-302, our product candidate for FSHD, that demonstrated robust and durable DUX4 suppression and functional benefit in a mouse model. We generated these data using an innovative hTfR1/iFLExD mouse model we developed that expresses the human transferrin 1 receptor, or TfR1, and enables tunable DUX4 induction in skeletal muscle. In hTfR1/iFLExD mice, a single intravenous dose of DYNE-302 resulted in dose-dependent and robust reduction of the DUX4 transcriptome that lasted up to three months, with benefit on muscle structure. DYNE-302 also demonstrated prevention as well as reversal of muscle weakness. We are progressing DYNE-302 through IND/CTA-enabling studies.

We are developing a product candidate, DYNE-401, to deliver an enzyme replacement therapy to address the deficiency of the lysosomal enzyme, GAA, that causes Pompe disease. We engineered FORCE-GAA by leveraging the FORCE platform and evaluated efficacy *in vivo* using hTfR1/6^{Neo} mice, that were developed by crossing the well-established 6^{Neo} mouse model of Pompe with mice expressing human transferrin receptor 1. Using this approach, intravenous, or IV, administration cleared glycogen in muscle and the CNS and normalized lysosomal size in hTfR1/6^{Neo} mice. This approach reduced serum neurofilament light chain, a biomarker of axonal injury, providing evidence of benefit in the CNS and displayed superior dose potency compared to GAA alone. Additional data with this approach supported the potential for monthly dosing which is less frequent than approved enzyme replacement therapies for Pompe.

We were incorporated and commenced operations in 2017. Since our incorporation, we have devoted substantially all of our financial resources and efforts to organizing and staffing our company, business planning, raising capital, conducting research and development activities and filing and prosecuting patent applications. We do not have any products for sale and have not generated any revenue from product sales or otherwise. To date, we have principally raised capital through sales of equity securities and our initial borrowing under our Loan and Security Agreement, or the Loan Agreement, with Hercules Capital, Inc., or Hercules.

Since our inception, we have incurred significant operating losses. Our ability to generate any product revenue or product revenue sufficient to achieve profitability will depend on the successful development and eventual commercialization of

one or more product candidates. For the nine months ended September 30, 2025 and 2024, we reported net losses of $334.3 million and $227.9 million, respectively. As of September 30, 2025, we had an accumulated deficit of $1.3 billion.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We expect that our expenses and capital expenditure requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- advance our product candidates for DM1, DMD, FSHD and Pompe and conduct research programs in additional indications;
- expand the capabilities of our proprietary FORCE platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- establish manufacturing sources for any product candidate we may develop, including the Fab antibody, linkers and therapeutic payload that will comprise the product candidate, and secure supply chain capacity to provide sufficient quantities for preclinical and clinical development and commercial supply;
- 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.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for any product candidates we may develop. If we obtain regulatory approval for or otherwise commercialize any product candidates we may develop, we expect to incur significant expenses related to developing our commercialization capabilities to support product sales, marketing and distribution. Further, we expect to continue to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed, on favorable terms, or at all. If we fail to raise capital or enter into such agreements or arrangements as and when needed, we may have to significantly delay, reduce or eliminate the development or future commercialization of one or more product candidates we may develop.

Because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to raise capital, maintain our research and development efforts, expand our business or continue our operations at planned levels, and as a result we may be forced to substantially reduce or terminate our operations.

We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses, debt service obligations and capital expenditure requirements into the third quarter of 2027. Based on our current plans and anticipated timelines and assuming positive data results from the Registrational Expansion Cohorts of our ACHIEVE and DELIVER clinical trials and assuming no additional testing is required by the FDA, we estimate that these cash resources would be sufficient to enable us to obtain data from the Registrational Expansion Cohorts of our ACHIEVE and DELIVER clinical trials, submit applications for marketing approval to the FDA for z-rostudirsen in DMD and z-basivarsen in DM1 using the Accelerated Approval pathway and obtain a decision from the FDA with respect to the application for marketing approval for z-rostudirsen and, if approved, commercially launch z-rostudirsen in the United States.

We have based our estimates as to how long we expect we will be able to fund our operations, debt service obligations and capital expenditure requirements on assumptions that may prove to be wrong. We could use our available capital

resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. See "—Liquidity and capital resources" below. These estimates do not give effect to any additional funding tranches we may obtain access to under our Loan Agreement with Hercules, subject to the achievement of specified clinical, regulatory and commercial milestones, and do not give effect to any revenue we may generate on commercial sales of any products for which we obtain regulatory approval.

Impact of Tariffs

The new U.S. administration has announced or imposed a series of tariffs on U.S. trading partners. In response, several countries have threatened or imposed retaliatory measures. While we have not experienced, and do not currently expect to experience, any significant direct impact from these tariffs and retaliatory measures, the full extent of the future impact of these and other threatened measures remains uncertain. We continue to monitor these tariffs and retaliatory measures and their possible effects on our business.

Components of our results of operations

Revenue

We have not generated any revenue since our inception and do not expect to generate any revenue from the sale of products at least until 2027, if at all. If our development efforts are successful and we commercialize products, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales, as well as upfront, milestone and royalty payments from such collaboration or license agreements, or a combination thereof.

Research and development expenses

Research and development expenses consist primarily of costs incurred for our research activities and development of our product candidates. These expenses include:

- development and operation of our proprietary FORCE platform;
- employee-related expenses, including salaries, related benefits and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with our research programs and development of our product candidates, including those incurred under agreements with third parties, such as consultants and contract research organizations, or CROs, to conduct preclinical studies and clinical trials;
- the cost of laboratory supplies and acquiring, developing and manufacturing materials for use in our research, preclinical studies and clinical trials, including those incurred under agreements with third parties, such as consultants and CMOs;
- facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance; and
- costs related to compliance with regulatory requirements.

We expense research and development costs as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct external research and development expenses consist of costs that include fees, reimbursed materials and other costs paid to consultants, contractors, CMOs and CROs in connection with our development, manufacturing and clinical activities. We have not allocated our direct external research and development costs to specific programs or product candidates that are not in clinical development.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. Accordingly, we expect that our research and development expenses will increase substantially as we advance z-basivarsen and z-rostudirsen through clinical trials, in connection with our preclinical and clinical development activities of DYNE-302, our FSHD product candidate, and DYNE-401, our Pompe disease product candidate, and if, and as, we advance any other

product candidates through preclinical studies and clinical trials. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. The successful development of any product candidate is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of programs we decide to pursue and their regulatory paths to market;
- the need to raise funding to complete preclinical and clinical development of any product candidates we may develop;
- our ability to establish new licensing or collaboration arrangements and the progress of the development efforts of third parties with whom we may enter into such arrangements;
- our ability to maintain our current research and development programs and to establish new programs;
- the successful initiation, enrollment and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities for any product candidates we may develop;
- the availability of specialty raw materials for use in production of any product candidate we may develop;
- establishing agreements with third-party manufacturers for supply of product candidate components for our clinical trials;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our other rights in our intellectual property portfolio;
- commercializing product candidates, if and when approved, whether alone or in collaboration with others; and
- obtaining and maintaining third-party insurance coverage and adequate reimbursement for any approved products.

A change in the outcome of any of these variables with respect to the development of any product candidate we may develop could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any product candidate we may develop.

General and administrative expenses

General and administrative expenses consist primarily of employee-related expenses, including salaries, related benefits and stock-based compensation, for employees in executive, finance, corporate and business development and administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters; professional fees for accounting, auditing, tax and administrative consulting services; insurance costs; administrative travel expenses; and facility-related expenses, which include allocated expenses for rent, depreciation and maintenance of facilities and other operating costs.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our growth strategy. In addition, if we obtain regulatory approval for a product candidate and do not enter into a third-party commercialization collaboration, we expect to incur significant expenses related to building a sales and marketing team to support product sales, marketing and distribution activities.

Interest income

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

Interest expense

Interest expense consists of amortization of debt issuance costs and discount and interest expense under the Loan Agreement with Hercules.

Other income (expense), net

Other income (expense), net consists of realized gains and losses on sales of marketable securities and foreign currency gains and losses.

Income taxes

Since our inception, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in any year or for our earned research and development tax credits, due to our uncertainty of realizing a benefit from those items.

Results of operations

Comparison of the three months ended September 30, 2025 and 2024

The following table summarizes our results of operations for the three months ended September 30, 2025 and 2024:

(in thousands)	Three Months Ended September 30, 2025	2024	Change
Operating expenses:			
Research and development	$ 97,219	$ 92,800	$ 4,419
General and administrative	16,673	12,859	3,814
Total operating expenses	113,892	105,659	8,233
Loss from operations	(113,892)	(105,659)	(8,233)
Other income (expense):			
Interest income	8,476	9,276	(800)
Interest expense	(2,904)	—	(2,904)
Other income (expense), net	279	(742)	1,021
Total other income (expense), net	5,851	8,534	(2,683)
Net loss	$ (108,041)	$ (97,125)	$ (10,916)

Research and development expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2025 and 2024:

(in thousands)	Three Months Ended September 30, 2025	2024	Change
Direct research and development expenses by product candidate:			
Z-basivarsen (DM1)	$ 37,853	$ 30,922	$ 6,931
Z-rostudirsen (DMD)	22,666	29,021	(6,355)
Unallocated research and development expenses:			
Platform and external research and development	5,769	10,439	(4,670)
Personnel related (including stock-based compensation)	23,811	17,223	6,588
Facility-related and other	7,120	5,195	1,925
Total research and development expenses	$ 97,219	$ 92,800	$ 4,419

Expenses related to z-basivarsen increased in the three months ended September 30, 2025 compared to the three months ended September 30, 2024. This was attributable to higher manufacturing activity in the three months ended September 30, 2025 to produce a sufficient clinical supply of drug product for the ongoing ACHIEVE trial and an increase in clinical trial activity primarily for the registrational expansion cohort of the ACHIEVE trial. Expenses related to z-rostudirsen decreased in the three months ended September 30, 2025 compared to the three months ended September 30, 2024. This was attributable to lower manufacturing cost in the three months ended September 30, 2025 due to the timing of a new raw material purchase in the three months ended September 30, 2024.

The decrease in platform and external research and development expenses in the three months ended September 30, 2025 compared to the three months ended September 30, 2024 was primarily due to timing of raw material purchases in manufacturing. The increase in personnel-related expenses was primarily due to increased headcount in our research and

development function. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of research and development personnel.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three months ended September 30, 2025 and 2024:

(in thousands)	Three Months Ended September 30, 2025	2024	Change
Personnel-related	$ 4,964	$ 2,847	$ 2,117
Stock-based compensation expense	4,824	4,295	529
Professional and consulting fees	4,557	4,077	480
Facility-related and other	2,328	1,640	688
Total general and administrative expenses	$ 16,673	$ 12,859	$ 3,814

The increase in personnel-related and stock-based compensation expenses in the three months ended September 30, 2025 compared to the three months ended September 30, 2024 was due to increased headcount in our general and administrative function. Professional and consulting fees increased due to the higher consulting costs to support the growth of the organization in the three months ended September 30, 2025. Facility-related and other expenses increased due to higher costs of supporting a larger number of general and administrative personnel in the three months ended September 30, 2025.

Interest income

Interest income for the three months ended September 30, 2025 and 2024 was $8.5 million and $9.3 million, respectively, due to interest earned on invested cash balances. The decrease in interest income was due to an overall decrease in interest rates in the three months ended September 30, 2025 compared to the three months ended September 30, 2024.

Interest expense

Interest expense for the three months ended September 30, 2025 was $2.9 million due to our entry into the Loan Agreement with Hercules in June 2025. No interest expense was incurred in the three months ended September 30, 2024.

Other income (expense), net

Other income for the three months ended September 30, 2025 was $0.3 million while other expense for the three months ended September 30, 2024 was $0.7 million due to realized foreign currency gains.

Comparison of the nine months ended September 30, 2025 and 2024

The following table summarizes our results of operations for the nine months ended September 30, 2025 and 2024:

(in thousands)	Nine Months Ended September 30, 2025	2024	Change
Operating expenses:			
Research and development	$ 302,902	$ 199,601	$ 103,301
General and administrative	49,153	47,177	1,976
Total operating expenses	352,055	246,778	105,277
Loss from operations	(352,055)	(246,778)	(105,277)
Other income (expense):			
Interest income	22,202	19,330	2,872
Interest expense	(2,998)	—	(2,998)
Other income (expense), net	(1,408)	(428)	(980)
Total other income (expense), net	17,796	18,902	(1,106)
Net loss	$ (334,259)	$ (227,876)	$ (106,383)

Research and development expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2025 and 2024:

(in thousands)	Nine Months Ended September 30, 2025	2024	Change
Direct research and development expenses by product candidate:			
Z-basivarsen (DM1)	$ 117,964	$ 53,047	$ 64,917
Z-rostudirsen (DMD)	73,314	62,677	10,637
Unallocated research and development expenses:			
Platform and external research and development	21,399	20,622	777
Personnel related (including stock-based compensation)	69,708	48,731	20,977
Facility related and other	20,517	14,524	5,993
Total research and development expenses	$ 302,902	$ 199,601	$ 103,301

Expenses related to z-basivarsen increased in the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024. This was attributable to higher manufacturing activity in the nine months ended September 30, 2025 to produce a sufficient clinical supply of drug product for the ongoing ACHIEVE trial and an increase in clinical trial activity for the registrational expansion cohort of the ACHIEVE trial. Expenses related to z-rostudirsen increased in the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024. This was attributable to higher manufacturing activity in the nine months ended September 30, 2025 to produce a sufficient clinical supply of drug product for the ongoing DELIVER trial and an increase in clinical trial activity for the registrational expansion cohort of the DELIVER trial.

The increase in platform and external research and development expenses in the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024 was primarily due to increased external research activity associated with our preclinical programs and product candidates. The increase in personnel-related expenses was primarily due to increased headcount in our research and development function. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of research and development personnel.

General and administrative expenses

The following table summarizes our general and administrative expenses for the nine months ended September 30, 2025 and 2024:

(in thousands)	Nine Months Ended September 30, 2025	2024	Change
Personnel-related	$ 13,117	$ 8,507	$ 4,610
Stock-based compensation expense	14,530	23,135	(8,605)
Professional and consulting fees	15,320	11,025	4,295
Facility-related and other	6,186	4,510	1,676
Total general and administrative expenses	$ 49,153	$ 47,177	$ 1,976

The increase in personnel-related expenses in the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024 was due to increased headcount in our general and administrative function. The decrease in stock-based compensation expense in the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024 was primarily the result of entering into separation and consulting agreements with our former chief executive officer in March 2024 and former chief business officer in September 2024, resulting in the acceleration of vesting and modification of previously granted awards. Professional and consulting fees increased due to the higher consulting costs to support the growth of the organization in the nine months ended September 30, 2025. Facility-related and other expenses increased due to higher costs of supporting a larger number of general and administrative personnel in the nine months ended September 30, 2025.

Interest income

Interest income for the nine months ended September 30, 2025 and 2024 was $22.2 million and $19.3 million, respectively, due to interest earned on invested cash balances. The increase in interest income was due to an increased

cash, cash equivalents and marketable securities balance throughout the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024.

Interest expense

Interest expense for the nine months ended September 30, 2025 was $3.0 million due to our entry into the Loan Agreement with Hercules in June 2025. No interest expense was incurred in the nine months ended September 30, 2024.

Other income (expense), net

Other expense for the nine months ended September 30, 2025 was $1.4 million due to net foreign currency losses. Other income for the nine months ended September 30, 2024 was $0.4 million due to net foreign currency gains.

Liquidity and capital resources

Sources of liquidity

Since our inception, we have incurred significant operating losses. We expect to incur significant expenses and operating losses for the foreseeable future as we support our continued research activities and development of our product candidates and platform. We have not yet commercialized any product candidates, and we do not expect to generate revenue from sales of any product candidates at least until 2027, if at all. To date, we have funded our operations primarily with proceeds from sales of equity securities and our initial borrowing under the Loan Agreement with Hercules. As of September 30, 2025 we had cash, cash equivalents and marketable securities of $791.9 million.

In November 2021, we filed a universal shelf registration statement on Form S-3, or the 2021 Shelf Registration Statement, to register for sale from time to time up to $400.0 million of common stock, preferred stock, debt securities, warrants and/or units in one or more offerings. Further, in November 2021, we entered into an Open Market Sale Agreement℠, or the Sales Agreement, with Jefferies LLC, or Jefferies, and included in the 2021 Shelf Registration Statement a prospectus relating to the Sales Agreement, pursuant to which, from time to time, we may offer and sell shares of our common stock having an aggregate offering price of up to $150.0 million, which we refer to as our at-the-market offering program. Sales of common stock under the Sales Agreement through Jefferies may be made by any method that is deemed an "at-the-market" offering as defined in Rule 415(a)(4) under the Securities Act of 1933, as amended, or Securities Act. On January 4, 2024, we notified Jefferies that we were suspending and terminating the prospectus filed under our 2021 Shelf Registration Statement relating to the Sales Agreement for our at-the-market offering program.

On March 5, 2024, we filed a universal shelf registration statement on Form S-3, or the 2024 Shelf Registration Statement, and included a prospectus relating to the Sales Agreement. Under the 2024 Shelf Registration Statement, we may offer and sell debt securities, common stock, preferred stock, units and/or warrants from time to time at an indeterminate aggregate offering price in one or more offerings. In November 2024, we filed a prospectus supplement relating to the Sales Agreement, pursuant to which, in accordance with the Sales Agreement, we may offer and sell shares of our common stock having an aggregate offering price of up to $300.0 million in our at-the-market offering program.

During the nine months ended September 30, 2025, we issued and sold an aggregate of 10,660,159 shares of common stock pursuant to the Sales Agreement for aggregate net proceeds of $140.6 million, after deducting commissions and offering expenses payable by us. We sold such shares at a weighted average price of $13.60 per share.

In June 2025, we entered into the Loan Agreement with Hercules, in its capacity as administrative agent and collateral agent and as a lender, and certain other financial institutions that from time to time become parties to the Loan Agreement as lenders, which we refer to collectively as the Lenders. The Loan Agreement provides for term loans in an aggregate principal amount of up to $275.0 million under multiple tranches, available as follows: (i) an initial term loan tranche funded on the closing date of the Loan Agreement in aggregate principal amount of $100.0 million; (ii) subject to the achievement of specified clinical, regulatory and commercial milestones, three additional term loan tranches totaling up to $115.0 million; and (iii) subject to approval by the Lenders' investment committee in their discretion, a final term loan tranche of up to $60.0 million. On June 27, 2025, we received net proceeds of $98.8 million from the initial term loan tranche, after deducting debt issuance costs payable by us. Refer to Note 7, "Long-Term Debt" in the accompanying notes to the condensed consolidated financial statements for a discussion of the Loan Agreement with Hercules.

In July 2025, we completed the July 2025 offering, pursuant to which we issued and sold 27,878,788 shares of our common stock. We received net proceeds from the offering of $215.8 million, after deducting underwriting discounts and commissions and offering expenses paid by us.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

(in thousands)	Nine Months Ended September 30, 2025	2024
Net cash used in operating activities	$ (291,375)	$ (208,034)
Net cash used in investing activities	(29,733)	(188,066)
Net cash provided by financing activities	459,342	806,259
Net increase in cash, cash equivalents and restricted cash	$ 138,234	$ 410,159

Operating activities

During the nine months ended September 30, 2025, operating activities used $291.4 million of cash, due to our net loss of $334.3 million, partially offset by non-cash charges of $35.0 million and changes in our operating assets and liabilities of $7.9 million. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $17.7 million increase in accounts payable and other liabilities and a $3.2 million decrease in prepaid expenses and other current assets, partially offset by a $13.0 million increase in other non-current assets. During the nine months ended September 30, 2024, operating activities used $208.0 million of cash, due to our net loss of $227.9 million and net cash used by changes in our operating assets and liabilities of $14.4 million, partially offset by non-cash charges of $34.2 million. Net cash used by changes in our operating assets and liabilities primarily consisted of a $8.0 million decrease in accounts payable and other liabilities and a $6.4 million increase in prepaid expenses and other current assets. Changes in our operating assets and liabilities during these periods were generally due to the growth of our business, increased clinical trial activity, increased manufacturing activities, advancement of our product candidates, the timing of vendor invoices and payments and annual bonus payments.

Investing activities

During the nine months ended September 30, 2025, net cash used in investing activities was $29.7 million due to purchases of marketable securities of $153.8 million, an advance payment for long-lead equipment for $18.8 million, and purchases of property and equipment of $1.4 million, partially offset by maturities of marketable securities of $135.8 million and sales of marketable securities of $8.5 million. During the nine months ended September 30, 2024, net cash used in investing activities was $188.1 million due to purchases of marketable securities of $264.7 million and purchases of property and equipment of $1.3 million, partially offset by maturities of marketable securities of $76.2 million and sales of marketable securities of $1.7 million.

Financing activities

During the nine months ended September 30, 2025, net cash provided by financing activities was $459.3 million, consisting of $215.8 million in aggregate net proceeds from sales of common stock under our follow-on offering in July 2025, $140.6 million in aggregate net proceeds from sales of common stock under our at-the-market offering program, $98.8 million in net proceeds from the initial term loan tranche under the Loan Agreement with Hercules and $4.6 million in proceeds received from stock option exercises. These cash inflows are partially offset by $0.5 million in payment of debt issuance costs. During the nine months ended September 30, 2024, net cash provided by financing activities was $806.3 million, consisting of $675.2 million in aggregate net proceeds from sales in our public offerings in January 2024 and May 2024, $97.9 million in aggregate net proceeds from sales of our common stock under our at-the-market offering program and $33.2 million in net proceeds received from stock option exercises.

Funding requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we advance the clinical development of z-basivarsen and z-rostudirsen, the development of our FSHD and Pompe programs and additional research programs. The timing and amount of our operating expenditures will depend largely on:

- the identification of additional product candidates;

- the scope, progress, costs and results of preclinical and clinical development of any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- changes in laws or regulations applicable to any product candidates we may develop, including but not limited to clinical trial requirements for approvals;
- the cost and timing of obtaining materials to produce adequate product supply for any preclinical or clinical development of any product candidate we may develop;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate we may develop for which we obtain marketing approval;
- the legal costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims;
- additions or departures of key scientific or management personnel;
- our ability to establish and maintain collaborations on favorable terms, if at all, as well as the costs and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder; and
- the costs of operating as a public company.

We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses, debt service obligations and capital expenditure requirements into the third quarter of 2027. Based on our current plans and anticipated timelines and assuming positive data results from the registrational expansion cohorts of our ACHIEVE and DELIVER clinical trials and assuming no additional testing is required by the FDA, we estimate that these cash resources would be sufficient to enable us to obtain data from the registrational expansion cohorts of our ACHIEVE and DELIVER clinical trials, submit applications for marketing approval to the FDA for z-rostudirsen in DMD and z-basivarsen in DM1 using the Accelerated Approval pathway and obtain a decision from the FDA with respect to the application for marketing approval for z-rostudirsen and, if approved, commercially launch z-rostudirsen in the United States. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaborations, strategic alliances or marketing, distribution 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. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed, on favorable terms, or at all. If we fail to raise capital or enter into such agreements other arrangements as and when needed, we may have to significantly delay, reduce or eliminate the development or future commercialization of one or more of our product candidates we may develop. See Item 1A. “Risk factors” in this Quarterly Report for additional risks associated with our substantial capital requirements.

Contractual and other obligations

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancelable by us upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation and in the case of certain arrangements with CROs and CMOs may include non-cancelable fees.

We have also entered into a license agreement with the University of Mons under which we are obligated to make specified milestone and royalty payments. The payment obligations under this agreement are contingent upon future events, such as our achievement of specified development, regulatory and commercial milestones, or generating product sales. We are unable to estimate the timing or likelihood of achieving these milestones or generating future product sales.

On December 4, 2020, we entered into a lease agreement for office and laboratory space, which was amended in January 2021, March 2021 and June 2021. The lease has a term of 8.5 years that commenced when we gained access to the office and laboratory space in September 2021. Our obligation for the payment of the base rent began in April 2022 and is $0.4 million per month, increasing to $0.5 million per month during the term of the lease. We have two options to extend the term of the lease, each for a period of an additional five years.

On January 15, 2025, we entered into a master manufacturing services agreement with a CMO which secures capacity at the CMO's manufacturing facilities for certain of our product candidates and components thereof. As of September 30, 2025, we have paid $31.2 million towards non-current assets under this agreement and pursuant to a mutually agreed rolling forecast we have committed to pay an additional $65.6 million in fees through September 2027. In specified termination circumstances, the agreement requires us to pay the CMO for services completed, the cost of the CMO's raw materials that cannot be repurposed and specified cancellation fees. This agreement formalizes and supersedes a letter agreement that we entered into with the CMO on July 18, 2024.

On October 31, 2025, we entered into another master manufacturing services agreement with a CMO which also secures capacity at the CMO's manufacturing facilities for certain of our product candidate components. The agreement obligates us to compensate the CMO for producing certain of our product candidate components pursuant to a mutually agreed rolling forecast. Upon execution, we committed to compensate the CMO at least $25.5 million in fees thorugh March 2027. In specified termination circumstances, the agreement requires us to pay the CMO for services completed, the cost of the CMO's raw materials that cannot be repurposed, capital equipment and certain manufacturing activities previously committed to.

Critical accounting estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those accounting principles generally accepted in the United States of America that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations as well as the specific manner in which we apply those principles. Management has determined that our most critical accounting policies are those relating to accrued research and development expenses and stock-based compensation and that these policies have the most significant level of estimation uncertainty and have and are reasonably likely to have a material impact on our financial condition and results of operations. As we advance our product candidates into and through clinical development, we expect research and development expenses and, in particular, our accounting for accrued research and development expenses to be an increasingly important critical accounting policy.

There have been no significant changes to our critical accounting policies or estimates from those described in our Annual Report on Form 10-K filed with the SEC on February 27, 2025.

Recently Issued and Adopted Accounting Pronouncements

Refer to Note 2, "Summary of Significant Accounting Policies" in the accompanying notes to the condensed consolidated financial statements for a discussion of significant accounting policies. There are no recently issued accounting pronouncements that have not yet been adopted that are expected to have a material impact on the Company’s financial statements as of September 30, 2025.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended September 30, 2025, there have been no material changes in our market risk or how our market risk is managed from that described in "Quantitative and Qualitative Disclosures About Market Risk," included in our Annual Report on Form 10-K for the year ended December 31, 2024 filed with the SEC on February 27, 2025.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2025. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of an issuer that are designed to ensure that information required to be disclosed by an issuer in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by an issuer in the reports that it files or submits under the Exchange Act is accumulated and communicated to the issuer's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any disclosure 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. Based on the evaluation of our disclosure controls and procedures as of September 30, 2025, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

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) that occurred during the three months ended September 30, 2025 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.

None.

Item 1A. Risk Factors.

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Quarterly Report on Form 10-Q, or this Quarterly Report, including our condensed consolidated financial statements and the related notes thereto in evaluating our company. The risks described below are not the only risks facing our company. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could cause our business, prospects, operating results and financial condition to suffer materially.

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 $334.3 million and $227.9 million for the nine months ended September 30, 2025 and 2024, respectively and $317.4 million for the year ended December 31, 2024. As of September 30, 2025, we had an accumulated deficit of $1.3 billion. To date, we have financed our operations with the proceeds raised from the sale of equity securities and our initial borrowing under the Loan Agreement with Hercules. We have devoted substantially all of our financial resources and efforts to research and development. We are still in the early stages of development of our programs and product candidates. Our product candidates are in varying stages of preclinical and clinical development and we have not completed clinical development of any product candidate. 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:

- •advance our product candidates for DM1, DMD, FSHD and Pompe and any additional product candidates we may develop;
- •expand the capabilities of our proprietary FORCE platform;
- •seek marketing approvals for any product candidates that successfully complete clinical trials;
- •obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- •hire additional clinical, regulatory and scientific personnel;
- •establish manufacturing sources for any product candidate we may develop, including the Fab antibody, linker and therapeutic payload that will comprise the product candidate, and secure supply chain capacity to provide sufficient quantities for preclinical and clinical development and commercial supply;
- •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 any product candidates we may develop, 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.

Our product candidates are in varying stages of preclinical and clinical development. We have not completed clinical development of any product candidate, and we do not expect to have a product candidate ready for commercialization at least until 2027, if ever. 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 and clinical development of any product candidates we may identify and determine to develop;
- •obtaining regulatory approval for any product candidates we may develop;
- •manufacturing, 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. Our product candidates are in varying stages of preclinical and clinical development, and we have not completed clinical development of any product candidate. 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 any product candidate we may develop 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 products, 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 our stockholders to lose all or part of their investment.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development activities or commercialization efforts.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the clinical development of z-basivarsen and z-rostudirsen, and the preclinical and clinical development of DYNE-302, our FSHD product candidate, and DYNE-401, our Pompe disease product candidate and any additional product candidates we may develop, and arrange for the manufacturing of, and potentially seek marketing approval for any product candidates we may develop. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. 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, 2025, we had cash, cash equivalents and marketable securities of $791.9 million.

We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses, debt service obligations and capital expenditure requirements into the third quarter of 2027. Based on our current plans and anticipated timelines and assuming positive data results from the registrational expansion cohorts of our ACHIEVE and DELIVER clinical trials and assuming no additional testing is required by the FDA, we estimate that these cash resources would be sufficient to enable us to obtain data from the registrational expansion cohorts of our ACHIEVE and DELIVER clinical trials, submit applications for marketing approval to the FDA for z-rostudirsen in DMD and z-basivarsen in DM1 using the Accelerated Approval pathway and obtain a decision from the FDA with respect to the application for marketing approval for z-rostudirsen and, if approved, commercially launch z-rostudirsen in the United States. However, we have based these estimates on assumptions that may prove to be wrong, and our operating plan may change as a result of many 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 identification of additional product candidates;
- the scope, progress, costs and results of preclinical and clinical development of any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- changes in laws or regulations applicable to any product candidates we may develop, including but not limited to clinical trial requirements for approvals;
- the cost and timing of obtaining materials to produce adequate product supply for any preclinical or clinical development of any product candidate we may develop;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate we may develop for which we obtain marketing approval;
- the legal costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims;
- additions or departures of key scientific or management personnel;

- our ability to establish and maintain collaborations on favorable terms, if at all, as well as the costs and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder; and
- the costs of operating as a public company.

Identifying 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 identify and 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. Other than the Loan Agreement with Hercules, 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 candidates we may develop, 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.

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. For example, in June 2025, we entered into the Loan Agreement with Hercules providing for term loans in an aggregate principal amount of up to $275.0 million under multiple tranches, available as follows: (i) an initial term loan tranche funded on the closing date of the Loan Agreement in aggregate principal amount of $100.0 million; (ii) subject to the achievement of specified clinical, regulatory and commercial milestones, three additional term loan tranches totaling up to $115.0 million; and (iii) subject to approval by the lenders' investment committee in their discretion, a final term loan tranche of up to $60.0 million. However, if we do not satisfy the specified clinical, regulatory and commercial milestones or the lenders do not otherwise approve additional borrowing, we may not have access to the remaining amounts under term loans. Other than the Loan Agreement with Hercules, 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, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of common stockholders. 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.

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

We commenced operations in 2017, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting research and development activities and filing and prosecuting patent applications. Our product candidates are in varying stages of preclinical and clinical development, and their risk of failure

is high. We have not yet demonstrated our ability to complete the clinical development of any product candidate, 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 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 clinical-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 are in the process of transitioning from a company focused on conducting development activities to a company capable of supporting commercial activities. We may not be successful in such a transition.

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, 2024, we had federal NOL carryforwards of $315.7 million and state NOL carryforwards of $332.2 million.

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 taxable income. We completed a Section 382 study of transactions in our stock through January 25, 2021 and concluded that we have experienced ownership changes since inception that we believe under Sections 382 and 383 of the Code will result in limitation on our ability to use certain pre-change NOLs and credits. With additional stock issuances during 2024 and the first and third quarters of 2025, the Company continues to analyze the impacts of Section 382. In the future, we may experience additional ownership changes as a result of equity offerings or other changes in the ownership of our stock, some of which are beyond our control. 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.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs could expire or otherwise become unavailable to offset future income tax liabilities. Legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Risks related to our indebtedness

Our Loan Agreement contains restrictive and financial covenants that may limit our operating flexibility.

On June 27, 2025, we entered into the Loan Agreement with Hercules providing for term loans in an aggregate principal amount of up to $275.0 million available as follows: (i) an initial term loan tranche funded on June 27, 2025 in aggregate principal amount of $100.0 million; (ii) subject to the achievement of specified clinical, regulatory and commercial milestones, three additional term loan tranches totaling up to $115.0 million; and (iii) subject to approval by the lenders' investment committee in their discretion, a final term loan tranche of up to $60.0 million. Our obligations under the Loan Agreement are secured by a first-priority security interest in substantially all of our property, inclusive of intellectual property, subject to customary permitted liens and other exceptions set forth in the Loan Agreement.

The Loan Agreement contains customary representations and warranties, events of default and affirmative and negative covenants, including a minimum cash covenant, which we refer to as the Minimum Cash Covenant, requiring that we maintain specified levels of cash in accounts subject to a control agreement in favor of Hercules as the agent for the Loan

Agreement, or the Qualified Cash, during the period commencing on April 1, 2026 (or January 1, 2027 if we have raised at least $350.0 million of cumulative net cash proceeds between June 4, 2025 and December 15, 2026 from the issuance of equity or indebtedness or payments under certain royalty or license transactions). The Minimum Cash Covenant will initially be set at 60% of the then outstanding obligations under the Loan Agreement, is subject to adjustment and will not be tested at any time when our market capitalization is greater than $1.65 billion. We are also required to maintain minimum net product revenue from the sale of z-basivarsen and z-rostudirsen starting nine months after FDA approval of z-basivarsen or z-rostudirsen, which we refer to as the Minimum Revenue Covenant, if the outstanding obligations under the Loan Agreement exceeds $100.0 million. The Minimum Revenue Covenant will not be tested for any month to the extent that for each day during such month either (i) Qualified Cash is at least 100% of our outstanding obligations under the Loan Agreement or (ii) our market capitalization is greater than $1.65 billion and Qualified Cash is at least 50% of our outstanding obligations under the Loan Agreement. Certain negative covenants under the Loan Agreement limit our ability, among other things, to incur future debt, grant liens, make investments, make acquisitions, distribute dividends and sell assets, subject in each case to certain exceptions. Our business may be adversely affected by these restrictions on our ability to operate our business. If we raise any additional debt financing, as permitted by the Loan Agreement, the terms of such additional indebtedness could further restrict our operating and financial flexibility.

We may not be able to generate sufficient cash flow or sales to meet the Minimum Cash Covenant or the Minimum Revenue Covenant or pay the outstanding obligations under the Loan Agreement when due. Furthermore, our future working capital, borrowings or equity financings could be unavailable to repay or refinance the amounts outstanding under the Loan Agreement. In the event of a liquidation of our business, we would be required to repay all outstanding obligations under the Loan Agreement prior to the distribution of assets to unsecured creditors, and the holders of our common stock would receive a portion of any liquidation proceeds only if all of our creditors then existing, including the lenders under our Loan Agreement with Hercules, were first repaid in full.

Our failure to comply with the covenants or other terms of the Loan Agreement, including as a result of events beyond our control, could result in a default under the Loan Agreement that could materially and adversely affect our business.

We may be required to repay the outstanding indebtedness under the Loan Agreement if an event of default occurs under the Loan Agreement or, if applicable, any future debt facility. The Loan Agreement includes customary events of default, including payment defaults, breaches of covenants following any applicable cure period, the occurrence of certain events that could reasonably be expected to have a "material adverse effect" as set forth in the Loan Agreement and cross acceleration. We may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time any such event of default occurs. In this case, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our business, financial condition and results of operations could be materially adversely affected as a result of any of these events.

Risks related to discovery and development

Our product candidates are in varying stages of preclinical and clinical development and we have not completed clinical development of any product candidate. We do not expect to complete the commercialization of any product candidate at least until 2027, if ever. If we are unable to identify and advance 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 have focused our efforts to date on developing our platform, identifying our programs and conducting the clinical development of our product candidates. Our product candidates are in varying stages of preclinical and clinical development, and we have not completed clinical development of any product candidate. Our ability to generate product revenue, which we do not expect will occur until at least 2027, if ever, will depend heavily on the successful 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 United States is subject to acceptance 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 requests prior to commencing clinical trials, the start of any future clinical trials may be delayed. Even after we receive and incorporate guidance from the FDA, the FDA could disagree that we have satisfied their requirements to commence any clinical trial or change their position on

the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect.

For example, the FDA placed on clinical hold our IND application to initiate a clinical trial of z-rostudirsen in patients with DMD amenable to skipping exon 51. We received a clinical hold letter from the FDA in January 2022 requesting additional clinical and non-clinical information for z-rostudirsen, which we submitted before the FDA ultimately cleared the IND in July 2022.

There are equivalent processes and risks applicable to clinical trial applications in other countries, including countries in the European Union.

Commercialization of any product candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in any jurisdiction where we seek to commercialize such product candidates, such as the FDA and the European Medicines Agency, or EMA; manufacturing supply, capacity and expertise; a commercial organization; and significant marketing efforts. The success of product candidates we may develop will depend on many factors, including the following:

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

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 we may develop, which would materially harm our business. If we are unable to advance our product candidates into and through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We may encounter substantial delays in commencement, enrollment or completion of our clinical trials and the data from the clinical trials of our product candidates may fail to demonstrate sufficient safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing any 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 and then conduct

extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans. We have not yet completed clinical development of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. 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 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 our INDs and other regulatory filings. We cannot be certain of the timely identification of a product candidate or the completion or outcome of our preclinical testing and studies and cannot predict whether the FDA will accept our proposed clinical programs or whether the outcome of our preclinical testing and studies will ultimately support the further development of any product candidates. 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. As a result, we cannot be sure that we will be able to submit INDs for our preclinical product candidates on the timelines we expect, if at all, and we cannot be sure that submission of INDs will result in the FDA allowing clinical trials to begin in the United States.

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 contract research organizations, or 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 United States, at each clinical trial site;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event 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 cGCPs;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of any product candidates we may develop 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;
- delays in having patients complete participation in a 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; or

•lack of adequate funding to continue the clinical trial.

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 any product candidates, we may need to conduct additional studies or trials 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 any product candidates we may develop or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize any product candidates we may develop and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with any product candidates we may develop, 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 modified risk evaluation and mitigation strategy;

•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 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 to try alternative therapies.

In addition, if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted. For example, in December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a Diversity Action Plan, or DAP, for each phase 3 clinical trial or any other “pivotal study” of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for the DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance, when finalized, will have the force of law as FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance.

On January 27, 2025, in response to an executive order issued by President Trump on January 21, 2025 on diversity, equity and inclusion programs, the FDA removed the draft DAP guidance from its website. That action, along with similar actions by the Trump administration to remove many other healthcare webpages, is currently the subject of ongoing

litigation. On July 3, 2025, the U.S. District Court for the District of Columbia ruled that the administration's actions to remove these webpages, including the draft DAP guidance, is unlawful under the Administrative Procedure Act, or APA. The U.S. District Court for the District of Columbia ordered the restoration of many of these webpages. In late July, 2025, the FDA restored the draft DAP guidance to its website with a statement that "information on this page may be modified and/or removed in the future subject to the terms of the court's order and implemented consistent with applicable law." Accordingly, in light of these ongoing actions, there is considerable uncertainty surrounding the draft DAP guidance and how the FDA will consider diversity action plans in connection with its review of applications for marketing approval.

The regulatory landscape related to clinical trials in the EU has also evolved. The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the Clinical Trials Directive required a separate clinical trial application, or CTA, to be submitted in each member state, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application to all member states concerned. If we are not able to fulfill these new requirements, our ability to conduct clinical trials may be delayed or halted.

Our approach to the discovery and development of product candidates based on our FORCE 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 FORCE platform. Our therapeutics are constructed from three components: a proprietary Fab, a linker and a therapeutic payload that we attach to our Fab using the linker. The Fab is engineered to bind to TfR1 to enable targeted delivery of nucleic acids and other molecules to skeletal, cardiac and smooth muscle.

All of our product candidates are still in varying stages of preclinical and clinical development, and our approach to treating muscle disease is unproven. Our research programs may fail to identify additional product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying additional potential product candidates and our potential product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies. In addition, our 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 product candidates and programs are based on our FORCE platform, adverse developments with respect to one of our product candidates and programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other product candidates and programs.

In addition, we have not completed clinical development of any product candidate or successfully developed any product candidates, and our ability to identify and develop product candidates may never materialize. The process by which we identify and develop product candidates may fail to yield 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 product candidates obsolete or less attractive;
- product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- 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;
- product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a 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.

The outcome of preclinical studies and data from earlier-stage clinical trials may not be predictive of final results or future results of clinical trials or the success of later clinical trials and data from clinical trials in one indication may not be predictive of results of clinical trials in other indications.

We have not completed clinical development of any product candidate. As a result, our belief in the capabilities of our platform, including our belief that we have demonstrated proof of concept of our FORCE platform, is based on early research, preclinical studies and data from clinical trials of our product candidates. However, the results of preclinical studies may not be predictive of the results of later preclinical studies or clinical trials, and the initial results of any clinical trials, such as initial results of ACHIEVE and DELIVER that we have reported, may not be predictive of the final results of those trials or the results of any later clinical trials, and may also not be predictive of results of clinical trials in other indications. 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. Further, certain of our hypotheses regarding the potential benefits of our product candidates compared to alternative therapies and treatments are based on cross-trial comparisons of results that were not derived from head-to-head clinical trials. Such clinical trial data may not be directly comparable due to differences in study protocols, conditions and patient populations. Accordingly, these cross-trial comparisons may not be reliable predictors of the relative efficacy or other benefits of our product candidates compared to other product candidates that may have been approved previously.

Our clinical trials may not ultimately be successful or support further clinical development of any product candidates we may develop. 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.

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 any product candidates we may develop 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;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

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

to obtain additional financing. Furthermore, we 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.

If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent us from conducting clinical trials or seeking or obtaining regulatory approval, limit the commercial potential of our product candidates or result in significant negative consequences to the extent such effects or adverse properties are observed following any marketing approval.

We have not completed clinical development of any product candidate. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. There can be no assurance that our technologies will not cause undesirable side effects.

Although other oligonucleotide therapeutics have received regulatory approval, our approach for our DM1, DMD and FSHD programs, which combine oligonucleotides with a Fab, is a novel approach to oligonucleotide therapy. As a result, there is uncertainty as to the safety profile of product candidates we may develop 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 conjugated oligonucleotide therapeutics.

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. 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.

If in the future we are unable to demonstrate that such side effects were caused by factors other than our product candidates, the FDA, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates for any or all targeted indications. Even if we are able to demonstrate that any future serious adverse events are not product-related and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

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 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.

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 our product candidates in clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no product candidates that have been approved for commercial sale, the 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 will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates we may develop;
- 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 any product candidates we may develop.

We have increased our insurance coverage in countries in which we plan to conduct clinical trials and will need to increase our insurance coverage if we conduct clinical trials in additional countries or of additional product candidates 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.

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, or our 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, or our 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 contract manufacturing organizations, or CMOs, for the manufacturing of any product candidates we test in preclinical or clinical development, as well as CROs for portions of our animal testing, preclinical research and for the conduct of our 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, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND/CTA-enabling studies and clinical trials are conducted in accordance with the study plan and protocols. Moreover, the FDA requires us to comply with cGCPs 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 cGCPs, 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 cGCP regulations. In addition, our clinical trials must be conducted with product produced under conditions that comply with the FDA's current Good

Manufacturing Practices. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

Although we design the clinical trials for our product candidates, CROs conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct preclinical studies and 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 any product candidates we may develop 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 submissions and approval of any product candidates we may develop.

We currently depend on a small number of third-party suppliers for the manufacture of our Fab, the linkers and payloads. 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. In the future, we may seek to establish our own manufacturing facility for the long-term commercial supply of any product candidates we may develop and which receive regulatory approval. We rely on a small number of third-party suppliers for the manufacture of our Fab, linkers and payloads. We expect to continue to depend on third-party suppliers for the manufacture of any product candidates that we evaluate in preclinical studies and clinical trials, 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 and any comparable foreign regulatory authority pursuant to inspections that will be conducted after we submit a biologics license application, or BLA, to the FDA or any comparable filing to a 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 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 or any comparable foreign regulatory authority, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities.

Our product candidates consist of a proprietary Fab conjugated with a linker to a payload. Our Fab is manufactured by starting with cells which are stored in a cell bank. If we lose multiple cell banks, our manufacturing will be adversely impacted by the need to replace the cell banks.

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 or any comparable foreign regulatory authority does not approve these facilities for the manufacture of any product candidates we may develop 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 any product candidates we may develop, 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. In addition, certain Chinese biotechnology companies and CMOs that supply us with drug components may become subject to trade restrictions, sanctions, and other regulatory requirements by the U.S. government, which could restrict or even prohibit our ability to work with such entities, thereby potentially disrupting the supply of material to us. Such disruption could have adverse effects on the development of our product candidates and our business operations.

We may also seek to eventually establish our own manufacturing facility for the long-term commercial supply of any product candidates we may develop and which receive regulatory approval. 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 preclinical studies or clinical trials of product candidates;
- delays 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 may compete with third parties for access to manufacturing facilities. 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 through a BLA supplement which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. 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.

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 the product candidates we may develop.

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

We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. 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 the product candidates we may develop. 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 any product candidates we may develop 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 any product candidates we may develop 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 any product candidates we may develop 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 any product candidates we may develop 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 applicable product candidates we may develop; 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 "Risk Factors" section 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. 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 the product candidates we may develop 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 research programs and product candidates and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, 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, and the negotiation process is time-consuming and complex. 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 similar regulatory authorities outside the United States, 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 IT 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 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 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 any product candidates that we may develop 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 and development 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 any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

There are currently no approved therapies to treat the underlying cause of DM1. Product candidates currently in clinical development to treat DM1 include: SAR446268, a microRNA targeting DMPK via an adeno-associated virus, being evaluated in a Phase 1/2 study of non-congenital DM1 patients initiated in July 2025 by Sanofi S.A.; tideglusib, a GSK3-ß inhibitor in late-stage clinical development by AMO Pharma Ltd. for children and adults with DM1; pitolisant, a selective histamine 3 receptor antagonist / inverse agonist being evaluated in a Phase 2 clinical trial for non-muscular symptoms of DM1 by Harmony Biosciences Holdings, Inc.; delpacibart etedesiran (formerly AOC-1001), an antibody linked siRNA being evaluated in a Phase 3 clinical trial by Avidity Biosciences, Inc., or Avidity; PGN-EDODM1, a peptide-linked PMO currently being evaluated in a Phase 1 clinical trial by Pepgen, Inc.; SRP-1003 (ARO-DM1), a peptide-linked siRNA being evaluated in a Phase 1/2a clinical trial in Australia and New Zealand by Arrowhead Pharmaceuticals, Inc. in collaboration with Sarepta Therapeutics, Inc., or Sarepta; ATX-01, a lipophilic peptide conjugated anti-miR designed to target microRNA 23b currently being evaluated in a Phase 1/2 clinical trial by ARTHEx Biotech S.L.; and VX-670, an endosomal escape vehicle technology with a CUG steric blocker oligonucleotide by Entrada Therapeutics, Inc. in collaboration with Vertex being evaluated in a Phase 1/2 clinical trial in Canada, the United Kingdom, the European Union and Australia.

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. A novel steroid, AGAMREE (vamorolone) is also approved by the FDA for treatment of DMD in patients 2 years of age and older and is marketed by Catalyst Pharmaceuticals, Inc. DUVYZAT (givinostat), an HDAC inhibitor, received FDA approval for treatment of DMD in patients 6 years of age and older and is marketed in the U.S. by ITF Therapeutics, LLC. In addition, there are four FDA-approved exon skipping drugs: EXONDYS 51 (eteplirsen), VYONDYS 53 (golodirsen) and AMONDYS 45 (casimersen), which are naked PMOs approved for the treatment of DMD patients amenable to exon 51, exon 53 and exon 45 skipping, respectively, and are marketed by Sarepta, and VILTEPSO (vitolarsen), a naked PMO approved for the treatment of DMD patients amenable to exon 53 skipping, which is marketed by Nippon Shinyaku Co. Ltd. Additionally, there is one FDA-approved gene therapy for patients with a confirmed mutation in the dystrophin gene, ELEVIDYS (delandistrogene moxeparvovec-rokl), which is marketed by Sarepta. Companies focused on developing treatments for DMD that target dystrophin mechanisms, as does our DMD program, include Wave Life Sciences Ltd. with WVE-N531, a stereopure oligonucleotide being evaluated in a Phase 2 clinical trial for patients amenable to exon 53 skipping; SQY Therapeutics, S.A. with SQT51,a palmitoyl-conjugated tc-DNA antisense oligonucleotide currently being evaluated in a Phase 1/2a clinical trial; Entrada Therapeutics, Inc. with ENTR-601-44, an endosomal escape vehicle technology for the treatment of DMD patients amenable to exon 44 skipping currently being evaluated in a Phase 1 clinical trial; BioMarin Pharmaceuticals, Inc. with BMN 351, an oligonucleotide therapy that targets dystrophin production which is being evaluated in a Phase 1/2 clinical trial and Avidity with AOC 1044, an antibody oligonucleotide conjugate that targets dystrophin production for patients amenable to exon 44 skipping being evaluated in a Phase 1/2 clinical trial. In addition, several companies are developing gene therapies to treat DMD, including Sarepta (SRP-9001 and Galgt2 gene therapy program), Solid Biosciences Inc. (SGT-003), REGENXBIO Inc. (RGX-202), Genethon (GNT-0004), and Insmed (INS1201). Gene editing treatments that are in preclinical development are also being pursued by Vertex, Sarepta and GenAssist Ltd. We are also aware of several companies targeting non-dystrophin mechanisms for the treatment of DMD.

There are currently no therapies approved to treat FSHD. Products currently in development for FSHD include: ARO-DUX4, an siRNA therapy being evaluated in a Phase 1/2 clinical trial and licensed by Arrowhead Pharmaceuticals, Inc. to Sarepta; delpacibart braxlosiran (formerly AOC-1020), an antibody oligonucleotide conjugate being evaluated in a Phase 1/2 clinical trial by Avidity and RO7204239, an anti-latent myostatin antibody by Roche Pharmaceuticals that is in a Phase 2 clinical trial. Additionally, satralizumab, an anti-IL-6 antibody, is being evaluated in a Phase 1 clinical trial by the University Hospital of Nice and clenbuterol, a beta (2) agonist, is being evaluated in a Phase 2 clinical trial by Springbok Analytics, Inc. Epicrispr Biotechnologies, Inc. recently announced that the FDA has cleared an IND application for EPI-321, an epigenetic therapy for the treatment of FSHD and plans to initiate a global Phase 1/2 clinical trial in 2025.

There are three currently approved medicines for Pompe disease, all of which are enzyme replacement therapies: Myozyme/Lumizyme (alglucosidase alfa) and Nexviazyme/Nexviadyme (avalglucosidase alfa) by Sanofi, and Pombiliti + Opfolda (cipaglucosidase alfa-atga in combination with miglustat) by Amicus Therapeutics, Inc. Beyond these marketed products, the Pompe clinical pipeline consists of early-stage product candidates that aim to address Pompe disease via alternative strategies. ACTUS-101, a gene therapy delivered to the liver for continuous, endogenous production of GAA currently being evaluated in a Phase 1/2 clinical trial by AskBio, Inc. and AT-845, a muscle-targeted gene therapy currently being evaluated in a Phase 1/2 clinical trial Astellas Pharma US, Inc. ABX-1100 by ARO Biotherapeutics Co. and MZE-001 by Maze Therapeutics, Inc. are substrate reduction therapies in Phase 1 clinical trials.

We also expect to compete more generally with other companies developing alternative scientific and technological approaches to the treatment of muscle diseases, including other companies working to develop conjugates with oligonucleotides for extra-hepatic delivery, including Alnylam Pharmaceuticals, Inc., Aro Biotherapeutics, Inc., Arrowhead Pharmaceuticals, Inc., Avidity, Denali Therapeutics, Inc., Novo Nordisk A/S, DTx Pharma, Inc., Gennao Bio, Inc., Ionis Pharmaceuticals, Inc. 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 products we may develop. Competitive products or technological approaches may make any products we develop, or our FORCE 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 we may develop, if approved, could be adversely affected.

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

If any product candidate we may develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payers 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 payers on the benefits of any product candidates we may develop may require significant resources and may not be successful. If any product candidates we may develop 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 any product candidates we may develop, 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 any product candidates we may develop, 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 there is currently no standard of care treatment directed at some of our target indications, such as FSHD. As a result, the pricing and reimbursement of any product candidates we may develop, 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 pricing, insurance coverage and reimbursement status of newly approved products is 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.

The initial target platforms in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payers, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payers will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payers. 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.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payers tend to follow CMS to a substantial degree. However, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage for the drug product. Further, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the European Union may be more conservative than CMS.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as any product candidates we may develop. In many countries, particularly the countries of the European Union, 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 United States. 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 United States, the reimbursement for any product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payers, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for any product candidates we may develop. We expect to experience pricing pressures in connection with the sale of any product candidates we may develop due to the trend toward managed healthcare, the increasing influence of certain third-party payers, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. There have been instances in which third-party payers have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payers, professional organizations such as the American Medical Association can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payers contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

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 any product candidates we may develop if and when they 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 some of the product candidates we may develop if and when they are approved. 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 payers;
- 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 any product candidates we may develop 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 any product candidates we may develop.

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

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Amendment, or the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. In December 2022, Congress clarified through FDORA that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA

approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product.

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

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 any product candidates we may develop 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 United States and other jurisdictions. We currently own and license patents and patent applications relating to our FORCE platform technology, including our Fabs, payloads and Fab-payload conjugates, as well as aspects of our manufacturing and methods of treatment. We and our licensors have sought, and will seek, to protect our proprietary position by filing additional patent applications in the United States and abroad related to certain technologies and our platform that are important to our business. However, while much of our patent portfolio is at an early stage, we own forty-nine issued U.S. patents and eleven granted foreign patents, and exclusively license three issued U.S. patents and one issued European patent. Moreover, 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 any product candidates we may develop 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 any product candidates we may develop and 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 selected 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 any product candidates we may develop 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 FORCE 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 any product candidates we may develop.

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 United States 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 United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know 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 United States and other jurisdictions. For example, we may be subject to a third-party submission of prior art to the United States 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 any product candidates we may develop 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 any product candidates we may develop. 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 United States, 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 any product candidates we may develop. 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 are party to a license from the University of Mons, or UMONS, to certain patent rights and know-how of UMONS. Our license agreement with UMONS imposes, and we expect that any future license agreement will impose, specified diligence, milestone 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 any product candidates we may develop 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, our license agreement with UMONS 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 current 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.

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 any product candidates we may develop in the future.

Moreover, some of our in-licensed patent and other intellectual property rights 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. 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. In addition, the U.S. government may have certain rights in such patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.

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 any product candidates we may develop in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the United States could be less extensive than those in the United States. 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 United States. 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 United States. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering any product candidates we may develop and our technology in all jurisdictions outside the United States and, as a result, may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. 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 United States. These products may compete with any product candidates we may develop 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 United States 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.

As another example, the complexity and uncertainty of European patent laws have also increased in recent years. In Europe, a new unitary patent system went into effect on June 1, 2023, which significantly impacts European patents, including those granted before the introduction of such system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court (UPC). Existing European patents and published applications may be opted out of the jurisdiction of the UPC at any time before the end of a transitional period (at least seven years from the UPC Agreement which went into effect on June 1, 2023), unless an action has already been brought before the UPC in which case an opt-out request cannot be filed. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. Patents granted before the implementation of the UPC will have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

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.

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 United States 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 product candidates we may develop through acquisitions and in-licenses.

We currently have rights to certain intellectual property through licenses from third parties. Because our product candidates 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 any product candidates we may develop 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 any product candidates we may develop 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 any product candidates we may develop could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States 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 any of the product candidates we may develop 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 United States, 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 United States 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 any product candidates we may develop or our technology or prevent third parties from competing with any product candidates we may develop 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 United States 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. In such circumstances, third parties could potentially use our trade secrets to compete with any product candidates we may develop 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 any product candidates we may develop 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 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 any product candidates we may develop, 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 any product candidates we may develop 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 United States and other jurisdictions owned by third parties that claim subject matter that relates to our program candidates and the FORCE platform. Although we believe that these patents are invalid and/or not infringed, such third parties may assert these patents against us in litigation in the United States 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 any product candidates we may develop 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 United States 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 any product candidates we may develop 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, any product candidates we may develop or the use of any product candidates we may develop.

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 any product candidates we may develop or any other of 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 any product candidates we may develop 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 any product candidates we may develop 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 any product candidates we may develop or at all. 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 any product candidates we may develop 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 any product candidates we may develop. 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 and data exclusivity for any product candidates we may develop, our business may be harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop 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 the Hatch-Waxman Act. The Hatch-Waxman Act permits 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 any product candidates we may develop 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.

We have filed trademark applications with the USPTO for our corporate name, logos and tagline and have filed trademark applications in foreign jurisdictions. Our current and future trademark applications in the United States 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 any product candidates we may develop 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 drug product and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our product engine and pipeline, we must, 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, collaborative research agreements, consulting agreements and other similar agreements with our collaborators, advisors, employees, consultants and contractors prior to beginning research or disclosing any proprietary information. 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. 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 regulatory approval and other regulatory and legal compliance matters

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 any product candidates we may develop. 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, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop 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 United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate we may develop 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 filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs 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, purity and potency. 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. Any product candidates we may develop 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 United States 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 or foreign regulatory approval processes and are commercialized. Even if any product candidates we may develop 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 and comparable 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. If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Further, under the Pediatric Research Equity Act, or PREA, a new drug application, or NDA, a BLA or supplement to an NDA or BLA for certain drugs and biological products must contain data to assess the safety and effectiveness of the drug or biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the EU also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the EMA, or to obtain a waiver or deferral from the conduct of these studies by the Pediatric Committee of the EMA. For any of our product candidates for which we seek regulatory approval in the United States or the EU, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

Moreover, principal investigators for our future clinical trials may serve as scientific advisors or consultants to us and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or a comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or 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 one or more of our product candidates.

Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for any product candidates, the FDA or the comparable foreign regulatory authority may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-market clinical trials. The FDA or the comparable 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 or comparable 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.

In addition, we could be adversely affected by several significant administrative law cases decided by the U.S. Supreme Court in 2024. In *Loper Bright Enterprises v. Raimondo*, for example, the court overruled *Chevron U.S.A., Inc. v. Natural Resources Defense Council, Inc.*, which for 40 years required federal courts to defer to permissible agency interpretations of statutes that are silent or ambiguous on a particular topic. The U.S. Supreme Court stripped federal agencies of this presumptive deference and held that courts must exercise their independent judgment when deciding whether an agency, such as the FDA, acted within its statutory authority under the APA. Additionally, in *Corner Post, Inc. v. Board of Governors of the Federal Reserve System*, the U.S. Supreme Court held that actions to challenge a federal regulation under the APA can be initiated within six years of the date of injury to the plaintiff, rather than the date the rule is finalized. The decision appears to give prospective plaintiffs a personal statute of limitations to challenge longstanding agency regulations. Another decision, *Securities and Exchange Commission v. Jarkesy*, overturned regulatory agencies' ability to impose civil penalties in administrative proceedings. These decisions could introduce additional uncertainty into the regulatory process and may result in additional legal challenges to actions taken by federal regulatory agencies, including the FDA and CMS, that we rely on. In addition to potential changes to regulations as a result of legal challenges, these decisions may result in increased regulatory uncertainty and delays and other impacts, any of which could adversely impact our business and operations.

In addition, our ability to develop and market new drug products may be impacted by litigation challenging the FDA's approval of mifepristone. In April 2023, the U.S. District Court for the Northern District of Texas invalidated the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various measures adopted under a Risk Evaluation and Mitigation Strategy.

On appeal, the U.S. Court of Appeals for the Fifth Circuit declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the U.S. Court of Appeals for the Fifth Circuit did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious and in violation of federal law. In June 2024, the U.S. Supreme Court reversed and remanded that decision after unanimously finding that the plaintiffs did not have standing to bring this legal action against the FDA. On October 11, 2024, the Attorneys General of three states filed an amended complaint in the district court in Texas challenging the FDA's actions. On January 16, 2025, the district court in Texas agreed to allow these states to file an amended complaint and continue to pursue this challenge. Thereafter, on September 30, 2025, the district court declined to dismiss the case and, instead, transferred it to federal district court in the Eastern District of Missouri. Depending on the outcome of this litigation, if it continues, our ability to develop new drug product candidates and to maintain approval of any then-existing drug products could be at risk and could be delayed, undermined or subject to protracted litigation.

Finally, with the change in presidential administrations in 2025, there is substantial uncertainty as to how the new administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates. This uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates.

We, or any future collaborator, may seek approval from the FDA or comparable foreign regulatory authorities to use accelerated development pathways for our product candidates. If we, or any future collaborator, are not able to use such pathways, we, or they, may be required to conduct additional clinical trials beyond those that are contemplated, which would increase the expense of obtaining, and delay the receipt of, necessary marketing approvals, if we, or they, receive them at all. In addition, even if an accelerated approval pathway is available to us, or any future collaborator, it may not lead to expedited approval of our product candidates, or approval at all.

We are currently pursuing accelerated development pathways for our product candidates for DM1 and DMD. Under the Federal Food, Drug and Cosmetic Act, or FDCA, and implementing regulations, the FDA may grant accelerated approval to a product candidate to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over

available therapies, upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. Similar risks to those described above are also applicable to any application that we, or any future collaborator, may submit in jurisdictions outside of the United States. Prior to seeking such accelerated approval, we, or any future collaborator, may continue to seek feedback from the FDA or comparable foreign regulatory agencies and otherwise evaluate our, or their, ability to seek and receive such accelerated approval.

There can be no assurance that the FDA or foreign regulatory agencies will agree with our, or any future collaborators', surrogate endpoints or intermediate clinical endpoints in any of our, or their, clinical trials, or that we, or future collaborator, will decide to pursue or submit any additional BLAs for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that, after feedback from the FDA or comparable foreign regulatory agencies, we, or any future collaborator, will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval. Furthermore, for any submission of an application for accelerated approval or application under another expedited regulatory designation, there can be no assurance that such submission or application will be accepted for filing or that any expedited development, review or approval will be granted on a timely basis, or at all.

Finally, there can be no assurance that we will satisfy all FDA requirements, including new provisions, that govern accelerated approval. For example, with passage of the FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded and to submit progress reports on its post-approval studies to FDA every six months until the study is completed. Moreover, FDORA established expedited procedures authorizing FDA to withdraw an accelerated approval if certain conditions are met, including where a required confirmatory study fails to verify and describe the predicted clinical benefit or where evidence demonstrates the product is not shown to be safe or effective under the conditions of use. The FDA may also use such procedures to withdraw an accelerated approval if a sponsor fails to conduct any required post-approval study of the product with due diligence, including with respect to "conditions specified by the Secretary." The new procedures include the provision of due notice and an explanation for a proposed withdrawal, and opportunities for a meeting with the Commissioner or the Commissioner's designee and a written appeal, among other things. We will need to fully comply with these and other requirements in connection with the development and approval of any product candidate that qualifies for accelerated approval.

In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval. The FDA indicated that the accelerated approval pathway is commonly used for approval of oncology drugs due to the serious and life-threatening nature of cancer. Although single-arm trials have been commonly used to support accelerated approval, a randomized controlled trial is the preferred approach as it provides a more robust efficacy and safety assessment and allows for direct comparisons to an available therapy. To that end, the FDA outlined considerations for designing, conducting, and analyzing data for trials intended to support accelerated approvals of oncology therapeutics. Subsequently, in December 2024 and January 2025, the FDA issued additional draft guidances relating to accelerated approval. While these guidances are currently only in draft form and will ultimately not be legally binding even when finalized, we will need to observe the FDA's guidances closely to ensure that our products qualify for accelerated approval.

Accordingly, a failure to obtain and maintain accelerated approval or any other form of expedited development, review or approval for our product candidates, or withdrawal of a product candidate, would result in a longer time period until commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

Obtaining and maintaining marketing approval or commercialization of our product candidates in the United States 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 any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union 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 United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, 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 United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Further, we could face heightened risks with respect to obtaining marketing authorization in the United Kingdom as a result of the withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit.

The United Kingdom is no longer part of the European Single Market and EU Customs Union. As of January 1, 2025, the Medicines and Healthcare Products Regulatory Agency, or the MHRA, is responsible for approving all medicinal products destined for the United Kingdom market (i.e., Great Britain and Northern Ireland). On April 28, 2025, the United Kingdom Parliament adopted amendments to improve and strengthen the United Kingdom's clinical trials regulatory regime; they will take effect on April 28, 2026. These changes were needed since the current United Kingdom requirements are based upon the now-repealed EU Clinical Trials Directive (2001/20/EC), which has been replaced by the European Clinical Trials Regulation (Regulation EU No 536/2014). In anticipation of these new requirements, on October 1, 2025, the MHRA updated its guidance for clinical trials to address, among other things, research transparency requirements for clinical trials, the approvals process, Research Ethics Committee review of clinical trials, simplified arrangements for consent in clinical trials and pharmacovigilance. Since the United Kingdom left the European Union prior to the date on which the European Clinical Trials Regulation took effect, the United Kingdom's legal framework did not benefit from the same revisions as occurred in the European Union.

At the same time, a new international recognition procedure, or IRP, will apply, which intends to facilitate approval of pharmaceutical products in the UK. The IRP is open to applicants that have already received an authorization for the same product from one of the MHRA's specified Reference Regulators, or RRs. The RRs notably include EMA and regulators in the EU/European Economic Area member states for approvals in the EU centralized procedure and mutual recognition procedure as well as the FDA (for product approvals granted in the U.S.). However, the concrete functioning of the IRP is currently unclear. Any delay in obtaining, or an inability to obtain, any marketing approvals may force us or our collaborators to restrict or delay efforts to seek regulatory approval in the United Kingdom for our product candidates, which could significantly and materially harm our business.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products, which may reduce the duration of regulatory data protection and exclusivity periods for orphan drugs, and revise the eligibility for expedited pathways in addition to other changes, was published in April 2023 and the European Parliament has requested several amendments. In June 2025, after almost two years of negotiations among the European Union Member States, the Council of the European Union adopted its position on the proposed overhaul of the European Union general pharmaceutical legislative framework, which is known as the new Pharma Package. This proposal will now be the subject of additional negotiations and technical meetings, with the objective of reaching agreement on issues such as the regulatory data protection framework and the access and supply obligations. At this point, it appears that the period of market exclusivity for innovator products may be reduced from two years to one, exclusions from patent infringement for studies and trials will likely expand, and there will be a new obligation to ensure sufficient supply of medicines. The revisions may have a significant impact on the pharmaceutical industry and our business in the long term.

Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of the Trade and Cooperation Agreement or otherwise, would prevent us from commercializing any product candidates in the United Kingdom and/or the

European Union 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 United Kingdom and/or the European Union for any product candidates we may develop, which could significantly and materially harm our business.

We are conducting and intend to conduct certain of our clinical trials globally. However, the FDA and other foreign equivalents may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We are conducting and intend to continue conducting certain of our clinical trials globally. The acceptance by the FDA or other regulatory authorities of study data from clinical trials conducted outside their jurisdiction may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to cGCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the clinical trial is well-designed and well-conducted in accordance with cGCP requirements and the FDA is able to validate the data from the trial through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

Conducting clinical trials outside the United States 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; diminished protection of intellectual property in some countries; and interruptions or delays in our trials resulting from geopolitical events, such as war or terrorism.

Fast Track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical need for this condition, we may apply for FDA Fast Track designation. In October 2022, the FDA granted Fast Track designation for z-rostudirsen, and in January 2025, the FDA granted Fast Track designation for z-basivarsen. However, a Fast Track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive Fast Track designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

Breakthrough or RMAT therapy designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development, the sponsor may apply for FDA breakthrough designation or a regenerative medicine advanced therapy, or RMAT, designation. In June 2025, the FDA granted Breakthrough Therapy Designation to z-basivarsen for the treatment of DM1. Additionally, in August 2025, the FDA granted Breakthrough Therapy Designation to z-rostudirsen for the treatment of DMD, amenable to exon 51 skipping. However, neither a breakthrough designation nor an RMAT designation ensures that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive breakthrough or RMAT designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw breakthrough or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Neither breakthrough nor RMAT designation alone guarantees qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If the FDA determines that a product candidate we may develop offers major advances in treatment or provides a treatment where no adequate therapy exists, 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 any product candidates we may develop. 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 we may develop is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review 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 thereafter. On October 16, 2025, the FDA announced nine voucher recipients under the CNPV Program.

On June 17, 2025, the FDA announced the creation of a new voucher program to expedite the development and approval of new drug products. Vouchers issued under the new program, which is known as the Commissioner's National Priority Voucher, or CNPV, Program, may reportedly be redeemed by sponsors to shorten the review time of a BLA from approximately 10 to 12 months to 1 to 2 months. The FDA has indicated that the new CNPV process will convene experts from the FDA's offices for a team-based review rather than using the standard review system of a drug application being sent to numerous FDA offices. Clinical information will be reviewed by a multidisciplinary team of physicians and scientists who will pre-review the submitted information and convene for a 1-day meeting. Vouchers under this program will reportedly be given to companies aligned with U.S. national priorities.

We may not be able to obtain orphan drug exclusivity for product candidates we may develop, and even if we do, that exclusivity may not prevent regulatory authorities from approving other competing products.

In March 2023 and April 2025, respectively, the EMA and FDA granted orphan drug designation to z-rostudirsen for the treatment of DMD in patients amenable to exon 51 skipping. In May and September 2023, respectively, the EMA and FDA granted orphan drug designation to z-basivarsen for the treatment of DM1. 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. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, 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 or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. 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 particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two genetic medicine products to be different drugs solely based on minor differences in the transgenes or vectors. 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 or EMA 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.

In 2017, the U.S. Congress passed the FDA Reauthorization Act of 2017, or the FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority.

The FDA and the U.S. Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit (*Catalyst Pharms., Inc. v. Becerra)* in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, the FDA announced that, in matters beyond the scope of that order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. In February 2025, a federal district court in Washington, D.C. fully embraced the reasoning in the 11th Circuit decision in another court decision (*Neurelis v. Brenner*) challenging the scope of orphan drug exclusivity. On April 17, 2025, the FDA appealed this decision to the U.S. Court of Appeals for the D.C. Circuit. The implications of this decision, and its impact on the FDA's implementation of the Orphan Drug Act, are unclear at this point.

Even if we, or any collaborators we may have, obtain marketing approvals for any product candidates we may develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, if ever, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA typically advises that patients treated with genetic medicine undergo follow-up observations for potential adverse events for a 15-year period. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine. Similar restrictions apply to the approval of products in the European Union.

Accordingly, assuming we, or any third parties we may collaborate with, receive marketing approval for one or more product candidates we may develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

If we fail to comply with applicable regulatory requirements following approval of any product candidates we may develop, a regulatory agency may:

- •issue a warning letter asserting that we are in violation of the law;
- •seek an injunction or impose civil or criminal penalties or monetary fines;
- •suspend or withdraw regulatory approval;
- •suspend any ongoing clinical trials;
- •refuse to approve a pending BLA or supplements to a BLA submitted by us;
- •seize product; or
- •refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we may develop and generate revenues.

Any product candidate we may develop 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 and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other regulatory agencies 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 or such other regulatory agencies as reflected in the product's approved labeling. If we do not market our medicines for their approved indications, 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, or DOJ. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws 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 FDA has also requested that companies enter into consent decrees or 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.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific communications concerning their products in certain circumstances. For example, in January 2025, the FDA published final guidance outlining the agency's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This final guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. If a company engages in such communications consistent with the guidance's recommendations, the FDA indicated that it will not treat such communications as evidence of unlawful promotion of a new intended use for the approved product. In addition, under some recent guidance from the FDA and the Pre-Approval Information Exchange Act, signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices. We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products.

Further, on September 9, 2025, the President issued a Memorandum directing the Department of Health and Human Services, or HHS, to "ensure transparency and accuracy in direct-to-consumer prescription drug advertising, including by increasing the amount of information regarding any risks associated with the use of any such prescription drug required to

be provided in prescription drug advertisements." The same day, the FDA declared that it will no longer tolerate what it characterized as "deceptive practices" in prescription drug advertising and that the agency would "aggressively deploy" its available enforcement tools, with "heightened scrutiny" of fair balance and disclosures in social media promotions. The FDA issued a generic "notice letter" to a substantial number of companies directing such companies to "remove any noncompliant advertising and bring all promotional communications into compliance." When and if our products are approved, we will need to maintain a robust compliance program and processes designed to ensure that all such advertising activities are performed in a legal and compliant manner.

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 product candidates we may develop receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy 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. 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 manufacturer must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's cGLP and 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 any product candidates we may develop or any of our other potential products. 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 our 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. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States.

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.

Our relationships with healthcare providers, physicians and third-party payers 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 payers 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 payers 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 the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the federal False Claims Act, and civil monetary penalty laws which can be enforced through civil whistleblower or qui tam actions, imposes civil and criminal penalties against individuals or entities for knowingly presenting or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid or other government payers that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which prohibits, among other things, knowingly and willfully executing, or attempting to execute, a scheme or artifice to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private), and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as further amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, which imposes certain requirements, including mandatory contractual terms, on covered entities subject

to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers, as well as their respective business associates and their subcontractors that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security, and transmission of such individually identifiable health information;

•the federal transparency requirements under the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report to the HHS information related to payments and other transfers of value to physicians, as defined by such law, other healthcare providers and teaching hospitals and ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations; and

•analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, and certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to drug pricing and payments to physicians and other healthcare providers or marketing expenditures and state and local laws that require the registration of 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 European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States and the United Kingdom, such as the UK Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union 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 European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations 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 these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Legislative and regulatory changes may increase the difficulty and cost for us and any future collaborators to obtain reimbursement for our product candidates, if and when approved.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of any product candidates we may develop, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on

average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payers.

The ACA, which became law in 2010, contains provisions of importance to our business. Our ability to commercialize and the prices we may obtain for any product candidates we may develop and that are approved for sale, may be affected by these provisions, including without limitation, the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which will remain in effect through 2031. However, as a result of other legislation, the actual reductions in Medicare payments may vary up to 4%. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous executive and legal challenges and U.S. congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Act, the U.S. Congress effectively repealed the "individual mandate" by reducing the applicable penalty to zero dollars. The modification of this provision, which required most Americans to carry a minimal level of health insurance, became effective in 2019. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

We expect that these healthcare reforms, 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 additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our products, if and when approved.

The prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. There have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation

designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid.

In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. A number of states have submitted Section 804 Importation Program proposals to the FDA with the goal of obtaining authority to import drugs from Canada, subject to conditions. On May 21, 2025, the FDA announced that it would offer individual states the opportunity to submit a draft proposal for pre-review and meet with the FDA to obtain initial feedback from the FDA prior to formally submitting their Section 804 importation program proposal. The intent of these meetings is to assist states in developing their proposals by further clarifying requirements, enhancing the quality of proposals submitted to the FDA and ultimately shortening the review timeline.

On August 16, 2022, the Inflation Reduction Act, or the IRA, was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, the U.S. Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it did not originally apply to drugs and biologics that have been approved for a single rare disease or condition. With passage of the OBBBA, on July 3, 2025, which was signed into law on July 4, 2025, Congress extended this exemption to drugs and biologics with multiple orphan drug designations. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products are the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on our drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years. The first cycle of negotiations for the Medicare Drug Price Negotiation Program commenced in the summer of 2023. On August 15, 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions, including diabetes, chronic kidney disease, and rheumatoid arthritis. The prices of these ten drugs will become effective January 1, 2026. On January 17, 2025, CMS announced its selection of 15 additional drugs covered by Part D for the second cycle of negotiations. While there had been some questions about the Trump administration's position on this program, CMS issued a public statement on January 29, 2025, declaring that lowering the cost of prescription drugs is a top priority of the new administration and CMS is committed to considering opportunities to bring greater transparency in the negotiation program. This second cycle of negotiations with participating drug companies will occur during 2025, and any negotiated prices for this second set of drugs will be effective starting January 1, 2027.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates if they raise prices for certain Part B and Part D drugs faster than the rate of inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year. In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

On June 6, 2023, Merck & Co., Inc., filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the U.S. Constitution. Subsequently, other parties, including the U.S. Chamber of Commerce, or Chamber of Commerce, Bristol Myers Squibb Company, the PhRMA, Astellas Pharma US, Inc., Novo Nordisk Inc., Janssen Pharmaceuticals, Inc., Novartis Pharmaceutical Corporation, AstraZeneca L.P. and Boehringer Ingelheim Pharmaceuticals, Inc. also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. HHS has generally won the substantive disputes in these cases or succeeded in getting claims dismissed for lack of standing. Most of these cases are now on appeal. On October 30, 2024, the U.S. Court of Appeals for the Third Circuit heard oral arguments in three of these cases. In April 2025, the U.S. Court of Appeals for the Second Circuit and the U.S. Court of Appeals for the Third Circuit heard arguments in an additional three cases. On May 8, 2025, the U.S. Court of Appeals for the Third Circuit rejected AstraZeneca L.P.'s challenge to the Medicare price negotiation program, finding that the program did not violate the company's due process rights under the Constitution since there is no protected property interest in selling goods to Medicare beneficiaries at a price higher than what the government is willing to pay in reimbursement.

We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results. Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

In April 2025, President Trump issued an executive order which directs HHS to take steps to reduce the prices of pharmaceutical products. The new executive order repeats many of the proposals advanced during the first Trump administration, including directing the FDA to streamline and improve its existing drug importation program so as to make it easier for states to obtain approval without sacrificing the safety or quality of drug products. Other provisions of the executive order relate to the 340B program. Specifically, one provision calls on the Secretary of HHS to determine the hospital acquisition cost for covered outpatient drugs at hospital outpatient departments and to consider and propose any appropriate adjustments for Medicare payment. The other provisions direct HHS to condition grant funding to certain health centers on those centers passing through the 340B discounts they receive on insulin and injectable epinephrine products to patients who meet certain requirements. With respect to the IRA's Medicare drug pricing program, the executive order, among other things, calls for alignment in "the treatment of small molecule prescription drugs with that of biological products, ending the distortion that undermines relative investment in small molecule prescription drugs, coupled with other reforms to prevent any increase in overall costs to Medicare and its beneficiaries."

Further, on May 12, 2025, President Trump issued an additional executive order calling on pharmaceutical manufacturers to voluntarily reduce the prices of medicines in the United States. The executive order directs the Secretary of HHS to communicate most-favored-nation, or MFN, price targets to pharmaceutical manufacturers to bring prices in line with comparably developed nations. The executive order further provides that if such actions do not lower the costs of pharmaceuticals, the Secretary of HHS would pursue other actions, including proposing a rulemaking that imposes MFN pricing in the United States. Subsequently, on May 20, 2025, HHS indicated that the proposed MFN pricing will apply only to brand products without generic or biosimilar competition and the referenced foreign countries will include only those in which the branded product similarly does not have generic or biosimilar competition. Second, HHS indicated that the MFN target price will be the lowest price in a country that is a member of the Organization for Economic Co-operation and Development, or OECD, with a gross domestic product, or GDP, per capita of at least 60% of the U.S. GDP per capita. Based on previous estimates, there are likely at least 22 OECD countries that would satisfy this criterion. The implications of these actions remain unclear and are likely to result in litigation if the administration pursues an MFN regulatory pricing requirement.

On July 31, 2025, the President issued letters to 17 pharmaceutical companies reiterating the requirements of the May 12, 2025, executive order and demanding that such companies extend MFN pricing to Medicaid patients, guarantee MFN pricing for newly-launched drug products, return increased revenues abroad to American patients and provide for direct purchasing at MFN pricing. The letters also urged these companies to stipulate that they will not offer other developed nations better prices for new drugs than the prices offered for such products in the U.S. The letters called for engagement with the FDA and CMS within 60 days to implement these changes and threatened to use "every tool in our arsenal" to address what the letter characterized as "abusive drug pricing practices."

On September 30, 2025, the administration announced that Pfizer had agreed to base its pharmaceutical prices in the U.S. on MFN pricing. According to a White House fact sheet, the agreement "will provide every State Medicaid program in

the country access to MFN drug prices" on the company's products, and requires the company "to offer medicines at a deep discount off the list price when selling directly to American patients." Pfizer indicated that the agreement "provides certainty from tariffs" and that the company "will also participate in a direct purchasing platform, TrumpRx.gov, that will allow American patients to purchase medicines … at a significant discount." Thereafter, on October 10, 2025, another pharmaceutical company (AstraZeneca) announced that it too had reached an agreement with the administration to lower prices for "eligible patients with prescriptions for chronic diseases" in the U.S. and that it will participate in the TrumpRx direct-to-patient marketing website.

In addition, the industry is awaiting the release of the Global Benchmark for Efficient Drug Pricing, or GLOBE Model, a proposed rule by the Centers for Medicare & Medicaid Services that is currently pending review at the Office of Information and Regulatory Affairs within the Office of Management and Budget. Once released, the GLOBE Model proposed rule will provide further insight into how the administration is seeking to advance drug pricing policy and provide an opportunity for interested parties to submit public comment as part of the rulemaking process. The implications and consequences of these actions and subsequent actions by the Trump administration to compel an MFN regulatory pricing requirement in the U.S. continue to remain unclear and uncertain and could ultimately result in litigation.

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 any product candidates we may develop or additional pricing pressures.

In addition, in some countries including those member states of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices, and in certain instances render commercialization in certain markets infeasible or disadvantageous from a financial perspective. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product and/or our product candidates to other available products in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third party payors or government authorities may lead to further pressure on the prices or reimbursement levels. If reimbursement of our products, if any, is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, the commercial launch of our product candidates may be delayed, possibly for lengthy periods of time, we or our collaborators may not launch at all in a particular country, we may not be able to recoup our investment in one or more product candidates, and there could be a material adverse effect on our business.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at containing or lowering the cost of healthcare. 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 product candidates that we may identify, if we obtain regulatory approval;
- our ability to receive or set a price that we believe is fair for our products;
- 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 are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, and contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may 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 and contractual obligations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the U.S., European Union and United Kingdom. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, claims for damages by affected individuals, 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.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have violated these privacy and security laws and/or breached certain contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face significant civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. 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.

In addition to potential enforcement by HHS, we are also potentially subject to privacy enforcement from the Federal Trade Commission, or the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the Federal Trade Commission Act, as well as the types of activities it views to trigger the Health Breach Notification Rule, which the FTC also has the authority to enforce. The FTC is also in the process of developing rules related to commercial surveillance and data security that may impact our business. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate our risk for a potential enforcement action, which may be costly. If we are subject to a potential FTC enforcement action, we may be subject to a settlement order that requires us to adhere to very specific privacy and data security practices, which may impact our business. We may also be required to pay fines as part of a settlement, depending on the nature of the alleged violations. If we violate any consent order that we reach with the FTC, we may be subject to additional fines and compliance requirements.

There are also increased restrictions at the federal level relating to transferring sensitive data outside of the United States to certain foreign countries. For example, in 2024, Congress passed H.B. 815, which included the Protecting Americans' Data from Foreign Adversaries Act of 2024. This law creates certain restrictions for entities that disclose sensitive data (including potential health data) to countries such as China. Failure to comply with these rules can lead to a potential FTC enforcement action. Additionally, the DOJ recently finalized a rule implementing Executive Order 14117, which implements the DOJ's Data Security Program, or DSP, and creates similar restrictions related to the transfer of sensitive U.S. data to countries such as China. Violations of the DSP can lead to both civil and criminal penalties, and it is unclear how aggressively the DOJ will enforce the new program. These data transfer restrictions (and others that may pass in the future) may create operational challenges and legal risks for our business.

States are also active in creating specific rules relating to the processing of personal information. In 2018, California passed into law the California Consumer Privacy Act, or the CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or the GDPR, which is further described below, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of the "sale" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or the CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, at least eighteen other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect over the next few years. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. Additional states are expected to consider such legislation in 2025 and beyond. Other states will be considering similar laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, Washington state passed a health privacy law in 2023 that regulates the collection and sharing of health information, and the law also has a private right of action, which further increases the relevant compliance risk. Other states have also passed similar laws regulating consumer health data and additional states are considering such legislation. These 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.

Similar to the laws in the U.S., there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the European Economic Area, or the EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which went into effect in May 2018 and which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

The GDPR places restrictions on the cross-border transfer of personal data from the European Union to countries that have not been found by the European Union to offer adequate data protection legislation, such as the U.S. There are ongoing concerns about the ability of companies to transfer personal data from the European Union to other countries. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the U.S.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The European Union initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022, and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data

Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business.

Following the withdrawal of the UK from the European Union, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the UK and includes parallel obligations to those set forth by GDPR. In relation to data transfers, both the UK and the European Union have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. The UK and the U.S. have also agreed to a to develop a U.S.-UK "Data Bridge", which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer data from the UK to the United States. In addition to the UK, Switzerland is also in the process of approving an adequacy decision in relation to the Swiss-U.S. Data Privacy Framework (which would function similarly to the EU-U.S. Data Privacy Framework and the U.S.-UK Data Bridge in relation to data transfers from Switzerland to the United States). Any changes or updates to these developments have the potential to impact our business.

Beyond GDPR and similar laws in the United States, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws may impact our ability to conduct our business activities.

While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices, which could adversely affect our business. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with federal, state and international laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines, penalties or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement actions, litigation and significant costs for remediation, reputational harm, diminished profits and earnings, additional reporting requirements and/or oversight, any of which could adversely affect our business, our results of operations or prospects. We also face a threat of consumer class actions related to these laws and the overall protection of personal data. 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.

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, partners and the principal investigators in our clinical trials. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union 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 United States 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 or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but 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 may preclude us from developing, manufacturing and selling certain product candidates outside of the United States or adversely impact our ability to operate our business outside the United States. In addition, changes in and uncertainty surrounding U.S. trade policy could have a material adverse impact on our business, financial condition and results of operations.

We are subject to numerous laws and regulations in each jurisdiction outside the United States 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 third 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 United States 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 DOJ. 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 United States, 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 United States embargoed countries or sanctioned countries, governments, persons and entities. Our operations outside of the United States have required, and will continue to require, us to dedicate additional resources to comply with laws governing international business practices, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with these laws 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.

Changes in and uncertainty surrounding United States trade policy could have a material adverse impact on our business, financial condition and results of operations.

The Trump administration has, in 2025, initiated a series of tariff-related actions against U.S. trading partners. On April 2, 2025, President Trump issued an executive order announcing a "baseline" reciprocal tariff of 10% on all U.S. trading partners effective April 5, 2025, and higher individualized reciprocal tariffs on 57 countries (with certain product exemptions for pharmaceutical-related products, among others). Previously, the administration had imposed a 25% tariff on Canada and Mexico for goods not covered by the United States-Mexico-Canada Agreement, or USMCA, and tariffs equaling 20% on China. In response, several countries threatened retaliatory measures, including Canada and China, which then imposed retaliatory tariffs. Prior to when the country-specific reciprocal tariffs were scheduled to take effect, the administration delayed the effective date of such tariffs for all countries except China to August 1, 2025. Several countries, including Japan, South Korea and the United Kingdom, among others, as well as the European Union, have reached deals with the U.S. that include reduced tariff rates to varying levels and other measures. On July 31, 2025, President Trump issued an executive order detailing new reciprocal tariff rates for individual countries that took effect on August 7, 2025. The new reciprocal rates, which are consistent with the rates reflected in the trade deals already announced, range from 10% to 41% with imports from Switzerland receiving a 39% rate. The new rates do not apply to Canada, China, Mexico and a few other countries.

The U.S. and China reached a framework agreement that resulted in the suspension of the higher reciprocal tariffs on China until November 10, 2025. For China, the 10% baseline reciprocal tariff announced in April 2025 remains in effect, in addition to a minimum of an additional 20%. Sustained uncertainty about, or the further escalation of, trade and political tensions between the United States and China could result in a disadvantageous research and manufacturing

environment in China, particularly for U.S. based companies, including retaliatory restrictions that hinder or potentially inhibit our ability to rely on contract development and manufacturing organizations and other service providers that operate in China.

Regarding Canada and Mexico, as of October 31, 2025, the rate remained 25% for goods that are not covered by the USMCA for Mexico and, effective August 1, 2025, was increased to 35% on imports from Canada that are not covered by the USMCA. Certain other countries, including Japan, South Korea and the United Kingdom, as well as the European Union, have reached agreements with the U.S. that cap pharmaceutical tariffs at 15%.

Separately, in April 2025, the U.S. Department of Commerce initiated an investigation under Section 232 of the Trade Expansion Act of 1962 into the impact on U.S. national security of the imports of pharmaceuticals and pharmaceutical ingredients, including finished drug products, medical countermeasures, critical inputs such as active pharmaceutical ingredients, and key starting materials, and derivative products of those items. On September 25, 2025, via a post on Truth Social, President Trump announced that, beginning October 1, 2025, all branded or patented drugs imported in the U.S. would face a 100% tariff. At the same time, Trump indicated that these tariffs could be avoided by building pharmaceutical manufacturing facilities in the U.S. Thereafter, Trump delayed the October 1st effective date of the tariffs, announcing that the Administration had now "begun preparing" tariffs on manufacturers that do not build in the U.S. or enter into a MFN drug pricing agreement with the Trump administration.

As a result of changes in tariffs that have been announced and/or implemented, and the underlying uncertainty currently surrounding international trade, we could experience a negative impact on our costs of materials and production processes, and supply chain disruptions and delays as a result of any new tariff policies or trade restrictions. If we are unable to obtain necessary raw materials or product components in sufficient quantity and in a timely manner due to disruptions in the global supply chain caused by macroeconomic events and conditions, the development, testing and clinical trials of our product candidates may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. We cannot yet predict the effect of the recently imposed U.S. tariffs on imports, or the extent to which other countries will impose quotas, duties, tariffs, taxes or other similar restrictions upon imports or exports in the future, nor can we predict future trade policy or the terms of any renegotiated trade agreements and their impact on our business.

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 and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at 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 biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

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.

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. For example, in 2024 and 2025, we had several changes in our executive management team. 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 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, 2025, we had 240 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 we may develop 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 are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for, and fully understanding the regulatory and manufacturing pathways to, all 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, 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.

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 any 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 security 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, 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, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including, but not limited to, intellectual property, proprietary business information and personal information). It is critical that we, and our vendors, collaborators or 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 any future vendors, collaborators and other contractors and consultants, and the increasing amounts of confidential information that they maintain, such information technology systems are vulnerable to damage or interruption from computer viruses, computer hackers, malicious code, 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 security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber

terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies.

Although we seek to protect our information technology systems from system failure, accident and security breach, our efforts may not be successful. 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 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 breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the 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 security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. 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 or security breach 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, penalties and fines, we could become the subject of regulatory action or investigation, 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 be in breach of our contractual obligations. Furthermore, 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, which could result in significant legal and financial exposure and reputational damage. 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 and could have a material adverse effect on our business, financial condition, results of operations or prospects. 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, CMOs and CROs, as well as regulatory agencies and other parties, for the continued operation of our business. Although 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 CMOs or CROs, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

Risks related to ownership of our common stock and our status as a public company

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

Our stock price has been, and is likely to continue to be, volatile. 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, our stockholders may not be able to sell their common stock at or above the price they paid for their shares. 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 any product candidates we may develop or those of our competitors or potential collaborators;
- adverse regulatory decisions, including failure to receive clearance to initiate clinical trials or obtain marketing approvals for any product candidates we may develop;
- our success in commercializing any product candidates that may be approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States 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 any product candidates we may develop;
- 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 results, development timelines or recommendations by securities analysts;
- variations in our financial results or the financial 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; and
- the other factors described in this “Risk Factors” section.

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 divert management’s attention and resources.

If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not have control over these analysts. There can be no assurance that existing analysts will continue to cover us or that new analysts will begin to cover us. There is also no assurance that any covering analysts will provide favorable coverage. If one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provide more favorable relative recommendations about our competitors, the price of our stock could decline. If one or more of these analysts cease to

cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline.

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 a pandemic, such as the COVID-19 pandemic, could result in a variety of risks to our business, including weakened demand for any product candidates we may develop. 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 and financial market conditions could adversely impact our business.

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

Our executive officers and directors and their affiliates, in the aggregate, beneficially owned shares representing approximately 11.2% of our common stock as of September 30, 2025. 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 different than 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 all or 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.

We have broad discretion in the use of our cash, cash equivalents and marketable securities and may not use them effectively.

Our management has broad discretion in the application of our cash, cash equivalents and marketable securities and could use such funds in ways that do not improve our results of operations or enhance the value of our common stock or in ways that our stockholders may not agree with. The failure by our management to apply these funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest these funds in a manner that does not produce income or that loses value.

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

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. In addition, the terms of our Loan Agreement with Hercules preclude us from paying dividends without the lender's consent or at all. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

A significant portion of our total outstanding shares may be sold into the market in the near future, which 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 or impair our ability to raise capital through the sale of equity securities in the future. As of October 31, 2025, we had 142,824,687 shares of common stock outstanding.

All of our outstanding shares of common stock are available for sale in the public market, subject to applicable securities laws.

Moreover, holders of a substantial number of shares of our common stock and shares of our common stock issuable upon exercise of outstanding options 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 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 and exercise of options.

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

As a public company, and particularly as we are no longer an emerging growth company, or EGC, or a smaller reporting company, or SRC, we will incur significant legal, accounting and other expenses that we did not previously incur as a private company or as an EGC and SRC. 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. As a result of no longer being able to take advantage of the exemptions from various reporting requirements that are applicable to EGCs and SRCs, we are required to comply with auditor attestation requirements, increased disclosure obligations and other reporting requirements which will likely increase our costs in the upcoming fiscal year. 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 will 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 will make some activities more time-consuming and costly compared to when we were a private company. For example, as a public company it is more difficult and more expensive for us to obtain director and officer liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantial costs. 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 costs we may incur to continue to operate as a public company, nor can we predict 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.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting and our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting. If we have an unremediated material weakness, we would receive an adverse opinion regarding our internal control over financial reporting from our independent registered public accounting firm. We are engaged in a continuous 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, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement 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.

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. Our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404. 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.

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.

Provisions in our restated certificate of incorporation and our 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 our stockholders might otherwise receive a premium for their 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 75% 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 the 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 restated certificate of incorporation designates 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 restated certificate of incorporation provides 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 restated certificate of incorporation provides 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 restated certificate of incorporation. 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 either exclusive forum provision contained in our restated certificate of incorporation 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.

General Risk Factors

Changes in patent law in the United States or worldwide could diminish the value of patents in general, thereby impairing our ability to protect any product candidates we may develop and our technology.

Changes in either the patent laws or interpretation of patent laws in the United States and worldwide, including patent reform legislation such as the Leahy-Smith America Invents Act, or the 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 United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States 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. Because patent applications in the United States 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 United States 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 biologics and 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 any product candidates we may develop or our technology will be held by the USPTO or equivalent foreign patent offices or by courts in the United States 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.

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

Income, sales, use or other tax laws, statutes, rules, or regulations could be enacted or amended at any time, which could affect our business or financial condition, including causing potentially adverse impacts to our effective tax rate, tax liabilities and cash tax obligations. For example, the IRA was signed into law in August 2022, and the OBBBA was signed into law in July 2025. The IRA introduced new tax provisions, and in particular imposes a 1% excise tax on certain stock repurchases by publicly traded corporations. The OBBBA contains numerous tax provisions that we are currently in the process of evaluating, and which may significantly affect our business or financial condition. The recent changes under the OBBBA include tax rate extensions and changes to the business interest deduction limitation, the expensing of domestic research and development expenditures (in contrast to the continued capitalization and amortization of foreign research and development expenditures), the bonus depreciation deduction rules and the international tax framework. Regulatory guidance under the IRA, the OBBBA, and additional tax-related legislation is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. In addition, it is uncertain if and to what extent various states will conform to such legislation.

Disruptions at the FDA and other government agencies from funding cuts, personnel losses, regulatory reforms, government shutdowns and other developments could hinder our ability to obtain guidance from the FDA regarding our clinical development programs and develop and secure approval of our product candidates in a timely manner, which would negatively impact our business.

The FDA and comparable regulatory agencies in foreign jurisdictions, such as the EMA and Committee for Medicinal Products for Human Use, play an important role in the development of our product candidates by providing guidance on our clinical development programs and reviewing our regulatory submissions, including INDs, requests for special designations and marketing applications. If these oversight and review activities are disrupted, then correspondingly our ability to develop and secure timely approval of our product candidates could be impacted in a negative manner.

For example, the recent loss and retirement of FDA leadership and personnel could lead to disruptions and delays in FDA guidance, review and approval of our product candidates. Pursuant to President Trump's executive order 14210, "Implementing the President's 'Department of Government Efficiency' Workforce Optimization Initiative," the Secretary of HHS announced on March 27, 2025, a reorganization and Reduction in Force, or RIF, across HHS of approximately 20,000 employees (82,000 to 62,000), with the FDA's workforce of approximately 20,000 to decrease by 3,500 full-time employees. Subsequently, the FDA indicated that roughly a quarter of those employees who received RIF notices had been reinstated. On July 14, 2025, following litigation reaching the U.S. Supreme Court, the administration began to carry out these layoffs across HHS, including the FDA. There are also ongoing deliberations within the administration and Congress over potentially substantial proposed cuts to the overall budget for HHS and funding of the FDA for the 2026 federal fiscal year.

While the FDA's review of marketing applications and other activities for new drugs and biologics is largely funded through the user fee program established under the Prescription Drug User Free Act, or PDUFA, it remains unclear how the administration's RIF and budget cuts will impact this program and the ability of the FDA to provide guidance and review our product candidates in a timely manner. For example, while the FDA's RIF did not reportedly specifically target FDA reviewers, many operations, administrative and policy staff that help support such reviews were affected and those losses could lead to delays in PDUFA reviews and related activities. There has been at least one report in which the FDA failed to meet a PDUFA goal date for approval of an NDA due to heavy workload and limited resources. In addition, while currently unclear, there is a risk that the RIF and budget cutbacks could threaten the integrity of the PDUFA program itself. That is because, for the FDA to obligate user fees collected under PDUFA in the first place, a certain amount of non-user fee appropriations must be spent on the process for the review of applications plus certain other costs during the same fiscal year.

There is also substantial uncertainty as to how regulatory reform measures being implemented by the Trump administration across the government will impact the FDA and other federal agencies with jurisdiction over our activities. For example, since taking office, President Trump has issued a number of executive orders that could have a significant impact on the manner in which the FDA conducts its operations and engages in regulatory and oversight activities. These include executive order 14192, "Unleashing Prosperity Through Deregulation," January 31, 2025; executive order 14212, "Establishing the President's Make America Healthy Again Commission," February 13, 2025; and executive order 14219, Ensuring Lawful Governance and Implementing the President's 'Department of Government Efficiency' Deregulatory Initiative," February 21, 2025. If these or other orders or executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Similarly, actions by the U.S. government have significantly disrupted the operations of U.S. government agencies such as the National Institutes of Health, National Science Foundation, Centers for Disease Control and Prevention, and the FDA, which have traditionally provided funding for basic research, research and development, and clinical testing. These U.S. government actions have included, among other things, suspending, terminating and withholding of disbursements of funds owed under ongoing contracts, grants, and other financial assistance agreements; declining to continue multi-year research projects for additional annual budget periods; canceling or delaying solicitations for new contract, grant and other financial assistance awards; canceling or delaying proposal evaluation processes and issuance of such new awards; substantially reducing federal agency staff responsible for managing contract and financial assistance programs; eliminating agency information and resources for facilitating research activity; delaying or terminating federal agency procedures for authorizing international transactions; initiating aggressive enforcement actions that may disrupt the operations of major research universities that are significant contributors to life sciences research in the U.S., and threatening access to federal agency contracts and other funding awards based on companies' otherwise lawful corporate policies and choice of counsel. These U.S. government actions could, directly or indirectly, significantly disrupt, delay, prevent, or increase the costs of our research and product commercialization programs, including our ability to develop new product candidates, conduct clinical trials, implement research collaborations with other companies or institutions, and obtain approvals to market and sell new products.

In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and

unpredictable. Over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions and could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

For example, the federal government shut down on October 1, 2025 and as of November 5, 2025, it has not reopened. With the shutdown, the FDA issued a public notice stating that agency operations would continue to the extent permitted by law, such as activities necessary to address imminent threats to the safety of human life and activities funded by carryover user fee funds. At the same time, the FDA declared that, during the shutdown period, it does not have legal authority to accept user fees assessed for fiscal year 2026 until an appropriation or continuing resolution for the FDA is enacted for such fiscal year. As a result, the FDA will not be able to accept any regulatory submissions for fiscal year 2026 that require a fee payment and that are submitted during the lapse period.

At the same time, disruptions at the FDA and other government agencies may result from public health events similar to the COVID-19 pandemic. For example, during the pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. In the event of a similar public health emergency in the future, the FDA may not be able to continue its current pace and review timelines could be extended. Regulatory authorities outside the United States facing similar circumstances may adopt similar restrictions or other policy measures in response to a similar public health emergency and may also experience delays in their regulatory activities.

Accordingly, if any of the foregoing developments or others impact the ability of the FDA to provide us with guidance regarding our clinical development programs or delay the FDA's review and processing of our regulatory submissions, including INDs and BLAs, our business would be negatively impacted. Further, any future government shutdown could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

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.

Item 2. 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.

Director and Officer Trading Arrangements

None of our directors or officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended) adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the quarterly period covered by this report.

Item 6. Exhibits.

Exhibit Number	Description
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
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	The cover page from the Company's Quarterly Report on Form 10-Q for the three months ended September 30, 2025, has been formatted in Inline XBRL.

* Filed herewith.

† The certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report, are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of Dyne Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report, irrespective of any general incorporation language contained in such filing.

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.

DYNE THERAPEUTICS, INC.

Date: November 5, 2025	By:	/s/ John G. Cox John G. Cox President and Chief Executive Officer (Principal Executive Officer)
Date: November 5, 2025	By:	/s/ Erick Lucera Erick Lucera Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)