UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 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 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**93-0797222**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
215 First Street, Suite 415 Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 274-4000

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

Title of each class	Trading Symbol	Name of exchange on which registered
Common Stock, $0.0001 par value per share	SRPT	The 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 ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock with $0.0001 par value	**97,713,438**
(Class)	**(Outstanding as of August 1, 2025)**

SAREPTA THERAPEUTICS, INC.
FORM 10-Q
INDEX

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited, in thousands, except share and per share amounts)

	As of June 30, 2025	As of December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 510,598	$ 1,103,010
Short-term investments	289,541	251,782
Accounts receivable, net	527,295	601,988
Inventory	994,036	749,960
Manufacturing-related deposits and prepaids	207,921	276,262
Other current assets	127,594	90,461
Total current assets	2,656,985	3,073,463
Property and equipment, net	371,857	340,336
Right of use assets	143,041	148,310
Non-current inventory	194,668	187,986
Strategic investments	195,522	3,710
Non-current investments	34,604	133,163
Other non-current assets	83,137	76,205
Total assets	$ 3,679,814	$ 3,963,173
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 136,702	$ 214,442
Accrued expenses	377,399	373,513
Deferred revenue, current portion	395,431	130,256
Other current liabilities	10,416	13,473
Total current liabilities	919,948	731,684
Long-term debt	1,139,458	1,137,124
Lease liabilities, net of current portion	214,419	192,473
Deferred revenue, net of current portion	—	325,000
Contingent consideration	47,400	47,400
Other non-current liabilities	1,204	1,750
Total liabilities	2,322,429	2,435,431
Commitments and contingencies (Note 17)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 98,356,950 and 97,706,074 issued and outstanding, respectively, at June 30, 2025 and 96,900,496 issued and outstanding at December 31, 2024	10	10
Treasury stock, at cost, 650,876 and 0 shares at June 30, 2025 and December 31, 2024, respectively	(25,263)	—
Additional paid-in capital	5,844,279	5,738,924
Accumulated other comprehensive loss, net of tax	(51)	(218)
Accumulated deficit	(4,461,590)	(4,210,974)
Total stockholders' equity	1,357,385	1,527,742
Total liabilities and stockholders' equity	$ 3,679,814	$ 3,963,173

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
Revenues:				
Products, net	$ 513,123	$ 360,548	$ 1,124,646	$ 720,032
Collaboration and other	97,968	2,383	231,301	56,363
Total revenues	611,091	362,931	1,355,947	776,395
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	152,558	44,545	290,122	95,104
Research and development	204,392	179,690	977,840	380,086
Selling, general and administrative	137,897	138,796	271,526	265,799
Amortization of in-licensed rights	667	601	1,268	1,202
Total cost and expenses	495,514	363,632	1,540,756	742,191
Operating income (loss)	115,577	(701)	(184,809)	34,204
Other income (loss), net:				
Other income (expense), net	38,061	14,278	(45,071)	20,821
Income (loss) before income tax expense	153,638	13,577	(229,880)	55,025
Income tax (benefit) expense	(43,254)	7,117	20,736	12,446
Net income (loss)	$ 196,892	$ 6,460	$ (250,616)	$ 42,579
Other comprehensive (loss) income:				
Unrealized (losses) gains on investments, net of tax	(85)	(339)	167	(1,948)
Total other comprehensive (loss) income	(85)	(339)	167	(1,948)
Comprehensive income (loss)	$ 196,807	$ 6,121	$ (250,449)	$ 40,631
Earnings (loss) per share:				
Basic	$ 2.01	$ 0.07	$ (2.57)	$ 0.45
Diluted	$ 1.89	$ 0.07	$ (2.57)	$ 0.44
Weighted average number of shares of common stock used in computing earnings (loss) per share:				
Basic	98,005	94,618	97,685	94,305
Diluted	106,623	99,144	97,685	99,129

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited, in thousands)

	Common Stock Issued		Treasury Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
BALANCE AT DECEMBER 31, 2024	**96,900**	**$ 10**	**—**	**$ —**	**$ 5,738,924**	**$ (218)**	**$ (4,210,974)**	**$ 1,527,742**
Exercise of options for common stock	146	—	—	—	9,036	—	—	9,036
Vest of restricted stock units	1,135	—	—	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	74	—	—	—	6,645	—	—	6,645
Stock-based compensation	—	—	—	—	46,556	—	—	46,556
Unrealized gains on investments, net of tax	—	—	—	—	—	252	—	252
Net loss	—	—	—	—	—	—	(447,508)	(447,508)
BALANCE AT MARCH 31, 2025	**98,255**	**$ 10**	**—**	**$ —**	**$ 5,801,161**	**$ 34**	**$ (4,658,482)**	**$ 1,142,723**
Exercise of options for common stock	57	—	—	—	1,500	—	—	1,500
Vest of restricted stock units	45	—	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	41,618	—	—	41,618
Repurchases of common stock	—	—	(651)	(25,263)	—	—	—	(25,263)
Unrealized losses on investments, net of tax	—	—	—	—	—	(85)	—	(85)
Net income	—	—	—	—	—	—	196,892	196,892
BALANCE AT JUNE 30, 2025	**98,357**	**$ 10**	**(651)**	**$ (25,263)**	**$ 5,844,279**	**$ (51)**	**$ (4,461,590)**	**$ 1,357,385**

	Common Stock Issued		Treasury Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
BALANCE AT DECEMBER 31, 2023	**93,732**	**$ 9**	**—**	**$ —**	**$ 5,304,623**	**$ 918**	**$ (4,446,213)**	**$ 859,337**
Exercise of options for common stock	207	—	—	—	15,579	—	—	15,579
Vest of restricted stock units	461	—	—	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	90	—	—	—	6,561	—	—	6,561
Stock-based compensation	—	—	—	—	45,205	—	—	45,205
Unrealized losses on investments, net of tax	—	—	—	—	—	(1,609)	—	(1,609)
Net income	—	—	—	—	—	—	36,119	36,119
BALANCE AT MARCH 31, 2024	**94,490**	**$ 9**	**—**	**$ —**	**$ 5,371,968**	**$ (691)**	**$ (4,410,094)**	**$ 961,192**
Exercise of options for common stock	468	1	—	—	39,917	—	—	39,918
Vest of restricted stock units	131	—	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	55,654	—	—	55,654
Issuance of common stock for conversion of 2024 Notes	194	—	—	—	14,184	—	—	14,184
Unrealized losses on investments, net of tax	—	—	—	—	—	(339)	—	(339)
Net income	—	—	—	—	—	—	6,460	6,460
BALANCE AT JUNE 30, 2024	**95,283**	**$ 10**	**—**	**$ —**	**$ 5,481,723**	**$ (1,030)**	**$ (4,403,634)**	**$ 1,077,069**

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Six Months Ended June 30,	
	2025	**2024**
Cash flows from operating activities:		
Net (loss) income	$ (250,616)	$ 42,579
Stock-based compensation	78,453	91,174
Loss (gain) on strategic investments	54,007	(1,079)
Depreciation and amortization	20,818	17,463
Reduction in the carrying amounts of the right of use assets	7,956	7,964
Accretion of investment discount, net	(3,778)	(22,859)
Non-cash change in the fair value of derivatives	—	10,100
Other	3,303	2,513
Changes in operating assets and liabilities, net:		
Decrease in accounts receivable	74,693	40,330
Decrease (increase) in manufacturing-related deposits and prepaids	68,869	(203,344)
Increase in inventory	(236,548)	(162,972)
(Increase) decrease in other assets	(37,258)	6,627
Decrease in deferred revenue	(59,825)	(40,380)
Decrease in accounts payable, accrued expenses, lease liabilities and other liabilities	(42,174)	(15,250)
Net cash used in operating activities	(322,100)	(227,134)
Cash flows from investing activities:		
Purchase of property and equipment	(75,447)	(61,611)
Purchase of available-for-sale securities	(44,658)	(547,282)
Maturity and sales of available-for-sale securities	109,384	739,161
Purchase of intangible assets and other	(2,426)	(10,000)
Acquisition of strategic investments	(245,819)	—
Net cash (used in) provided by investing activities	(258,966)	120,268
Cash flows from financing activities:		
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	17,181	62,058
Repurchases of common stock	(25,013)	—
Payments related to Revolving Credit Facility	(3,514)	—
Net cash (used in) provided by financing activities	(11,346)	62,058
Decrease in cash, cash equivalents and restricted cash	(592,412)	(44,808)
Cash, cash equivalents and restricted cash:		
Beginning of period	1,118,589	444,009
End of period	$ 526,177	$ 399,201
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 510,598	$ 383,622
Restricted cash in other assets	15,579	15,579
Total cash, cash equivalents and restricted cash	$ 526,177	$ 399,201
Supplemental disclosure of cash flow information:		
Cash paid during the period for income taxes	$ 20,093	$ 7,990
Cash paid during the period for interest	$ 7,188	$ 7,981
Supplemental schedule of non-cash activities:		
Intangible assets and property and equipment included in accounts payable and accrued expenses	$ 26,466	$ 30,483
Capitalized stock-based compensation and depreciation as inventory	$ 14,210	$ 13,287
Lease liabilities arising from obtaining right of use assets	$ 5,232	$ 2,018
Common stock issued for conversion or exchange of 2024 Notes	$ —	$ 14,184

See accompanying notes to unaudited condensed consolidated financial statements.

1. ORGANIZATION AND NATURE OF BUSINESS

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, “Sarepta” or the “Company”) is a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, small interfering RNA ("siRNA") platform, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying its proprietary, highly-differentiated and innovative technologies, and through collaborations with its strategic partners, the Company has developed multiple approved products for the treatment of Duchenne muscular dystrophy (“Duchenne”) and is developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne, Limb-girdle muscular dystrophies (“LGMDs”) and other neuromuscular and central nervous system (“CNS”) disorders.

The Company's products in the U.S., EXONDYS 51 (eteplirsen) Injection (“EXONDYS 51”), VYONDYS 53 (golodirsen) Injection (“VYONDYS 53”) and AMONDYS 45 (casimersen) Injection (“AMONDYS 45”), were granted accelerated approval by the U.S. Food and Drug Administration (the “FDA”) in 2016, 2019 and 2021, respectively. Indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51, exon 53 and exon 45 skipping, respectively, EXONDYS 51, VYONDYS 53 and AMONDYS 45 (collectively, the “PMO Products”) use the Company’s phosphorodiamidate morpholino oligomer (“PMO”) chemistry and exon-skipping technology to skip exon 51, exon 53 and exon 45 of the dystrophin gene. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

ELEVIDYS (delandistrogene moxeparvovec-rokl), an adeno-associated virus-(“AAV”) based gene therapy, was approved by the FDA on June 20, 2024 for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene, as well as for non-ambulatory patients under the accelerated approval pathway. ELEVIDYS was previously granted accelerated approval by the FDA in June 2023 for the treatment of ambulatory patients aged four through five years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene. In response to two recent safety events, the Company suspended all commercial shipments of ELEVIDYS to non-ambulatory patients in June 2025. As of the date of this Quarterly Report, the Company remains in ongoing discussions with FDA related to a labeling supplement for ELEVIDYS, including a boxed warning for acute liver injury and acute liver failure.

As of June 30, 2025, the Company had $850.3 million of cash, cash equivalents, restricted cash and investments, consisting of $510.6 million of cash and cash equivalents, $324.1 million of investments and $15.6 million of non-current restricted cash. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report, along with future cash inflows from operations, is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue additional cash resources through public or private debt and equity financings, seek funded research and development arrangements and additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States (“U.S. GAAP”), reflect the accounts of Sarepta and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases.

In the opinion of the Company’s management, all adjustments of a normal recurring nature necessary for a fair presentation have been reflected. Certain financial information that is normally included in annual financial statements prepared in accordance with U.S. GAAP, but that is not required for interim reporting purposes, has been omitted. These unaudited condensed consolidated financial statements should be read in conjunction with the consolidated financial statements and related notes for the year ended December 31, 2024, which are contained in the Company’s Annual Report on Form 10-K for the year ended December 31, 2024, filed with the U.S. Securities and Exchange Commission on February 28, 2025. The results for the three and six months ended June 30, 2025 are not necessarily indicative of the results to be expected for the full year.

Estimates and Uncertainties

The preparation of the unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenues, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist of cash held at financial institutions, cash equivalents, investments and accounts receivable, net, from customers. As of June 30, 2025, the Company's cash was concentrated at three financial institutions, which potentially exposes the Company to credit risks. However, the Company does not believe that there is significant risk of non-performance by the financial institutions. The Company also purchases commercial paper, government and government agency bonds, corporate bonds and certificates of deposit issued by highly rated corporations, financial institutions and governments and limits the amount of credit exposure to any one issuer. These amounts may at times exceed federally insured limits. The Company has not experienced any credit losses related to these financial instruments and does not believe to be exposed to any significant credit risk related to these instruments. As of June 30, 2025, three entities accounted for 47%, 24% and 17% of accounts receivable, net, respectively. As of December 31, 2024, three entities accounted for 54%, 21% and 13% of accounts receivable, net, respectively. For details about the Company's accounts receivable, please read *Note 6, Product Revenues, Net, Accounts Receivable, Net, And Reserves For Product Revenues.*

Significant Accounting Policies

For details about the Company's accounting policies, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the Annual Report on Form 10-K for the year ended December 31, 2024.

Acquired in-process research and development

Acquired in-process research and development ("IPR&D") is recognized at fair value and initially characterized as an indefinite-lived intangible asset, irrespective of whether the acquired IPR&D has an alternative future use. If the acquired net assets do not constitute a business under the acquisition method of accounting, the transaction is accounted for as an asset acquisition and no goodwill is recognized. In an asset acquisition, the amount allocated to acquired IPR&D with no alternative future use is recorded as an expense at the acquisition date.

Treasury stock

Treasury stock purchases are accounted for under the cost method whereby the entire cost of the acquired stock is recorded as treasury stock. Repurchased shares are held as treasury stock until they are retired or re-issued. The Company uses the weighted average purchase cost to determine the cost of treasury stock that is reissued, if any. Excise taxes incurred on share repurchases and broker fees paid represent direct costs of share repurchases. Excise taxes are recorded as a part of the cost basis of repurchased shares within treasury stock and are paid when due.

There have not been any other material changes to the Company's accounting policies or recent accounting pronouncements that could have a material impact through June 30, 2025.

3. LICENSE AND COLLABORATION AGREEMENTS

License Agreement with Arrowhead Pharmaceuticals, Inc.

On November 25, 2024, the Company and Arrowhead Pharmaceuticals, Inc. ("Arrowhead") entered into an exclusive global license and collaboration agreement and a stock purchase agreement (collectively, the "Arrowhead Agreement"), which became effective as of February 7, 2025 (the "Effective Date"), upon the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

The Arrowhead Agreement granted the Company an exclusive license under certain of Arrowhead's intellectual property rights to develop, manufacture and commercialize the lead candidate (and all backup candidates) for the following programs:

Development Stage	Indication
Arrowhead Clinical Programs	(a) DUX4 for the treatment of facioscapulohumeral muscular dystrophy, (b) DM1 for the treatment of type 1 myotonic dystrophy, (c) ATXN2 for the treatment of ataxias, and (d) MMP7 for the treatment of idiopathic pulmonary fibrosis.
Arrowhead Pre-clinical Programs	(a) ATXN1 for the treatment of ataxias,

	(b) ATXN3 for the treatment of ataxias, and (c) HTT for the treatment of Huntington's Disease.

In addition, the parties will collaborate on the discovery and development of compounds that are directed to six targets to be selected by the Company during the term (each an "Arrowhead Discovery Program," and together with the Arrowhead Clinical Programs and Pre-clinical Programs, the "Arrowhead Programs").

The development and manufacturing activities under the Arrowhead Agreement are governed by a series of committees established to facilitate transition and collaboration between the two parties.

Under the terms of the Arrowhead Agreement, Arrowhead will conduct development activities with respect to the Arrowhead Programs pursuant to agreed-upon development plans. At pre-determined transition points for each Arrowhead Program, Arrowhead will transfer development responsibility to the Company, who will then perform all development activities necessary to obtain and maintain regulatory approvals throughout the world. The Company will have the sole worldwide right to commercialize licensed products.

The Company will reimburse Arrowhead for certain pre-determined development activities for the Arrowhead Clinical Programs. Each party is responsible for the costs and expenses of other development activities under the Arrowhead Agreement. Arrowhead will complete all manufacturing activities necessary for their development activities and provide clinical supply for all Arrowhead Programs and commercial supply for the Arrowhead Clinical Programs. The parties will determine at a later date whether Arrowhead will provide commercial supply for the Arrowhead Pre-clinical Programs and Arrowhead Discovery Programs. Upon the occurrence of certain conditions, Arrowhead will transfer control of manufacturing and supply to the Company.

In connection with the Arrowhead Agreement, Arrowhead appointed the Company's Chief Executive Officer ("CEO"), Douglas Ingram, as a director, effective February 2025.

When the Arrowhead Agreement became effective, the Company paid Arrowhead an up-front payment of $500.0 million and invested $325.0 million in approximately 11.9 million shares of Arrowhead's common stock at a premium to the valuation on the closing date. The Company is contractually restricted from selling its investment in Arrowhead common stock until August 2025. Based on the closing price of Arrowhead's common stock traded on the Nasdaq Global Select Market ("Nasdaq") on the Effective Date, $241.4 million was allocated to the equity investment in Arrowhead and recorded within strategic investments on the Company's unaudited condensed consolidated balance sheets. For the six months ended June 30, 2025, the Company recorded $583.6 million as acquired in-process research and development expense, as the remainder of the up-front payments is allocated to the up-front license fee representing rights to potential future benefits associated with research and development activities that have no alternative future use. The Company concluded that it does not have a controlling financial interest in Arrowhead as the Company does not have the power to direct the activities that would most significantly impact the economic performance of Arrowhead. Additionally, the Company concluded that, as it does not have significant influence over Arrowhead, the Company's investment in Arrowhead is not subject to the equity method of accounting. Accordingly, the investment in Arrowhead's common stock is recorded at fair value and remeasured each reporting period, with changes recorded to other income (expense), net in the Company's unaudited condensed consolidated statements of comprehensive income (loss). For the three and six months ended June 30, 2025, the Company recognized a gain of $36.5 million and a loss of $53.0 million, respectively, related to the change in fair value of this strategic investment. The Arrowhead Agreement also provides the Company with the option to exchange its holding of Arrowhead's common stock into pre-funded warrants ("Warrants") with an exercise price of $0.001 per Warrant share. The Company currently does not have any plan to exercise this option. For the three and six months ended June 30, 2025, the Company recorded $6.5 million and $8.8 million, respectively, of research and development expense related to reimbursable development costs incurred by Arrowhead.

Additionally, the Company will pay Arrowhead an aggregate total of $250.0 million in annual installments of $50.0 million over five years ("Annual Fees"), with the first payment due on the first anniversary of the Effective Date. The Annual Fees are contingent upon the Company not terminating the Arrowhead Agreement. The Company may be obligated to make payments to Arrowhead totaling up to $10.3 billion upon the achievement of certain development, regulatory and sales milestones, inclusive of $300.0 million in near-term payments associated with the continued enrollment of certain cohorts of a Phase 1/2 study for an individual program. Furthermore, upon commercialization, the Company will be required to make tiered royalty payments based on net sales. Please refer to *Note 18, Subsequent Events* for further discussion of the Company's milestone payment due to Arrowhead.

Collaboration Agreement with F. Hoffman-La Roche Ltd.

For the three and six months ended June 30, 2025, the Company recognized $63.5 million and $175.5 million, respectively, of collaboration revenue associated with the license, collaboration and option agreement (the "Roche Collaboration Agreement") with F. Hoffman-La Roche Ltd. ("Roche"). Under the Roche Collaboration Agreement, the Company has granted Roche options to acquire ex-U.S. rights to certain future Duchenne development programs (the "Options") in exchange for separate option exercise payments, milestone and royalty considerations, and cost-sharing provisions. These Options were accounted for as material rights related to

individual programs and recorded in deferred revenue at the inception of the Roche Collaboration Agreement. The value assigned to the material rights is included in deferred revenue until such rights expire or are exercised.

In June 2025, the Company received a milestone payment of $63.5 million in connection with the receipt of regulatory approval of ELEVIDYS in Japan for individuals ages 3- to less than 8-years-old, who do not have any deletions in exon 8 and/or exon 9 in the Duchenne gene and who are negative for anti-AAVrh74 antibodies. The milestone payment is included in collaboration and other revenues for the three and six months ended June 30, 2025. As a result of the milestone received from Roche, the Company recorded a $5.1 million milestone due to Nationwide Children's Hospital ("Nationwide") in accordance with the Company's license agreement with Nationwide as an in-licensed right intangible asset in its unaudited condensed consolidated balance sheets as of June 30, 2025. As of June 30, 2025, the in-licensed right asset with a net carrying value of approximately $5.0 million is being amortized on a straight-line basis over the remaining life of the relevant patent as it reflects the expected time period that the Company will benefit from this in-licensed right.

In February 2025, an Option for a certain program expired, which resulted in the immediate recognition of $112.0 million of collaboration revenue for the six months ended June 30, 2025. In February 2024, Roche declined to exercise its option related to one external, early-stage development program, which resulted in the immediate recognition of $48.0 million of collaboration revenue for the six months ended June 30, 2024. As of June 30, 2025 and December 31, 2024, the Company had total deferred revenue of $395.4 million and $455.3 million, primarily related to the separate material rights for the Options associated with the Roche Collaboration Agreement, of which $395.4 million and $130.3 million are classified as current, respectively.

In accordance with the Roche Collaboration Agreement, the parties agreed to enter into a supply agreement in order to supply Roche with clinical and commercial batches of ELEVIDYS (the "Roche Supply Agreement"). Roche utilizes the supply for sales of ELEVIDYS in territories outside of the U.S. where Roche has received certain approvals for ELEVIDYS. While the Roche Supply Agreement is in the process of being negotiated at the issuance of this report, the Company delivered commercial ELEVIDYS supply to Roche that were agreed upon on a purchase order-by-purchase order basis. Contract manufacturing revenue and royalty revenue are included in collaboration and other revenues in the unaudited condensed consolidated statements of comprehensive income (loss). The following table summarizes certain Roche activity for each of the periods indicated:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands)			
Contract manufacturing revenue	$ 27,023	$ —	$ 44,402	$ 5,807
Royalty revenue	7,445	2,383	11,399	2,556
Cost of sales (inventory costs related to products sold to Roche)	(21,818)	—	(33,961)	(1,654)

The costs associated with co-development activities performed under the Roche Collaboration Agreement are included in operating expenses, with any reimbursement of costs by Roche reflected as a reduction of such expenses when the related expense is incurred. For the three and six months ended June 30, 2025, costs reimbursable by Roche and reflected as a reduction to operating expenses were $25.7 million and $54.2 million, respectively. For the three and six months ended June 30, 2024, costs reimbursable by Roche and reflected as a reduction to operating expenses were $17.9 million and $39.6 million, respectively. As of June 30, 2025 and December 31, 2024, there were $73.3 million and $34.6 million, respectively, of collaboration and other receivables included in other current assets on the unaudited condensed consolidated balance sheets.

Research and Option Agreements

The Company has research and option agreements with third parties in order to develop various technologies and biologics that may be used in the administration of the Company's genetic therapeutics. The agreements generally provide for research services related to pre-clinical development programs and options to license the technology for clinical development. Prior to the options under these agreements being executed, the Company may be required to make up to $13.0 million in research milestone payments. Under these agreements, there are $73.8 million in potential option payments to be made by the Company upon the determination to exercise the options. Additionally, if the options for each agreement are executed, the Company would incur additional contingent obligations and may be required to make development, regulatory, and sales milestone payments and royalty payments based on the net sales of the developed products upon commercialization. During the three and six months ended June 30, 2025 and 2024, the Company did not exercise any options or have any additional research milestone payments become probable of occurring.

Milestone Obligations

Including the agreements discussed above, the Company has license and collaboration agreements in place for which it could be obligated to pay, in addition to the payment of up-front fees upon execution of the agreements, certain milestone payments as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of June 30, 2025, the Company may be obligated to make up to $12.6 billion of future development, regulatory, commercial and up-front royalty payments associated with its collaboration and license agreements. These obligations exclude potential future option and milestone payments for options that have yet to be exercised within agreements entered into by the Company as of June 30, 2025, which are discussed above. For the six months ended June 30, 2025, the Company recognized up-front and development milestone expenses of $583.8 million as research and development expense in the accompanying unaudited condensed consolidated statements of comprehensive income (loss), with no similar activity for the three months ended June 30, 2025 and the three and six months ended June 30, 2024.

4. FAIR VALUE MEASUREMENTS

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

- Level 1 — quoted prices for identical instruments in active markets;
- Level 2 — quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3 — valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

During the six months ended June 30, 2025, there were no transfers into or out of Level 3. The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value and indicate the level within the fair value hierarchy of the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of June 30, 2025			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 153,270	$ 153,270	$ —	$ —
Commercial paper	3,534	—	3,534	—
Government and government agency bonds	255,065	—	255,065	—
Corporate bonds	55,208	—	55,208	—
Strategic investments*	195,522	190,091	—	5,431
Certificates of deposit	10,338	—	10,338	—
Deferred compensation plan assets	475	475	—	—
Total assets	$ 673,412	$ 343,836	$ 324,145	$ 5,431
Liabilities				
Contingent consideration	$ 47,400	$ —	$ —	$ 47,400
Total liabilities	$ 47,400	$ —	$ —	$ 47,400

	Fair Value Measurement as of December 31, 2024			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 455,535	$ 455,535	$ —	$ —
Government and government agency bonds	279,899	—	279,899	—
Corporate bonds	93,727	—	93,727	—
Strategic investments	3,710	2,710	—	1,000
Certificates of deposit	11,319	—	11,319	—
Total assets	$ 844,190	$ 458,245	$ 384,945	$ 1,000
Liabilities				
Contingent consideration	$ 47,400	$ —	$ —	$ 47,400
Total liabilities	$ 47,400	$ —	$ —	$ 47,400

* The balance at June 30, 2025 includes an investment with a fair value of $188.4 million, which is subject to a contractual sale restriction that expires in August 2025.

The Company’s assets with a fair value categorized as Level 1 within the fair value hierarchy include money market funds, the Company's strategic investments in Arrowhead and another biotechnology company, both listed on Nasdaq, and assets associated with the Company's deferred compensation plan that are held in a trust. For more information related to Arrowhead, please read *Note 3, License and Collaboration Agreements*. The Company's deferred compensation plan allows for certain employees and directors to defer the receipt of compensation until a later date based on the terms of the plan. The Company has recorded an asset within other non-current assets on the Company's unaudited condensed consolidated balance sheets which reflects the participants' investment elections and has been valued at daily quoted market prices.

The Company's assets with a fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds, corporate bonds and certificates of deposit. These assets have been initially valued at the transaction price and subsequently valued at the end of each reporting period. The Company uses observable market inputs to determine value, which primarily consist of reportable trades. Certain highly liquid investments with maturities of less than three months at the date of acquisition are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of June 30, 2025 and December 31, 2024.

The Company’s assets with a fair value categorized as Level 3 within the fair value hierarchy consist of strategic investments in private biotechnology companies whose fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. At the end of each reporting period, the fair value of the Company's strategic investments that are not listed securities are adjusted if the entities were to issue similar or identical securities or when there is a triggering event for impairment. There were no valuation measurement events related to the fair value of the Company's Level 3 strategic investments during the six months ended June 30, 2025 or 2024, as no impairment indicators were identified nor were similar securities issued.

The Company’s contingent consideration liability with a fair value categorized as Level 3 within the fair value hierarchy relates to the regulatory-related contingent payments to Myonexus Therapeutics, Inc. (“Myonexus”) selling shareholders as well as to an academic institution under a license agreement that meets the definition of a derivative. For more information related to Myonexus, please read *Note 3, License and Collaboration Agreements* to the financial statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2024. The contingent consideration liability was estimated using an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. Significant changes which increase or decrease the probabilities of achieving the milestone, or shorten or lengthen the time required to achieve the milestone, would result in a corresponding increase or decrease in the fair value of the liability. At the end of each reporting period, the fair value is adjusted to reflect the most current valuation assumptions through earnings. For the six months ended June 30, 2024, the Company recorded an increase of $10.1 million to account for the change in fair value of the Company's existing contingent consideration liabilities, with no similar activity for the six months ended June 30, 2025. As of June 30, 2025, the contingent consideration was recorded as a non-current liability on the Company's unaudited condensed consolidated balance sheets.

The fair value of the Company's 1.25% convertible senior notes due on September 15, 2027 (the “2027 Notes”) is based on open market trades and is classified as Level 1 in the fair value hierarchy. As of June 30, 2025 and December 31, 2024, the fair value of the 2027 Notes was $849.6 million and $1,254.9 million, respectively.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable, net and accounts payable approximated fair value because of the immediate or short-term maturity of these financial instruments.

5. CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

The following table summarizes the Company's financial assets with maturities of less than 90 days from the date of purchase included in cash equivalents in the unaudited condensed consolidated balance sheets for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Money market funds	$ 153,270	$ 455,535
Total	$ 153,270	$ 455,535

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The weighted average maturity of the Company's available-for-sale securities as of June 30, 2025 and December 31, 2024 was approximately six and eleven months, respectively. All of the Company's non-current investments as of June 30, 2025 and December 31, 2024 had maturities between one and two years.

The following tables summarize the Company's cash, cash equivalents, short-term investments and non-current investments for each of the periods indicated:

	As of June 30, 2025			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 510,598	$ —	$ —	$ 510,598
Commercial paper	3,534	—	—	3,534
Government and government agency bonds	255,122	64	(121)	255,065
Corporate bonds	55,203	25	(20)	55,208
Certificates of deposit	10,338	—	—	10,338
Total cash, cash equivalents and investments	$ 834,795	$ 89	$ (141)	$ 834,743
As reported:				
Cash and cash equivalents	$ 510,598	$ —	$ —	$ 510,598
Short-term investments	289,571	79	(109)	289,541
Non-current investments	34,626	10	(32)	34,604
Total cash, cash equivalents and investments	$ 834,795	$ 89	$ (141)	$ 834,743

	As of December 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 1,103,010	$ —	$ —	$ 1,103,010
Government and government agency bonds	280,001	227	(329)	279,899
Corporate bonds	93,816	67	(156)	93,727
Certificates of deposit	11,319	—	—	11,319
Total cash, cash equivalents and investments	$ 1,488,146	$ 294	$ (485)	$ 1,487,955
As reported:				
Cash and cash equivalents	$ 1,103,010	$ —	$ —	$ 1,103,010
Short-term investments	251,598	286	(102)	251,782
Non-current investments	133,538	8	(383)	133,163
Total cash, cash equivalents and investments	$ 1,488,146	$ 294	$ (485)	$ 1,487,955

6. PRODUCT REVENUES, NET, ACCOUNTS RECEIVABLE, NET, AND RESERVES FOR PRODUCT REVENUES

Net product revenues, which includes revenues associated with the PMO Products and ELEVIDYS, consisted of the following:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands)			
PMO Products				
United States	$ 202,922	$ 204,248	$ 388,750	$ 393,915
Rest of World	28,350	34,579	79,060	70,460
Total PMO product revenues, net	$ 231,272	$ 238,827	$ 467,810	$ 464,375
ELEVIDYS				
United States	281,851	121,721	656,836	255,657
Total ELEVIDYS product revenue, net	$ 281,851	$ 121,721	$ 656,836	$ 255,657
Total product revenues, net	$ 513,123	$ 360,548	$ 1,124,646	$ 720,032

For the three and six months ended June 30, 2025 and 2024, no individual country outside the U.S. exceeded 10% of total net product revenues.

The following table summarizes the Company's net product revenues, by customer, for those customers that exceeded 10% for the periods indicated:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
Product revenues, net				
Customer 1	25%	35%	22%	35%
Customer 2	15%	21%	13%	20%

As of June 30, 2025 and December 31, 2024, the Company's accounts receivable, net were $527.3 million and $602.0 million, respectively, both of which were related to product sales, net of discounts and allowances. As of June 30, 2025, the majority of the Company’s accounts receivable arose from product sales in the U.S. and all customers have standard payment terms that generally require payment within 65 to 90 days. Outside of the U.S., the majority of the Company’s customers have payment terms ranging between 90 and 150 days.

The following tables summarize an analysis of the change in reserves for discounts and allowances for each of the periods indicated:

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2024	$ 45,904	$ 107,843	$ 5,941	$ 34,611	$ 194,299
Provision	131,842	109,973	10,965	45,012	297,792
Adjustments relating to prior periods	—	(9,497)	—	(625)	(10,122)
Payments/credits	(130,222)	(64,359)	(11,251)	(43,412)	(249,244)
Balance, as of June 30, 2025	$ 47,524	$ 143,960	$ 5,655	$ 35,586	$ 232,725

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2023	$ 27,486	$ 98,194	$ 3,831	$ 35,261	$ 164,772
Provision	41,935	72,684	8,585	35,527	158,731
Adjustments relating to prior periods	783	(5,171)	—	(88)	(4,476)
Payments/credits	(54,393)	(62,647)	(8,462)	(33,210)	(158,712)
Balance, as of June 30, 2024	$ 15,811	$ 103,060	$ 3,954	$ 37,490	$ 160,315

The following table summarizes the total reserves above included in the Company's unaudited condensed consolidated balance sheets for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Reduction to accounts receivable, net	$ 87,450	$ 85,142
Component of accrued expenses	145,275	109,157
Total reserves	$ 232,725	$ 194,299

7. INVENTORY

The following table summarizes the components of the Company's inventory for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Raw materials	$ 314,853	$ 280,045
Work in progress	802,867	610,692
Finished goods	70,984	47,209
Total inventory	$ 1,188,704	$ 937,946

No material inventory reserves existed as of June 30, 2025 or December 31, 2024. The Company classifies inventory associated with its PMO Products as non-current inventory when consumption of the inventory is expected beyond the Company's normal PMO Product inventory operating cycle of two years. Non-current inventory consists of raw materials and work in progress associated with the PMO Products.

The following table summarizes the balance sheet classification of the Company's inventory for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Balance sheet classification		
Inventory	$ 994,036	$ 749,960
Non-current inventory	194,668	187,986
Total inventory	$ 1,188,704	$ 937,946

8. OTHER ASSETS

The following table summarizes the Company's other current assets for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Collaboration and other receivables	$ 73,256	$ 34,608
Prepaid maintenance services	15,592	13,407
Prepaid clinical and pre-clinical expenses	10,821	10,220
Tax-related receivables and prepaids	7,244	13,132
Prepaid insurance	4,113	3,668
Prepaid commercial expenses	3,729	3,371
Interest receivable	1,800	1,970
Other	11,039	10,085
Total other current assets	$ 127,594	$ 90,461

The following table summarizes the Company's other non-current assets for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Intangible assets, net	$ 30,522	$ 26,887
Manufacturing-related deposits and prepaids	25,436	25,964
Restricted cash*	15,579	15,579
Prepaid maintenance services	4,218	4,381
Other	7,382	3,394
Total other non-current assets	$ 83,137	$ 76,205

* The Company had $15.6 million in restricted cash included in other non-current assets on the Company's unaudited condensed consolidated balance sheets as of both June 30, 2025 and December 31, 2024. Restricted cash for both periods relates to (i) letters of credit established under the Company's various property leases that serve as security for potential future default of lease payments, (ii) a letter of credit established under a certain commercial supply agreement and (iii) collateralized cash for the Company's credit cards. The restricted cash is unavailable for withdrawal or use for general obligations.

9. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024
	(in thousands)	
Product revenue related reserves	$ 145,275	$ 109,157
Accrued contract manufacturing costs	59,513	77,842
Accrued employee compensation costs	55,232	91,119
Accrued income taxes	18,730	17,391
Accrued professional fees	26,611	17,691
Accrued clinical and pre-clinical costs	26,152	26,849
Accrued royalties	13,996	16,625
Accrued fixed assets	8,150	5,305
Accrued milestone and license expense	6,080	2,000
Accrued clinical collaboration costs	7,003	—
Accrued interest expense	4,727	4,192
Other	5,930	5,342
Total accrued expenses	$ 377,399	$ 373,513

10. EQUITY

In November 2024, the Board of Directors approved a share repurchase program (the "2024 Repurchase Program") of up to $500.0 million of the Company's outstanding common stock over the next 18 months. Correspondingly, the Company entered into a Rule 10b-18 repurchase plan that allows it to conduct open market repurchases periodically up to the remaining authorization under the 2024 Repurchase Program.

During the three and six months ended June 30, 2025, the Company purchased a total of 650,876 shares of common stock under the 2024 Repurchase Program at an average price of $38.41 per share for a total cost of $25.3 million, inclusive of approximately $0.3 million in excise taxes and other fees. As of June 30, 2025, the remaining amount authorized under the 2024 Repurchase Program was $475.0 million.

Repurchased shares are held as treasury stock. The amount paid to repurchase shares is recorded as a reduction to stockholders' equity. As treasury stock is not considered outstanding, these shares are excluded from weighted average common stock outstanding for both basic and diluted earnings per share.

11. STOCK-BASED COMPENSATION

The following table summarizes the Company's stock awards granted for each of the periods indicated:

	For the Three Months Ended June 30,				For the Six Months Ended June 30,			
	2025		2024		2025		2024	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	22,016	$ 13.98	5,100	$ 72.37	488,192	$ 46.93	321,478	$ 67.63
Restricted stock units	317,317	$ 17.10	54,905 [(2)]	$ 138.91	1,815,501 [(1)]	$ 83.72	1,216,890 [(2)]	$ 129.05

[(1)] Included in restricted stock units ("RSUs") for the six months ended June 30, 2025 are 120,378 shares with performance conditions which are related to the achievement of certain financial performance goals, regulatory development and approval of certain of the Company's product candidates and certain manufacturing achievements. As of June 30, 2025, none of the performance conditions were probable of being achieved. Stock options and the remaining RSUs granted during the three and six months ended June 30, 2025 have only service-based criteria and vest over four years.

[(2)] Included in RSUs for the three and six months ended June 30, 2024 are 8,860 and 97,460 shares with performance conditions (the "March 2024 PSUs"), respectively, which are related to the achievement of certain financial performance goals and regulatory approval of certain of the Company's product candidates. The expanded regulatory approval of ELEVIDYS in June 2024 resulted in the cliff-vesting of 44,300 shares during the year ended December 31, 2024. The achievement of certain financial performance targets during the three months ended June 30, 2025 resulted in 38,050 shares of the March 2024 PSUs becoming eligible for vesting, which is contingent on the fulfillment of remaining service conditions. As of June 30, 2025, none of the remaining performance conditions associated with the March 2024 PSUs were probable of being achieved.

Stock-based Compensation Expense

For the three months ended June 30, 2025 and 2024, total stock-based compensation expense was $37.0 million and $50.5 million, respectively. For the six months ended June 30, 2025 and 2024, total stock-based compensation expense was $78.5 million and $91.2 million, respectively.

The following table summarizes stock-based compensation expense by grant type and by function included within the unaudited condensed consolidated statements of comprehensive income (loss):

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands)			
Stock options	$ 11,689	$ 15,515	$ 24,578	$ 31,796
Restricted stock units	28,114	37,808	59,657	65,335
Employee stock purchase plan	1,815	2,331	3,939	3,728
Subtotal	$ 41,618	$ 55,654	$ 88,174	$ 100,859
Capitalized stock-based compensation costs	(4,593)	(5,172)	(9,721)	(9,685)
Total stock-based compensation expense included in expenses	$ 37,025	$ 50,482	$ 78,453	$ 91,174
Research and development	15,277	19,806	32,594	36,079
Selling, general and administrative	21,748	30,676	45,859	55,095
Total stock-based compensation expense included in expenses	$ 37,025	$ 50,482	$ 78,453	$ 91,174

12. OTHER INCOME (LOSS), NET

The following table summarizes other income (loss), net for each of the periods indicated:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands)			
Interest income	$ 5,254	$ 8,076	$ 15,444	$ 15,169
Accretion of investment discount, net	1,887	10,766	4,125	23,570
Gain (loss) on strategic investments	36,721	148	(54,007)	1,079
Interest expense	(5,228)	(4,832)	(9,731)	(8,998)
Change in fair value of derivatives*	—	—	—	(10,100)
Other, net	(573)	120	(902)	101
Total other income (expense), net	$ 38,061	$ 14,278	$ (45,071)	$ 20,821

*Related to the change in fair value of the contingent consideration derivative liabilities. For more information, please read *Note 4, Fair Value Measurements*.

13. INCOME TAXES

During the three and six months ended June 30, 2025, the Company recorded an income tax (benefit) expense of $(43.3) million and $20.7 million, representing an effective tax rate of (28.2)% and (9.0)%, respectively. During the three and six months ended June 30, 2024, the Company recorded an income tax expense of $7.1 million and $12.4 million, representing an effective tax rate of 8.4% and 22.6%, respectively. The income tax (benefit) expense, for each of the periods, primarily relates to the current tax provision on taxable profits, including in certain states which restrict the amount of net operating loss carryforwards which may be utilized to offset taxable income and the requirement to capitalize research and development costs for tax purposes.

On a periodic basis, the Company reassesses the valuation allowance on its deferred tax assets, weighing positive and negative evidence to assess the recoverability of such deferred tax assets. In assessing the Company's ability to realize its net deferred tax assets, the Company considered various factors, including future reversals of existing taxable temporary differences, projected future taxable income, potential carryback claims and tax planning strategies to determine whether it is more likely than not that some portion or all of its net deferred tax assets will not be realized. Based upon these factors, the Company has determined that the uncertainty regarding the realization of these assets is sufficient to warrant the need to maintain a full valuation allowance against its net deferred tax assets as of June 30, 2025. It is possible the Company may release a portion or all of its valuation allowance in future periods. The release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, the Company's level of profitability, revenue growth, and expectations regarding future profitability. Release of the valuation allowance would result in a benefit to income tax expense for the period the release is recorded, which could have a material impact on net earnings. The total deferred tax asset balance subject to the valuation allowance was approximately $1,131.8 million as of June 30, 2025. The Company will continue to assess the realizability of its deferred tax assets on a quarterly basis.

For tax years beginning on or after January 1, 2022, the Tax Cuts and Jobs Act of 2017 eliminates the option to currently deduct research and development expenses and requires taxpayers to capitalize and amortize the costs over five years for research activities performed in the U.S. and 15 years for research activities performed outside the U.S.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. Management is currently evaluating the financial impact of the OBBBA. Please refer to *Note 18, Subsequent Events* for further discussion over the bill.

14. REVOLVING CREDIT FACILITY

On February 13, 2025 (the "Closing Date"), the Company entered into a credit agreement (the "Credit Agreement") with JPMorgan Chase Bank, N.A., as administrative agent (the "Administrative Agent") and as collateral agent and the lenders party thereto. The Credit Agreement provides for a five-year, $600.0 million senior secured revolving credit facility (the "Revolving Credit Facility"). The Company's obligations under the Credit Agreement are secured by substantially all of the Company's assets and the assets of certain wholly-owned material subsidiaries, subject to certain customary exceptions and exclusions.

Interest rates under the Revolving Credit Facility are variable and equal to the Secured Overnight Financing Rate plus a credit spread adjustment of 0.10% per annum ("Adjusted SOFR"), plus a margin of 1.125% to 1.75% per annum, or, at the Company's option, at a base reference rate equal to the highest of (a) the federal funds rate plus 0.50%, (b) the rate of interest last quoted by the Administrative Agent as its "base rate" and (c) the one-month Adjusted SOFR rate plus 1.00%, plus a margin of 0.125% to 0.75% per annum. The Company also will pay an unused commitment fee ranging from 0.20% to 0.35% per annum on the unused commitments.

The Credit Agreement contains customary representations and warranties, affirmative covenants, negative covenants, conditions and events of default. The Credit Agreement also contains financial covenants that are assessed on the last day of each of the Company's fiscal quarters, including certain financial ratios such as a maximum secured net leverage ratio and minimum consolidated interest coverage ratio. The Company may voluntarily prepay the outstanding revolving loans under the Revolving Credit Facility in whole or in part without premium or penalty provided that the prepayment shall be in certain amounts as specified therein.

The Company paid $3.2 million in arrangement and up-front fees on the Closing Date. The arrangement and up-front fees associated with the Revolving Credit Facility are being amortized over the five-year term of the Revolving Credit Facility. As of June 30, 2025, there were no amounts outstanding under the Revolving Credit Facility and the Company was in compliance with the covenants described above.

15. EARNINGS (LOSS) PER SHARE

Basic earnings (loss) per share is computed by dividing net income (loss) by the weighted-average number of shares of common stock outstanding. Diluted earnings per share is computed based on the treasury stock method for stock awards and the if-converted method for convertible debt by dividing net income by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company recorded a net loss for the six months ended June 30, 2025, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and are, therefore, excluded from the diluted net loss per share calculation.

The following table sets forth the computation of basic and diluted earnings (loss) per common share:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands, except per share amounts)			
Numerator:				
Net income (loss) - basic	$ 196,892	$ 6,460	$ (250,616)	$ 42,579
Add: interest expense, net of tax, on the Company's convertible debt	4,303	340	—	769
Net income (loss) - diluted	$ 201,195	$ 6,800	$ (250,616)	$ 43,348
Denominator:				
Weighted-average common shares outstanding, basic	98,005	94,618	97,685	94,305
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	518	3,095	—	3,388
Common stock issuable under the Company's convertible debt	8,100	1,431	—	1,436
Weighted-average common shares outstanding, diluted	106,623	99,144	97,685	99,129
Earnings (loss) per common share, basic	$ 2.01	$ 0.07	$ (2.57)	$ 0.45
Earnings (loss) per common share, diluted	$ 1.89	$ 0.07	$ (2.57)	$ 0.44

The following table summarizes potential shares of common stock that were excluded from the computation of diluted earnings per share as they were anti-dilutive:

	As of June 30, 2025	As of June 30, 2024
	(in thousands)	
Common stock issuable under the Company's equity incentive plans	8,308	2,879 [(1)]
Common stock issuable under the Company's convertible debt	—	8,100
Total number of potentially issuable common stock	8,308	10,979

[(1)] As of June 30, 2024, the anti-dilutive common stock issuable under the Company's equity incentive plans excludes 1.2 million shares that are dilutive but have performance or market conditions that were not met as of the end of the period.

16. SEGMENT INFORMATION

The measure of segment profit or loss that the CEO uses to allocate resources and assess performance as the chief operating decision-maker ("CODM") is the Company's consolidated net income (loss). The table below includes information about the Company's segment, including significant segment expenses, and a reconciliation to net income (loss):

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
	(in thousands)			
Total revenues	$ 611,091	$ 362,931	$ 1,355,947	$ 776,395
Segment expenses and other segment items				
Cost of sales (excluding amortization of in-licensed rights)	152,558	44,545	290,122	95,104
Compensation and other personnel expenses	89,839	77,535	182,923	161,122
Up-front and milestone expenses	—	—	583,787	—
Manufacturing expenses	80,685	50,659	148,150	120,359
Clinical trial expenses	33,511	39,861	66,658	84,008
Facility- and technology-related expenses (excluding depreciation and amortization)	28,580	26,651	56,237	51,629
Research and development- other (excluding non-cash items) (a)	31,843	27,646	60,314	56,494
Selling, general and administrative- other (excluding non-cash items) (b)	56,322	55,474	107,464	104,436
Roche collaboration reimbursement	(25,689)	(17,940)	(54,170)	(39,598)
Other segment items (c)	(1,314)	(10,886)	(3,223)	(13,571)
(Gain) loss on strategic investments	(36,721)	(148)	54,007	(1,079)
Interest expense	5,228	4,832	9,731	8,998
Interest income	(5,254)	(8,076)	(15,444)	(15,169)
Income tax (benefit) expense	(43,254)	7,117	20,736	12,446
Depreciation and amortization expense	10,840	8,719	20,818	17,463
Stock-based compensation expense	37,025	50,482	78,453	91,174
Segment net income (loss)	$ 196,892	$ 6,460	$ (250,616)	$ 42,579
Reconciliation of profit or loss				
Adjustments and reconciling items	—	—	—	—
Consolidated net income (loss)	$ 196,892	$ 6,460	$ (250,616)	$ 42,579

(a) Research and development-other includes professional services, pre-clinical expenses and research and other expenses.
(b) Selling, general and administrative-other includes professional services and other expenses.
(c) Other segment items included in segment net income (loss) include accretion of investment discount, net, change in fair value of derivatives and other, net, as well as the items separately presented and not defined as significant expenses below.

Significant expense categories that are regularly provided to the CODM include cost of sales (excluding amortization of in-licensed rights), compensation and other personnel expenses, up-front and milestone expenses, manufacturing expenses, clinical trial expenses, facility- and technology-related expenses, research and development- other and selling, general and administrative- other. The other expense or income information are other segment items and include separate presentation of (gain) loss on strategic investments, interest expense, interest income, income tax (benefit) expense, depreciation and amortization and stock-based compensation, which are included in the measure of segment net income (loss), but are not significant segment expenses.

Assets provided to the CODM for the single segment are consistent with those reported on the unaudited condensed consolidated balance sheets.

17. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

The following table summarizes the aggregate non-cancelable contractual obligations arising from the Company's manufacturing obligations:

	As of June 30, 2025**
	(in thousands)
2025 (July-December)	$ 626,052
2026	320,563
2027	81,604
2028	68,772
Total manufacturing commitments*	$ 1,096,991

*Total manufacturing commitments include the Catalent, Inc. ("Catalent") manufacturing and supply agreement, for which the Company has ROU assets and lease liabilities recorded on the unaudited condensed consolidated balance sheets as of June 30, 2025. For more information, please read *Note 22, Commitments and Contingencies* to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024.

**Total manufacturing commitments include obligations related to the global supply of ELEVIDYS, including inventories necessary to supply Roche for sales of ELEVIDYS in territories outside of the U.S. where Roche has received certain approvals for ELEVIDYS.

Thermo Fisher Scientific, Inc.

The Company entered into a development, commercial manufacturing, and supply agreement as related to the Company's adherent manufacturing process for its gene therapy programs in June 2018 and, subsequently, entered into the first, second, third and fourth amendments in May 2019, July 2020, October 2021 and March 2023, respectively, with Brammer Bio MA, LLC ("Brammer"), an affiliate of Thermo Fisher Scientific, Inc. ("Thermo") (collectively, the "Thermo Agreement").

On July 18, 2024, the Company issued a termination notice to Thermo to terminate the Thermo Agreement. The termination was effective as of August 21, 2024.

On December 20, 2024, Brammer filed an arbitration demand against the Company relating to the Company's termination of the Thermo Agreement. Brammer alleged claims for breach of contract, breach of the implied covenant of good faith and fair dealing and a violation of MGL c. 93A, and sought relief including damages, treble damages, and attorneys' fees and costs. On January 24, 2025, Sarepta filed its answer and asserted counterclaims for declaratory judgment and breach of contract, seeking relief including damages and attorneys' fees and costs.

On July 12, 2025, the Company entered into a settlement agreement with Brammer to resolve the outstanding claims related to the termination of the Thermo Agreement (the "Brammer Settlement"). Under the Brammer Settlement, the Company agreed to pay Brammer an aggregate amount of $13.0 million. Upon payment, both parties are required to take all necessary action to dismiss any arbitration. The Brammer Settlement is recorded in the Company's unaudited condensed consolidated statements of comprehensive income (loss) within research and development expense for the three and six months ended June 30, 2025.

Litigation

In the normal course of business, the Company from time to time is named as a party to various legal claims, actions and complaints, which have included and may include matters involving securities, employment, intellectual property, arising from the use of therapeutics utilizing its technology, or others. The Company records a loss contingency reserve for a legal proceeding when it considers the potential loss probable and it can reasonably estimate the amount of the loss or determine a probable range of loss. The Company provides disclosure when it considers a loss reasonably possible or when it determines that a loss in excess of a reserve is reasonably possible. The Company provides an estimate of such reasonably possible losses or an aggregate range of such reasonably possible losses, unless the Company believes that such an estimate cannot be made. The Company has not recorded any material accruals for loss contingencies, and in management's opinion, no material range of loss is estimable for the matters described below as of June 30, 2025.

On September 15, 2020, REGENXBIO INC. ("Regenx") and the Trustees of the University of Pennsylvania ("U-Penn") filed a lawsuit against the Company and Sarepta Therapeutics Three, LLC, in the U.S. District Court for the District of Delaware. The plaintiffs assert patent infringement of U.S. Patent No. 10,526,617 ("the '617 Patent") under 35 U.S.C.§§ 271(a)-(c) based on Sarepta's alleged direct or indirect manufacture and use of the patented cultured host cell technology allegedly used to make AAV gene therapy products, including SRP-9001 (approved June 22, 2023 in the U.S. as ELEVIDYS®). Specifically, the Complaint essentially includes the allegation that Sarepta's use, and the use by its contract manufacturers on its behalf, of a host cell containing a

recombinant acid molecule that encodes a capsid protein having at least 95% amino acid identity to AAVrh10 infringes the ’617 Patent asserted by Regenx. Plaintiffs seek injunctive relief, a judgment of infringement and willful infringement, damages that are no less than a reasonable royalty (treble damages), attorneys’ fees and costs, and such other relief as the court deems just and proper. On January 5, 2024, the Court granted Sarepta’s motion for summary judgment on the grounds that the asserted claims of Regenx’s ’617 Patent are invalid because they cover patent ineligible subject matter under 35 U.S.C. § 101. On January 12, 2024, the Court entered judgment and closed the case. Plaintiffs have appealed to the U.S. Court of Appeals for the Federal Circuit.

On June 20, 2023, Regenx and U-Penn commenced a second patent infringement lawsuit against Sarepta and its contract manufacturer, Catalent, asserting patent alleged infringement of U. S. Patent No. 11,680,274 (“the ’274 Patent”). In the second lawsuit, Regenx and U-Penn allege that Sarepta and Catalent’s manufacture, use and commercial launch of ELEVIDYS® (formerly/also known as SRP-9001) infringe the ’274 Patent. Sarepta answered the complaint on August 10, 2023. On February 21, 2024, Sarepta submitted a petition for Inter Partes Review for filing with the Patent Trial and Appeal Board (“PTAB”) at the U.S. Patent and Trademark Office (“USPTO”). The petition seeks to invalidate the ’274 Patent. On March 20, 2024, the court in the patent infringement litigation ordered that the case be stayed pending final resolution of the Inter Partes Review proceedings. On August 22, 2024, the PTAB instituted inter partes review of all challenged claims of the ’274 Patent on all asserted grounds. Oral argument in the PTAB proceedings was held on May 28, 2025, and the PTAB’s final written decision is expected in August 2025.

On July 13, 2021, Nippon Shinyaku Co., Ltd. (“Nippon Shinyaku” or “NS”) filed a lawsuit against the Company in the U.S. District Court for the District of Delaware. NS asserted a claim for breach of contract arising from Sarepta filing seven petitions for Inter Partes Review (“IPR Petitions”) with the PTAB at the USPTO, in which Sarepta sought to invalidate certain NS patents concerning exon 53 skipping technology (U.S. Patent Nos. 9,708,361, 10,385,092, 10,407,461, 10,487,106, 10,647,741, 10,662,217, and 10,683,322, respectively, and collectively the “NS Patents”). In addition, NS asserted claims for patent infringement and willful infringement of each of the NS Patents allegedly arising from Sarepta’s activities, including the sale of, its exon 53 skipping product, VYONDYS 53 (golodirsen). NS further sought a determination of non-infringement by NS alleged to arise from NS’s activities, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and invalidity of certain patents licensed to the Company from UWA (U.S. Patent Nos. 9,994,851, 10,227,590, and 10,266,827, collectively the “UWA Patents”). In its complaint, NS sought legal fees and costs, an unspecified amount of monetary relief (treble damages) attributed to Sarepta’s alleged infringement, and such other relief as the court deems just and proper. In January 2022, the PTAB granted institution of all claims of all NS Patents in response to Sarepta’s IPR Petitions and determined that Sarepta demonstrated a reasonable likelihood of success in proving that the NS Patents are unpatentable. NS filed a motion for preliminary injunction solely seeking Sarepta’s withdrawal of the IPR Petitions, which was ultimately granted after the U.S. Court of Appeals for the Federal Circuit reversed and remanded to the district court on February 8, 2022. Sarepta subsequently withdrew the IPRs, which were terminated on June 14, 2022. On December 27, 2021, the district court partially granted and denied the motion to dismiss by Sarepta and ordered NS to file a Second Amended Complaint (“SAC”), which it did on January 14, 2022. In the SAC, NS maintained all claims of the original complaint of July 13, 2021, except a determination of non-infringement of the UWA Patents. On January 28, 2022, Sarepta filed its answer to the SAC, with defenses and counterclaims against NS and NS Pharma Inc. that include infringement of the UWA Patents allegedly arising from their activities concerning, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and breach of contract. Sarepta also sought a determination of invalidity of the NS Patents. In its counterclaim complaint, Sarepta sought an award of relief in its defenses to NS’ allegations, a judgment of breach of contract, a determination of invalidity of the NS Patents, a judgment of infringement and willful infringement of the UWA Patents, legal fees and costs, an unspecified amount of monetary relief (treble damages) attributable to NS’ alleged infringement, and such other relief as the court deems just and proper. UWA has since been joined as a Plaintiff in Sarepta’s counterclaims against NS. On August 14, 2023, the Court granted cross motions to amend the pleadings, allowing Sarepta to add a counterclaim against NS for inequitable conduct, and NS to add counterclaims against Sarepta for inequitable conduct and Walker Process fraud. The parties have since stipulated to the dismissal of NS’s claim of infringement of its ’361 Patent and certain claims of the ’322 Patent, and NS’s breach of contract claim. The Court bifurcated the Walker Process fraud claim on April 18, 2024, and granted Sarepta's motion for summary judgment of infringement of the ’851 Patent and NS’s motion for partial summary judgment of infringement of certain NS patents on May 1, 2024. After a jury trial in December 2024, the jury found that NS’s ’092 Patent is invalid as obvious and Sarepta’s and UWA’s ’851 Patent is not invalid. The jury did not find that NS’s infringement was willful. The jury awarded Sarepta approximately $115.2 million in damages for NS’s infringement relating to its sales of Viltepso in the United States, and the parties stipulated to approximately $0.8 million in reasonable royalty damages for NS's sales of Viltepso outside of the United States, both through December 15, 2024. Judgment was entered on January 7, 2025. On February 14, 2025, Sarepta filed a motion seeking supplemental damages and NS filed post-trial motions challenging the jury's verdict, as well as briefing relating to its inequitable conduct claim, which was tried to the court in December 2024. Those motions remain pending.

On July 26, 2024, Genzyme Corporation filed a lawsuit against Sarepta Therapeutics, Inc. and Sarepta Therapeutics Three, LLC, in the U.S. District Court for the District of Delaware. The complaint asserts infringement of United States Patent Nos. 9,051,542 (the “’542 Patent”) and 7,704,721 (the “’721 Patent”) arising from Sarepta’s alleged manufacture and sale of ELEVIDYS® (delandistrogene moxeparvovec-rokl). In its complaint, Genzyme seeks, inter alia, damages for the alleged infringement, including increased damages up to three times the amount found or assessed, together with prejudgment and post-judgment interest and costs. Following a partial motion to dismiss by Sarepta, Genzyme filed its First Amended Complaint on November 21, 2024. In its First Amended Complaint, Genzyme no longer alleges willfulness or indirect infringement before the filing of the complaint. Sarepta

answered the First Amended Complaint on December 12, 2024. On May 27, 2025, the court subsequently granted Genzyme's motion to amend the complaint to include new allegations of infringement related to five patents: United States Patent Nos. 12,031,894 (the "'894 Patent"), 12,013,326 (the "'326 Patent"), 11,698,377 (the "'377 Patent"), 12,123,880 (the "'880 Patent"), and 12,298,313 (the "'313 Patent"). The Court also entered an amended scheduling order, with trial scheduled for June 14, 2027. Sarepta answered the Second Amended Complaint on June 17, 2025.

On December 20, 2024, Brammer filed an arbitration demand against the Company relating to the Company's termination of the Thermo Agreement. Brammer alleged claims for breach of contract, breach of the implied covenant of good faith and fair dealing and a violation of MGL c. 93A, and seeks relief including damages, treble damages, and attorneys' fees and costs. On January 24, 2025, Sarepta filed its answer and asserted counterclaims for declaratory judgment and breach of contract, seeking relief including damages and attorneys' fees and costs. On July 12, 2025, the parties entered into the Brammer Settlement, pursuant to which the Company agreed to pay Brammer an aggregate amount of $13.0 million. Upon payment, both parties are required to take all necessary action to dismiss any arbitration.

On June 26, 2025, a putative securities class action complaint was filed against the Company, Chief Executive Officer Douglas Ingram, former Chief Customer Officer Dallan Murray, and President of Research and Development and Technical Operations Louise Rodino-Klapac in the U.S. District Court for the Southern District of New York (the "Securities Action"). The complaint alleges violations of the Securities Exchange Act of 1934 (the "Exchange Act") and Rule 10b-5 in connection with disclosures made regarding ELEVIDYS's safety and efficacy and the Company's financial statements and projections. The plaintiff seeks to represent a class of shareholders who purchased or otherwise acquired the Company's securities between June 22, 2023 and June 24, 2025. The complaint seeks unspecified damages.

On July 15, 2025, a shareholder derivative lawsuit concerning substantially the same disclosures underlying the Securities Action (the "Derivative Action") was filed in the U.S. District Court for the Southern District of New York, naming Company directors M. Kathleen Behrens, Richard J. Barry, Kathryn Boor, Michael Chambers, Deirdre Connelly, Stephen L. Mayo, Claude Nicaise, and Hans Wigzell, as well as Douglas Ingram, Dallan Murray, and Louise Rodino-Klapac (the "Individual Defendants"). The Company is named as a nominal defendant. The Derivative Action alleges, among other claims, breaches of the Individual Defendants' fiduciary duties in connection with substantially similar disclosures at issue in the Securities Action and violations of Section 14(a) of the Exchange Act in connection with the Company's 2024 and 2025 annual proxy statements. The Derivative Action seeks unspecified damages, including costs incurred in defending the Company against the Securities Action. The Derivative Action also seeks an order that the Company take all necessary action to reform and improve its corporate governance and internal procedures.

18. SUBSEQUENT EVENTS

One Big Beautiful Bill Act

On July 4, 2025, the OBBBA was enacted in the U.S. The OBBBA includes provisions that could have a significant impact on the Company, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. The Company is currently evaluating the financial impact of the OBBBA.

Restructuring Plan

On July 16, 2025, the Company announced its plan to reprioritize its pipeline and reduce its workforce, representing approximately 36% of the Company's workforce (the "Restructuring"). As a result of this reduction in force, the Company estimates that it will record a charge in the range of $32.0 million to $37.0 million during the three and nine months ended September 30, 2025, related to employee termination benefits, including severance, all of which is anticipated to result in cash expenditures. The Company expects the reduction in force to be substantially complete by the end of the third quarter of 2025.

LGMD FDA Clinical Hold

On July 21, 2025, the Company announced that the FDA placed a clinical hold on the Company's investigational gene therapy clinical trials for LGMD (the "LGMD Clinical Hold"). The LGMD Clinical Hold includes the Company's LGMD clinical trials related to its product candidates SRP-9003 (LGMD2E/R4/bidridistrogene xeboparvovec), SRP-9004 (LGMD2D/patidistrogene bexoparvovec), SRP-6004 (LGMD2B/R2) and SRP-9005 (LGMD2C/R5 g-sarcoglycan). The Company previously announced on July 16, 2025, that it had suspended the development of each of the LGMD programs mentioned above as part of the Restructuring, with the exception of SRP-9003. As of June 30, 2025, there were $47.4 million of regulatory-related contingent payments included in contingent consideration liability on the unaudited condensed consolidated balance sheets, of which $47.1 million relates to regulatory-related contingent payments to Myonexus' selling shareholders associated with the Company's LGMD product candidates. The Company is currently evaluating the financial impact of the LGMD Clinical Hold on its contingent consideration liabilities.

Milestone Payment to Arrowhead

On July 28, 2025, the Company recognized a $100.0 million milestone payment obligation to Arrowhead pursuant to the terms of the Arrowhead Agreement (the "Arrowhead Milestone"). The Arrowhead Milestone was triggered by Arrowhead's achievement of the first of two predetermined enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 study for an individual program. In accordance with the Arrowhead Agreement, the Company is required to remit the milestone payment within 60 days of achievement of the Arrowhead Milestone. The Arrowhead Milestone will be recorded in the Company's unaudited condensed consolidated statements of comprehensive income (loss) within research and development expense for the three and nine months ended September 30, 2025.

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

The purpose of Management's Discussion and Analysis of Financial Condition and Results of Operations is to provide an understanding of the financial condition, changes in financial condition and results of operations of Sarepta Therapeutics, Inc. This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2024 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This Quarterly Report on Form 10-Q contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our belief that our proprietary technology platforms and collaborations can be used to develop potential therapeutic candidates to treat a broad range of diseases;*
- *our expectation that our partnerships with manufacturers will support our clinical and commercial manufacturing capacity for our Duchenne muscular dystrophy ("Duchenne") gene therapy programs and Limb-girdle muscular dystrophy ("LGMD") programs, while also acting as a manufacturing platform for potential future gene therapy programs, and our belief that our current network of manufacturing partners is able to fulfill the requirements of our commercial plan;*
- *the possible impacts of the ELEVIDYS Suspension (as defined herein);*
- *the possible impacts of the clinical hold the U.S. Food and Drug Administration (the "FDA") has placed on our investigational use gene therapy clinical trials for LGMD in July 2025 and the revocation of the platform technology designation for our AAVrh74 platform technology previously granted on June 2, 2025;*
- *the estimated impacts of the strategic restructuring plan announced in July 2025;*
- *our expectation that our partnership with Catalent, Inc. ("Catalent") will support our clinical and commercial manufacturing demand for our Duchenne gene therapy program and LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs;*
- *our expectation that Aldevron LLC ("Aldevron") will provide Good Manufacturing Processes ("GMP")-grade plasmid for our Duchenne gene therapy program and our SRP-9003 LGMD program, as well as plasmid source material for any future gene therapy programs;*
- *the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business as well as the development of our product candidates and our financial and contractual obligations;*
- *estimated timelines and milestones for the remainder of 2025 and beyond, including seeking to discuss with the FDA in 2025 a SRP-9003 biologics license application ("BLA");*
- *our engagement with regulatory authorities outside of the U.S. including the European Medicines Agency (the "EMA");*
- *our plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved;*
- *our plan to expand our pipeline through internal research and development and through strategic transactions;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for our products in confirmatory trials;*
- *our ability to further secure long-term supply of our commercial products and our product candidates to satisfy our planned commercial, early access programs ("EAP") and clinical needs;*
- *the possible impact of any executive, legislative or regulatory action and competing products on the commercial success of our products and our product candidates and our ability to compete against such products;*
- *our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;*

- *our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future;*
- *our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*
- *the potential benefits of our technologies and programs, including those with strategic partners;*
- *our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*
- *our expectation regarding the impact of environmental laws and regulations on our business; and*
- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this Quarterly Report on Form 10-Q after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission (the "SEC"). We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements, including the risks, uncertainties and assumptions identified under the heading "Risk Factors" in this Quarterly Report on Form 10-Q.

Overview

We are a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, siRNA knockdown therapies, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying our proprietary, highly differentiated and innovative technologies, and through collaborations with our strategic partners, we have developed multiple approved products for the treatment of Duchenne and are developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne and LGMD, as well as those through our partnered programs with Arrowhead Therapeutics Inc. ("Arrowhead"), including Facioscapulohumeral muscular dystrophy ("FSHD"), myotonic dystrophy type 1 ("DM1"), Spinocerebellar ataxia type 2 ("SCA2"), Idiopathic Pulmonary Fibrosis ("IPF"), Huntington's disease and other neuromuscular and skeletal diseases.

We commercialized four products that have been approved by the FDA:

- The PMO Products:
    - EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), granted accelerated approval by the FDA on September 19, 2016, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51 skipping. EXONDYS 51 uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene.
    - VYONDYS 53 (golodirsen) Injection ("VYONDYS 53"), granted accelerated approval by the FDA on December 12, 2019, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping. VYONDYS 53 uses our PMO chemistry and exon-skipping technology to skip exon 53 of the dystrophin gene.
    - AMONDYS 45 (casimersen) Injection ("AMONDYS 45"), granted accelerated approval by the FDA on February 25, 2021, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping. AMONDYS 45 uses our PMO chemistry and exon-skipping technology to skip exon 45 of the dystrophin gene.
- ELEVIDYS (delandistrogene moxeparvovec-rokl), an AAV-based gene therapy, was approved by the FDA on June 20, 2024 for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene as well as non-ambulatory patients under the accelerated approval pathway. ELEVIDYS was previously granted accelerated approval by the FDA on June 22, 2023 for the treatment of ambulatory patients aged four through five years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene. In response to safety events announced in March and June 2025, in June 2025, we suspended all shipments of ELEVIDYS to

non-ambulatory patients in the U.S. In response to a request from FDA that we voluntarily stop all shipments of ELEVIDYS in the U.S., we temporarily suspended all shipments of ELEVIDYS in the U.S., effective July 22, 2025, to allow us the necessary time to respond to FDA's requests for information and complete a labeling supplement process (the "ELEVIDYS Suspension"). On July 28, 2025, the FDA informed us that it recommended the removal of the voluntary hold for ambulatory patients. As of the date of this Quarterly Report, we have resumed shipments of ELEVIDYS for ambulatory patients in the U.S. We remain in ongoing discussions with FDA regarding the labeling supplement process for ELEVIDYS, including a boxed warning for acute liver injury ("ALI") and acute liver failure ("ALF").

We are in the process of conducting various clinical trials for our approved products, including studies that are required to comply with our post-marketing FDA requirements/commitments to verify and describe the clinical benefit of these products.

Our pipeline includes programs at various stages of discovery, pre-clinical and clinical development. Through our collaborations with our strategic partners, we are expanding into adjacent therapeutic areas. Our pipeline reflects our aspiration to apply our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

In July 2025, we announced a strategic restructuring plan designed to enhance financial flexibility and meet our 2027 financial obligations, including a revised program portfolio that prioritizes our siRNA platform assets. A summary description of our key product candidates, including those in collaboration with our strategic partners, is as follows:

- *SRP-9003 (LGMD, gene therapy program).* We are developing gene therapy programs for various forms of LGMD. The most advanced of our LGMD product candidates, SRP-9003, aims to treat LGMD2E, also known as beta-sarcoglycanopathy, a severe and debilitating form of LGMD characterized by progressive muscle fiber loss, inflammation and muscle fiber replacement with fat and fibrotic tissue. SRP-9003 is designed to transfect a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. SRP-9003 has generated positive pre-clinical safety and efficacy data utilizing the AAVrh.74 vector, the same vector used in our SRP-9001 gene therapy program. A Phase 1/2a trial of SRP-9003 commenced in the fourth quarter of 2018. In June 2020, we announced safety and expression results from three clinical trial participants in the high-dose cohort measured at 60 days, and one-year functional data from three clinical trial participants in the low-dose cohort. In March 2022, we announced 36-month functional data from three clinical trial participants in the low-dose cohort and 24-month functional data from two clinical trial participants in the high-dose cohort. In December 2024, we announced that we had completed enrollment and dosing in EMERGENE (Study SRP-9003-301), a Phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec).

  On July 21, 2025, we announced that the FDA placed a clinical hold on our investigational use gene therapy clinical trials for LGMD, including our trials for product candidates SRP-9003 (LGMD2E/R4/bidridistrogene xeboparvovec), SRP-9004 (LGMD2D/patidistrogene bexoparvovec), SRP-6004 (LGMD2B/R2) and SRP-9005 (LGMD2C/R5 g-sarcoglycan), following the death of a patient in our Phase 1/2 LGMD clinical trial for SRP-9004. We previously announced on July 16, 2025 that we had suspended each of the LGMD programs mentioned above as part of the Restructuring, with the exception of SRP-9003. We intend to seek to discuss with FDA in 2025 the timing of a BLA submission for SRP-9003.

- *SRP-1003 (DM1)*. DM1 is an autosomal dominant, debilitating, chronic progressive multisystem disorder characterized by an expansion of a highly unstable CUG_{exp} in the DMPK gene. Patients with DM1 have muscle weakness and wasting, myotonia, cataracts, and often have cardiac conduction abnormalities, and may become physically disabled and have a shortened life span. SRP-1003 is designed to reduce expression of the dystrophia myotonica protein kinase (DMPK) gene. There is currently no approved disease-modifying therapy for DM1. We are currently investigating SRP-1003 in a Phase 1/2a clinical trial.

- *SRP-1001 (FSHD)*. FSHD is a rare genetic disease in which the body is unable to maintain complete epigenetic suppression of DUX4 expression in differentiated skeletal muscle, leading to overexpression of DUX4, which is myotoxic and can lead to muscle degeneration. SRP-1001 is designed to selectively target and knockdown DUX4 using RNAi, with the goal of preventing or reversing downstream myotoxicity and lead to muscle repair and improvement in muscle function in patients. There are currently no cures or approved disease-modifying treatments for FSHD. We are currently investigating SRP-1001 in a Phase 1/2a clinical trial.

Manufacturing, Supply and Distribution

We have developed proprietary state-of-the-art Chemistry, Manufacturing and Controls (“CMC”) capabilities that allow manufacturing and testing of our products and product candidates to support both clinical development and commercialization. We continue to refine and optimize our manufacturing processes and test methods. We have entered into certain manufacturing and supply

arrangements with third-party suppliers which will in part utilize these capabilities to support production of certain of our product candidates and their components. We have also opened facilities over the past several years that significantly enhanced our internal research and development capabilities. However, we currently do not have internal GMP manufacturing capabilities to produce our products and product candidates for commercial and/or clinical use. For our current and future manufacturing needs, we have entered into supply agreements with specialized contract manufacturing organizations (each a "CMO") to produce custom raw materials, the active pharmaceutical ingredients ("APIs"), drug product and finished goods for our products and product candidates for both commercial and clinical use. All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing our specific technology.

For our commercial Duchenne PMO program, we have worked with our existing CMOs to increase production capacity from mid-scale to large-scale. While there is a limited number of companies that can produce raw materials and APIs in the quantities and with the quality and purity that we require for our commercial products, based on our diligence to date, we believe our current network of CMOs is able to fulfill these requirements, and is capable of expanding capacity as needed. Additionally, we have evaluated, and will continue to evaluate further relationships with additional suppliers to increase overall capacity as well as further reduce risks associated with relying on a limited number of suppliers for manufacturing.

Our gene therapy manufacturing capabilities have been greatly enhanced through partnerships with Aldevron and Catalent. We have adopted a hybrid development and manufacturing strategy in which we have built internal expertise relative to all aspects of AAV-based manufacturing, including gene therapy and gene editing, while closely partnering with experienced manufacturing partners to expedite development and commercialization of our gene therapy programs. We have secured manufacturing capacity at Catalent to support our clinical and commercial manufacturing demand for ELEVIDYS and our LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs. The collaboration integrates process development, clinical and commercial production and testing. Aldevron provides GMP-grade plasmid for ELEVIDYS and is expected to provide plasmid source material for our LGMD programs and future gene therapy programs.

Manufacturers and suppliers of our commercial products and product candidates are subject to the current GMP ("cGMP") requirements and other rules and regulations prescribed by the FDA and other foreign regulatory authorities. We depend on our third-party partners for continued compliance with cGMP requirements and applicable foreign standards.

Our PMO commercial products are distributed in the U.S. through a limited network of home infusion specialty pharmacy providers that deliver the medication to patients and a specialty distributor that distributes our products to hospitals and hospital outpatient clinics. With respect to the precommercial distribution of our products to patients outside of the U.S., we have contracted with third-party distributors and service providers to distribute our products in certain countries through our EAPs. We plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved.

The U.S. distribution model for ELEVIDYS employs multiple distribution partners that include third-party logistics providers as well as a limited network of specialty pharmacy providers that provide the medication to hospitals for infusion.

With respect to our siRNA programs, we are relying on our partner Arrowhead to supply drug substance manufacturing and testing services for ongoing and future clinical trials. Arrowhead's cGMP manufacturing facility has a variety of manufacturing lines at multiple scales available and is capable of potential future commercial-scale manufacture and capacity expansion as needed. In addition, we plan to leverage existing CMOs, which have technical expertise in manufacturing RNA therapies, for the manufacture of drug product.

Leadership Transition

On July 14, 2025, the Board appointed Ian Estepan as President and Chief Operating Officer, Louise Rodino-Klapac, Ph.D. as President, Research and Development and Technical Operations and Ryan Wong as Executive Vice President, Chief Financial Officer, each effective as of July 16, 2025. Douglas Ingram, who previously served as President and Chief Executive Officer, continues to serve as Chief Executive Officer. Mr. Wong replaced Mr. Estepan as principal financial officer and principal accounting officer.

Cash, Cash Equivalents, Restricted Cash and Investments

As of June 30, 2025, we had $850.3 million of cash, cash equivalents, restricted cash and investments, consisting of $510.6 million of cash and cash equivalents, $324.1 million of investments and $15.6 million of non-current restricted cash. We believe that our balance of cash, cash equivalents and investments, along with cash inflows from operations is sufficient to fund our current operational plan for at least the next twelve months.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the risks associated with government sponsored reimbursement programs and the complex regulatory environment in which we operate.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the U.S. requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates. We believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our unaudited condensed consolidated financial statements:

- inventory; and
- income tax.

There have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2024.

Results of Operations for the Three and Six Months Ended June 30, 2025 and 2024

The following tables set forth selected unaudited condensed consolidated statements of income (loss) data for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 513,123	$ 360,548	$ 152,575	42%
Collaboration and other	97,968	2,383	95,585	NM*
Total revenues	611,091	362,931	248,160	68%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	152,558	44,545	108,013	242%
Research and development	204,392	179,690	24,702	14%
Selling, general and administrative	137,897	138,796	(899)	(1)%
Amortization of in-licensed rights	667	601	66	11%
Total cost and expenses	495,514	363,632	131,882	36%
Operating income (loss)	115,577	(701)	116,278	NM*
Other income, net				
Other income, net	38,061	14,278	23,783	167%
Income before income tax expense	153,638	13,577	140,061	NM*
Income tax (benefit) expense	(43,254)	7,117	(50,371)	NM*
Net income	$ 196,892	$ 6,460	$ 190,432	NM*
Earnings per share				
Basic	$ 2.01	$ 0.07	$ 1.94	NM*
Diluted	$ 1.89	$ 0.07	$ 1.82	NM*

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 1,124,646	$ 720,032	$ 404,614	56%
Collaboration and other	231,301	56,363	174,938	310%
Total revenues	1,355,947	776,395	579,552	75%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	290,122	95,104	195,018	205%
Research and development	977,840	380,086	597,754	157%
Selling, general and administrative	271,526	265,799	5,727	2%
Amortization of in-licensed rights	1,268	1,202	66	5%
Total cost and expenses	1,540,756	742,191	798,565	108%
Operating (loss) income	(184,809)	34,204	(219,013)	NM*
Other (loss) income, net				
Other (expense) income, net	(45,071)	20,821	(65,892)	NM*
(Loss) income before income tax expense	(229,880)	55,025	(284,905)	NM*
Income tax expense	20,736	12,446	8,290	67%
Net (loss) income	$ (250,616)	$ 42,579	$ (293,195)	NM*
(Loss) earnings per share				
Basic	$ (2.57)	$ 0.45	$ (3.02)	NM*
Diluted	$ (2.57)	$ 0.44	$ (3.01)	NM*

* NM: not meaningful

Revenues

Revenues from product sales are recorded at the time of sale at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, patient assistance programs and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payments are required of us) or a current liability (if a payment is required of us). Our estimates take into consideration current contractual and statutory requirements. Actual amounts of consideration ultimately received or paid may differ from our estimates.

The following tables summarize the components of our net product revenues, by product, for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
ELEVIDYS	$ 281,851	$ 121,721	$ 160,130	132%
PMO Products	231,272	238,827	(7,555)	(3)%
Products, net	$ 513,123	$ 360,548	$ 152,575	42%

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
ELEVIDYS	$ 656,836	$ 255,657	$ 401,179	157%
PMO Products	467,810	464,375	3,435	1%
Products, net	$ 1,124,646	$ 720,032	$ 404,614	56%

Net product revenues for our products for the three and six months ended June 30, 2025 increased by $152.6 million and $404.6 million compared with the three and six months ended June 30, 2024. The increase primarily reflects an increase in net product

revenues of ELEVIDYS of $160.1 million and $401.2 million in the three and six months ended June 30, 2025 as a result of its expanded label approval in June 2024.

The following tables summarize the components of our collaboration and other revenues for the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Collaboration revenue	$ 63,500	$ —	$ 63,500	NM*
Contract manufacturing	27,023	—	27,023	NM*
Royalty revenue	7,445	2,383	5,062	212%
Total collaboration and other	$ 97,968	$ 2,383	$ 95,585	NM*

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Collaboration revenue	$ 175,500	$ 48,000	$ 127,500	266%
Contract manufacturing	44,402	5,807	38,595	NM*
Royalty revenue	11,399	2,556	8,843	346%
Total collaboration and other	$ 231,301	$ 56,363	$ 174,938	310%

* NM: not meaningful

Collaboration and other revenues primarily relate to our collaboration arrangement (the "Roche Collaboration Agreement") with F. Hoffman-La Roche Ltd. ("Roche"). In addition, in accordance with the Roche Collaboration Agreement, the parties agreed to enter into a supply agreement in order for us to supply Roche with clinical and commercial batches of ELEVIDYS (the "Roche Supply Agreement"). Roche utilizes the supply for sales of ELEVIDYS in territories outside of the U.S. where Roche has received regulatory approvals for ELEVIDYS. We are eligible to receive royalties on these sales.

For the three months ended June 30, 2025 and 2024, we recognized $98.0 million and $2.4 million of collaboration and other revenues, respectively. The increase is primarily due to $63.5 million of collaboration revenue recognized in the three months ended June 30, 2025 related to milestone payments received under the Roche Collaboration Agreement from the regulatory approval of ELEVIDYS in Japan for individuals ages 3- to less than 8-years-old, who do not have any deletions in exon 8 and/or exon 9 in the Duchenne gene and who are negative for anti-AAVrh74 antibodies (the "Japan Approval Milestone"). Please refer to *Note 3, License and Collaboration Agreements* for further discussion of the Roche Collaboration Agreement. While the Roche Supply Agreement is in the process of being negotiated, we delivered batches of commercial ELEVIDYS supply to Roche that were agreed upon on a purchase order-by-purchase order basis. For the three months ended June 30, 2025, we recognized $27.0 million of contract manufacturing revenue, which is related to these shipments, with no similar activity for the three months ended June 30, 2024. In addition, we recognized $7.4 million and $2.4 million of royalty revenue from sales of ELEVIDYS by Roche outside of the U.S. for the three months ended June 30, 2025 and 2024, respectively.

For the six months ended June 30, 2025 and 2024, we recognized $231.3 million and $56.4 million of collaboration and other revenues, respectively. The increase is primarily due to $112.0 million of collaboration revenue recognized in the six months ended June 30, 2025 related to the expiration of an option for a certain program previously recorded as deferred revenue and the Japan Approval Milestone payment of $63.5 million under the Roche Collaboration Agreement, as compared to $48.0 million of collaboration revenue recognized in the six months ended June 30, 2024 related to Roche's declined option to acquire the rights to a certain external, early stage Duchenne development program previously recorded as deferred revenue. For the six months ended June 30, 2025 and 2024, we recognized $44.4 million and $5.8 million of contract manufacturing revenue related to shipments of ELEVIDYS to Roche, respectively. In addition, we recognized $11.4 million and $2.6 million of royalty revenue from sales of ELEVIDYS by Roche outside of the U.S. for the six months ended June 30, 2025 and 2024, respectively.

Cost of sales (excluding amortization of in-licensed rights)

Our cost of sales (excluding amortization of in-licensed rights) consists of inventory costs that relate to sales of our products and royalty payments primarily to BioMarin Pharmaceuticals, Inc. ("BioMarin") and the University of Western Australia ("UWA") for our PMO Products and to Nationwide Children's Hospital ("Nationwide") for ELEVIDYS. Prior to receiving regulatory approval for our products, we expensed manufacturing and material costs as research and development expenses. For the PMO Products, all previously expensed manufacturing costs had been fully consumed prior to the three and six months ended June 30, 2025 and 2024. For ELEVIDYS sold in the three and six months ended June 30, 2025, a portion of related manufacturing costs incurred had previously been expensed as research and development expenses. For ELEVIDYS sold in the three and six months ended June 30, 2024, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to FDA approval of the product. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental inventory costs related to ELEVIDYS sold, including products sold to Roche under the Roche Collaboration Agreement, would have been $4.5 million and $18.2 million higher for the three and six months ended June 30, 2025, respectively, as compared to $15.9 million and $39.7 million for the three and six months ended June 30, 2024, respectively.

The following tables summarize the components of our cost of sales (excluding amortization of in-licensed rights) for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold (excluding products sold to Roche**)	$ 116,743	$ 36,814	$ 79,929	217%
Inventory costs related to products sold to Roche**	21,818	—	21,818	NM*
Royalty payments	13,997	7,731	6,266	81%
Total cost of sales (excluding amortization of in-licensed rights)	$ 152,558	$ 44,545	$ 108,013	242%

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold (excluding products sold to Roche**)	$ 226,501	$ 74,513	$ 151,988	204%
Inventory costs related to products sold to Roche**	33,961	1,654	32,307	NM*
Royalty payments	29,660	18,937	10,723	57%
Total cost of sales (excluding amortization of in-licensed rights)	$ 290,122	$ 95,104	$ 195,018	205%

* NM: not meaningful

** See above for further details regarding product supply sold to Roche via contract manufacturing under the Roche Collaboration Agreement.

The cost of sales (excluding amortization of in-licensed rights) for the three months ended June 30, 2025 increased by $108.0 million, or 242%, compared with the same period in 2024. The cost of sales (excluding amortization of in-licensed rights) for the six months ended June 30, 2025 increased by $195.0 million, or 205%, compared with the same period in 2024. For both comparative periods, the changes primarily reflects (1) depletion of previously expensed ELEVIDYS inventory (2) increased demand following expanded label approval of ELEVIDYS in June 2024 (3) an increase in products sold to Roche under the Roche Collaboration Agreement and (4) an increase in the write-offs of certain batches of our products not meeting our quality specifications.

Research and development expenses

Research and development expenses consist of costs associated with research activities as well as those associated with our product development efforts, conducting pre-clinical trials, clinical trials and manufacturing activities. Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants, up-front fees and milestones paid to third parties in connection with technologies that have not reached technological feasibility and do not have an alternative future use, and other external services, such as data management and statistical analysis support, and materials and supplies used in support of clinical programs. Indirect costs of our programs include salaries, stock-based compensation and allocation of our facility- and technology-related costs.

Research and development expenses represent a substantial percentage of our total operating expenses. We do not maintain or evaluate and, therefore, do not allocate internal research and development costs on a project-by-project basis. As a result, a

significant portion of our research and development expenses are not tracked on a project-by-project basis, as the costs may benefit multiple projects.

The following tables summarize our research and development expenses by project for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change $	Change %
	(in thousands)			
SRP-9001	$ 63,232	$ 41,454	$ 21,778	53%
LGMD platform	21,494	20,964	530	3%
Eteplirsen (exon 51)	11,103	19,992	(8,889)	(44)%
Other gene therapies	11,065	7,919	3,146	40%
siRNA platform	6,512	—	6,512	NM*
PPMO platform	5,349	5,821	(472)	(8)%
Golodirsen (exon 53)	1,577	2,529	(952)	(38)%
Casimersen (exon 45)	1,388	5,812	(4,424)	(76)%
Other projects	4,536	8,278	(3,742)	(45)%
Internal research and development expenses	103,687	84,640	19,047	23%
Roche collaboration reimbursement	(25,551)	(17,719)	(7,832)	44%
Total research and development expenses	$ 204,392	$ 179,690	$ 24,702	14%

	For the Six Months Ended June 30,			
	2025	2024	Change $	Change %
	(in thousands)			
Up-front and milestone expenses	$ 583,787	$ —	$ 583,787	NM*
SRP-9001	132,089	112,854	19,235	17%
LGMD platform	49,149	37,918	11,231	30%
Other gene therapies	23,446	16,529	6,917	42%
Eteplirsen (exon 51)	21,443	38,822	(17,379)	(45)%
siRNA platform	8,805	—	8,805	NM*
PPMO platform	5,885	18,332	(12,447)	(68)%
Casimersen (exon 45)	5,554	11,058	(5,504)	(50)%
Golodirsen (exon 53)	3,797	4,982	(1,185)	(24)%
Other projects	7,833	16,096	(8,263)	(51)%
Internal research and development expenses	189,863	162,686	27,177	17%
Roche collaboration reimbursement	(53,811)	(39,191)	(14,620)	37%
Total research and development expenses	$ 977,840	$ 380,086	$ 597,754	157%

* NM: not meaningful

The following tables summarize our research and development expenses by category for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change $	Change %
	(in thousands)			
Manufacturing expenses	$ 80,685	$ 50,659	$ 30,026	59%
Compensation and other personnel expenses	43,582	37,407	6,175	17%
Clinical trial expenses	33,511	39,861	(6,350)	(16)%
Facility- and technology-related expenses	25,045	22,030	3,015	14%
Stock-based compensation	15,277	19,806	(4,529)	(23)%
Professional services	9,044	7,746	1,298	17%
Pre-clinical expenses	1,431	1,443	(12)	(1)%
Research and other	21,368	18,457	2,911	16%
Roche collaboration reimbursement	(25,551)	(17,719)	(7,832)	44%
Total research and development expenses	$ 204,392	$ 179,690	$ 24,702	14%

Research and development expenses for the three months ended June 30, 2025 increased by $24.7 million compared with the three months ended June 30, 2024. The increase was primarily driven by the following:

- $30.0 million increase in manufacturing expenses primarily due to ELEVIDYS clinical batch releases of $27.0 million as certain commercial batches being utilized for clinical activity and $13.0 million of costs associated with our settlement agreement with Brammer Bio MA, LLC ("Brammer") to resolve outstanding claims related to the termination of the Thermo Agreement (the "Brammer Settlement"). Please refer to *Note 17, Commitments and Contingencies* for further discussion of the Brammer Settlement;
- $6.2 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $6.4 million decrease in clinical trial expenses primarily due to our decision to discontinue our PPMO programs, partially offset by increased activity in our LGMD programs;
- $3.0 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts;
- $4.5 million decrease in stock-based compensation expense primarily due to the achievement of performance conditions related to certain PSUs during the three months ended June 30, 2024 and fulfillment of remaining service conditions associated with certain PSUs in March 2025;
- $1.3 million increase in professional services primarily due to an increase in reliance on third-party research and development contractors for clinical programs;
- $2.9 million increase in research and other expenses primary due to reimbursements to Arrowhead, with no similar activity for the three months ended June 30, 2024, partially offset by a decrease in collaboration cost-sharing expenses related to our license and collaboration agreement with Genethon; and
- $7.8 million increase in the offset to expense associated with a collaboration reimbursement from Roche primarily due to an increase in ELEVIDYS clinical supply batch releases.

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Up-front and milestone expenses	$ 583,787	$ —	583,787	NM*
Manufacturing expenses	148,150	120,359	27,791	23%
Compensation and other personnel expenses	90,881	78,942	11,939	15%
Clinical trial expenses	66,658	84,008	(17,350)	(21)%
Facility- and technology-related expenses	49,267	43,395	5,872	14%
Stock-based compensation	32,594	36,079	(3,485)	(10)%
Professional services	18,614	14,280	4,334	30%
Pre-clinical expenses	3,193	4,892	(1,699)	(35)%
Research and other	38,507	37,322	1,185	(—)%
Roche collaboration reimbursement	(53,811)	(39,191)	(14,620)	37%
Total research and development expenses	$ 977,840	$ 380,086	$ 597,754	157%

* NM: not meaningful

Research and development expenses for the six months ended June 30, 2025 increased by $597.8 million compared with the six months ended June 30, 2024. The increase was primarily driven by the following:

- $583.8 million increase in up-front and milestone expenses primarily due to the $583.6 million in acquired in-process research and development expense associated with our exclusive global licensing and collaboration agreement and stock purchase agreement with Arrowhead (the "Arrowhead Agreement");
- $27.8 million increase in manufacturing expenses primarily due to ELEVIDYS clinical batch releases of $23.0 million due to certain commercial batches being utilized for clinical activity and $13.0 million of costs associated with the Brammer Settlement, partially offset by a decrease in PMO manufacturing activity of $10.7 million;
- $11.9 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $17.4 million decrease in clinical trial expenses primarily due to our decision to discontinue our PPMO programs, partially offset by increased activity in our LGMD programs;
- $5.9 million increase in facility- and technology-related expenses primarily due to our expansion efforts;
- $3.5 million decrease in stock-based compensation expense primarily due to the achievement of performance conditions related to certain PSUs during the three months ended June 30, 2024 and fulfillment of remaining service conditions associated with certain PSUs in March 2025, partially offset by changes in headcount;
- $4.3 million increase in professional services primarily due to an increase in reliance on third-party research and development contractors for clinical programs;
- $1.7 million decrease in pre-clinical expenses primarily due to a decrease in capsid and toxicology studies across our gene therapy programs; and
- $14.6 million increase in the offset to expense associated with a collaboration reimbursement from Roche primarily due to an increase in ELEVIDYS clinical supply batch releases.

Selling, general and administrative expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resources, commercial and other general and administrative functions. Other general and administrative expenses include professional fees for legal, consulting and accounting services and an allocation of our facility- and technology-related costs.

The following tables summarize our selling, general and administrative expenses by category for each of the periods indicated:

	For the Three Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Professional services	$ 50,515	$ 44,033	$ 6,482	15%
Compensation and other personnel expenses	46,257	40,128	6,129	15%
Stock-based compensation	21,748	30,676	(8,928)	(29)%
Facility- and technology-related expenses	13,708	12,739	969	8%
Other	5,807	11,441	(5,634)	(49)%
Roche collaboration reimbursement	(138)	(221)	83	(38)%
Total selling, general and administrative expenses	$ 137,897	$ 138,796	$ (899)	(1)%

Selling, general and administrative expenses for the three months ended June 30, 2025 decreased by $0.9 million compared with the three months ended June 30, 2024. This decrease was primarily driven by the following:

- $6.5 million increase in professional service expenses primarily due to marketing and consulting related to our PMO programs, LGMD site readiness and our continuing efforts to commercialize ELEVIDYS;
- $6.1 million increase in compensation and other personnel expenses primarily due to annual merit-based salary increases and an increase in certain employer-related payroll taxes;
- $8.9 million decrease in stock-based compensation primarily due to the achievement of performance conditions related to certain PSUs during the three months ended June 30, 2024 and fulfillment of remaining service conditions associated with certain PSUs in March 2025; and

- $5.6 million decrease in other expenses primarily due to timing of charitable contribution activity.

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Professional services	$ 92,745	$ 83,698	$ 9,047	11%
Compensation and other personnel expenses	92,042	82,180	9,862	12%
Stock-based compensation	45,859	55,095	(9,236)	(17)%
Facility- and technology-related expenses	26,520	24,495	2,025	8%
Other	14,719	20,738	(6,019)	(29)%
Roche collaboration reimbursement	(359)	(407)	48	(12)%
Total selling, general and administrative expenses	$ 271,526	$ 265,799	$ 5,727	2%

Selling, general and administrative expenses for the six months ended June 30, 2025 increased by $5.7 million compared with the six months ended June 30, 2024. This increase was primarily driven by the following:

- $9.0 million increase in professional service expenses primarily related to our continuing efforts to commercialize ELEVIDYS, partially offset by a decrease in expenses related to ongoing litigation matters;
- $9.9 million increase in compensation and other personnel expenses primarily due to annual merit-based salary increases and an increase in certain employer-related payroll taxes;
- $9.2 million decrease in stock-based compensation primarily due to the achievement of performance conditions related to certain PSUs during the six months ended June 30, 2024 and fulfillment of remaining service conditions associated with certain PSUs in March 2025;
- $2.0 million increase in facility- and technology-related expenses primarily due to our expansion efforts; and
- $6.0 million decrease in other expenses primarily due to timing of charitable contribution activity.

Amortization of in-licensed rights

Amortization of in-licensed rights relates to the agreements we entered into with UWA, Nationwide, BioMarin and Parent Project Muscular Dystrophy in April 2013, December 2016, July 2017 and May 2018, respectively. Each in-licensed right is being amortized on a straight-line basis over the remaining life of the relevant patent from the date the related fee was incurred, either the regulatory approval or the first commercial sale of the applicable product. For the three and six months ended June 30, 2025, we recorded amortization of in-licensed rights of approximately $0.7 million and $1.3 million, respectively. For the three and six months ended June 30, 2024, we recorded amortization of in-licensed rights of approximately $0.6 million and $1.2 million, respectively.

Other income (expense), net

Other income (expense), net primarily consists of the unrealized gain or loss from our investments in our strategic equity investments, interest expense on our 1.25% convertible senior notes due on September 15, 2027 (the "2027 Notes"), interest income on our cash, cash equivalents and investments, accretion of investment discount and the change in the fair value of contingent consideration related to regulatory-related contingent payments meeting the definition of a derivative liability. Our cash equivalents and investments consist of money market funds, corporate bonds, commercial paper, government and government agency debt securities and certificates of deposit.

For the three months ended June 30, 2025, other income, net increased by $23.8 million compared with the three months ended June 30, 2024. The change is primarily due to a $36.6 million increase in the fair value of our strategic investments, which includes the recurring fair value adjustments of investments in publicly-traded companies, including Arrowhead, offset by a decrease of $8.9 million in accretion of investment discount, net due to changes in the investment mix of our investment portfolio.

For the six months ended June 30, 2025, other income (expense), net changed by $65.9 million compared with the six months ended June 30, 2024. The unfavorable change is primarily due to a $55.1 million increase in the loss on our strategic investments and a $19.4 million decrease in accretion of investment discount, net due to changes in the investment mix of our investment portfolio. These decreases were partially offset by a loss of $10.1 million associated with the change in fair value of contingent consideration for the six months ended June 30, 2024 with no similar activity during the six months ended June 30, 2025.

Income tax (benefit) expense

Income tax (benefit) expense for the three and six months ended June 30, 2025 was $(43.3) million and $20.7 million, respectively. Income tax (benefit) expense for all periods presented primarily relates to state, federal and foreign income taxes for which available tax losses or credits were not available to offset. As of June 30, 2025, we continued to maintain a full valuation allowance against our deferred tax assets, with the exception of deferred tax assets in certain foreign jurisdictions. We continue to monitor the available evidence relative to recovery of our deferred tax assets and whether such evidence would be sufficient to conclude that it is more likely than not that such deferred tax assets may be partially or fully recoverable. If we were to remove our valuation allowance in part or full, any such adjustment could have a material impact on our effective tax rate in the applicable period and beyond.

On July 4, 2025, President Trump signed into law the One Big Beautiful Bill Act ("OBBBA"). The OBBBA includes provisions that could have a significant impact on us, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. We are currently evaluating the impact of the OBBBA and expect the results of such evaluations to be reflected in our future filings.

Liquidity and Capital Resources

There have been no material changes to our obligations under lease or debt arrangements as reported in our Annual Report on Form 10-K for the year ended December 31, 2024, except for the five-year $600.0 million senior secured revolving credit facility entered into in February 2025 (the "Revolving Credit Facility"), as discussed in *Note 14, Revolving Credit Facility*.

The following table summarizes our financial condition for each of the periods indicated:

	As of June 30, 2025	As of December 31, 2024	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 510,598	$ 1,103,010	$ (592,412)	(54)%
Short-term investments	289,541	251,782	37,759	15%
Non-current investments	34,604	133,163	(98,559)	(74)%
Restricted cash	15,579	15,579	—	(—)%
Total cash, cash equivalents and investments	$ 850,322	$ 1,503,534	$ (653,212)	(43)%
Borrowings:				
Convertible debt	$ 1,139,458	$ 1,137,124	$ 2,334	(—)%
Total borrowings	$ 1,139,458	$ 1,137,124	$ 2,334	(—)%
Working capital				
Current assets	$ 2,656,985	$ 3,073,463	$ (416,478)	(14)%
Current liabilities	919,948	731,684	188,264	26%
Total working capital	$ 1,737,037	$ 2,341,779	$ (604,742)	(26)%

For the periods ended June 30, 2025 and December 31, 2024, our principal sources of liquidity were primarily derived from the sales of our products, our collaboration arrangement with Roche and proceeds from the settlement of capped call options associated with our convertible senior notes due on November 15, 2024. Our principal uses of cash were our $583.6 million up-front payment to Arrowhead and $241.4 million equity investment in Arrowhead's common stock (collectively, the "Arrowhead Payments"), inventory commitments, research and development expenses, selling, general and administrative expenses, investments, capital expenditures, settlement of long-term debt, share repurchases under our $500.0 million share repurchase program approved by the Board of Directors in November 2024 (the "2024 Repurchase Program") and other working capital requirements. Please refer to *Note 10, Equity*, for further discussion of share repurchases under the 2024 Repurchase Program during the period. The changes in our working capital primarily reflect use of cash in operating activities, as well as a reduction in our cash, cash equivalents and investments to fund the Arrowhead Payments. The Arrowhead Agreement includes a commitment of $250.0 million in guaranteed payments to be paid in five equal annual installments of $50.0 million, beginning in February 2026. These payments are not contingent on clinical or regulatory milestones. The Arrowhead Agreement also includes $300.0 million in anticipated near-term milestone payments tied to the clinical progress of ARO-DM1, our investigational siRNA therapeutic for skeletal muscle diseases. Of this amount, $100.0 million was earned in July 2025 upon certain patient enrollment targets and subsequent authorization to dose

escalate in our ARO-DM1 program (the "Arrowhead Milestone") and the remaining $200.0 million is contingent upon further enrollment and trial progression milestones. Refer to *Note 18. Subsequent Events* for further discussion of the Arrowhead Milestone. While our contractual obligations, commitments and debt service requirements over the next several years are significant, we intend to continue to fund our short-term financing needs and working capital requirements from cash flows of operating activities as well as cash on hand, and such sources are anticipated to be adequate to fund working capital requirements for at least twelve months from the date these unaudited condensed consolidated financial statements were issued.

In response to two reported cases of ALF resulting in death of non-ambulatory patients, we suspended all commercial shipments of ELEVIDYS to non-ambulatory patients in June 2025. In July 2025, we also disclosed a recent death from ALF of a non-ambulatory LGMD patient participating in a Phase 1/2 trial for SRP-9004, who was not treated with ELEVIDYS. Thereafter, in response to a request from the FDA that we voluntarily stop all shipments of ELEVIDYS in the U.S., including to ambulatory patients, we temporarily suspended all shipments of ELEVIDYS in the U.S., effective July 22, 2025, to allow us the necessary time to respond to FDA's requests for information and complete a labeling supplement process. Although the FDA informed the Company on July 28, 2025 that it recommended the removal of the voluntary hold for ambulatory patients, the ELEVIDYS Suspension could materially impact our near-term revenue generation. The suspension of shipments for non-ambulatory patients remains in effect as of the date of issuance of this Quarterly Report. It is currently unclear whether or when we might resume shipments of ELEVIDYS for non-ambulatory patients, or whether the FDA will pursue further actions relating to the use of ELEVIDYS in ambulatory or non-ambulatory patients, such as additional studies, additional product modifications or controls, or withdrawal in the future. We considered the ELEVIDYS Suspension and the partial resumption of shipments when concluding that our cash, cash equivalents and investments as of the date of issuance of this report, along with future cash inflows from operations, are sufficient to fund our current operational plan for at least the next twelve months.

Further, in July 2025, we announced a strategic restructuring plan designed to enhance financial flexibility and meet our financial obligations associated with our 2027 Notes (the “Restructuring”). This plan includes a revised cost structure, program portfolio and a reduction in force. The workforce reduction represented approximately 36% of our workforce. As a result of this reduction in force, we estimate that we will record a one-time charge in the third quarter of 2025 related to employee termination benefits, including severance, between approximately $32.0 million and $37.0 million, all of which is anticipated to result in cash expenditures. While we anticipate that the Restructuring will result in a reduction in our future expenditures, actual savings realized may vary and there can be no assurance that the Restructuring will achieve our anticipated cost savings or operational efficiencies.

Beyond June 30, 2026, our cash requirements will depend extensively on our ability to advance our research, development and commercialization of product candidates. We may seek additional financings primarily from, but not limited to, the sale and issuance of equity and debt securities, the licensing or sale of our technologies, and entering into additional government contracts and/or funded research and development agreements. Our future expenditures and long-term capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- our ability to continue to generate revenues from sales of commercial products and potential future products;
- our ability to resume shipments of shipments of ELEVIDYS for non-ambulatory patients in the U.S.;
- our ability to implement the Restructuring;
- the risk that our Restructuring and pipeline reprioritization efforts may not generate their intended benefits to the extent or as quickly as anticipated;
- the timing and costs associated with repurchases of our common stock under our 2024 Repurchase Program;
- the timing and costs of building out our manufacturing capabilities;
- the timing of payments related to our future inventory commitments and manufacturing obligations;
- the timing and costs associated with our existing lease obligations and new obligations expected to be entered into in future years;
- the timing and costs associated with our clinical trials and pre-clinical trials;
- the attainment of milestones and our obligations to make milestone payments to Arrowhead, Myonexus Therapeutics, Inc.'s selling shareholders, BioMarin, Nationwide, UWA and other institutions;
- the timing and repayment of future borrowings on our Revolving Credit Facility;
- obligations to holders of our 2027 Notes; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution. Additional information regarding our Revolving Credit Facility is provided in *Note 14, Revolving Credit Facility* to the unaudited condensed consolidated financial statements contained in Item 1.We believe that existing cash, cash equivalents and investments along with future cash generated from operations will be sufficient to meet the capital requirements of our operations for the next 12 months and foreseeable future.

We have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and issuance of debt securities, among others. Additional information regarding our obligations under manufacturing arrangements is provided in *Note 17, Commitments and Contingencies* to the unaudited condensed consolidated financial statements contained in Item 1. There have been no material changes to our obligations under debt or leasing arrangements as reported in our Annual Report on Form 10-K for the year ended December 31, 2024.

For products and product candidates that are currently approved or are in various research and development stages, we may be obligated to make up to $12.6 billion of future development, regulatory, up-front royalty and sales milestone payments associated with our license and collaboration agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable, and payment is not required as of June 30, 2025, such contingencies have not been recorded in our unaudited condensed consolidated financial statements. Amounts related to contingent milestone payments are not yet considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and commercial milestones.

Cash Flows

The following table summarizes our cash flow activity for each of the periods indicated:

	For the Six Months Ended June 30,			
	2025	2024	Change	Change
	(in thousands)		$	%
Cash (used in) provided by				
Operating activities	$ (322,100)	$ (227,134)	$ (94,966)	42%
Investing activities	(258,966)	120,268	(379,234)	NM*
Financing activities	(11,346)	62,058	(73,404)	(118)%
Decrease in cash, cash equivalents and restricted cash	$ (592,412)	$ (44,808)	$ (547,604)	NM*

* NM: not meaningful

Operating Activities

Cash used in operating activities, which consists of our net (loss) income adjusted for non-cash items and changes in net operating assets and liabilities, totaled $322.1 million and $227.1 million for the six months ended June 30, 2025 and 2024, respectively. Cash used in operating activities for the six months ended June 30, 2025 was primarily driven by the net loss of $250.6 million, adjusted for the following:

- $78.5 million in stock-based compensation expense;
- $54.0 million loss on strategic investments;
- $20.8 million in depreciation and amortization expense; and
- $11.3 million in other non-cash items.

These non-cash charges were partially offset by $3.8 million in accretion of investment discount, net.

The net cash outflow from changes in our operating assets and liabilities was primarily driven by the following:

- $236.5 million increase in inventory primarily due to a continuing build-up of ELEVIDYS inventory as a result of label expansion of the therapy in June 2024;
- $59.8 million decrease in deferred revenue primarily related to our collaboration with Roche, partially offset by our contract manufacturing activities with Roche;

- $37.3 million increase in other assets primarily due to an increase in receivables from Roche associated with contract manufacturing revenue;
- $42.2 million decrease in accounts payable, accrued expenses, lease liabilities and other liabilities, primarily due to payments on accrued employee compensation costs and the timing and invoicing of payments with our contract research organizations and contract manufacturing organizations, partially offset by an increase in our product revenue;
- $68.9 million decrease in manufacturing-related deposits and prepaids primarily due to timing of payments to Catalent; and
- $74.7 million decrease in accounts receivable, net primarily due to a decrease in demand for ELEVIDYS from year-end.

Cash used in operating activities for the six months ended June 30, 2024 was primarily driven by the net income of $42.6 million, adjusted for the following:

- $91.2 million in stock-based compensation expense;
- $17.5 million in depreciation and amortization expense;
- $10.1 million change in the fair value of contingent consideration; and
- $9.4 million in other non-cash items.

These non-cash charges were partially offset by the $22.9 million in accretion of investment discount, net.

The net cash outflow from changes in our operating assets and liabilities was primarily driven by the following:

- $203.3 million increase in manufacturing-related deposits and prepaids primarily due to an increase in prepaid raw materials and batch fees with Catalent;
- $163.0 million increase in inventory primarily due to capitalized inventory related to ELEVIDYS;
- $40.4 million decrease in deferred revenue primarily related to the collaboration with Roche; and
- $15.3 million decrease in accounts payable, accrued expenses, lease liabilities and other liabilities, primarily due to the timing and invoicing of payments with our contract research organizations and contract manufacturing organizations and payments on accrued employee compensation costs.

These amounts were partially offset by the following:

- $6.6 million decrease in other assets primarily due to a decrease in the collaboration receivable related to Roche; and
- $40.3 million decrease in accounts receivable due to a reduction in payment terms for product sales related to ELEVIDYS.

Investing Activities

Cash used in investing activities was $259.0 million for the six months ended June 30, 2025, compared to $120.3 million of cash provided by for the six months ended June 30, 2024. Cash used in investing activities for the six months ended June 30, 2025 primarily consisted of $245.8 million in the acquisition of strategic investments primarily related to Arrowhead, $75.4 million of purchases of property and equipment and $44.7 million of purchases of available-for-sale securities, partially offset by $109.4 million from the maturity and sale of available-for-sale securities.

Cash provided by investing activities for the six months ended June 30, 2024 primarily consisted of $739.2 million from the maturity and sale of available-for sale securities, partially offset by $547.3 million of purchases of available-for-sale securities, $61.6 million of purchases of property and equipment and $10.0 million of purchases of intangible assets.

Financing Activities

Cash used in financing activities was $11.3 million for the six months ended June 30, 2025 and cash provided by financing activities was $62.1 million for the six months ended June 30, 2024. Cash used in financing activities for the six months ended June 30, 2025 consisted of $25.0 million of purchases of our common stock under our 2024 Repurchase Program and $3.5 million in arrangement, up-front and commitment fees related to the Revolving Credit Facility, partially offset by $17.2 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program.

Cash provided by financing activities for the six months ended June 30, 2024 consisted of $62.1 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest-Rate-Sensitive Financial Instruments

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, certificates of deposit, government and government agency bonds and high-grade corporate bonds with maturities of 24 months or less. Our cash is primarily deposited in and invested through highly rated financial institutions in the U.S. As of June 30, 2025, we had $850.3 million of cash, cash equivalents, restricted cash and investments, comprised of $510.6 million of cash and cash equivalents, $324.1 million of investments and $15.6 million non-current restricted cash. The Company only holds debt securities classified as available-for-sale. The fair value of cash equivalents and investments is subject to change as a result of potential changes in market interest rates. Our future investment income may fluctuate due to changes in interest rates or we may suffer losses in principal if we sell securities that decline in market value due to changes in interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of June 30, 2025, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.1 million to our interest rate sensitive instruments.

Our $1,150.0 million aggregate principal amount of our 2027 Notes has a fixed interest rate of 1.25% per annum, payable semi-annually in cash on each March 15 and September 15, and therefore are not subject to fluctuations in market interest rates.

Market-Price-Sensitive Financial Instruments

Our strategic investment portfolio includes investments in equity securities of certain publicly traded biotechnology companies as a result of certain business development transactions. While we are holding such securities, we are subject to equity price risk and this may increase the volatility of our income in future periods due to changes in the fair value of strategic equity investments. As of June 30, 2025, strategic equity investments with a fair value of $188.4 million were subject to a contractual sale restriction that expires in August 2025. Changes in the fair value of these equity securities are impacted by the volatility of the stock market and changes in general economic conditions, among other factors. The potential change in fair value for market-price-sensitive instruments has been assessed on a hypothetical 10.0% adverse movement. As of June 30, 2025, we estimate that such hypothetical adverse 10.0% movement would result in a hypothetical loss in fair value of approximately $19.0 million to our market-price-sensitive financial instruments.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Quarterly Report on Form 10-Q for the period ended June 30, 2025, under the supervision and with the participation of our management, including our principal executive officer and our principal financial officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934, as amended (the “Exchange Act”). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms and (ii) is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of June 30, 2025, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended June 30, 2025, there were no changes in our internal controls over financial reporting 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

For material legal proceedings, please read *Note 17, Commitments and Contingencies* to our unaudited condensed consolidated financial statements included in this report.

Item 1A. Risk Factors.

Set forth below and elsewhere in this report and in other documents we file with the SEC are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

We are highly dependent on the commercial success of our products. We may not be able to meet expectations with respect to sales of our products or maintain profitability and positive cash-flow from operations.

The commercial success of our products continues to depend on, and the commercial success of any future products would depend on, a number of factors attributable to one of our products or the products of our competitors, including, but not limited to:

- the effectiveness of our sales, managed markets, marketing efforts and support for our products;
- the generation and dissemination of new data and analyses and the consistency of any new data and analyses with prior results, whether they support a favorable safety, efficacy and effectiveness profile of our products and any potential impact on our FDA approval status and/or FDA package insert for our products, including our ongoing discussions with FDA for ELEVIDYS in response to incidents of ALF resulting in patient death;
- the effectiveness of our ongoing commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, ongoing manufacturing efforts and hiring any additional personnel as needed to support commercial efforts;
- our ability to timely comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy, effectiveness and safety of our products and acceptance of the same by the FDA and medical community since continued approval of accelerated approval products may be contingent upon verification of a clinical benefit in confirmatory trials, particularly in light of FDA's expanded expedited withdrawal procedures as set forth in FDORA;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas, including recent patient deaths associated with our products and product candidates and the associated public coverage;
- the generation of evidence describing payers, patients and/or societal value of our products;
- whether we can consistently manufacture our products and product candidates at acceptable costs;
- the rate and consistency with which our products are prescribed by physicians, which depends on physicians' views on the safety, effectiveness and efficacy of our products;
- our ability to secure and maintain adequate reimbursement for our products, including the duration of the prior-authorization as well as the number and duration of re-authorization processes required for patients who initially obtained coverage by third parties, including by government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for our products, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties;
- the development, commercialization or pricing of competing products or therapies for the treatment of Duchenne, or its symptoms, and the existence of competing clinical trials;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding Duchenne mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with evolving laws and regulations that apply to us and our commercial activities;

- the actual market-size, ability to identify patients and the demographics of patients eligible for our products, which may be different than expected;
- executive, legislative or regulatory action that restricts pricing, coverage or reimbursement of our current or future products
- the sufficiency of our drug supply to meet commercial and clinical demands and standards, which are negatively impacted by various factors, including when our projections on the potential number of amenable patients and their average weight are inaccurate; the potential impacts of future pandemics; if regulatory requirements increase our drug supply needs; if our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit; failure to meet cGMP requirements; or if we encounter delays expanding the number of patients on our products and portions of our products' supply expire before sale;
- our ability to obtain and maintain regulatory approvals to commercialize our product candidates, and to commercialize our products in markets outside of the U.S.;
- the process leading to a patient's first infusion of our products and any future commercial products may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. In addition, the capacity of any infusion centers responsible for the administration of ELEVIDYS may impact timing. Delays in the process prior to infusion could negatively impact the sales of our products, including any future gene therapy products; and
- the exercise by Roche of its option to obtain an exclusive license to commercialize one or more of our Duchenne products beyond ELEVIDYS outside of the U.S. and Roche's subsequent commercialization efforts.

We experience significant fluctuations in sales of our products from period to period and, ultimately, we may never generate sufficient revenues from our products to maintain profitability or sustain our anticipated levels of operations.

Even though EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS have received accelerated approval from the FDA, they face future post-approval development and regulatory requirements, which present additional challenges for us to successfully navigate.

The accelerated approvals for EXONDYS 51, VYONDYS 53 and AMONDYS 45 granted by the FDA were based on an increase in the surrogate biomarker of dystrophin in skeletal muscles observed in some patients treated with these products. The accelerated approval for ELEVIDYS in non-ambulatory patients granted by the FDA was based on an effect on the surrogate endpoint of expression of ELEVIDYS micro-dystrophin, the protein produced by ELEVIDYS. These products are subject to ongoing FDA requirements governing labeling, packaging, storage, advertising, promotion and recordkeeping, and we are required to submit additional safety, efficacy and other post-marketing information to the FDA.

Under the accelerated approval pathway, continued approval may be contingent upon verification of a clinical benefit in confirmatory trials. These post-marketing requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of our products; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and in particular, any failure to obtain safety and efficacy data that supports clinical benefits from our ongoing and planned studies of our products, could lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51, VYONDYS 53, AMONDYS 45 or ELEVIDYS. FDORA, enacted in 2022, has expanded FDA's expedited withdrawal procedures for drugs approved via the accelerated approval pathway if a sponsor fails to conduct any required post-approval study with due diligence.

Further, the current administration has also undertaken significant efforts to reduce the size and spending of the federal government, including at the FDA. A significant reduction in FDA's workforce or FDA's budget, or other disruptions at FDA, could materially impact FDA's ability to engage in a variety of activities that may affect our business, including routine regulatory and oversight activities. For example, any reduction in FDA's workforce could lead to disruptions and delays in FDA's review and oversight of our post-approval confirmatory trials.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with FDA requirements, including cGMP regulations. Drug product manufacturers are required to continuously monitor and report adverse events from clinical trials and commercial use of the product. If we or a regulatory agency discover previously unknown adverse events or events of unanticipated severity or frequency, a regulatory agency may establish additional regulatory requirement including, among other things, labeling changes, implementation of risk evaluation and mitigation strategy program, or additional post-marketing studies or clinical trials. For example, following two patient deaths due to ALF in non-ambulatory patients associated with the use of ELEVIDYS, FDA proposed a safety label supplement for ELEVIDYS to include a boxed warning for ALI and ALF. Subsequently, on July 18, 2025, we announced a reported case of ALF resulting in death in a patient following dosing in the Company's Phase 1/2 LGMD trial for SRP-9004. As of the date of this Quarterly Report, the Company remains in ongoing discussions with FDA regarding the label supplement for ELEVIDYS. If we or a regulatory agency discover previously unknown problems with a product, such as problems with a facility where the API or drug product is manufactured or tested, a regulatory agency may impose restrictions on that product and/or the manufacturer, including removal of specific product lots from the market, withdrawal of the product from the market, suspension of manufacturing or suspension of clinical trials using the same manufacturing materials. Sponsors of drugs approved under FDA accelerated approval provisions also

are required to submit to the FDA, at least 30 days before initial use, all promotional materials intended for use after the first 120 days following marketing approval. If we or the manufacturing facilities for our products fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to product labeling or to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

We are subject to uncertainty relating to reimbursement policies which, if not favorable, could hinder or prevent the commercial success of our products and/or product candidates.

Our ability to successfully maintain and/or increase sales of our products in the U.S. depends in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors. Third party payors are increasingly challenging the effectiveness of, and the prices charged for medical products and services. We may not be able to obtain or maintain adequate third-party coverage or reimbursement for our products, and/or we may be required to provide discounts or rebates on our products in order to obtain or maintain adequate coverage.

We expect that private insurers will continue to consider the efficacy, effectiveness, cost-effectiveness and safety of our products, including any new data and analyses that we are able to collect and make available in a compliant manner, in determining whether to approve reimbursement for our products and at what levels. If there are considerable delays in the generation of new evidence or if any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of our products. For example, in light of the ELEVIDYS Suspension and ongoing discussions with FDA, certain third-party payors have restricted reimbursement for ELEVIDYS for ambulatory patients, notwithstanding FDA's recommendation that we resume shipments of ELEVIDYS to ambulatory patients in the U.S. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of our products from additional insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we are not able to maintain favorable coverage decisions and/or fail to receive additional favorable coverage decisions from third party insurers, in particular during re-authorization processes for patients that have already initiated therapy. Our business could also be adversely affected if government health programs, private health insurers, including managed care organizations, or other reimbursement bodies or payors limit the indications for which our products will be reimbursed or fail to recognize approval or accelerated approval and surrogate endpoints as clinically meaningful.

Furthermore, we cannot predict to what extent an economic recession, changes in fiscal policy, restrictions in eligibility for government health care programs such as Medicaid or general increase in unemployment rates may disrupt global healthcare systems and access to our products or result in a widespread loss of individual health insurance coverage due to unemployment or trends in employee attrition, a shift from commercial payor coverage to government payor coverage, or an increase in demand for patient assistance and/or free drug programs, any of which would adversely affect access to our products and our net sales.

In some foreign countries, particularly Canada and the countries of Europe, Latin America and Asia Pacific, the pricing and reimbursement of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing and reimbursement negotiations with governmental authorities can take 12 to 24 months or longer after the receipt of regulatory approval and product launch. In order to obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to collect additional data, including conducting additional studies. Furthermore, several countries around the world have implemented government measures to either freeze or reduce pricing of pharmaceutical products. If reimbursement for our products is

unavailable in any country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed. In addition, many foreign countries reference to other countries' official public list price, hence an unsatisfactory price level in one country could consequently impinge negatively upon overall revenue.

We expect to experience pricing pressures in connection with the sale of our current and future products due to a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including managed care plans) to reduce healthcare costs, the scrutiny of pharmaceutical pricing, the ongoing debates on reducing government spending and additional legislative proposals. These healthcare reform efforts or any future legislation or regulatory actions aimed at controlling and reducing healthcare costs, including through measures designed to limit reimbursement, restrict access or impose unfavorable pricing modifications on pharmaceutical products, could impact our and our partners' ability to obtain or maintain reimbursement for our products at satisfactory levels, or at all, which could materially harm our business and financial results.

Additionally, ELEVIDYS and our gene therapy product candidates represent novel approaches to treatment that will call for new levels of innovation in both pricing, reimbursement, payment and drug access strategies. Current reimbursement models may not accommodate the unique factors of our gene therapy product and product candidates, including high up-front costs, lack of long-term efficacy and safety data and fees associated with complex administration, dosing and patient monitoring requirements. Hence, it may be necessary to restructure approaches to payment, pricing strategies and traditional payment models to support these therapies.

The downward pressure on healthcare costs in general has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our products and product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our products and product candidates (e.g., for administration of our products to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

Healthcare policy reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of our products and product candidates.

The U.S. government and individual states continue to aggressively pursue healthcare reform, which includes ongoing attempts to manage utilization as well as control and/or lower the cost of prescription drugs and biologics. See "Item 1. Business – Government Regulation – U.S. Healthcare and Other Reform" There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare policy will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid and private insurance healthcare costs, including proposed or implemented reforms involving price controls, waivers from Medicaid drug rebate law requirements, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs and implementing new requirements for, or eliminating caps on, rebates paid on products under government healthcare programs. Recent workforce reductions in and restructuring of the U.S. Department of Health and Human Services, including at the U.S. Food and Drug Administration, may create regulatory uncertainty, potentially impacting drug and biologic development programs and approvals. These cost containment measures may include, among other possible actions, implementation or modification of:

- controls on government funded reimbursement for drugs;
- mandatory discount requirements under certain government sponsored programs;
- caps on drug reimbursement under commercial insurance;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of coverage and reimbursement requirements;
- mechanisms utilized by managed care organizations to control utilization of drugs and other health care; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

In recent years, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their commercial products, which has resulted in several Congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products.

Additionally, in its 2024 decision in Loper Bright Enterprises v. Raimondo, the U.S. Supreme Court overruled the "Chevron doctrine," which gives deference to regulatory agencies' statutory interpretations in litigation against federal government agencies, such as the FDA, the Centers for Medicare & Medicaid Services ("CMS") and other federal agencies where the law is ambiguous. The Loper decision could result in additional legal challenges to regulations and guidance issued by federal agencies, including the FDA and the CMS, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the Loper decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the U.S. or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, our business could be materially harmed.

Under the current presidential administration, there have been significant and wide-ranging reforms to federal policy and the federal government. In particular, as in recent preceding presidential administrations, drug pricing and reimbursement reform is a focus of reform efforts. Other healthcare reform efforts or other actions under the current presidential administration may adversely affect the development of new drug therapies, access to healthcare coverage or the funding of healthcare benefits, although the full impact of such efforts or actions cannot be predicted. For example, the Congressional Budget Office has estimated that Medicaid provisions in the One Big Beautiful Bill Act ("OBBBA"), including restrictions in eligibility and funding for Medicaid, as well as changes to the healthcare marketplace will increase the number of uninsured by 16 million by 2034. As another example, recent workforce reductions in and restructuring of the U.S. Department of Health and Human Services, including at the U.S. Food and Drug Administration, may create regulatory uncertainty, potentially impacting drug and biologic development programs and approvals.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. There is ongoing uncertainty regarding the nature or impact of any drug or broader healthcare reform implemented by the current presidential administration through executive or administrative action or by Congress and the extent to which such action may be subject to litigation or other challenges. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for our products and product candidates, which would have an adverse effect on our net revenues and operating results.

Our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

The commercial success of our products, particularly in the U.S., depends upon the level of market adoption by patients, payors and healthcare providers. If our products do not achieve an adequate level of market adoption for any reason, or if market adoption does not persist, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of our products depends on a number of factors, including:

- our ability to demonstrate to the medical and payor community, including specialists who may purchase or prescribe our products, the clinical efficacy, effectiveness and safety of our products as the prescription products of choice for their respective indications;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for our products in a timely manner from government and private payors;
- the ability to timely demonstrate to the satisfaction of payors real world effectiveness and the economic, humanistic, societal and clinical benefits of our products;
- the burden or efficiency of payer prior authorization processes and the ability of families and physicians to navigate them;
- the actual and perceived efficacy and safety profile of our products, particularly if new safety signals arise or there are unanticipated adverse events related to our products' treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for our products do not support, or are interpreted by some parties to not support, the efficacy of our products; and
- the efficacy and safety of our other exon-skipping and gene therapy product candidates and third parties' competitive therapies.

For example, we recently announced two reported cases of ALF resulting in death in non-ambulatory patients following treatment with ELEVIDYS. Following these announcements, FDA proposed a safety label supplement for ELEVIDYS to include a

boxed warning for ALI and ALF. Subsequently, on July 18, 2025 we announced a reported case of ALF resulting in death in a patient following dosing in the Company's Phase 1/2 LGMD trial for SRP-9004. These announcements have impacted, and may continue to, impact the market adoption of our products and create uncertainty among patients, providers, and payers. Although we have resumed shipments to ambulatory patients in the U.S., we may experience continued hesitation from patients, payers and healthcare providers which could adversely impact our business.

Further, the potential commercial success of our product candidates as well as continued commercialization of ELEVIDYS will depend on additional factors, including the capacity of any infusion centers responsible for the administration of our product candidates and ELEVIDYS.

ELEVIDYS and our gene therapy product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events. New safety signals, failure of other gene therapy programs, negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of ELEVIDYS or our gene therapy product candidates and harm our ability to conduct our business, make accurate financial forecasts, or obtain regulatory approvals for ELEVIDYS or our gene therapy product candidates.

Gene therapy remains a newly applied technology, with only a few gene therapy products approved to date in the U.S., the EU or elsewhere, including ELEVIDYS.

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

In addition, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our products or product candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

More restrictive government regulations or negative public opinion would harm our business, financial condition, results of operations, financial guidance, ability to accurately forecast key financial metrics, and prospects and may delay or impair the development and commercialization of our gene therapy product candidates or demand ELEVIDYS or any other products we may develop. For example, earlier gene therapy trials of other sponsor's products led to several well-publicized adverse events, including death, and other gene therapy trials have failed to demonstrate efficacy. In addition, we recently announced two reported cases of ALF resulting in death of non-ambulatory patients following treatment with ELEVIDYS, as well as one case of ALF resulting in death of a non-ambulatory patient following dosing in the Company's Phase 1/2 LGMD trial for our gene therapy product candidate, SRP-9004. In response to these announcements, FDA revoked the platform technology designation for the Company's AAVrh74 Platform Technology associated with both ELEVIDYS and SRP-9004. The degree to which these events have impacted or will impact market acceptance of ELEVIDYS in ambulatory patients, or any of our other drug products, is uncertain and difficult to estimate, which may result in unpredictable variability in our financial forecasts.

Lack of efficacy and/or serious adverse events related to clinical trials or our commercial products we, our strategic partners or other companies conduct, even if such adverse events are not ultimately attributable to the relevant product candidates or products, and/or failed commercialization of gene therapy products may result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates, all of which could adversely impact our business.

We may not be able to expand the global footprint of our products outside of the U.S.

In addition to receiving accelerated approval in the U.S., EXONDYS 51 has been approved for marketing in Israel, Libya, Kuwait, and Georgia, AMONDYS 45 in Libya, Kuwait, and Georgia, and VYONDYS 53 in Libya, Kuwait, and Georgia. We may not receive approval to commercialize these products in additional countries. Our partner for ELEVIDYS, Roche, has received certain approvals for ELEVIDYS in territories outside of the U.S. In November 2016, we submitted a MAA for eteplirsen to the EMA and the application was validated in December 2016. As we announced on June 1, 2018, the CHMP of the EMA adopted a negative opinion for eteplirsen. In September 2018, the CHMP of the EMA confirmed its negative opinion for eteplirsen, and the European Commission adopted the CHMP opinion in December 2018. During 2019, we sought follow-up EMA scientific advice for eteplirsen.

Once data from our ongoing studies are available, we plan to evaluate future engagement with the EMA on potential next steps. We also recently announced that our ENVISION study is on pause.

In order to market any product in a country outside of the U.S., we must comply with numerous and varying regulatory requirements for approval in those countries regarding demonstration of evidence of the product's safety and efficacy and governing, among other things, labeling, distribution, advertising, and promotion, as well as pricing and reimbursement of the product. Obtaining marketing approval in a country outside of the U.S. is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject an application or delay, limit or deny approval of any of our products for many reasons, including:

- we may not be able to demonstrate to the satisfaction of regulatory authorities outside the U.S. the risk benefit of our products;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by regulatory authorities outside the U.S.;
- regulatory authorities outside the U.S. may disagree with the adequacy (number, design, size, controls, conduct or implementation) of our clinical trials prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities outside the U.S. may conclude that data we submit to them fail to demonstrate an appropriate level of safety or efficacy of our products, or that our products' respective clinical benefits outweigh their safety risks;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites or require us to generate additional data or information;
- regulatory authorities outside the U.S. may impose limitations or restrictions on the approved labeling of our products, thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes, or may require us to change our manufacturing process or specifications; and
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ significantly from that required to obtain approval in the U.S. In particular, in many foreign countries, it is required that a product receives pricing and reimbursement approval before the product can be distributed commercially. Many foreign countries undertake cost-containment measures that could affect pricing or reimbursement of our products. This can result in substantial delays, and the price that is ultimately approved in some countries may be lower than the price for which we expect to offer our products.

Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for our products and could adversely affect our business and financial condition. In addition, failure to obtain approval in one country or area may affect sales under the EAP in other countries or areas. Even if we are successful in obtaining regulatory approval of our products in additional countries, our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors.

In addition, we have granted Roche an exclusive option to obtain an exclusive license to commercialize certain products, including eteplirsen, golodirsen and casimersen, outside of the U.S. If this option is exercised, Roche will have sole control over and decision-making authority with respect to the commercialization of such products outside the U.S.

Historical revenues from eteplirsen, golodirsen and casimersen through our EAP outside the U.S. may not continue and we may not be able to continue to distribute our products through our EAP.

We established a global EAP for our products in some countries where these products currently have not been approved. While we generate revenue from the distribution of these products through our EAP, we cannot predict whether historical revenues from this program will continue, whether we will be able to continue to distribute our products through our EAP, or whether revenues will exceed revenues historically generated from sales through our EAP. Reimbursement of aforementioned products through our EAPs may cease to be available if authorization for an EAP expires or is terminated. For example, healthcare providers in EAP jurisdictions may not be convinced that their patients benefit sufficiently from our products or alternatively, may prefer to wait until such time as our products are approved by a regulatory authority in their country before prescribing any of our products. Even if a healthcare provider is interested in obtaining access to our products for its patient through the EAP, the patient may not be able to

obtain access to our products if funding for the drug is not secured. Also geo-political changes and challenges might negatively impinge upon future revenue generated through our EAP.

Our business and financial results have not yet been materially adversely affected by the ongoing conflict between Russia and Ukraine, or the conflict in the Middle-East. However, access to and reimbursement for patients in those regions through our EAP and consequently, our ability to generate revenue from sales of our products in Russia, Ukraine or the Middle East may be adversely affected in the future. The U.S. and other nations have raised the possibility of sanctions on companies that do business with Russia or its allies, including Belarus. We also may be adversely impacted by sanctions imposed on third parties with which we do business, such as third-party distributors and service providers of our EAP.

Any failure to maintain revenues from sales of our products through our EAP and/or to generate revenues from commercial sales of these products exceeding historical sales due to geo-political challenges like those potentially resulting from the ongoing conflict between Russia and Ukraine or the instability in the Middle-East, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Failure to obtain or maintain regulatory exclusivity for our products could result in our inability to protect our products from competition and our business may be adversely impacted. If a competitor obtains an authorization to market the same or substantially same product before a product of ours is authorized in a given country and is granted regulatory exclusivity, then our product may not be authorized for sale as a result of the competitor's regulatory exclusivity and as a result, our investment in the development of that product may not be returned.

In addition to any patent protection, we rely on various forms of regulatory exclusivity to protect our products. During the development of our products, we anticipate any one form of regulatory exclusivities becoming available upon approval of our products. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity, or failure to obtain or maintain the extent or duration of such protections that we expect in each of the markets for our products due to challenges, changes or interpretations in the law or otherwise, could affect our revenues for our products or our decision on whether to market our products in a particular country or countries or could otherwise have an adverse impact on our results of operations. We are not guaranteed to receive or maintain regulatory exclusivity for our current or future products, and if our products that are granted orphan status were to lose their status as orphan drugs or the data or marketing exclusivity provided for orphan drugs, our business and operations could be adversely affected.

Due to the nature of our products and product candidate pipeline, in addition to NCE exclusivity and new biologic exclusivity, orphan drug exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on orphan drug exclusivity to maintain a competitive position. If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the United States for seven years, we would not be able to exclude other companies from obtaining regulatory approval of products using the same or similar active ingredient for the same indication during or beyond the exclusivity period applicable to our product on the basis of orphan drug status. For example, the exclusivity period for EXONDYS 51 ended in September 2023. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process. A decision in 2021 by the U.S. Court of Appeals for the Eleventh Circuit in Catalyst Pharmaceuticals, Inc. vs. Becerra regarding interpretation of the Orphan Drug Act's exclusivity provisions as applied to drugs and biologics approved for orphan indications narrower than the product's orphan designation has the potential to significantly broaden the scope of orphan exclusivity for such products. While the FDA has since taken the position that it will continue to apply orphan drug exclusivity only on the basis of the specific indication, the Supreme Court's recent decision in 2024 in Loper Bright Enterprises v. Raimondo has the potential to impact how the Agency applies the Catalyst decision. Our ability to obtain or seek to work around orphan exclusivity, as well as our ability to retain orphan exclusivity that the FDA previously has recognized for our products, may be impacted depending on how the Catalyst decision is ultimately implemented. Legislation has been introduced to amend the Orphan Drug Act in a way that may prevent these effects of the Catalyst decision, but it is unclear if or when such legislation could be enacted.

In addition, we may face risks with maintaining regulatory exclusivities for our products, and our protection may be circumvented, even if maintained. For instance, orphan drug exclusivity in the U.S. may be rescinded if (i) an alternative, competing product demonstrates clinical superiority to our product with orphan exclusivity; or (ii) we are unable to assure the availability of sufficient quantities of our orphan products to meet the needs of patients. Moreover, competitors may receive approval of different drugs or biologics for indications for which our prior approved orphan products have exclusivity. In Europe, the granted orphan exclusivity period may be reduced to six years if, at the end of the fifth year, it is established, in respect of the medicinal product concerned, that the criteria for orphan designation are no longer met, among other things, where it is shown on the basis of available evidence that the product is sufficiently profitable not to justify maintenance of market exclusivity. The granted market exclusivity may also be ineffective against a similar medicinal product where the originator is unable to supply sufficient quantities of the medicinal product or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial

orphan drug. The scope of the orphan drug exclusivity in Europe may be modified after grant of the market authorization of the orphan product (e.g., the approved therapeutic indication based on the benefit-risk assessment is narrower than or a subset of the designated orphan indication). Where the therapeutic indication being sought for approval does not fall within the scope of the designated orphan condition, a request should be sought for the designation decision to be amended. An amendment is possible only if the new condition differs slightly from that designated previously.

Thus, other companies may have received, or could receive, approval to market a product candidate that is granted orphan drug exclusivity for the same drug or similar drug and same orphan indication as any of our product candidates for which we plan to file an NDA, BLA or MAA. If that were to happen, our prior approved orphan products may face competition and any pending NDA, BLA or MAA for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the U.S. or the EU, as applicable. For example, in September 2021, the FDA issued guidance concerning its position on interpreting when gene therapy products would be considered the "same" or "different" for purposes of orphan drug exclusivity. The guidance states that if two gene therapy products have or use different vectors, the FDA generally intends to consider them to be "different" drugs. Further, according to the guidance, the FDA generally intends to consider vectors from the same viral group (e.g., AAV2 vs. AAV5) to be different, when the differences between the vectors impact factors such as tropism, immune response avoidance, or potential insertional mutagenesis. However, there is considerable uncertainty as to the interpretation of these guidelines. As illustrated by this guidance, orphan drug exclusivity as applied to gene therapy products is an evolving area subject to change and interpretation by the FDA and therefore, we cannot be certain as to how the FDA will apply those rules to ELEVIDYS or our gene therapy product candidates. Similarly, pursuant to the 2018 Commission Regulation, two gene therapy medicinal products are not considered similar when there are differences in the therapeutic sequence, viral vector, transfer system, regulatory sequences or manufacturing technology that significantly affect the biological characteristics and/or biological activity relevant for the intended therapeutic effect and/or safety attributes of the product.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of our products may be negatively impacted.

We have hired and trained a commercial team and put in the organizational infrastructure we believe we need to support the commercial success of our products in the U.S. Factors that may inhibit our efforts to maintain and further develop commercial capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or our products, to deliver a consistent message regarding our products and be effective in educating physicians on how to prescribe our products;
- an inability to equip sales personnel with compliant and effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding our products and their proper administration and educate payors on the safety, efficacy and effectiveness profile of our products to support favorable coverage decisions;
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization; and
- an inability to develop effective commercial, sales and marketing infrastructure to support new product launches.

If we are not successful in maintaining an effective commercial, sales and marketing infrastructure, we will encounter difficulty in achieving, maintaining or increasing projected sales of our products in the U.S., which would adversely affect our business and financial condition.

The patient population suffering from Duchenne, LGMD, FSHD and DM1 is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected.

Duchenne and LGMD are rare, fatal genetic disorders. FSHD is a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 13,000. DM1 is also a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 30,000. Duchenne affects an estimated one in approximately every 3,500 to 5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon 51 skipping, up to 8% are estimated to be amenable to exon 53 skipping and up to 8% are estimated to be amenable to exon 45 skipping. LGMDs as a class affect an estimated range of approximately one in every 14,500 to one in every 123,000 individuals. Our estimates of the size of these patient populations are based on a limited number of published studies as well as internal analyses. Various factors may decrease the market size of our products and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our products and product candidates. If the results of these studies or our analysis of them do not accurately reflect the relevant patient population, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to maintain profitability.

We face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products.

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change, including the use of artificial intelligence ("AI"). We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas in which our products and product candidates are aimed. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our products or product candidates. For example, we face competition in the field of Duchenne by third parties who are developing or who had once developed:

(i) exon skipping product candidates, such as Wave (targeting various exons, including 53 and 51), Nippon Shinyaku (targeting various exons, including 51 and 45, and notably for exon 53 for which it has received accelerated FDA approval for its product Viltepso (viltolarsen)), Dyne Therapeutics pursuing antibody-oligonucleotide conjugates for exons 44, 45, 51, and 53, Avidity Biosciences pursuing antibody-oligonucleotide conjugates for exons 44, 45 and 51, SQY Therapeutics and BioMarin (BMN-351 for exon 51), Entrada (notably for exon 44, 45, 50, and 51);

(ii) gene therapies, such as Genethon and Solid (also in partnership with Ultragenyx), and Regenxbio;

(iii) gene editing, including CRISPR/Cas 9 approaches, such as GenAssist, CRISPR Therapeutics, and Precision Biosciences;

(iv) other disease modifying approaches, such as PTC Therapeutics and Satellos, which has a small molecule candidate, ataluren, that targets nonsense mutations; and

(v) other approaches that may be palliative in nature or potentially complementary with our products and product candidates and that are or were once being developed including but not limited to, Santhera (Reveragen), Capricor Therapeutics (in partnership with Nippon Shinyaku), BioPhytis, Italfarmaco (approved product Givinostat), Dystrogen and Edgewise Therapeutics. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of Duchenne, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of Duchenne. Indeed, BioMarin is conducting clinical trials for BMN-351, an oligonucleotide therapy. In addition, while Wave announced its intention to discontinue development of suvodirsen and suspend development of WVE-N531, it is conducting clinical trials for its exon 53 oligonucleotide, WVE-N531.

In addition, we are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development using platform technologies that may be viewed as competing with ours beyond and including those companies mentioned immediately above, such as Alnylam Pharmaceuticals, Inc., Arbutus (formerly Tekmira Pharmaceuticals Corp.), Deciphera Pharmaceuticals, Ionis Pharmaceuticals, Inc., Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Shire plc (now Takeda), Biogen, Moderna Therapeutics, Avidity, Dyne Therapeutics, Stoke Therapeutics, Ultragenyx, Sanofi and PepGen. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene therapy and gene editing (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Astellas Pharma, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire (now Takeda), Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Akashi, Capricor Therapeutics (in partnership with Nippon Shinyaku), Oxford University, Exonics Therapeutics (acquired by Vertex Pharmaceuticals), and Editas Medicine.

If any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to enter into the market, gain market share or maintain market share in the Duchenne space or other diseases targeted by our platform technologies, products and product candidate pipeline.

It is possible that our competitors will succeed in developing technologies that, in addition to limiting the market size for our products or product candidates, impact the regulatory approval and post-marketing process for our products and product candidates, are more effective than our products or product candidates or would render our technologies obsolete or noncompetitive. Our competitors may, among other things, relative to our products or product candidates:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- have lower cost of goods;
- receive more favorable reimbursement coverage;
- obtain preferred formulary status;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

Further, development and commercialization of ELEVIDYS and any expansion of its currently approved label, and development of our gene therapy product candidates, may compete with or supersede our current approved products, which may impact future revenues from sales of our current approved products. Our gene therapy product candidates are being developed for potential treatment of overlapping patient populations with our current approved products, and we have not determined if our gene therapy product candidates will be used in patients in combination with our existing approved products or in separate treatment regiments.

Our revenue could face competitive pressures for any of the above reasons. Moreover, if competing products are marketed in a territory in which we also have the authority to market our products, our sales may diminish, or our business could be otherwise materially adversely affected.

Future sales of ELEVIDYS may decrease sales growth, or reduce sales, of our PMO products, which could negatively impact our operating results, including through potential inventory write-offs.

Substantial overlap may exist between the addressable patient population for ELEVIDYS and the patient populations eligible for treatment with our PMO products. In the future, ELEVIDYS may be used in combination with our PMO products or may be adopted as a separate treatment regimen. Accordingly, ELEVIDYS may compete with our PMO products. As a result, successful commercialization of ELEVIDYS may reduce sales of our PMO products, potentially resulting in significant accounting charges relating to write-off of inventory if such inventory becomes in excess, obsolete or unusable.

We have entered into multiple collaborations and strategic transactions and may seek or engage in future strategic collaborations, alliances, acquisitions or licensing agreements or other relationships that complement or expand our business. We may not be able to complete such transactions, and such transactions, if executed, may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

In order to achieve our long-term business objectives, we actively evaluate various strategic opportunities on an ongoing basis, including licensing or acquiring products, technologies or businesses. We may face competition from other companies in pursuing such opportunities. This competition is most intense for approved drugs and late-stage drug candidates, which have the lowest risk in terms of probability of success but would have a higher risk and more immediate effect on our financial performance. Our ability to complete transactions may also be limited by applicable antitrust and trade regulation laws and regulations in the relevant U.S. and foreign jurisdictions in which we or the operations or assets we seek to acquire carry on business.

We have entered into multiple collaborations, including with Roche, Arrowhead, Nationwide, Duke University, Dyno Therapeutics, and Hansa Biopharma. We may not realize the anticipated benefits of such collaborations, and the anticipated benefits of any future collaborations or strategic relationships, each of which involves numerous risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- collaborators may not pursue development and commercialization of our products or product candidates based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates, or otherwise undermine or devalue the efforts of our collaboration;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our products or product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may eliminate our rights to commercialize certain product candidates or may result in a need for additional capital;
- failure to successfully develop the acquired or licensed drugs or technology or to achieve strategic objectives, including successfully developing and commercializing the drugs, drug candidates or technologies that we acquire or license;
- entry into markets in which we have no or limited direct prior experience or where competitors in such markets have stronger market positions;
- disruption of our ongoing business, distraction of our management and employees from other opportunities and challenges and retention of key employees;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired company, or acquired or licensed product or technology, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, accounting practices, employee, customer or third-party relations and other known and unknown liabilities;
- liability for activities of the acquired company or licensor before the acquisition or license, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities;
- exposure to litigation or other claims in connection with, or inheritance of claims or litigation risk as a result of an acquisition or license, including but not limited to, claims from terminated employees, customers, former equity holders or other third-parties;
- difficulty in integrating the products, product candidates, technologies, business operations and personnel of an acquired asset or company; and
- difficulties in the integration of the acquired company's departments, systems, including accounting, human resource and other administrative systems, technologies, books and records, and procedures, as well as in maintaining uniform standards, controls, including internal control over financial reporting required by the Sarbanes-Oxley Act of 2002 and related procedures and policies.

For example, we will have limited influence and control over the development and commercialization activities of Roche in the territories in which it leads development and commercialization of ELEVIDYS, and if the exclusive option is exercised, in the territories in which it may lead commercialization of certain other products or product candidates. Roche's development and commercialization activities in the territories where it is the lead party may adversely impact our own efforts in the U.S. Failure by Roche to meet its obligations under the Roche Agreement, to apply sufficient efforts at developing and commercializing collaboration products, or to comply with applicable legal or regulatory requirements, may materially adversely affect our business and our results of operations. In addition, to the extent we rely on Roche to commercialize any products for which we obtain regulatory approval, we will receive less revenues than if we commercialized these products ourselves.

Even if we achieve the long-term benefits associated with strategic transactions, our expenses and short-term costs may increase materially and adversely affect our liquidity and short-term net income (loss). Future licenses or acquisitions could result in potentially dilutive issuances of our equity securities, the incurrence of debt, the creation of contingent liabilities, impairment or expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition.

Risks Related to the Development of our Product Candidates

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology, delays in our ability to expand the labels of any of our approved products or termination of the clinical trials altogether.

We, or our strategic partners, may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete clinical trials within the expected timeframe. Patient enrollment can be impacted by factors including, but not limited to:

- design and complexity and/or commitment of participation required in the study protocol;
- size of the patient population;
- diagnostic capabilities within patient population;
- eligibility criteria for the study in question;
- clinical supply availability;
- delays in participating site identification, qualification and subsequent activation to enroll;
- perceived risks and benefits of the product candidate under study, including in response to adverse effects observed in our products and product candidates and similar or competing therapies;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- competition of site efforts to facilitate timely enrollment in clinical trials;
- participating site motivation;
- patient referral practices of physicians;
- activities of patient advocacy groups;
- ability to monitor patients adequately during and after treatment; and
- severity of the disease under investigation.

In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because newborn screening for these diseases is not widely adopted, and 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, pandemics and other national or regional health emergencies may impact patient ability and willingness to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact enrollment in our clinical trials.

We may not be able to initiate or continue clinical trials if we cannot enroll the required eligible patients per protocol to participate in the clinical trials required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate,

enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations ("CROs") and physicians;
- different standards for the conduct of clinical trials;
- our inability to locate qualified local consultants, physicians and partners;
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment;
- ability to procure and deliver necessary clinical trial materials needed to perform the study; and
- inability to implement adequate training at participating sites remotely when in person training cannot be completed.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business and on our ability to maintain our accelerated approval in the U.S.

Failures or delays in the commencement or completion of ongoing and planned clinical trials of our product candidates could negatively impact commercialization efforts; result in increased costs; and delay, prevent or limit our ability to gain regulatory approval of product candidates and to generate revenues and continue our business.

Successful completion of clinical trials at each applicable stage of development is a prerequisite to submitting a marketing application to the regulatory agencies and, consequently, the ultimate approval and commercial marketing of any of our product candidates for the indications in which we develop them. We do not know whether any of our clinical trials, or those with our strategic partners, will begin or be completed, and results announced, as planned or expected, if at all, as the commencement and completion of clinical trials and announcement of results is often delayed or prevented for a number of reasons, including, among others:

- denial by the regulatory agencies of permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or placement of a clinical trial on hold. For example, on July 21, 2025, we announced that the FDA placed the LGMD FDA Clinical Hold following a case of ALF resulting in the death of a patient in our Phase 1/2 LGMD clinical trial for SRP-9004, which could impact the timing of a BLA submission for SRP-9003;
- delays in filing or receiving approvals of additional INDs that may be required;
- negative and/or unanticipated results from our ongoing non-clinical trials or clinical trials;
- challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials;
- challenges with subject compliance within clinical trials;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs/ vendors involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards ("IRBS"), such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting us to various risks;
- inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials, for example as a result of delays in defining and implementing the manufacturing process for materials used in pivotal trials or for the manufacture of larger quantities or other delays or issues arising in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining IRB approval, and equivalent (Ethics Committees or ECs) approval for sites outside the U.S., to conduct a clinical trial at a prospective site or sites;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- delays or problems in analyzing data, or the need for additional analysis or data or the need to enroll additional patients;
- the occurrence of serious adverse events or unexpected drug-related side effects experienced by patients in a clinical trial or unexpected results in ongoing non-clinical trials;

- delays in validating endpoints utilized in a clinical trial;
- delays in validating outcome assessments needed in a clinical trial;
- our inability to have formal meetings with the regulatory agencies or to interact with them on a regular basis;
- our inability to satisfy the requirements of the regulatory agencies to commence clinical trials, such as developing potency assays and lot release specifications that correlate with the activity or response of the product candidate or other CMC requirements;
- the regulatory agencies disagreeing with our clinical trial design and our interpretation of data from clinical trials, or changing the requirements for approval even after the regulatory authority has reviewed and commented on the design for our clinical trials;
- reports from non-clinical or clinical testing of competing therapies that raise safety or efficacy concerns; and
- the recruitment and retention of employees, consultants or contractors with the required level of expertise.

Further, any reduction in FDA's workforce could delay or materially impact FDA's feedback on our development programs, including through meetings and other informal interactions, and affect FDA's review and oversight of our product candidates. Additionally, changes in FDA personnel under the new presidential administration may lead to changes in the regulations, policies and operations of the FDA, which may impact our clinical development plans. Any of these actions could adversely affect the development and approval of our product candidates.

Any inability to complete successfully pre-clinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties, as well as our ability to maintain our accelerated approvals. In addition, manufacturing or formulation changes to our product candidates often require additional studies to demonstrate comparability of the modified product candidates to earlier versions. Clinical study delays also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which impairs our ability to successfully commercialize our product candidates and harms our business and results of operations.

Clinical development is lengthy and uncertain. Clinical trials of our product candidates may be delayed, and certain programs may never advance in the clinic or may be more costly to conduct than we anticipate, any of which could have a material adverse impact on our business.

Clinical testing is expensive and complex and can take many years to complete, and its outcome is inherently uncertain. We may not be able to initiate, may experience delays in, or may have to discontinue clinical trials for our product candidates as a result of numerous unforeseen events, including:

- the FDA, other regulators, IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site for any number of reasons, including concerns regarding safety and aspects of the clinical trial design;
- we may experience delays in reaching, or fail to reach, agreement on favorable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the outcome of our pre-clinical studies and our early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results;
- we may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful;
- clinical trials of any product candidates may fail to show safety or efficacy, or produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional nonclinical studies or clinical trials, or we may decide to abandon product development programs;
- differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials;
- pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many product candidates believed to have performed satisfactorily in pre-clinical studies and clinical trials have nonetheless failed to obtain marketing approval; and
- regulators may elect to impose a clinical hold, or we or our investigators, IRBs, or ethics committees may elect to suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding

that the participants are being exposed to unacceptable benefit risk ratio. For example, in the past we have received clinical holds from the FDA, and, on July 21, 2025, we announced that the FDA placed the LGMD FDA Clinical Hold. Although these holds have generally not materially affected our development timelines, there is no assurance that our current hold or any future hold would not have a material adverse effect. A clinical hold, or any of the above factors, may be out of our control and could materially impair our development timelines, expenses and results of operations.

Results from pre-clinical and early-stage clinical trials may not be indicative of safety or efficacy in late-stage clinical trials, and pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical trials, that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials, including those with our strategic partners, of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although we believe the data for SRP-9003 collected to date are positive, the additional data we collect may not be consistent with the pre-clinical and/or early clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval.

Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials. For example, announcements for SRP-9003 include: in March 2022, we announced 24-month functional data from two clinical trial participants in the high-dose cohort, and 36-month functional data from three clinical trial participants in the low-dose cohort for SRP-9003. These data are based on small patient samples, and, given the heterogeneity of LGMD patients and potential lot-to-lot variability, the data may not be predictive of future results. In addition, we cannot assure that the results of additional data or data from any future trial will yield results that are consistent with the data presented, that we will be able to demonstrate the safety and efficacy of these product candidates, that later trial results will support further development, or even if such later results are favorable, that we will be able to successfully complete the development of, obtain accelerated, conditional or standard regulatory approval for, or successfully commercialize any of such product candidates. Similarly, we cannot provide assurances that data from our ongoing and planned studies with respect to our commercially approved products and product candidates will be positive and consistent or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our products or product candidates will be consistent with our interpretations.

Our products or product candidates may cause undesirable side effects, result in new safety signals or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

We have recently reported new safety signals in the non-ambulatory population for ELEVIDYS and for our LGMD product candidate SRP-9004. For example, on March 18, 2025, Sarepta announced that a young man with Duchenne passed away following treatment with ELEVIDYS after having suffered from ALF. On June 15, 2025, Sarepta announced a second reported case of ALF resulting in death in a patient following treatment with ELEVIDYS, and that both cases of ALF occurred in non-ambulatory individuals. Such adverse events have resulted in FDA's proposal of a label supplement for ELEVIDYS to include a boxed warning for ALI and ALF. On July 18, 2025, Sarepta announced a reported case of ALF resulting in death in a patient following dosing in the Company's Phase 1/2 LGMD trial for SRP-9004.

In addition to side effects caused by our product candidates or products, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our trials, we may decide, or the FDA, the EMA or other regulatory authorities could order us, to halt, delay or amend pre-clinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. For example, the FDA placed SRP-9003, SRP-9004, SRP-6005 and SRP-9005 on clinical hold following the reported death in a patient following dosing in the Company's Phase 1/2 LGMD trial of SRP-9004.

Even if we are able to demonstrate that all future serious adverse events are not product-related, 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 of our product candidates, 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.

If there are significant delays in obtaining, or if we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the U.S., approvals and oversight from federal (e.g., the FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the U.S. or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application to the FDA or an MAA to the EMA (or NCA of an EU member state) and even fewer are approved for commercialization.

Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates in any jurisdiction, including in the U.S. or the EU, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, chemistry, manufacturing and controls and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA, BLA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that our data are insufficient to provide a positive recommendation for approval of any NDA or BLA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA or BLA submission, the FDA could still disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- The regulatory approval process for product candidates targeting orphan diseases, such as Duchenne, that use new technologies and processes, such as antisense oligonucleotide therapies, gene therapy and other alternative approaches or endpoints for the determination of efficacy is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, small safety databases, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. As a result of uncertainty in the approval process for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs, BLAs and MAAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional safety data, patient muscle biopsies, dystrophin analyses and the use of assays), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or BLA or result in a decision by the Company not to proceed with an NDA or BLA submission for a product candidate based on feedback from regulators.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates.

Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for PMO, gene therapy-based product candidates or other product candidates. Responding to requests from regulators and meeting requirements for clinical trials, submissions and approvals may require substantial personnel, financial or other resources, which, as a small biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third party vendors and associates may be complicated by our own limitations and those of the parties we work with. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including guidance related to clinical trial design with respect to any NDA, BLA or MAA submissions.

Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Disruptions at regulatory agencies that are unrelated to our products and product candidates could delay the review and approval of our products, which could adversely affect our business. For example, changes in government, the ability to hire and retain key personnel and statutory and regulatory

changes could result in delays. In addition, government funding of regulatory, government agencies, and programs on which our operations may rely is subject to the impacts of political events, which are inherently unpredictable and fluid. Further, additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval. Since the start of the new presidential administration in 2025, U.S. policy changes have been implemented at a rapid pace and additional changes are likely. It is difficult to predict how executive actions that may be taken under the current administration may affect the FDA's ability to exercise its regulatory authority. If any actions impose constraints on the FDA's ability to engage in routine oversight and product review activities in the normal course, our business may be negatively impacted. Additionally, the new administration and federal government could adopt legislation, regulations, or policies that adversely affect our business or create a more challenging and costly environment to pursue the development, approval, and commercialization of our products.

In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. Furthermore, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. Finally, some of our product candidates may require diagnostic tests to ensure we appropriately select patients suitable for treatment. If we are unable to successfully develop diagnostic tests for these product candidates, experience significant delays in doing so, or are unable to obtain required regulatory clearances or approvals for any diagnostic tests, the commercialization of our product candidates may be delayed or prevented. Even if we receive the required regulatory clearance or approvals for certain diagnostic tests, the commercial success of any of our product candidates that require such tests will be dependent upon the continued availability of such tests.

In addition, adverse events or new safety signals have in the past resulted and could result in the future in regulatory agency actions or cause delays in commercialization. For example, in response to two reported cases of ALF resulting in death of non-ambulatory patients, we suspended shipment of ELEVIDYS in the U.S. to non-ambulatory patients in June 2025. Additionally, in July 2025, we disclosed a reported case of ALF in a non-ambulatory patient participating in our Stage 1/2 LGMD trial for SRP-9004, who was not treated with ELEVIDYS. Thereafter, in response to a request from FDA that we voluntary stop all shipments of ELEVIDYS in the U.S., we suspended shipment of ELEVIDYS in the U.S. to ambulatory patients effective July 22, 2025. On July 28, 2025, the FDA informed the Company that it recommended the removal of the voluntary hold for ambulatory patients. In response, the Company has resumed commercial shipments of ELEVIDYS for ambulatory patients in the U.S. The suspension of shipments for non-ambulatory patients remains in effect as of the date of issuance of this Quarterly Report. It is currently unclear whether or when the Company might resume shipments of ELEVIDYS to non-ambulatory patients, or whether the FDA will pursue further actions, such as additional studies, additional product modifications or controls, or withdrawal in the future

We are investing significant resources in the development of siRNA and gene therapy products and product candidates. If we are unable to show the safety and efficacy of these product candidates, experience delays in doing so or are unable to successfully commercialize at least one of these drugs, our business would be materially harmed.

We have invested significant resources in the development of our gene therapy products and product candidates, and are investing significant resources in the development of our siRNA product candidates. Within the FDA, the Center for Drug Evaluation and Research ("CDER") typically regulates siRNA products. We believe that a significant portion of the long-term value attributed to our company by investors is based on the commercial potential of these product candidates. There can be no assurance that any development problems we experience in the future related to our siRNA programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Development problems and delays in one program may delay the development of other programs. Early results from ongoing clinical trials may differ materially from final results from such clinical trials. The results from pre-clinical and early clinical studies do not always accurately predict results in later, large-scale clinical trials. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have been approved in the western world. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene therapy product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA and the EC may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The CBER works closely with the National Institutes of Health (the "NIH"). The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. For example, on January 28, 2020, the FDA issued final guidance documents that updated draft guidance documents that were originally released in July 2018 to reflect recent advances in the field, and to set forth the framework for the development, review and approval of gene therapies. These final guidance documents pertain to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. The FDA also issued a new guidance document in September 2021 describing the FDA's approach for determining whether two gene therapy products were the same or different for the purpose of assessing orphan drug exclusivity, as well as a final guidance document in January 2024 on human gene therapy product incorporating human genome editing. The FDA also issued a draft guidance in December 2023 that provides recommendations for developing a potency assurance strategy for gene therapy products. In addition, the FDA can put an IND on hold if the information in an IND is not sufficient to assess the risks in pediatric patients.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines, failure of which may lead to delayed or discontinued development of our product candidates. For example, we intend to seek FDA alignment on a clinical trial for ELEVIDYS related to the use of sirolimus immunosuppression in non-ambulatory patients. FDA may also seek additional clinical trials or studies related to ELEVIDYS, which could impact the timing of resuming shipments of ELEVIDYS to non-ambulatory patients in the U.S. and adversely impact the Company.

If the anticipated or actual timing of marketing approvals for our gene therapy product candidates, or the market acceptance of these product candidates, if approved, including treatment reimbursement levels agreed to by third-party payors, do not meet the expectations of investors or public market analysts, the market price of our common stock would likely decline.

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to determine the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. Achieving appropriate statistical power may be challenging for some of the ultra-rare genetically defined diseases we are targeting in our programs, especially if the acceptance of descriptive data is not yet established. In addition, different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn.

Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy ("RMAT") designation by the FDA, or access to the Priority Medicine scheme ("PRIME") by the EMA, for our product candidates, if granted, may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek fast track, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies such as our gene therapy product candidates. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme built on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment administered by the EMA to enhance support for the development of medicines that are considered of major public health interest, in particular from the viewpoint of therapeutic innovation to address an unmet medical need. By engaging with medicine developers early on, PRIME aims at improving scientific evidence-generation so that the data generated are suitable for evaluating a marketing-authorization application. Once admitted to the PRIME scheme, the sponsor will benefit from scientific and regulatory advice on the overall development plan and at major milestones, with an opportunity to involve stakeholders such as health technology bodies responsible for determining adoption of new treatment methods in the EU national health systems. PRIME-designated medicinal products may be eligible for accelerated assessment where the centralized assessment timeframe for 210 days, not counting procedural clock stops, can be reduced to 150 days.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review. This application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RMAT designations will accelerate approval and will include all the benefits of fast

track and breakthrough therapy designations, including early interactions with the FDA, but the exact mechanisms have not yet been announced by FDA.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product, the FDA or the EMA may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the relevant agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

Even though our products are PRIME designated, the EMA may not accept that our products are eligible for expedited assessment. The EMA may decide to return to the standard assessment timeframe of 210 days if an application initially granted accelerated assessment does not meet the criteria for accelerated assessment.

We may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success.

Our pipeline includes programs in various stages of development for a broad range of diseases and disorders. Because we have limited resources, we may not be able to advance all of our programs. We may also forego or delay pursuit of opportunities with certain programs or for indications that later prove to have greater commercial potential. For example, in connection with our recent restructuring, we have paused a number of our programs, including those for LGMD and CMT. 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 for product candidates may not yield any commercially viable products. 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 strategic 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, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Risks Related to Third Parties

If we are unable to maintain our agreements with third parties to distribute our products to patients, our results of operations and business could be adversely affected.

We rely on third parties to commercially distribute our products to patients in the U.S. We have contracted with a third-party logistics company to warehouse our products and with distributors and specialty pharmacies to sell and distribute our products to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management.

This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from our products. If we are unable to effectively manage the distribution process, the sales of our products, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of third parties involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using our products or serious adverse events and/or product complaints regarding our products;
- not effectively sell or support our products;
- reduce or discontinue their efforts to sell or support our products;
- not devote the resources necessary to sell our products in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or

- cease operations.

Any such events may result in decreased product sales, lower product revenue, loss of revenue, and/or reputational damage, which would harm our results of operations and business.

With respect to the pre-commercial distribution of our products to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute our products in certain countries through our EAP. We will need to continue building out our network for commercial distribution in jurisdictions in which our products are approved, which will also require third party contracts. The use of distributors and service providers involves certain risks, including, but not limited to, risks that these organizations will not comply with applicable laws and regulations, or not provide us with accurate or timely information regarding serious adverse events and/or product complaints regarding our products. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of our products in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

We rely on third parties to conduct some aspects of our early stage research and pre-clinical and clinical development. The inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development.

We have relied upon, and plan to continue to rely upon, third parties to conduct some aspects of our early stage research and pre-clinical and clinical development with respect to certain of our product candidates, including our follow-on exon-skipping product candidates, gene therapy, gene editing product candidates and siRNA product candidates. Our third-party collaborators may not commit sufficient resources or adequately develop our programs for these candidates. If our third-party collaborators fail to commit sufficient resources to any of our product candidates or to carry out their contractual duties or obligations, our programs related to any particular product candidate could be delayed, terminated, or unsuccessful. Furthermore, if we fail to make required payments to these third-party collaborators, including up-front, milestone, reimbursement or royalty payments, or to observe other obligations in our agreements with them, these third parties may not be required to perform their obligations under our respective agreements with them and may have the right to terminate such agreements. In addition, if our strategic partners experience regulatory delays for the development of their clinical product candidates, including clinical holds, our opportunities to commercialize products may be delayed.

We also have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data completeness for our ongoing pre-clinical and clinical programs. We rely on these parties for execution of our pre-clinical and clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on collaborators and CROs does not relieve us of our regulatory responsibilities.

The individuals at our third-party collaborators and CROs who conduct work on our behalf, including their sub-contractors, are not always our employees, and although we participate in the planning of our early stage research and pre-clinical and clinical programs, we cannot control whether or not they devote sufficient time and resources or exercise appropriate oversight of these programs, except for remedies available to us under our agreements with such third parties. If our collaborators and CROs do not successfully carry out their contractual duties or obligations or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our pre-clinical and clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Our reliance on third parties requires us to share our proprietary information, which increases the possibility that a competitor will discover them or that our proprietary information will be misappropriated or inadvertently disclosed.

Our reliance on third-party collaborators requires us to disclose our proprietary information to these parties, which could increase the risk that a competitor will discover this information or that this information will be misappropriated or disclosed without our intent to do so. If any of these events were to occur, then our ability to obtain patent protection or other intellectual property rights could be irrevocably jeopardized, and costly, distracting litigation could ensue. Furthermore, if these third parties cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our products or product candidates, our development programs may be delayed. Although we carefully manage our relationships with our third-party collaborators and CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Some of the third parties we rely for early-stage research and pre-clinical development are located in China. There has been increased governmental focus in the U.S. on the role of Chinese companies in the life sciences industry. This focus has included U.S. legislative proposals, such as the proposed BIOSECURE Act, which is pending before the U.S. Senate. If enacted, the BIOSECURE Act would, among other things, prohibit U.S. federal agencies from entering into or renewing any contract with any entity that uses biotechnology equipment or services produced or provided by a "biotechnology company of concern" to perform that contract with the government. If adopted, the BIOSECURE Act could cause us to seek to exit some or all of our arrangements with China-based service providers determined to be "biotechnology companies of concern" and transition these services to alternative companies.

Risks Related to Manufacturing

We currently rely on third parties to manufacture our products and to produce our product candidates; our dependence on these parties, including failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial, EAP, clinical and pre-clinical product demand may impair the availability of product for commercial supply or to successfully support various programs, including research and development and the potential commercialization of additional product candidates in our pipeline.

We rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product and to provide labeling and packaging of vials and storage of our products and product candidates. The limited number of third parties with facilities and capabilities suited for the manufacturing process of our products and product candidates creates a risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. As of the date of this Quarterly Report, we have dual sourcing for the APIs and drug product for all three of our PMO commercial products and one source for ELEVIDYS drug substance and drug product manufacturing.

In addition, the process for adding new manufacturing capacity is lengthy and often causes delays in development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as a future pandemic, order delays for equipment or materials, equipment malfunctions, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, such as earthquakes or fires, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in supply of our products, product candidates or materials. Any delay or interruption in the supply of finished products could hinder our ability to distribute our products to meet commercial demand or execute our commercialization plans on the timing that we expect, which could result in the loss of potential revenues, adversely affect our ability to gain market acceptance, or otherwise adversely affect our business, financial condition and prospects.

If these third parties cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture our products or product candidates in sufficient quality and quantity required for our planned commercial, pre-clinical and clinical or EAPs, our various product research, development and commercialization efforts would be adversely affected.

Furthermore, any problems in our manufacturing process or the facilities with which we contract make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs.

We, through our third-party manufacturers, seek to produce or produce supply of our products and product candidates. In light of the limited number of third parties with the expertise to produce our products and product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our products and product candidates. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our products and product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which impacts our ability to execute our business plans on expected or required timelines in connection with the commercialization of our products and the continued development of our product candidates. When we enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, we constrain our operational flexibility.

We also rely on a third party to design, manufacture, obtain and maintain regulatory approval for necessary diagnostic tests for ELEVIDYS. Any delay or failure by us or our collaborators to develop or obtain regulatory approval of the necessary diagnostic tests could harm our business, possibly materially.

The operations at one of our partner sites could also be disturbed by man-made or natural disasters, public health pandemics or epidemics or other business interrupts such as potential supply chain disruptions caused by the ongoing conflict between Russia and Ukraine. In addition, the need to prioritize rated orders issued by the Federal Emergency Management Agency pursuant to the U.S. Defense Production Act could impact the manufacturing, supply chain and distribution of our products and product candidates.

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems or inaccurately forecast demand, which could result in delays in commercialization or development of other gene therapy programs, limit the supply of our product candidates or future approved products or otherwise harm our business.

We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of ELEVIDYS and our gene therapy product candidates. Several factors could cause production interruptions, including talent acquisition/retention, equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers. In addition, several of the components used in our testing are currently from single-source suppliers. If these single-source suppliers fail to satisfy the Company's requirements on a timely basis, or if the Company is forced to change suppliers, the Company could suffer delays, a possible loss of revenue, or incur higher costs, any of which could adversely affect its operating results.

The physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in delay in product release, product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical and/or commercial-grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs. Lot failures or product recalls could cause us to delay clinical trials or product launches, or may result in an inability to fulfill demand for commercial supply of ELEVIDYS, or other future gene therapy products, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the competent authority authorizes its release.

As our product candidates advance to later stage clinical trials, it is customary that various CMC aspects of the development program, such as manufacturing, formulation and other processes, and route of administration, may be altered to optimize the candidates and processes for scale-up necessary for later stage clinical trials and potential approval and commercialization. These changes may not produce the intended optimization, including production of drug substance and drug product of a quality and in a quantity sufficient for Phase 3 clinical stage development or for commercialization, which may cause delays in the initiation or completion of clinical trials and greater costs. We may also need to conduct additional studies to demonstrate comparability between newly manufactured drug substance and/or drug product for commercialization relative to previously manufactured drug substance and/or drug product for clinical trials. Demonstrating comparability may require us to incur additional costs or delay initiation or completion of clinical trials and, if unsuccessful, could require us to complete additional pre-clinical studies or clinical trials.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

In addition, if our third-party manufacturers are unable to satisfy requirements related to the manufacturing ELEVIDYS, our ability to meet commercial demand may be adversely impacted, which could result in the loss of potential revenues, adversely affect our ability to gain market acceptance of ELEVIDYS, or otherwise adversely affect our business, financial condition and prospects. ELEVIDYS is our first gene therapy product. We may not be able to accurately estimate commercial demand for this new type of product. If commercial demand for ELEVIDYS is greater than we estimate, we and our manufacturers may be unable to fulfill all orders for ELEVIDYS in a timely manner, which may adversely affect our business, financial condition and prospects.

Currently the capacity to produce our viral vectors or gene therapy product candidates at commercial levels is limited and the availability of sufficient GMP compliance capacity may result in delays in our development plans or increased capital expenditures, and the development and sales of any gene therapy products, if approved, may be materially harmed.

The third parties we use in the manufacturing process for our products and product candidates may fail to comply with cGMP regulations.

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. In addition, before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization also must be obtained from the appropriate EU regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we need to demonstrate that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. In complying with cGMP, we are obligated to expend time, money and effort in production, record keeping and quality control to seek to assure that the product meets applicable specifications and other requirements.

We do not have direct operational control over a third-party manufacturer's compliance with regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our products and product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. Failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, results in product recalls, clinical holds, delayed or withheld approvals, patient injury or death.

Failure by our contract manufacturers to adhere to applicable cGMP and other applicable government regulations, or our contract manufacturers experiencing manufacturing problems, may result in significant negative consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these consequences, the success of our commercialization of our products and/or our development efforts for our product candidates could be significantly delayed, fail or otherwise be negatively impacted.

We may not be able to successfully optimize manufacturing of our product candidates in sufficient quality and quantity or within targeted timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the commercial success of our products and/or the development of our product candidates.

Our focus remains on optimizing manufacturing, including for our product candidates, gene therapy and other programs. We may not be able to successfully increase manufacturing capacity for the production of materials, APIs and drug products, whether in collaboration with third party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all.

Challenges complying with cGMP requirements and other quality issues arise during efforts to increase manufacturing capacity and scale up production. We experience such issues in connection with manufacturing, packaging and storage of our products and product candidates, and during shipping and storage of the APIs or finished drug product. In addition, in order to release our products for commercial use and demonstrate stability of product candidates for use in clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. Failure to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of our products or product candidates in a timely or cost-effective manner, or at all, may undermine our commercial efforts. Failure to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, will negatively impact the commercial availability of our products and the continued development and/or regulatory approval of our product candidates, which could significantly harm our business.

During our work with our third-party manufacturers to increase and optimize manufacturing capacity, they may make proprietary improvements in the manufacturing processes for our products or product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, we may need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Failure to secure the intellectual property rights required for the manufacturing process needed for large-scale clinical trials or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the continued development of our product candidates.

Risks Related to our Intellectual Property

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain, maintain and defend the patent protection for our products, product candidates, and platform technologies, to preserve our trade secrets, and to prevent third parties from infringing on our proprietary rights.

We currently directly hold various issued patents and patent applications, or have exclusive license or option rights to issued patents and patent applications, in each case in the U.S. as well as other countries that protect our products, product candidates and platform technologies. We anticipate filing additional patent applications both in the U.S. and in other countries. Our success will depend, in significant part, on our ability to obtain, maintain and defend our U.S. and foreign patents covering our products, product candidates and platform technologies as well as preserving our trade secrets for these assets. The patent process is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining, maintaining, or defending our patents. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect our products, product candidates or platform technologies or may be challenged in post-grant proceedings by third parties.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. This uncertainty is heightened for our PMO- based products and product candidates and gene therapy-based products and product candidates for which there has not been a significant number of patent litigations involving such technologies. Congress periodically considers changes to patent law, and that such changes could have adverse effects. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the U.S. and tests used for determining the patentability of patent claims in all technologies are in flux. The USPTO and patent offices in other jurisdictions have often required that patent applications directed to pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated. Thus, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. Patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful, particularly as a tactic to impose a price control. Additionally, competitors may leverage such pressure to enhance their ability to exploit these laws to create, develop and market competing products.

We may be able to assert that certain activities engaged in by our competitors infringe on our current or future patent rights. To the extent that we enforce our patents, an alleged infringer may deny infringement and/or counter-claim that our patents are not valid or enforceable, and if successful, could negatively impact our patent estate. We may not be able to successfully defend patents necessary to prevent competitors from developing, manufacturing, or commercializing competing product candidates or products. To the extent we assert infringement of a patent that covers a competing product candidate or product as well as our own product candidate(s) or product(s), or such a patent is otherwise challenged without our initiation, the patent protection for our own product candidate(s) or product(s) could be materially adversely affected should an infringing competitor be successful in challenging the validity, enforceability, or scope of our patent(s). Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage. Defending our patent positions may require significant financial resources and could negatively impact other Company objectives. Even if we successfully enforce our patent rights against a competitor, we may not be able to recover adequate damages or obtain other desired relief.

Under the Hatch-Waxman Act, one or more motivated third parties may file an ANDA, seeking approval of a generic copy of an innovator product approved under the NDA pathway such as our PMO Products, or an NDA under Section 505(b)(2), for a new or improved version of the original innovator products. In certain circumstances, motivated third parties may file such an ANDA or NDA under Section 505(b)(2) as early as the so-called "NCE-1" date that is one year before the expiry of the five-year period of NCE exclusivity or more generally four years after NDA approval. The third parties are allowed to rely on the safety and efficacy data of the innovator's product, may not need to conduct clinical trials and can market a competing version of a product after the expiration or loss of patent exclusivity or the expiration or loss of regulatory exclusivity and often charge significantly lower prices. Upon the expiration or loss of patent protection or the expiration or loss of regulatory exclusivity for a product, the major portion of revenues for that product may be dramatically reduced in a very short period of time. If we are not successful in defending our patents and regulatory exclusivities, we will not derive the expected benefit from them. As such, a third party could be positioned to market an ANDA or Section 505(b)(2) product that competes with one of our products prior to the expiry of our patents if the third party successfully challenges the validity, enforceability, or scope of our patents protecting the product.

The patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. As such, the patents and patent applications that we own, license, have optioned, and rely on for exclusivity for our product candidates may be challenged.

Uncertainty over intellectual property in the pharmaceutical and biotechnology industry has been the source of litigation and other disputes, which is inherently costly and unpredictable.

Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are, have been and may in the future be necessary in some instances to determine the validity and scope of certain of our proprietary rights, and in other instances to determine the validity, enforceability, scope or non-infringement of certain patent rights claimed by third parties to be pertinent to the manufacture, use or sale of our product candidates or products. We may also face challenges to our patent and regulatory exclusivities covering our products by third parties, including manufacturers of generics and/or biosimilars who may choose to launch or attempt to launch their products before the expiration of our patents or regulatory exclusivity. Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are unpredictable and may be protracted, expensive and distracting to management. The outcomes of such proceedings could adversely affect the validity, enforceability, and scope of our patents or other proprietary rights, hinder our ability to manufacture and market our products, require us to seek a license for the infringed products or technology or result in the assessment of significant monetary damages against us that may exceed amounts, if any, accrued in our financial statements. An adverse determination in a judicial or administrative proceeding or a failure to obtain necessary licenses could prevent us from developing, manufacturing or selling our products. Furthermore, payments under any licenses that we are able to obtain would reduce our profits derived from our products. Any of these circumstances could result in financial, business or reputational harm to us or could cause a decline or volatility in our stock price.

Our business prospects will be impaired if third parties successfully assert that our products, product candidates, or platform technologies infringe proprietary rights of such third parties.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, particularly gene therapy programs, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years and have been protected with third party patent rights. Due to the amount of intellectual property in our various fields of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or other third parties or that we will not infringe intellectual property rights of competitors or other third parties granted or created in the future. Moreover, activities we conduct or those conducted on our behalf in connection with the development of our product candidates may not be protected from infringement under the so-called Safe Harbor provision of 35 U.S.C. § 271(e)(1) and thus may be found to infringe the patent rights of third parties. Our competitors or other third parties might have obtained, or could obtain in the future, patents that threaten, limit, interfere with or eliminate our ability to make, use and sell our products, product candidates or platform technologies in important commercial markets.

Due to the nature of our various partnerships, collaborators, licensors, CROs, CMOs and the like, we may be subjected to claims of infringement arising from activities conducted by these third parties in connection with our product candidates, whether or not such activities are authorized by us. In addition, we may have contractual obligations to indemnify these partners from claims of infringement or declaratory relief. As a result, we may be subject to substantial unforeseen costs, distraction, and financial liability if a third party making such a claim was successful in obtaining a final judgment of infringement and validity.

In order to maintain or obtain freedom to operate for our products and product candidates, we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. Additionally, if we were to challenge the patent rights of our competitors or otherwise defend against allegations of infringement, misappropriation, breach of contract or related claims, we could incur substantial costs and ultimately might not be successful.

If our products, product candidates, or platform technologies are alleged to infringe or are determined to infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate, or cease commercialization of an infringing product;
- redesign our products, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could result in product and product candidate development delays or cessation, and as such substantially harm our potential earnings, financial condition and operations. The patent landscape of our product candidates and products is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that our products, product candidates or platform technologies infringe on the intellectual property rights of such parties. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights.

Risks Related to our Business Operations

Failure to comply with healthcare and other regulations is subject to substantial penalties and our business, operations and financial condition could be adversely affected.

As a manufacturer of pharmaceuticals, within the U.S., certain federal and state healthcare laws and regulations apply to or affect our business. These laws may constrain the business or financial arrangements and relationships through which we conduct business, including how we conduct research regarding, market, sell, and distribute our products. The laws and regulations include:

- federal healthcare anti-kickback law, which prohibit, among other things, persons from soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, information or claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent;
- the Federal Food, Drug and Cosmetic Act, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;
- the so-called “federal sunshine” law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with teaching hospitals, physicians and certain non-physician practitioners as well as physician ownership interests to the federal government for re-disclosure to the public; and
- state law equivalents of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third party payor, including commercial insurers, state laws regulating interactions between pharmaceutical manufactures and healthcare providers, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by federal laws, thus complicating compliance efforts.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. We anticipate that government scrutiny of pharmaceutical sales and marketing practices and other activities will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. Given the breadth of the laws and regulations, limited guidance for certain laws and regulations, and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices are non-compliant.

We have implemented a compliance program, which is based on industry best practices and is designed to ensure that our activities comply with all applicable laws, regulations and industry standards. While our compliance program is intended to detect and prevent potential non-compliance, we cannot be certain that compliance will be assured. If our operations are found to be in violation of any of the laws described above or any other laws, rules or regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business. Even if we successfully defend against an action against us for violation of a law, the action and our defense could nonetheless cause us to incur significant legal expenses and divert our management’s attention from the operation of our business.

Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

Our announced strategic restructuring plan may not result in anticipated reductions in our annual combined research and development and selling, general and administrative expenses and may disrupt our business in unexpected ways.

In July 2025, we announced a strategic restructuring plan, which included a revised cost structure and program portfolio and a reduction in force. The planned workforce reduction represents approximately 36% of our workforce. We may not realize, in full or in part, the anticipated benefits, savings and improvements in our cost structure from these efforts due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the potential cost savings from the strategic restructuring plan, our business strategy, operating results and financial condition would be adversely affected. Our workforce reductions could yield unanticipated consequences, such as attrition beyond planned workforce reductions or disruptions in our day-to-day operations. Our strategic restructuring plan, including our revised cost structure and reduction in force, could also harm our ability to attract and retain qualified management and development personnel who are critical to our business. If we are unable to realize the expected benefits from the strategic restructuring plan, we may decide to undertake additional workforce reductions.

Failure to comply with data privacy and security laws and regulations could adversely affect our operating results and business.

We may collect, use, transfer, or otherwise process proprietary, confidential, and sensitive information, including personal information and health-related data, which subjects us to numerous evolving and complex data privacy and security obligations, including various laws, regulations, guidance, and industry standards. Within the U.S., there are numerous federal and state laws and regulations related to the privacy and security of personal information. For example, at the federal level, HIPAA, as amended, and its implementing regulations 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. While we have determined that we are neither a "covered entity" nor a "business associate" directly subject to HIPAA, many of the U.S. health care providers with which we interact are subject to HIPAA, and we may have assumed obligations related to protecting the privacy of personal information. States are increasingly regulating the privacy and security of personal information. In some states, such as California and Washington, state privacy laws are even more protective than HIPAA. For example, the CCPA, regulates companies' use and disclosure of the personal information of California residents and grants California residents several rights with respect to their personal information. The CCPA also provides for civil penalties for violations, including statutory fines for noncompliance, as well as a limited private right of action in connection with certain data breaches, and establishes a new regulatory agency to implement and enforce the law. In addition, almost 20 other states have now passed comprehensive privacy laws that have taken effect or will come into effect at various times over the next few years. All of these evolving compliance and operational requirements impose significant costs that are likely to increase over time, may require us to modify our data processing practices and policies, divert resources from other initiatives and projects and could restrict the way services involving data are offered, all of which may adversely affect our results of operations. Certain state laws may be more stringent or broader in scope, or offer greater individual rights, than federal or other state laws, and such laws may differ from each other, which may complicate compliance efforts. State laws are changing rapidly and there is ongoing discussion in Congress of a new federal data protection and privacy law to which we may be subject. We will continue to monitor and assess the impact of these state laws, which may impose substantial penalties for violations, impose significant costs for investigations and compliance, and carry significant potential liability for our business.

Outside of the U.S., data protection laws, including the GDPR, which also forms part of the law of England and Wales, Scotland and Northern Ireland by virtue of section 3 of the European Union (Withdrawal) Act 2018 and as amended by the UK GDPR, also apply to some of our operations. The GDPR and UK GDPR increase our obligations with respect to clinical trials conducted in the member states of the EEA and the UK by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR and the UK GDPR increase the scrutiny that clinical trial sites located in the EEA and the UK should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. The GDPR and the UK GDPR impose substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue or 20 million Euros (£17.5 million in the UK), whichever is greater, and they also confer a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives is a rigorous and time-intensive process that requires review and updates that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European and UK activities. Other governmental authorities around the world are considering and, in some cases, have enacted, similar privacy and data security laws. Failure to comply with federal, state and international data protection laws and regulations could result in government investigations and/or enforcement actions (which could include substantial civil and/or criminal penalties), private litigation and adverse publicity and could negatively affect our business, financial condition and results of operations.

Government pricing requirements, such as those under the Medicaid Drug Rebate Program, other federal government programs, and state price transparency laws, and their related reporting and payment obligations require strict adherence; our failure to adhere to such requirements could subject us to penalties, sanctions, and fines that could have a material adverse effect on our business, financial condition, results of operations, and growth prospects.

We participate in the Medicaid Drug Rebate Program, the Public Health Services ("PHS") 340B drug pricing program, the U.S. Department of Veterans Affairs, Federal Supply Schedule pricing program, and the Tricare Retail Pharmacy program, and have obligations to report the average sales price for certain drug products to the Medicare program. Compliance is challenging. Pricing and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies, and the courts, which can change and evolve over time.

Requirements are subject to challenge and change. For instance, the PHS 340B drug pricing program continues to be subject to legal and regulatory activity, including litigation, at the federal and state levels, and any related developments could alert the scope of the program and our obligation to offer discounts. Continued expansion of the PHS 340B drug pricing program and growth of entities claiming entitlement to 340B pricing, including in ways that may be inconsistent with the statutory scheme, could impact our revenue. Changes to the calculation of rebates under the Medicaid program could increase our Medicaid rebate obligations and decrease the prices charged to 340B covered entities. On September 20, 2024, CMS issued a final rule specifying penalties for misclassification of drugs, and otherwise altering manufacturer obligations, under the Medicaid Drug Rebate Program.

If we become aware that our reporting for a prior quarter or other time period was incorrect or has changed as a result of recalculation of pricing data, we generally are obligated to resubmit the corrected data and provide refunds or other reconciliations. Price recalculations may affect the ceiling price at which we are required to offer our products to certain customers under the PHS 340B drug pricing program and increase our general costs.

Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we are found to have made a misrepresentation in the reporting of our average sales price, if we fail to submit the required price data on a timely basis, or if we are found to have charged certain customers more than the statutorily mandated ceiling price. The CMS also could decide to terminate our Medicaid Drug Rebate agreement. Our failure to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program and other governmental programs could negatively impact our financial results.

Several states have passed or are considering legislation that requires or purports to require companies to report pricing information, including proprietary pricing information. Such reporting requirements are not always clearly defined and failure to appropriately disclose in accordance with these requirements may lead to the imposition of penalties.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Our operations involve the use of hazardous materials, including organic and inorganic solvents and reagents. Although we believe that our activities conform in all material respects with such environmental laws, there can be no assurance that violations of these laws will not occur in the future as a result of human error, accident, equipment failure or other causes. Liability under environmental, health and safety laws can be joint and several and without regard to fault or negligence. The failure to comply with past, present or future laws could result in the imposition of substantial fines and penalties, remediation costs, property damage and personal injury claims, loss of permits or a cessation of operations, and any of these events could harm our business and financial condition. We expect that our operations will be affected by other new environmental, health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

Further, with respect to the operations of any current or future collaborators or third party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our product or product candidates, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product or product candidates.

Comprehensive tax reform in the U.S. and future guidance could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act (the "TCJA") was enacted on December 22, 2017 in the U.S. The TCJA contains significant changes to corporate taxation, including reduction of the U.S. corporate tax rate from 35% to 21%, elimination of U.S. tax on foreign

earnings (subject to certain important exceptions), one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, limitation of the tax deduction for interest expense, immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. On March 27, 2020, President Trump signed into law the "Coronavirus Aid, Relief, and Economic Security Act" or the CARES Act, which included certain changes in tax law intended to stimulate the U.S. economy in light of the COVID-19 outbreak, including temporary beneficial changes to the treatment of net operating losses, interest deductibility limitations and payroll tax matters.

We continue to monitor changes in tax laws in the U.S. and the impact of proposed and enacted legislation in the international jurisdictions in which the company operates, which could materially impact our tax provision, cash tax liability and effective tax rate. For example, we are evaluating the potential impact of the provisions of the One Big Beautiful Bill Act relating to tax laws.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited by provisions of the Internal Revenue Code, and it is possible that certain transactions or a combination of certain transactions may result in material additional limitations on our ability to use our net operating losses.

We have generated net operating loss and tax credit carryforwards in certain historical periods as we pursued our business strategy. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset a portion of future taxable income, if any, subject to expiration of such carryforwards in the case of carryforwards generated prior to January 1, 2018. In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets (including R&D tax credits) to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such "ownership change." Such limitations may result in expiration of a portion of the net operating loss carryforwards incurred prior to 2018 before utilization and may be substantial. If such change has occurred or does occur, the tax benefits related to the net operating loss carryforwards and certain other tax assets may be limited or lost. Moreover, proposed U.S. Treasury Regulations promulgated under Section 382 of the Code could, if finalized, significantly impact a corporation's ability to use its pre-change net operating loss carryforwards or other attributes following an ownership change. Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes. At the state level, there may also be periods during which the use of net operating loss carryforwards or other attributes is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. These net operating losses have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits.

The Inflation Reduction Act of 2022, among other things implements, a corporate book minimum tax ("BMT") 15% rate that could apply to consolidated groups of companies with adjusted financial statement income in excess of $1.0 billion over a three-year period. The BMT has various limitations, including a more restrictive limit on availability of net operating loss carryforwards, which if applied to us, could impact its cash tax liability and ability to utilize tax attributes.

In addition, many of the jurisdictions in which we operate have or are expected to adopt changes to tax laws as a result of the Base Erosion and Profit Shifting final proposals from the Organization for Economic Co-operation and Development and specific country anti-avoidance initiatives. In addition, the current proposal of the BMT may result in increases in tax imposed by non-U.S. jurisdictions. Such tax law changes and anti-avoidance initiatives increase uncertainty and may adversely affect our tax provision, cash tax liability and effective tax rate. The impact to the Company was not material in 2024 and the Company does not expect the impact to be material in future periods but will continue to monitor and evaluate new legislation and guidance.

Our employees, principal investigators, consultants and strategic 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, principal investigators, consultants and strategic partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately or disclose unauthorized activities to us. We 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 governmental 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, including the imposition of significant fines or other sanctions.

Failure to retain our key personnel or an inability to attract and retain additional qualified personnel would cause our future growth and our ability to compete to suffer.

We are highly dependent on the efforts and abilities of the principal members of our senior management. Additionally, we have scientific personnel with significant and unique expertise in RNA-targeted therapeutics and gene therapy technologies. The loss of the services of any one of the principal members of our managerial team or staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain, motivate and support such personnel. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

Turnover rates of key employees have varied substantially in recent years. Over the last few years, we have had several executive management changes, including the departure of Dallan Murray in July 2025. Leadership transitions can be inherently difficult to manage and may cause uncertainty or a disruption to our business or may increase the likelihood of turnover in other key officers and employees. Further, our recent restructuring and reduction in force could lead to unforeseen disruptions to our business and increase the likelihood of turnover of our key employees or officers. If we lose the services of one or more of our senior management or key employees, or if one or more of them decides to join a competitor or otherwise to compete with us, our business could be harmed.

Risks Related to our Financial Condition and Capital Requirements

We have previously incurred operating losses and we may not maintain profitability.

We incurred an operating loss of $184.8 million for the six months ended June 30, 2025. Our accumulated deficit was $4.5 billion as of June 30, 2025. Although we currently have four commercially approved products in the U.S., we believe that it will take us some time to attain positive cash flow from operations. Since our products and product candidates target small patient populations, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have generally incurred expenses related to research and development of our technologies and product candidates and from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

- continue the commercialization of our products in the U.S.;
- expand the global footprint of our products outside of the U.S.;
- establish our sales, marketing and distribution capabilities;
- continue our research, pre-clinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- acquire or in-license other product candidates;
- maintain, expand and protect our intellectual property portfolio;

- increase manufacturing capabilities, including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict our ability to continue to generate profitability or the extent of it.

Our existing and any future indebtedness could adversely affect our ability to operate our business.

On February 13, 2025, we entered into a $600.0 million revolving credit agreement with JPMorgan Chase Bank, N.A., as administrative agent and as collateral agent, the lenders party thereto, and Sarepta Therapeutics Investments, Inc., a Delaware corporation and wholly owned subsidiary, which we refer to as the "Credit Agreement". To the extent we draw amounts under the Credit Agreement in the future, our payment obligations under the Credit Agreement may reduce cash available to fund working capital, capital expenditures, research and development and general corporate needs. In addition, indebtedness incurred under the Credit Agreement bears interest at a variable rate, which would make us vulnerable to increases in interest rates. If interest rates increase, we would be required to pay additional interest on any indebtedness incurred under the Credit Agreement, which would further reduce cash available for our other business needs. We may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under or refinance any indebtedness outstanding under the Credit Agreement, which is repayable on the maturity date, February 13, 2030.

Our obligations under the Credit Agreement are secured by substantially all of our assets and the assets of certain wholly owned material subsidiaries, subject to certain customary exceptions and exclusions. The security interest granted over our assets could limit our ability to obtain additional debt financing. In addition, the Credit Agreement contains financial covenants that are tested on the last day of each of the Company's fiscal quarters. These financial covenants include a (x) maximum secured net leverage ratio of 3.5:1.0, subject to a 4.0:1.0 covenant holiday following certain permitted acquisitions or permitted collaborations, and (y) minimum consolidated interest coverage ratio of 2.5:1.0. Failure to comply with the covenants in the Credit Agreement, including the financial covenants, could result in the acceleration of our obligations under the Credit Agreement and prevent us from borrowing under the Credit Agreement. If an event of default (other than certain events of bankruptcy or insolvency) occurs and is continuing, JPMorgan Chase Bank, N.A. may terminate the commitments under the Credit Agreement, prevent additional borrowing and declare all or any portion of the outstanding principal amount of the loans plus accrued and unpaid interest to be due and payable. Upon the occurrence of certain events of bankruptcy or insolvency, all of the outstanding principal amount of the loans plus accrued and unpaid interest will automatically become due and payable. If such acceleration were to occur, it would materially and adversely affect our business, financial condition, operating results, cash flows and prospects.

Any outstanding indebtedness, combined with our other financial obligations, could increase our vulnerability to adverse changes in general economic, industry and market conditions, limit our flexibility in planning for, or reacting to, changes in our business and the industry and impose a competitive disadvantage compared to our competitors that have less debt, fewer operational restrictions or better debt servicing options.

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

We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, the ownership interest of our stockholders in our company may be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect the rights of our stockholders. Debt financing, if available, may increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our consolidated financial statements and condensed consolidated financial statements could prove inaccurate.

Our consolidated financial statements and condensed consolidated financial statements are prepared in accordance with accounting principles generally accepted in the U.S. (the "U.S. GAAP") The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our consolidated financial statements or condensed consolidated financial statements, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements or condensed consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Risks Related to Our Common Stock

Our stock price is volatile and may fluctuate due to factors beyond our control.

The market prices for and trading volumes of securities of biotechnology companies, including our securities, have historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities and the adverse events observed in non-ambulatory patients receiving ELEVIDYS and in our Phase 1/2 LGMD trial for SRP-9004. For example, over the last 12 months, as of the date of this report, our stock has increased as much as 20% in a single day or decreased as much as 42% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the commercial performance of our products in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical trials for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of our products or product candidates or entry into strategic relationships on terms that are not deemed to be favorable to us;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of a company's stock, including ours, regardless of actual operating performance. For example, the trading prices of biopharmaceutical companies have been highly volatile as a result of inflation, announced tariffs and increased interest rates and overall market volatility. In addition, our operations and performance may be affected by political or civil unrest or military action, including the ongoing conflict between Russia and Ukraine. Additionally, in the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management's attention and resources.

Our revenues and operating results could fluctuate significantly, which may adversely affect our stock price and our ability to maintain profitability.

Our revenues and operating results may vary significantly from year-to-year and quarter-to-quarter as well as in comparison to the corresponding quarter of the preceding year. Variations may result from one or more factors, including, without limitation:

- timing of purchase orders;
- changes in coverage and reimbursement policies of health plans and other health insurers, especially in relation to those products that are currently manufactured, under development or identified for future development by us;
- re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- transition from temporary billing codes established by the CMS to permanent medical codes;
- timing of approval of applications filed with the FDA;
- timing of product launches and market acceptance of products launched;
- changes in the amounts spent to research, develop, acquire, license or promote new and existing products;
- results of clinical trial programs;
- serious or unexpected health or safety concerns with our product or product candidates and any resulting clinical holds;
- introduction of new products by others that render one or more of our products obsolete or noncompetitive;
- the ability to maintain selling prices and gross margins on our products;
- increases in the cost of raw materials contained within our products and product candidates;
- manufacturing and supply interruptions, including product rejections or recalls due to failure to comply with manufacturing specifications;
- timing of revenue recognition relating to our distribution agreements;
- changes in estimates or potential asset impairments;
- the ability to protect our intellectual property from being acquired by other entities;
- the ability to avoid infringing the intellectual property of others;
- the impact of global pandemics; and
- the addition or loss of customers.

In addition, in one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

Provisions of our certificate of incorporation, bylaws and Delaware law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the then-current management and board of directors.

Certain provisions of our certificate of incorporation and bylaws may make it more difficult for a third party to acquire control of us or effect a change in our board of directors and management. These provisions include:

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These and other provisions in our certificate of incorporation and our bylaws and in the Delaware General Corporation Law could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and we expect to issue additional stock awards and shares of common stock to attract and retain employees, directors and consultants. We may also issue shares of common stock to finance our operations and in connection with our strategic goals. The vesting and exercise of these awards and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

Currently, our Amended and Restated Certificate of Incorporation authorizes the issuance of up to 198.0 million shares of common stock. As of June 30, 2025, there were approximately 97.7 million shares of common stock outstanding and outstanding awards to purchase 10.9 million shares of common stock under various incentive stock plans. Additionally, as of June 30, 2025, there were approximately 5.6 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.4 million shares of common stock available for issuance under our Amended and Restated 2013 Employee Stock Purchase Plan, and approximately 1.6 million shares of common stock available for issuance under our 2024 Employment Commencement Incentive Plan.

We may issue additional shares to grant equity awards to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2024 Employment Commencement Incentive Plan. We may also issue additional common stock and warrants from time to time to finance our operations and in connection with strategic transactions, such as acquisitions and licensing. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance Ltd in connection with the entry into the collaboration agreement with Roche.

The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or stock options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of our common stock in the public market, including sales by members of our management or board of directors, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity or equity-related securities.

Risks Related to Our Convertible Senior Notes

Servicing our 1.25% notes due 2027 (the "2027 Notes") requires a significant amount of cash, and we may not have sufficient cash flow to pay our debt.

In September 2022, we issued $1,150.0 million aggregate principal amount of 2027 Notes, pursuant to that certain indenture dated as of September 16, 2022, between us, as issuer, and U.S. Bank National Association, as trustee, including $20.0 million of 2027 Notes issued to the Michael A. Chambers Living Trust in a private placement. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the 2027 Notes, depends on our future performance, which is subject to many factors, including, economic, financial, competitive and other, beyond our control. We do not expect our business to be able to generate cash flow from operations in the foreseeable future, sufficient to service our debt and make necessary capital expenditures and we may therefore be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance the remaining outstanding 2027 Notes, which mature in 2027, will depend on the capital markets and our financial condition at such times. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, and limit our flexibility in planning for and reacting to changes in our business.

We may not have the ability to raise the funds necessary to repurchase the 2027 Notes as required upon a fundamental change, and our future debt may contain limitations on our ability to repurchase the 2027 Notes.

Holders of the 2027 Notes will have the right to require us to repurchase their 2027 Notes for cash upon the occurrence of a fundamental change at a fundamental change repurchase price equal to 100% of the principal amount of the 2027 Notes to be repurchased, plus accrued and unpaid interest, if any. A fundamental change may also constitute an event of default or prepayment under, and result in the acceleration of the maturity of, our then-existing indebtedness. We cannot assure you that we will have sufficient financial resources, or will be able to arrange financing, to pay the fundamental change repurchase price in cash with respect to any 2027 Notes surrendered by holders for repurchase upon a fundamental change. In addition, restrictions under our then existing credit facilities or other indebtedness, if any, may not allow us to repurchase the 2027 Notes upon a fundamental change. Our failure to repurchase the 2027 Notes upon a fundamental change when required would result in an event of default with respect to the 2027 Notes which could, in turn, constitute a default under the terms of our other indebtedness, if any. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the 2027 Notes.

Capped call transactions entered into in connection with the 2027 Notes may impact the value of our common stock.

In connection with the 2027 Notes, we entered into capped call transactions (the "Capped Call Transactions") with certain financial institutions. The Capped Call Transactions are expected to generally reduce the potential dilution upon conversion of the 2027 Notes into shares of our common stock.

In connection with establishing their initial hedges of the Capped Call Transactions, these financial institutions or their respective affiliates may have entered into various derivative transactions with respect to our common stock and/or purchased our common stock. The financial institutions, or their respective affiliates, may modify their hedge positions by entering into or unwinding various derivatives with respect to our common stock and/or purchasing or selling our common stock or other securities of ours in secondary market transactions prior to the maturity of the 2027 Notes. This activity may have an impact on the value of our common stock.

General Risks

Unfavorable and uncertain global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including the impact of increased interest rates, tariffs and inflation (such as the recent rise in inflation in the U.S.), could result in a variety of risks to our business, including weakened demand for our product candidates and our

ability to raise additional capital when needed on acceptable terms, if at all. Significant uncertainty regarding general political and geopolitical conditions, as well as the stability of financial markets related to any future changes in policies, could adversely impact our business. In addition, a weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

We may be subject to product liability claims and our insurance may not be adequate to cover damages.

The current and future use of our product candidates by us and our collaborators in clinical trials, EAPs, the sale of our products, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained commercial general liability insurance coverage for our clinical trials and the sale of commercial products. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

We have expanded, and may continue to expand, our organization and may experience difficulties in managing this growth, which could disrupt our operations.

To support the expansion of our business activities, we have expanded, and may continue to expand, our full-time employee base, as well as our consultant and contractor base. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy.

Our sales and operations are subject to the risks of doing business internationally.

We are increasing our presence in international markets, including emerging markets, subjecting us to many risks that could adversely affect our business and revenues, such as:

- the inability to obtain necessary foreign regulatory or pricing approvals of products in a timely manner;
- uncertainties regarding the collectability of accounts receivable;
- fluctuations in foreign currency exchange rates that may adversely impact our revenues, net income and value of certain of our investments;
- difficulties in staffing and managing international operations;
- the imposition of governmental controls;
- less favorable intellectual property or other applicable laws;
- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations;
- the far-reaching anti-bribery and anti-corruption legislation in the UK, including the UK Bribery Act 2010, and elsewhere and escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and trade restrictions; and
- changes in tax laws and tariffs.

In addition, our international operations are subject to regulation under U.S. law. For example, the Foreign Corrupt Practices Act ("FCPA") prohibits U.S. companies and their representatives from paying, offering to pay, promising to pay or authorizing the payment of anything of value to any foreign government official, government staff member, political party or political candidate for the purpose of obtaining or retaining business or to otherwise obtain favorable treatment or influence a person working in an official capacity. In many countries, the healthcare professionals we regularly interact with may meet the FCPA's definition of a foreign government official. Failure to comply with domestic or foreign laws could result in various adverse consequences, including: possible delay in approval or refusal to approve a product, recalls, seizures or withdrawal of an approved product from the market, disruption in the supply or availability of our products or suspension of export or import privileges, the imposition of civil or criminal sanctions, the prosecution of executives overseeing our international operations and damage to our reputation. Any significant impairment of our ability to sell products outside of the U.S. could adversely impact our business and financial results.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, as well as personally identifiable information of the patients using our commercially approved products, clinical trial participants and employees. Similarly, our third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Our ongoing operating activities also depend on functioning computer systems. Cyberattacks have increased in frequency and potential harm over time, and the methods used to gain unauthorized access constantly evolve, making it increasingly difficult to anticipate, prevent, and/or detect incidents successfully in every instance. We are required to expend significant resources in an effort to protect against security incidents and may be required or choose to spend additional resources or modify our business activities, particularly where required by applicable data privacy and security laws or regulations or industry standards. Our security measures may be insufficient, and our information technology and infrastructure, as well as that of our vendors, contractors, and other third-party partners who process information on our behalf or have access to our systems, may be susceptible to security incidents, disruptions, cyberattacks, ransomware, breaches, viruses, phishing attacks and other forms of social engineering, denial-of-service attacks, third-party or employee theft or misuse and other negligent actions. Any such breach could result in a material compromise of our networks, and the information stored there could be accessed, publicly disclosed, lost, stolen, or rendered, permanently or temporarily, inaccessible. Any perceived or actual unauthorized or inadvertent disclosure of personal or other confidential information, cyberattack, or other breach or theft of information could have a material impact on our business, operations or financial results. Any such access, disclosure or other loss of information, including our data being breached at third party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business.

We may incur substantial costs in connection with litigation and other disputes.

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, contractual disputes, and employee matters. We may expend significant amounts of money and company resources in connection with these disputes and it is possible that we may not prevail in claims made against us in such disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

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

Social media is increasingly being used to communicate about our products, technologies and programs, and the diseases our product and product candidates are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to comment on the effectiveness of a product or to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations or we may not be able to defend ourselves or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product and/or product candidates.

Additionally, AI tools are increasingly being used in our industry. We are evaluating, and will continue to evaluate, the use of AI tools throughout our organization. There are risks involved in developing and using AI in our operations, including related to enhanced governmental or regulatory scrutiny and our development and use of AI may not be beneficial to our business, including the development of our product candidates or our profitability or efficiency.

In addition, any misuse of social media or AI may result in inappropriate disclosure of sensitive information or cause reputational harm, give rise to liability, lead to the loss of trade secrets and other IP, or lead to other consequences. If any of these events described above were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face overly restrictive regulatory actions or incur other harm to our business.

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

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage, terrorism attack or other event occurred that prevented us from using all or a significant portion of our office, manufacturing and/or lab spaces, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

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

The following table contains information regarding our purchases of our common stock during the quarter.

ISSUER PURCHASES OF EQUITY SECURITIES

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly announced Plans or Programs (a)	Approximate Dollar Value (in thousands) of Shares that May Yet Be Purchased Under the Plans or Programs (a)
April 1-30, 2025	—	$ —	—	$ 500,000
May 1-31, 2025	650,876	38.41	650,876	475,000
June 1-30, 2025	—	—	—	475,000
Total	650,876	$ 38.41	650,876	

(a) All Common Stock repurchases during the quarter were made under the November 2024 authorization from our Board of Directors to purchase up to $500.0 million of our outstanding Common Stock. See *Note 10, Equity* to our unaudited condensed consolidated financial statements included in this Quarterly Report for additional information.

Repurchases under the authorized stock repurchase program may be made using a variety of methods, which may include, but are not limited to, open market purchases, privately negotiated transactions, accelerated share repurchase agreements or purchases pursuant to a Rule 10b5-1 plan under the Securities Exchange Act of 1934, as amended. The authorization from our Board expires in May 2026 and may be suspended, delayed or discontinued at any time.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

During the six months ended June 30, 2025, no director or officer (as defined in Rule 16a-1(f) under the Exchange Act) of the Company adopted or terminated contracts, instructions or written plans for the purchase or sale of our securities that are intended to satisfy the conditions specified in Rule 10b5-1(c) under the Exchange Act for an affirmative defense against liability for trading in securities on the basis of material nonpublic information.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report on Form 10-Q.

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
3.1	Amended and Restated Certificate of Incorporation.	8-K12B	001-14895	3.1	6/6/13	
3.2	Amendment to the Amended and Restated Certificate of Incorporation.	8-K	001-14895	3.1	6/30/15	
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	6/8/20	
3.4	Second Amended and Restated Bylaws	8-K	001-14895	3.1	12/13/22	
3.5	Amendment No. 1 to the Second Amended and Restated Bylaws	8-K	001-14895	3.1	9/16/24	
10.1	Amendment No. 4 to the Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	8-K	001-14895	10.1	6/6/25	
10.2	Amendment No. 3 to the Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan	8-K	001-14895	10.2	6/6/25	
31.1	Certification of the Company's Principal Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Principal Financial and Accounting Officer, Ryan Wong, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1**	Certification of the Company's Principal Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2**	Certification of the Company's Principal Financial and Accounting Officer, Ryan Wong, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document					X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents					X
104	Cover page formatted as Inline XBRL and contained in Exhibit 101					X

† Indicates management contract or compensatory plan, contract or arrangement.

* Certain identified information has been excluded from the exhibit.

** The Certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filings of Sarepta 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 Form 10-Q, 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.

SAREPTA THERAPEUTICS, INC.
(Registrant)

Date: August 6, 2025

By: /s/ DOUGLAS S. INGRAM
Douglas S. Ingram
Chief Executive Officer
(Principal Executive Officer)

Date: August 6, 2025

By: /s/ RYAN H. WONG
Ryan H. Wong
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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

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

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

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

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

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

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

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

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

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

August 6, 2025

/s/ DOUGLAS S. INGRAM
Douglas S. Ingram
Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Ryan H. Wong, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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

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

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

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

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

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

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

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

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

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

August 6, 2025

/s/ RYAN H. WONG

Ryan H. Wong
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Douglas S. Ingram, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended June 30, 2025, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

August 6, 2025

/s/ DOUGLAS S. INGRAM
Douglas S. Ingram
Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Ryan H. Wong, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended June 30, 2025, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

August 6, 2025

/s/ RYAN H. WONG
Ryan H. Wong
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.