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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2026

OR

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

For the transition period from to

Commission file number 001-14895

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	**107,235,848**
(Class)	**(Outstanding as of November 2, 2026)**

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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 September 30, 2026	As of December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 472,760	$ 801,282
Short-term investments	406,016	138,368
Accounts receivable, net	369,178	398,233
Inventory	876,118	914,744
Manufacturing-related deposits and prepaids	62,206	113,455
Other current assets	86,611	171,856
Total current assets	2,272,889	2,537,938
Property and equipment, net	319,288	345,125
Right of use assets	117,709	125,495
Non-current inventory	200,579	184,543
Non-current investments	108,815	1,048
Other non-current assets	146,786	155,554
Total assets	$ 3,166,066	$ 3,349,703
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 45,704	$ 280,841
Accrued expenses	321,764	359,659
Deferred revenue, current portion	104,887	443,397
Current portion of long-term debt	157,971	—
Other current liabilities	26,027	11,393
Total current liabilities	656,353	1,095,290
Long-term debt	699,393	828,974
Lease liabilities, net of current portion	195,663	199,378
Deferred revenue, net of current portion	83,910	83,910
Other non-current liabilities	1,894	1,529
Total liabilities	1,637,213	2,209,081
Commitments and contingencies (Note 16)		
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; 107,888,367 and 107,232,725 issued and outstanding, respectively, at September 30, 2026 and 105,615,096 and 104,964,220 issued and outstanding, respectively, at December 31, 2025	11	11
Treasury stock, at cost, 655,642 and 650,876 shares at September 30, 2026 and December 31, 2025, respectively	(25,013)	(25,263)
Additional paid-in capital	6,134,817	6,042,586
Accumulated other comprehensive (loss) income, net of tax	(1,586)	272
Accumulated deficit	(4,579,376)	(4,876,984)
Total stockholders' equity	1,528,853	1,140,622
Total liabilities and stockholders' equity	$ 3,166,066	$ 3,349,703

See accompanying notes to unaudited condensed consolidated financial statements.

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues:				
Products, net	$ 323,763	$ 370,043	$ 982,967	$ 1,494,689
Collaboration and other	57,588	29,313	530,438	260,614
Total revenues	381,351	399,356	1,513,405	1,755,303
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	188,512	150,775	446,665	440,897
Research and development	82,549	218,890	327,767	1,196,730
Selling, general and administrative	118,612	91,893	335,163	363,419
Litigation settlement charge	—	—	39,000	—
Restructuring charge	—	40,510	—	40,510
Amortization of in-licensed rights	718	677	2,126	1,945
Total cost and expenses	390,391	502,745	1,150,721	2,043,501
Operating (loss) income	(9,040)	(103,389)	362,684	(288,198)
Other (loss) income, net:				
Other (expense) income, net	(16,726)	34,958	(47,025)	(10,113)
Gain on debt extinguishment	—	3,761	—	3,761
Total other (loss) income, net	(16,726)	38,719	(47,025)	(6,352)
(Loss) income before income tax expense (benefit)	(25,766)	(64,670)	315,659	(294,550)
Income tax expense (benefit)	2,695	(14,102)	18,051	6,634
Net (loss) income	$ (28,461)	$ (50,568)	$ 297,608	$ (301,184)
Other comprehensive (loss) income:				
Unrealized (losses) gains on investments, net of tax	(1,021)	271	(1,858)	438
Total other comprehensive (loss) income	(1,021)	271	(1,858)	438
Comprehensive (loss) income	$ (29,482)	$ (50,297)	$ 295,750	$ (300,746)
(Loss) earnings per share:				
Basic	$ (0.27)	$ (0.50)	$ 2.82	$ (3.06)
Diluted	$ (0.27)	$ (0.50)	$ 2.78	$ (3.06)
Weighted average number of shares of common stock used in computing (loss) earnings per share:				
Basic	105,930	100,237	105,453	98,545
Diluted	105,930	100,237	107,646	98,545

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 Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
BALANCE AT DECEMBER 31, 2025	**105,615**	**$ 11**	**(651)**	**$ (25,263)**	**$ 6,042,586**	**$ 272**	**$ (4,876,984)**	**$ 1,140,622**
Exercise of options for common stock	25	—	—	—	241	—	—	241
Vest of restricted stock units	587	—	(5)	—	—	—	—	—
Stock-based compensation	—	—	—	—	33,673	—	—	33,673
Unrealized losses on investments, net of tax	—	—	—	—	—	(393)	—	(393)
Net income	—	—	—	—	—	—	330,959	330,959
BALANCE AT MARCH 31, 2026	**106,227**	**$ 11**	**(656)**	**$ (25,263)**	**$ 6,076,500**	**$ (121)**	**$ (4,546,025)**	**$ 1,505,102**
Vest of restricted stock units	52	—	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	28,030	—	—	28,030
Unrealized losses on investments, net of tax	—	—	—	—	—	(444)	—	(444)
Net loss	—	—	—	—	—	—	(4,890)	(4,890)
BALANCE AT JUNE 30, 2026	**106,279**	**$ 11**	**(656)**	**$ (25,263)**	**$ 6,104,530**	**$ (565)**	**$ (4,550,915)**	**$ 1,527,798**
Vest of restricted stock units	1,609	—	—	—	—	—	—	—
Reversal of excise tax on repurchases of common stock	—	—	—	250	—	—	—	250
Stock-based compensation	—	—	—	—	30,287	—	—	30,287
Unrealized losses on investments, net of tax	—	—	—	—	—	(1,021)	—	(1,021)
Net loss	—	—	—	—	—	—	(28,461)	(28,461)
BALANCE AT SEPTEMBER 30, 2026	**107,888**	**$ 11**	**(656)**	**$ (25,013)**	**$ 6,134,817**	**$ (1,586)**	**$ (4,579,376)**	**$ 1,528,853**

	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**
Cumulative effect of accounting change to adopt ASU 2025-07	—	—	—	—	—	—	47,400	47,400
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**	**$ (4,611,082)**	**$ 1,190,123**
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)**	**$ (4,414,190)**	**$ 1,404,785**
Exercise of options for common stock	2	—	—	—	26	—	—	26
Vest of restricted stock units	25	—	—	—	—	—	—	—
Issuance of common stock under Employee Stock Purchase Plan	77	—	—	—	1,188	—	—	1,188
Issuance of common stock for the August 2025 Exchange, net of issuance costs	5,851	1	—	—	104,934	—	—	104,935
Issuance of common stock	1,107	—	—	—	20,000	—	—	20,000
Restructuring charge	—	—	—	—	(134)	—	—	(134)
Stock-based compensation	—	—	—	—	16,396	—	—	16,396
Unrealized gains on investments, net of tax	—	—	—	—	—	271	—	271
Net loss	—	—	—	—	—	—	(50,568)	(50,568)
BALANCE AT SEPTEMBER 30, 2025	**105,419**	**$ 11**	**(651)**	**$ (25,263)**	**$ 5,986,689**	**$ 220**	**$ (4,464,758)**	**$ 1,496,899**

See accompanying notes to unaudited condensed consolidated financial statements.

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

	For the Nine Months Ended September 30,	
	2026	**2025**
Cash flows from operating activities:		
Net income (loss)	$ 297,608	$ (301,184)
Adjustments to reconcile net income (loss) to net cash used in operating activities		
Stock-based compensation	80,614	93,380
Depreciation and amortization	26,903	39,268
Non-cash interest expense	28,926	5,810
Non-cash inventory write-downs for obsolescence	11,599	—
Reduction in the carrying amounts of the right of use assets	9,614	11,326
Loss on strategic investments	2,683	17,268
Accretion of investment discount, net	(2,464)	(4,911)
In-kind milestone payment to Arrowhead Pharmaceuticals, Inc.	—	50,000
Non-cash write-off of prepaid deposits	—	17,041
Gain on debt extinguishment	—	(3,761)
Other	1,379	549
Changes in operating assets and liabilities, net:		
Decrease in accounts receivable	29,055	206,249
Decrease in manufacturing-related deposits and prepaids	53,384	67,072
Decrease (increase) in inventory	29,793	(319,568)
Decrease (increase) in other assets	87,158	(100,070)
(Decrease) increase in deferred revenue	(338,510)	30,193
Decrease in accounts payable	(236,564)	(89,860)
Decrease in accrued expenses, lease liabilities and other liabilities	(26,825)	(55,487)
Net cash provided by (used in) operating activities	54,353	(336,685)
Cash flows from investing activities:		
Purchase of investments	(557,299)	(45,316)
Maturity and sales of investments	180,953	195,935
Purchase of property and equipment	(5,570)	(98,381)
Purchase of intangible assets	(4,200)	(7,080)
Acquisition of strategic investment	—	(245,819)
Proceeds from sale of investment in Arrowhead Pharmaceuticals, Inc.	—	174,095
Net cash used in investing activities	(386,116)	(26,566)
Cash flows from financing activities:		
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	241	18,395
Proceeds from borrowings under the Revolving Credit Facility	425,000	—
Repayments of borrowings under the Revolving Credit Facility	(425,000)	—
Repayment of 2027 Notes	—	(123,333)
Debt issuance costs for the August 2025 Exchange	—	(12,787)
Proceeds from issuance of common stock	—	20,000
Common stock issuance costs	—	(2,276)
Repurchases of common stock	—	(25,013)
Payments related to obtaining Revolving Credit Facility	—	(4,122)
Net cash provided by (used in) financing activities	241	(129,136)
Decrease in cash, cash equivalents and restricted cash	(331,522)	(492,387)
Cash, cash equivalents and restricted cash:		
Beginning of period	814,407	1,118,589
End of period	$ 482,885	$ 626,202
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 472,760	$ 613,077
Restricted cash in other assets	10,125	13,125
Total cash, cash equivalents and restricted cash	$ 482,885	$ 626,202
Supplemental disclosure of cash flow information:		
Cash paid during the period for income taxes	$ 11,862	$ 21,249
Cash paid during the period for interest	$ 46,940	$ 13,962
Supplemental schedule of non-cash activities:		

Intangible assets and property and equipment included in accounts payable and accrued expenses	$	3,523	$	5,697
Capitalized stock-based compensation and depreciation as inventory	$	18,802	$	18,253
Lease liabilities arising from obtaining right of use assets	$	155	$	5,232
Issuance of 2030 Notes in exchange for 2027 Notes	$	—	$	602,002
Non-cash settlement of 2027 Notes	$	—	$	(602,002)
Common stock issued for conversion or exchange of 2027 Notes	$	—	$	107,320
Accrued debt issuance costs for the August 2025 Exchange	$	—	$	618
Accrued common stock issuance costs	$	—	$	110
Contingent consideration liabilities derecognized in connection with adoption of ASU 2025-07	$	—	$	47,400

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") knockdown therapies, gene therapies and other genetic therapeutic modalities for the treatment of rare diseases. Applying its proprietary, 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, Myotonic Dystrophy type 1 (“DM1"), facioscapulohumeral muscular dystrophy (“FSHD”), Huntington's Disease ("HD"), limb girdle muscular dystrophy ("LGMD"), and other neuromuscular and central nervous system disorders.

The Company's RNA-targeted products EXONDYS 51 (eteplirsen) Injection (“EXONDYS 51”), VYONDYS 53 (golodirsen) Injection (“VYONDYS 53”) and AMONDYS 45 (casimersen) Injection (“AMONDYS 45”) (collectively, the “PMO Products”) 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 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.

The Company's gene therapy product, ELEVIDYS (delandistrogene moxeparvovec-rokl), an adeno-associated virus-(“AAV”) based gene therapy, was initially 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 subsequently received traditional approval by the FDA in June 2024 for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS was also approved for non-ambulatory patients under the accelerated approval pathway in June 2024. In response to safety events announced in March and June 2025, the Company suspended all shipments of ELEVIDYS to non-ambulatory patients in the U.S. in June 2025. In November 2025, the Company announced a boxed warning for acute liver injury and acute liver failure and removal of non-ambulatory population from the Indication and Usage section of the Prescribing Information. The Company is currently in the process of conducting various clinical trials for ELEVIDYS, including a study to evaluate the use of sirolimus as an enhanced immunosuppressive regimen as part of treatment with ELEVIDYS for non-ambulatory individuals living with Duchenne. The Company intends to discuss with the FDA the results of this study and a potential pathway forward to resume commercial dosing in the non-ambulatory population. Resumption of dosing in the non-ambulatory population will depend in part on the FDA's analysis of whether the sirolimus data positively changes ELEVIDYS' risk/benefit profile and on aligning with the FDA on the process for revising the label, both of which involve risks and uncertainties.

As of September 30, 2026, the Company had $998.7 million of cash, cash equivalents, restricted cash and investments, consisting of $472.8 million of cash and cash equivalents, $514.8 million of investments and $11.1 million of non-current restricted cash and investments. 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 and availability under the Company's Revolving Credit Facility (please refer to *Note 13, Revolving Credit Facility* for further information) 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/or establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

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, 2025 which are contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2025, filed with the U.S. Securities and Exchange Commission on March 2, 2026. The results for the three and nine months ended September 30, 2026 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, revenue, 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 September 30, 2026, the Company's cash was concentrated at seven 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, certificates of deposit and bank time deposits 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 September 30, 2026, three entities accounted for 35%, 24% and 21% of accounts receivable, net, respectively. As of December 31, 2025, four entities accounted for 33%, 26%, 20% and 10% of accounts receivable, net, respectively.

Correction of Immaterial Error

As disclosed in *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the Company's Annual Report on Form 10-K for the year ended December 31, 2025, the Company recorded an immaterial error correction associated with the accounting for its partial refinancing of its convertible senior notes due on September 15, 2027 (the "2027 Notes") that was completed on August 28, 2025 (the "August 2025 Exchange"). The Company concluded that the error related to the August 2025 Exchange was not material to any previously issued financial statements and the unaudited condensed consolidated financial statements for the three and nine months ended September 30, 2025 presented herein have been revised to reflect the correction.

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, 2025.

Recent Accounting Pronouncements

Recently adopted

Effective in the fourth quarter of 2025, the Company early adopted Accounting Standards Update ("ASU") 2025-07, *Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606): Scope Refinements* ("ASU 2025-07") using the modified retrospective transition method. The Company recorded the cumulative effect of this accounting change to remove the previously recognized derivative liabilities as of January 1, 2025, reducing the contingent consideration liability by $47.4 million, with an offsetting adjustment to accumulated deficit. Accordingly, the comparative stockholders' equity balance as of September 30, 2025 presented herein reflects this $47.4 million reduction to opening accumulated deficit and differs from the previously reported stockholders' equity balance in the Company's Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2025. The elimination of this derivative liability increased the previously reported net loss for both the three and nine months ended September 30, 2025 by $11.1 million, or $0.11 per basic and diluted share. For additional information regarding the adoption of ASU 2025-07, please refer to *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, 2025.

Recently issued but not yet adopted

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU 2024-03"), which requires

public entities to provide disaggregated disclosure of income statement expenses. Public entities are required to disaggregate, in a tabular presentation, each relevant expense caption on the face of the consolidated statements of comprehensive (loss) income such as the following expenses: purchases of inventory, employee compensation, intangible asset amortization, and depreciation. ASU 2024-03 is effective for annual reporting periods beginning after December 15, 2026 and interim periods beginning after December 15, 2027, with early adoption permitted. The Company is currently evaluating the impact of this guidance and does not expect its adoption to have a material impact on its consolidated financial statements and related disclosures.

3. LICENSE AND COLLABORATION AGREEMENTS

Arrowhead Pharmaceuticals, Inc.

In November 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 Collaboration Agreement") that subsequently became effective as of February 7, 2025 (the "Arrowhead Collaboration Agreement Effective Date").

The Arrowhead Collaboration 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 programs listed below. The following table summarizes the current development stage of each program:

Development Stage	Indication
Arrowhead Clinical Programs	(a) DUX4 for the treatment of FSHD; (b) DM1 for the treatment of myotonic dystrophy type 1; (c) ATXN2 for the treatment of ataxias; (d) MMP7 for the treatment of idiopathic pulmonary fibrosis; and (e) HD for the treatment of Huntington's Disease.
Arrowhead Pre-clinical Programs	(a) ATXN1 for the treatment of ataxias; and (b) ATXN3 for the treatment of ataxias.

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").

Under the terms of the Arrowhead Collaboration 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, which 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.

Upon the effectiveness of the Arrowhead Collaboration Agreement, 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. On the Arrowhead Collaboration Agreement 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, based on the closing price of Arrowhead's common stock traded on the Nasdaq Global Select Market ("Nasdaq"), and
- $583.6 million was 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 and recorded as research and development expense, on the Company's unaudited condensed consolidated statement of comprehensive (loss) income.

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 Collaboration 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. For the three and nine months ended September 30, 2026, the Company recorded $5.7 million and $20.5 million, respectively, of research and development expense related

to reimbursable development costs incurred by Arrowhead. For the three and nine months ended September 30, 2025, the Company recorded $7.0 million and $15.8 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 Collaboration License Fees"), with the first payment due on the first anniversary of the Arrowhead Collaboration Agreement Effective Date. The Annual Collaboration License Fees are contingent upon the Company not terminating the Arrowhead Collaboration Agreement. For the nine months ended September 30, 2026, the Company paid and recorded the first $50.0 million Annual Collaboration License Fee in research and development expense in the unaudited condensed consolidated statements of comprehensive (loss) income.

Over the term of the Arrowhead Collaboration 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 two milestones associated with the continued enrollment of certain cohorts of a Phase 1/2 study for the DM1 program (the "Arrowhead DM1 Milestones"), totaling $300.0 million, both of which were achieved and recognized during the year ended December 31, 2025. In January 2026, the Company paid $200.0 million in cash related to the second Arrowhead DM1 Milestone, which was included in accounts payable as of December 31, 2025. For details about the Arrowhead DM1 Milestones, please read *Note 3, License and Collaboration Agreements* of the Annual Report on Form 10-K for the year ended December 31, 2025. Furthermore, upon commercialization, the Company will be required to make tiered royalty payments based on net sales.

F. Hoffmann-La Roche Ltd.

For the nine months ended September 30, 2026 and 2025, the Company recognized $365.0 million and $175.5 million, respectively, of collaboration revenue associated with the license, collaboration and option agreement (the "Roche Collaboration Agreement") with F. Hoffmann-La Roche Ltd. ("Roche"), with no collaboration revenue recognized during the three months ended September 30, 2026 or 2025. Under the Roche Collaboration Agreement, the Company 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 deemed as material rights related to individual programs and, thus, 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 February 2026, Roche declined to exercise an Option for certain program rights, which resulted in the immediate recognition of $325.0 million of collaboration revenue during the nine months ended September 30, 2026. Additionally, Roche dosed the first commercial patient in Japan in March 2026, which resulted in the recognition of a $40.0 million milestone payment as collaboration revenue under the Roche Collaboration Agreement.

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 was included in collaboration and other revenues for the nine months ended September 30, 2025. 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 nine months ended September 30, 2025.

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, the Company has delivered commercial ELEVIDYS supply to Roche that is 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 (loss) income. During the nine months

ended September 30, 2026, Roche product cost of sales exceeded Roche contract manufacturing revenue as a result of the following period costs being allocated to Roche product cost of sales:

- write-offs of certain batches of ELEVIDYS that did not meet quality specifications
- the Company's reserve for obsolete ELEVIDYS inventory on hand, and
- scrapped and expired materials.

The following table summarizes certain Roche activity for each of the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands)			
Contract manufacturing revenue	$ 50,100	$ 18,705	$ 135,604	$ 63,107
Royalty revenue	7,488	10,608	19,834	22,007
Roche product cost of sales	(47,581)	(17,527)	(145,437)	(51,488)

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 nine months ended September 30, 2026, costs reimbursable by Roche and reflected as a reduction to operating expenses were $7.1 million and $22.5 million, respectively. For the three and nine months ended September 30, 2025, costs reimbursable by Roche and reflected as a reduction to operating expenses were $12.2 million and $66.4 million, respectively. As of September 30, 2026 and December 31, 2025, there was $41.5 million and $104.1 million of collaboration and other receivables included in other current assets on the unaudited condensed consolidated balance sheets, respectively.

As of September 30, 2026 and December 31, 2025, the Company had total deferred revenue of $188.8 million and $527.3 million, of which $104.9 million and $443.4 million are classified as current, respectively. As of September 30, 2026 and December 31, 2025, all deferred revenue was related to the Roche Collaboration Agreement and Roche Supply Agreement.

Milestone Obligations

Including the Arrowhead Collaboration Agreement, 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 September 30, 2026, the Company may be obligated to make up to $12.0 billion of future development, regulatory, commercial and up-front royalty payments associated with its collaboration and license agreements.

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 nine months ended September 30, 2026, 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 September 30, 2026			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 194,009	$ 194,009	$ —	$ —
Government and government agency bonds	294,612	—	294,612	—
Certificates of deposit	142,898	—	142,898	—
Corporate bonds	92,191	—	92,191	—
Strategic investments	5,823	1,392	—	4,431
Commercial paper	4,538	—	4,538	—
Deferred compensation plan assets	1,345	1,345	—	—
Total assets	$ 735,416	$ 196,746	$ 534,239	$ 4,431

	Fair Value Measurement as of December 31, 2025			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 474,914	$ 474,914	$ —	$ —
Government and government agency bonds	179,765	—	179,765	—
Corporate bonds	42,583	—	42,583	—
Strategic investments	9,520	4,089	—	5,431
Commercial paper	3,614	—	3,614	—
Certificates of deposit	3,368	—	3,368	—
Deferred compensation plan assets	883	883	—	—
Total assets	$ 714,647	$ 479,886	$ 229,330	$ 5,431

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 investment in a biotechnology company listed on Nasdaq and assets associated with the Company's deferred compensation plan that are held in a trust.

The Company's assets with a fair value categorized as Level 2 within the fair value hierarchy consist of government and government agency bonds, corporate bonds, commercial paper 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 September 30, 2026 and December 31, 2025.

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 is 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 issuers were to issue similar or identical securities or when there is a triggering event for impairment. Impairments and other measurement events related to the Company's Level 3 strategic investments were not material during the nine months ended September 30, 2026 and 2025.

The fair values of the 2027 Notes and the Company's 4.875% convertible senior notes due September 1, 2030 (the "2030 Notes") are based on open market trades and are classified as Level 1 in the fair value hierarchy. As of September 30, 2026, the carrying value of the 2027 Notes was classified within current liabilities on the unaudited condensed consolidated balance sheets as the 2027 Notes mature on September 15, 2027. The following table summarizes the Company's debt instruments by indenture for the periods indicated:

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Net carrying value of the 2027 Notes	$ 157,971	$ 157,478
Net carrying value of the 2030 Notes	699,393	671,496
Total carrying value of debt instruments	$ 857,364	$ 828,974
Fair value of 2027 Notes	$ 152,100	$ 143,076
Fair value of 2030 Notes	752,014	721,791
Total fair value of debt instruments	$ 904,114	$ 864,867

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

5. FINANCIAL INSTRUMENTS

The following table summarizes the Company's financial assets with maturities of less than three months at the date of acquisition included in cash equivalents in the unaudited condensed consolidated balance sheets for each of the periods indicated:

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Money market funds	$ 194,009	$ 474,914
Government and government agency bonds	21,914	89,914
Total	$ 215,923	$ 564,828

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 September 30, 2026 and December 31, 2025 was approximately eight and three months, respectively. All of the Company's non-current investments as of September 30, 2026 and December 31, 2025 had maturities between one and two years.

The following tables summarize the Company's financial instruments for each of the periods indicated:

	As of September 30, 2026			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 450,847	$ —	$ —	$ 450,847
Government and government agency bonds	295,847	—	(1,235)	294,612
Certificates of deposit	142,898	—	—	142,898
Corporate bonds	92,664	75	(548)	92,191
Commercial paper	4,538	—	—	4,538
Bank time deposits	2,505	—	—	2,505
Total cash, cash equivalents and investments	$ 989,299	$ 75	$ (1,783)	$ 987,591
As reported:				
Cash and cash equivalents	$ 472,761	$ —	$ (1)	$ 472,760
Short-term investments	406,848	75	(907)	406,016
Non-current investments	109,690	—	(875)	108,815
Total cash, cash equivalents and investments	$ 989,299	$ 75	$ (1,783)	$ 987,591

	As of December 31, 2025			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 711,368	$ —	$ —	$ 711,368
Government and government agency bonds	179,615	150	—	179,765
Corporate bonds	42,527	56	—	42,583
Commercial paper	3,614	—	—	3,614
Certificates of deposit	3,368	—	—	3,368
Total cash, cash equivalents and investments	$ 940,492	$ 206	$ —	$ 940,698
As reported:				
Cash and cash equivalents	$ 801,269	$ 13	$ —	$ 801,282
Short-term investments	138,175	193	—	138,368
Non-current investments	1,048	—	—	1,048
Total cash, cash equivalents and investments	$ 940,492	$ 206	$ —	$ 940,698

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 September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands)			
PMO Products				
United States	$ 204,331	$ 209,132	$ 591,725	$ 597,882
Rest of World	35,982	29,413	107,702	108,473
Total PMO product revenues, net	$ 240,313	$ 238,545	$ 699,427	$ 706,355
ELEVIDYS				
United States	$ 83,450	$ 131,498	$ 283,540	$ 788,334
Total ELEVIDYS product revenues, net	$ 83,450	$ 131,498	$ 283,540	$ 788,334
Total product revenues, net	$ 323,763	$ 370,043	$ 982,967	$ 1,494,689

For the three and nine months ended September 30, 2026 and 2025, 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 September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Product revenues, net				
Customer 1	38%	34%	37%	25%
Customer 2	24%	22%	22%	15%

As of September 30, 2026 and December 31, 2025, the Company's accounts receivable, net were $369.2 million and $398.2 million, respectively, both of which were related to product sales, net of discounts and allowances. As of September 30, 2026, 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, 2025	$ 25,563	$ 116,585	$ 3,831	$ 54,057	$ 200,036
Provision	66,296	115,320	11,900	64,511	258,027
Adjustments relating to prior periods	—	(10,274)	(1,429)	(21)	(11,724)
Payments/credits	(76,682)	(113,427)	(10,158)	(76,403)	(276,670)
Balance, as of September 30, 2026	$ 15,177	$ 108,204	$ 4,144	$ 42,144	$ 169,669

	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	159,510	148,822	15,702	65,864	389,898
Adjustments relating to prior periods	—	(10,327)	—	(625)	(10,952)
Payments/credits	(185,724)	(118,600)	(17,498)	(62,232)	(384,054)
Balance, as of September 30, 2025	$ 19,690	$ 127,738	$ 4,145	$ 37,618	$ 189,191

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 September 30, 2026	As of December 31, 2025
	(in thousands)	
Reduction to accounts receivable, net	$ 60,571	$ 82,518
Component of accrued expenses	109,098	117,518
Total reserves	$ 169,669	$ 200,036

7. INVENTORY

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

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Raw materials	$ 132,256	$ 141,278
Work in progress	883,808	892,689
Finished goods	60,633	65,320
Total inventory	$ 1,076,697	$ 1,099,287

For the nine months ended September 30, 2026, inventory losses for write-downs totaled $11.6 million related to obsolete ELEVIDYS inventory. There were no inventory losses for write-downs of excess and obsolete inventory for the nine months ended September 30, 2025.

The Company classifies inventory associated with its PMO Products and ELEVIDYS as non-current inventory when consumption of the inventory is expected beyond the Company's normal inventory operating cycle of two years for the PMO Products and approximately 3.5 years for ELEVIDYS. Non-current inventory consists of raw materials and work in progress associated with the PMO Products and raw materials associated with ELEVIDYS.

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

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Balance sheet classification:		
Inventory	$ 876,118	$ 914,744
Non-current inventory	200,579	184,543
Total inventory	$ 1,076,697	$ 1,099,287

8. OTHER ASSETS

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

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Collaboration and other receivables	$ 41,499	$ 104,117
Tax-related receivables and prepaids	13,807	15,604
Prepaid maintenance services	11,643	12,639
Prepaid clinical and pre-clinical expenses	6,432	7,976
Prepaid commercial expenses	3,189	2,290
Interest receivable	2,994	1,656
Prepaid insurance	2,094	4,004
Other	4,953	23,570
Total other current assets	$ 86,611	$ 171,856

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

	As of September 30, 2026	As of December 31, 2025
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 90,755	$ 92,890
Intangible assets, net	29,834	28,949
Restricted cash and investments*	11,125	13,125
Strategic investments	5,823	9,520
Prepaid maintenance services	3,061	3,058
Deferred tax asset	2,854	2,822
Other	3,334	5,190
Total other non-current assets	$ 146,786	$ 155,554

* Restricted cash and investments 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 and investments are 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 September 30, 2026	As of December 31, 2025
	(in thousands)	
Product revenue related reserves	$ 109,098	$ 117,518
Accrued contract manufacturing costs	58,946	92,281
Accrued employee compensation costs	45,812	58,607
Accrued litigation settlement charge	39,000	—
Accrued professional fees	22,015	17,840
Accrued clinical and pre-clinical costs	14,303	18,894
Accrued income taxes	10,217	4,755
Accrued royalties	9,427	10,863
Accrued interest expense	4,575	20,714
Accrued clinical collaboration costs	3,336	10,222
Other	5,035	7,965
Total accrued expenses	$ 321,764	$ 359,659

10. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2026		2025		2026		2025	
	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	2,224,342 [1]	$ 10.48	—	$ —	2,224,342 [1]	$ 10.48	488,192	$ 46.93
Restricted stock units	967,174 [1]	$ 16.04	3,496,155 [2]	$ 18.95	1,755,359 [1]	$ 17.00	5,191,278 [2]	$ 39.73
Performance stock units	—	$ —	225,000 [3]	$ 18.95	—	$ —	345,378 [4]	$ 47.09

[1] On July 28, 2026 (the "Grant Date"), the Company granted its Chief Executive Officer, Dr. Michael Severino, approximately 2.2 million premium-priced stock options that vest 25% on the first anniversary of the Grant Date, with the remainder vesting monthly over the following three years and approximately 0.8 million restricted stock units ("RSUs") that vest in equal annual installments over four years, in each case generally subject to continued employment.

[2] In September 2025, approximately 3.4 million RSUs were granted, which will vest over two years while the remaining vest over four years.

[3] In September 2025, RSUs with performance conditions ("PSUs") were granted (the "September 2025 PSUs"). The performance condition is related to the achievement of a certain financial performance goal. The September 2025 PSUs have a three-year cliff vesting schedule. As of September 30, 2026, the performance condition associated with the September 2025 PSUs has been achieved.

[4] In addition to the September 2025 PSUs, included in PSUs for the nine months ended September 30, 2025 are approximately 0.1 million 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 (the "March 2025 PSUs"). As of September 30, 2026, none of the performance conditions for the March 2025 PSUs were probable of being achieved.

Stock-based Compensation Expense

For the three months ended September 30, 2026 and 2025, total stock-based compensation expense included in total expenses was $26.7 million and $14.9 million, respectively. For the nine months ended September 30, 2026 and 2025, total stock-based compensation expense included in total expenses was $80.6 million and $93.4 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 (loss) income:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands)			
Stock options	$ 5,663	$ 8,939	$ 17,737	$ 33,517
Restricted and performance stock units	24,145	10,724	73,774	70,381
Employee Stock Purchase Plan	479	(3,267)	479	672
Subtotal	$ 30,287	$ 16,396	$ 91,990	$ 104,570
Capitalized stock-based compensation costs	(3,544)	(1,469)	(11,376)	(11,190)
Total stock-based compensation expense included in expenses	$ 26,743	$ 14,927	$ 80,614	$ 93,380
Research and development	8,509	4,139	27,295	36,733
Selling, general and administrative	18,234	10,788	53,319	56,647
Total stock-based compensation expense included in expenses	$ 26,743	$ 14,927	$ 80,614	$ 93,380

11. OTHER (LOSS) INCOME, NET

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands)			
Interest income	$ 7,086	$ 7,249	$ 19,653	$ 22,693
Accretion of investment discount, net	1,117	1,132	2,714	5,257
Interest expense	(21,616)	(9,460)	(64,837)	(19,191)
(Loss) gain on strategic investments	(523)	36,739	(2,683)	(17,268)
Other, net	(2,790)	(702)	(1,872)	(1,604)
Other (expense) income, net	$ (16,726)	$ 34,958	$ (47,025)	$ (10,113)
Gain on debt extinguishment	—	3,761	—	3,761
Total other (loss) income, net	$ (16,726)	$ 38,719	$ (47,025)	$ (6,352)

12. INCOME TAXES

During the three and nine months ended September 30, 2026, the Company recorded an income tax expense of $2.7 million and $18.1 million, representing an effective tax rate of (10.5)% and 5.7%, respectively. The provision for income taxes primarily reflects current foreign and state income tax expense. State tax expense primarily relates to taxable income in certain states in which the Company operates that continue to require the capitalization of research and development costs or limit the utilization of net operating loss carryforwards.

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. Under the applicable accounting standards, management has considered the Company's history of losses and concluded that it is more likely than not that the Company will not recognize the benefits of its net federal and state deferred tax assets. Accordingly, a full valuation allowance against the U.S. net deferred tax asset is maintained at September 30, 2026. It is possible the Company may release a portion or all of its valuation allowance in future periods. The Company will continue to assess the realizability of its deferred tax assets on a quarterly basis.

During the three and nine months ended September 30, 2025, the Company recorded an income tax (benefit) expense of $(14.1) million and $6.6 million, representing an effective tax rate of 21.8% and (2.3)%, respectively. The income tax expense, for the nine months ended September 30, 2025 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. The income tax (benefit), for the three months ended September 30, 2025, primarily relates to the tax (benefit) recorded on the quarter to date loss which offsets tax expense recorded on profits in prior interim periods.

13. REVOLVING CREDIT FACILITY

The Company entered into a credit agreement (the "Credit Agreement") on February 13, 2025, which was subsequently amended on December 18, 2025, 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 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.

During the nine months ended September 30, 2026, the Company drew down and repaid $425.0 million under the Revolving Credit Facility and paid approximately $0.8 million of interest expense. As of September 30, 2026, there were no amounts outstanding under the Revolving Credit Facility and the Company was in compliance with the covenants described above.

14. (LOSS) EARNINGS PER SHARE

Basic (loss) earnings per share is computed by dividing net (loss) income 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. In periods in which the Company records a net loss, 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 (loss) earnings per common share:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands, except per share amounts)			
Numerator:				
Net (loss) income - basic	$ (28,461)	$ (50,568)	$ 297,608 [1]	$ (301,184)
Add: interest expense, net of tax, on the Company's convertible debt	—	—	1,865 [1]	—
Net (loss) income - diluted	$ (28,461)	$ (50,568)	$ 299,473	$ (301,184)
Denominator:				
Weighted-average common shares outstanding, basic	105,930	100,237	105,453	98,545
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	—	—	1,076 [1]	—
Common stock issuable under the Company's convertible debt	—	—	1,117 [1]	—
Weighted-average common shares outstanding, diluted	105,930	100,237	107,646	98,545
(Loss) earnings per common share, basic	$ (0.27)	$ (0.50)	$ 2.82	$ (3.06)
(Loss) earnings per common share, diluted	$ (0.27)	$ (0.50)	$ 2.78	$ (3.06)

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
	(in thousands)			
Common stock issuable under the Company's equity incentive plans	13,124 [2][4]	12,842 [3][4]	11,191 [2]	12,842 [3][4]
Common stock issuable under the Company's convertible debt	16,007	13,203	14,890 [1]	13,203
Total number of potentially issuable common stock	29,131	26,045	26,081	26,045

1. For the nine months ended September 30, 2026, the interest expense, net of tax, and potential share settlement of the 2030 Convertible Notes were excluded from diluted net income and diluted weighted-average common stock outstanding, respectively, as the inclusion of such shares was anti-dilutive during the period.

2. For the three and nine months ended September 30, 2026, the anti-dilutive common stock issuable under the Company's equity incentive plans excludes 0.4 million shares that have performance conditions that were not met as of the end of the period.

3. For the three and nine months ended September 30, 2025, the anti-dilutive common stock issuable under the Company's equity incentive plans excludes 0.3 million shares that have performance conditions that were not met as of the end of the period.

4. For the three months ended September 30, 2026 and the three and nine months ended September 30, 2025, all shares issued under the Company's equity incentive plans were anti-dilutive as the Company was in a net loss position during these periods.

15. SEGMENT INFORMATION

The Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases. As of September 30, 2026, there have not been material changes in significant expense categories or assets provided to the Company's CEO, the Company's chief operating decision maker (the "CODM"). For further details regarding the Company's segment information, please read *Note 21, Segment Information* of the Annual Report on Form 10-K for the year ended December 31, 2025. The table below includes information about the Company's segment, including significant segment expenses and a reconciliation to net (loss) income:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
			(in thousands)	
Total revenues	$ 381,351	$ 399,356	$ 1,513,405	$ 1,755,303
Segment expenses and other segment items:				
Cost of sales (excluding amortization of in-licensed rights)	188,512	150,775	446,665	440,897
Compensation and other personnel expenses	62,644	44,428	185,299	227,351
Up-front and collaboration license fees	—	100,000	50,000	683,787
Manufacturing expenses	2,251	33,897	23,846	182,047
Clinical trial expenses	20,511	28,613	61,917	95,271
Facility- and technology-related expenses (excluding depreciation and amortization)	22,940	20,577	69,772	76,814
Restructuring charge	—	40,510	—	40,510
Research and development- other (excluding non-cash items) [1]	17,241	20,883	55,759	81,197
Selling, general and administrative- other (excluding non-cash items) [2]	50,787	41,883	133,442	149,347
Roche collaboration reimbursement	(7,138)	(12,198)	(22,496)	(66,368)
Other segment items [3]	1,673	(430)	(842)	(3,653)
Gain on debt extinguishment	—	(3,761)	—	(3,761)
Loss (gain) on strategic investments	523	(36,739)	2,683	17,268
Interest expense	21,616	9,460	64,837	19,191
Interest income	(7,086)	(7,249)	(19,653)	(22,693)
Income tax expense (benefit)	2,695	(14,102)	18,051	6,634
Depreciation and amortization expense	5,900	18,450	26,903	39,268
Stock-based compensation expense	26,743	14,927	80,614	93,380
Litigation settlement charge	—	—	39,000	—
Segment net (loss) income	$ (28,461)	$ (50,568)	$ 297,608	$ (301,184)
Reconciliation of profit or loss				
Adjustments and reconciling items	—	—	—	—
Consolidated net (loss) income	$ (28,461)	$ (50,568)	$ 297,608	$ (301,184)

[1] Research and development-other includes professional services, pre-clinical expenses and research and other expenses.

[2] Selling, general and administrative-other includes professional services and other expenses.

[3] Other segment items included in segment net (loss) income include accretion of investment discount, net and other, net, as well as 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 collaboration license fees, manufacturing expenses, clinical trial expenses, facility- and technology-related expenses, restructuring charge, 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 loss (gain) on strategic investments, interest expense, interest income, income tax expense (benefit), depreciation and amortization, stock-based compensation expense and litigation settlement charge, which are included in the measure of segment net (loss) income, 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.

16. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

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

	As of September 30, 2026**
	(in thousands)
2026 (October-December)	$ 168,990
2027	145,452
2028	39,640
Total manufacturing commitments*	$ 354,082

* Total manufacturing commitments include the Catalent Agreement (defined below), to which the Company has right of use assets and lease liabilities recorded on the unaudited condensed consolidated balance sheets as of September 30, 2026. For more information, please read *Note 23, Commitments and Contingencies* to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025.
**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.

Catalent, Inc.

Under the manufacturing and supply agreements with Catalent, Inc. ("Catalent") (the agreements, the "Catalent Agreements") described in N*ote 23, Commitments and Contingencies*, to the consolidated financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025, the Company has certain non-cancelable manufacturing obligations. In September 2026, the Company elected not to manufacture certain inventories subject to these obligations. As a result, the Company will receive no other future economic benefit, and accordingly, recorded a take-or-pay charge of $62.0 million within cost of sales (excluding amortization of in-licensed rights) during the three and nine months ended September 30, 2026. As of September 30, 2026, $22.1 million of the take-or-pay charge remained unpaid and is included within accrued expenses in the accompanying unaudited condensed consolidated balance sheets.

In October 2026, the Company amended its August 2025 supplemental letter agreement with Catalent in connection with the Catalent Agreements (the "Letter Agreement Amendment"). The Letter Agreement Amendment resolved the obligations underlying the charge described above and reduced the Company's committed manufacturing batch quantity. The Company's remaining non-cancelable manufacturing obligations under the Catalent Agreements as of September 30, 2026 are reflected in the manufacturing commitments table above.

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 accrual 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. Except as disclosed below, the Company has not recorded any material accruals or charges for loss contingencies or settlements, and in management's opinion, no material range of loss is reasonably estimable for the remaining matters described below as of September 30, 2026.

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 ("Sarepta Three" and, together with the Company, "Sarepta"), 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 (including 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 appealed to the U.S. Court of Appeals for the Federal Circuit (the "Federal Circuit"), which reversed the district court's decision on February 20, 2026, and remanded the case for further proceedings. On March 23, 2026, the Company filed a petition for rehearing en banc before the Federal Circuit, which the Federal Circuit denied on April 22, 2026. The Federal Circuit issued a mandate on April 29, 2026.

On June 20, 2023, Regenx and U-Penn commenced a second patent infringement lawsuit against Sarepta and its contract manufacturer, Catalent, asserting alleged patent 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 ("IPR") for filing with the Patent Trial and Appeal Board ("PTAB") at the U.S. Patent and Trademark Office ("USPTO"). The petition sought 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 IPR proceedings. On August 22, 2024, the PTAB instituted review of all challenged claims of the '274 Patent on all asserted grounds. U-Penn subsequently disclaimed five of the six challenged claims, and on August 20, 2025, the PTAB entered a final written decision in the IPR proceedings finding that the sole remaining claim was not unpatentable. On October 3, 2025, the parties submitted a joint status report to the court in the patent infringement litigation, in which they agreed that the stay of the patent litigation should remain in place until Sarepta has exhausted its right to review by the Federal Circuit. On October 20, 2025, Sarepta filed a notice of appeal of the PTAB's decision to the Federal Circuit.

On September 23, 2026 (the "Settlement Effective Date"), Sarepta, Regenx, U-Penn and Catalent entered into a settlement agreement (the "Settlement Agreement") regarding, among other matters, the resolution of certain patent infringement litigations and an appeal from inter partes review proceedings relating to the '274 Patent and the '617 Patent (collectively, the "Litigations").

Under the terms of the Settlement Agreement, Sarepta paid Regenx a lump sum of $39.0 million within ten days of the Settlement Effective Date of the Settlement Agreement. Regenx and U-Penn released Sarepta, Catalent and their affiliates from any claims relating to any Sarepta gene therapy product that uses the AAVrh74-based capsid, including ELEVIDYS, that are claims of infringement of the '274 Patent, the '617 Patent and U.S. Patent No. 9,198,984 and certain related patents (collectively, the "U-Penn Patents"), arising at any time up to and including the Effective Date.

In addition, Regenx and U-Penn agreed not to sue Sarepta, Catalent or their current affiliates, licensees, commercial partners, successors and permitted assigns with respect to any claims of patent infringement of the U-Penn Patents to the extent that infringement arises from or relates to any Sarepta gene therapy product that uses an AAVrh74-based capsid, including ELEVIDYS. Regenx further agreed not to sue Sarepta or its licensees, commercial partners, successors and permitted assigns with respect to claims of patent infringement of any Regenx-controlled or owned patents or patent applications that cover the existing form of ELEVIDYS or the AAVrh74 capsid sequence.

The Settlement Agreement provides for the dismissal, withdrawal or other disposal of the Litigations with prejudice. Each party will continue to bear its own costs, expenses and attorneys' fees in connection with the Litigations and the negotiation and execution of the Settlement Agreement. The Settlement Agreement does not include any admission of liability or wrongdoing by any party. During the nine months ended September 30, 2026, the Company recorded a litigation settlement charge of $39.0 million related to the settlement of the Litigations.

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 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 ("Genzyme") 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 treble damages, 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. 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. Sarepta submitted IPR petitions challenging the validity of the '542 and '721 Patents, for which the USPTO director discretionarily denied institution in November 2025. In November and December 2025, Sarepta submitted IPR petitions challenging the validity of the '894 Patent, '326 Patent, '377 Patent, '880 Patent, and '313 Patent. The USPTO director instituted review of the '894 Patent and denied institution of review of the '326 Patent, '377 Patent, '880 Patent, and '313 Patent. In June 2026, the USPTO director denied Genzyme's request for director review of the decision granting institution of review of the '894 Patent, and oral argument in that proceeding is currently scheduled for February 4, 2027. Proceedings in the patent litigation remain ongoing.

On June 26, 2025, a putative securities class action complaint was filed against the Company, former 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 sought 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 October 17, 2025, the court appointed lead plaintiffs and lead counsel. On October 24, 2025, lead plaintiffs filed a motion to transfer venue to the U.S. District Court for the District of Massachusetts, which the court granted on November 6, 2025. On January 22, 2026, the lead plaintiffs filed an amended complaint including a new plaintiff, removing Dallan Murray as a defendant, adding President and Chief Operating Officer Ian Estepan as a defendant, and expanding the class period to shareholders who purchased or otherwise acquired the Company's securities between June 22, 2023 and November 3, 2025 (the "Amended Complaint"). The Amended Complaint further alleges additional theories of liability, including that Sarepta and the named defendants made false or misleading public statements related to (i) the safety profile of ELEVIDYS and LGMD therapies that utilize the AAVrh74 viral vector; (ii) the status of the development of LGMD therapies; and (iii) the status of the ESSENCE trial. On March 9, 2026, Sarepta and the named defendants filed a motion to dismiss the Amended Complaint, and oral argument was held on August 4, 2026. On September 15, 2026, the court granted Sarepta and the named defendants' motion to dismiss and dismissed the case with prejudice. On October 13, 2026, the plaintiffs filed a notice of appeal with the U.S. Court of Appeals for the First Circuit.

On July 15, 2025, a shareholder derivative lawsuit concerning certain of the same disclosures underlying the first complaint in the Securities 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"). Two substantially similar shareholder derivative lawsuits were filed in the same court against the same defendants on August 20, 2025 and September 10, 2025, respectively (collectively, the "S.D.N.Y. Derivative Actions"). The Company is named as a nominal defendant in all three lawsuits. The S.D.N.Y. Derivative Actions allege, among other claims, breaches of the Individual Defendants' fiduciary duties in connection with certain of the same 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 S.D.N.Y. Derivative Actions seek unspecified damages, including costs incurred in defending the Company against the Securities Action. Two of the S.D.N.Y. Derivative Actions also seek an order that the Company take all necessary action to reform and improve its corporate governance and internal procedures. On October 10, 2025, the court consolidated the Derivative Actions and appointed co-lead counsel (the "S.D.N.Y. Consolidated Derivative Action"). On November 17, 2025, the court stayed the S.D.N.Y. Consolidated Derivative Action until the Securities Action is resolved.

On April 7, 2026, a separate shareholder derivative lawsuit was filed in the U.S. District Court for the District of Massachusetts, naming the same Individual Defendants as in the S.D.N.Y. Consolidated Derivative Action. On July 23, 2026, a similar lawsuit was filed in the same court against nearly all of the Individual Defendants, except it did not name Dallan Murray and added Ian Estepan as a defendant. The Company is named as a nominal defendant in both lawsuits (the "D. Mass. Derivative Actions"). The D. Mass. Derivative Actions assert claims based upon substantially similar statements and theories as the underlying Securities Action, along with allegations concerning statements regarding Company performance and compensation from the Company's 2025 annual proxy statement; the Company's November 2024 repurchase of Company common stock; and certain director stock sales. The D. Mass. Derivative Actions allege, among other claims, breaches of the Individual Defendants' fiduciary duties and violations of Section 14(a) of the Exchange Act. The D. Mass. Derivative Actions further seek, among other relief, unspecified damages and an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures. On June 22, 2026, the court stayed the first D. Mass. Derivative Action until the Securities Action is resolved and ordered that the terms of the stay shall apply to stockholder derivative actions later filed in that court. On September 14, 2026, the court ordered that the second D. Mass. Derivative Action be consolidated with the first (the "D. Mass. Consolidated Derivative Action") and stayed pursuant to the same terms.

On September 3, 2026, a different shareholder derivative lawsuit was filed in the Supreme Court of the State of New York (the "N.Y. State Derivative Action") against the same Individual Defendants named in the S.D.N.Y. Consolidated Derivative Action and the first D. Mass. Derivative Action. The Company is named as a nominal defendant. The N.Y. State Derivative Action asserts claims based on substantially similar statements and theories as the Securities Action, S.D.N.Y. Consolidated Derivative Action, and D. Mass. Consolidated Derivative Action. The N.Y. State Derivative Action seeks, among other relief, unspecified damages and an order directing the Individual Defendants to take all necessary actions to reform and improve the Company's corporate governance and internal procedures.

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, 2025 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, 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 and strategic partners will support our clinical and commercial manufacturing capacity for our products and product candidates, including our phosphorodiamidate morpholino oligomer ("PMO"), gene therapy, SRP-9003 Limb-girdle muscular dystrophy ("LGMD"), and small interfering RNA ("siRNA") programs, while also acting as a manufacturing platform for potential future programs, and our belief that our current network of manufacturing and strategic partners is able to fulfill the requirements of our commercial plan;*
- *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 possible impacts of the results of our MIS51ON trial of EXONDYS 51 and our ESSENCE confirmatory trial for VYONDYS 53 and AMONDYS 45, including the timing and outcome of additional results, potential regulatory actions from the FDA, including directives to remove these products from the market or alter labels, patient demand for these products and changes to reimbursement and coverage by insurance companies, as well the timing and outcome of FDA review of our supplemental new drug applications ("sNDAs") for VYONDYS 53 and AMONDYS 45, including the anticipated PDUFA date;*
- *the estimated and potential 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 SRP-9003 LGMD program, while also acting as a manufacturing platform for any potential future gene therapy programs;*
- *our expectation that Aldevron LLC ("Aldevron") will provide Good Manufacturing Processes ("GMP")-grade plasmid for our Duchenne muscular dystrophy ("Duchenne") gene therapy program and our SRP-9003 LGMD program, as well as plasmid source material for future gene therapy programs;*
- *the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, including the addition of a boxed warning for acute liver injury ("ALI") and acute liver failure ("ALF") and removal of the non-ambulatory population from the Indication and Usage section of the Prescribing Information for ELEVIDYS, as well as the development of our product candidates and our financial and contractual obligations;*
- *estimated timelines and milestones for the remainder of 2026 and beyond, including discussions with the FDA regarding our products and product candidates, and sharing data for certain of our clinical trials;*
- *our expectations regarding the ongoing study to evaluate the use of sirolimus as an enhanced immunosuppression regimen as part of treatment with ELEVIDYS for non-ambulant individuals living with Duchenne, including discussions with the FDA and possible impacts on the resumption of dosing in the non-ambulatory population;*
- *our engagement with regulatory authorities outside of the U.S. including the European Medicines Agency (the "EMA");*
- *our ability to maintain, and our plan to continue building out our distribution network in jurisdictions in which our products are approved or in which we are seeking to make our products available, commercially or through our early access programs ("EAP");*
- *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, 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 expectation 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;*
- *our expectation regarding the outcomes or impacts of our ongoing litigations that we are currently, or may in the future become, party to;*
- *our expectation regarding our ability to satisfy the conditions to borrow under our Credit Agreement (defined below); 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, 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 LGMD. We are also developing potential therapeutic candidates through our partnered program with Arrowhead Pharmaceuticals, Inc. ("Arrowhead"), including Facioscapulohumeral muscular dystrophy ("FSHD"), myotonic dystrophy type 1 ("DM1"), Spinocerebellar ataxia ("SCA"), and Huntington's disease ("HD").

To date, we have developed and 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 in 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 in 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 in 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 adeno-associated virus-("AAV") based gene therapy, received traditional approval by the FDA in June 2024 for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS was also approved for non-ambulatory patients under the accelerated approval pathway in June 2024. 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 safety events announced in March and June 2025, we suspended all shipments of ELEVIDYS to non-ambulatory patients in the U.S. in June 2025. In response to a request from the 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 (the "ELEVIDYS Suspension"). On July 28, 2025, the FDA informed us that it recommended the removal of the voluntary hold for ambulatory patients. On July 31, 2025, we resumed shipments of ELEVIDYS for ambulatory patients in the U.S. In November 2025, we announced a boxed warning for ALI and ALF and removal of non-ambulatory population from the Indication and Usage section of the Prescribing Information for ELEVIDYS.

  In the tables referenced in this section, when referring to manufactured inventory for commercial sale in the U.S. and ex-U.S. territories, we use the designation of ELEVIDYS, which is recognized as inventory in our unaudited condensed consolidated balance sheets. Separately, when referring to manufactured product for clinical use, such as clinical trials, we use the designation of SRP-9001, which is recognized as research and development expense in our unaudited condensed consolidated statements of comprehensive (loss) income.

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. In November 2025, we announced topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of two of our PMO Products: AMONDYS 45 and VYONDYS 53. The topline results did not show statistical significance on the study's primary endpoint. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026. As of the date of this Quarterly Report, we anticipate a PDUFA target action date of February 28, 2027. In addition, as part of an FDA postmarketing study requirement for EXONDYS 51, we are conducting a Phase 3 clinical trial, MIS51ON, comparing the safety and efficacy of higher doses of eteplirsen to the approved dose.

We are also in the process of conducting various clinical trials for ELEVIDYS, including Cohort 8 of Study 9001-103 (ENDEAVOR), a study to evaluate the use of sirolimus as an enhanced immunosuppression regimen as part of treatment with ELEVIDYS for non-ambulant individuals living with Duchenne. We intend to discuss with the FDA the results of this study and a potential pathway forward to resume commercial dosing in the non-ambulatory population. Resumption of commercial dosing in the non-ambulatory population will depend in part on the FDA's analysis of whether the sirolimus data positively changes ELEVIDYS' risk/benefit profile and on aligning with the FDA on the process for revising the label, both of which involve risks and uncertainties.

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 reduce operating expenses and align our cost structure with strategic priorities, aiming to enhance financial flexibility and meet our 2027 financial obligations (the "Restructuring"). The Restructuring suspended all development of our LGMD programs with the exception of SRP-9003.

Set forth below are our key clinical stage programs, including those in collaboration with our strategic partners, listed in the order of stage of development:

- *SRP-9003 (LGMD, gene therapy program).* 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). In October 2025, we announced safety and expression results from EMERGENE.

In July 2025, we announced that the FDA placed a clinical hold on our investigational use gene therapy 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 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. In December 2025, the FDA confirmed that we remain on clinical hold and informed us that it requires data from the study of sirolimus as an immunosuppressant before accepting a biologic license application ("BLA") for SRP-9003. We anticipate re-engaging with the agency on next steps for the program after we receive data from Cohort 8 of Study 9001-103 (ENDEAVOR).

- *SRP-1003 (DM1)*. DM1 is an autosomal dominant, debilitating, chronic progressive multisystem disorder characterized by an expansion of a highly unstable CUGexp in the dystrophia myotonica protein kinase ("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 DMPK gene. There is currently no approved disease-modifying therapy for DM1. We are currently investigating SRP-1003 in a Phase 1/2 clinical trial, which our strategic partner Arrowhead has conducted to date. We shared early results from this trial in March 2026, and expect to share multiple ascending dose data in the fourth quarter of 2026. We are in the process of transitioning sponsorship of this study to Sarepta.
- *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 1/2 clinical trial, which our strategic partner Arrowhead has conducted to date. We shared early results from this trial in March 2026 and expect to share multiple ascending dose data in the fourth quarter of 2026. We are in the process of transitioning sponsorship of this study to Sarepta.
- *SRP-1005* (*HD*). HD is a rare and ultimately fatal inherited neurodegenerative disorder which is passed down from generation to generation within affected families. HD is caused by a mutation in the gene for a protein called huntingtin, which leads to progressive deterioration of nerve cells in the brain, affecting cognition, movement, and behavior. Study SRP-1005-101 (INSIGHTT) is a Phase 1, multi-center, dose escalation study that will evaluate the safety and tolerability of subcutaneous dosing of SRP-1005 in approximately 24 participants. SRP-1005 leverages an advanced transferrin receptor protein 1 approach that uses monovalent fragment antigen binding (fAb) designed for efficient delivery to the central nervous system. Subcutaneous administration is intended to avoid saturating the transferrin receptor and achieve constant and robust penetration across the blood brain barrier. During the second quarter of 2026, we enrolled the first patient in the study.

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, or specialized contract manufacturing organizations ("CMOs"), which will in part utilize these capabilities to support production of certain of our products and product candidates and their components. Specifically, we have entered into agreements with CMOs to produce custom starting materials, active pharmaceutical ingredients ("APIs"), drug product and finished goods for our products and product candidates for both commercial and clinical use. We also have opened facilities over the past several years to further enhance 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.

All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing medicinal products and, for commercial products, significant experience utilizing our specific technologies. 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 applicable foreign regulatory authorities. We depend on our third-party partners for continued compliance with cGMP requirements and applicable foreign standards.

We believe our current network of CMOs is able to fulfill our requirements for quantity, quality and purity of our commercial products and product candidates, and is capable of expanding capacity as needed. Additionally, we have evaluated and will continue to evaluate further relationships with additional suppliers as appropriate for specific products, taking into account production volume, logistics, business continuity and other routine business considerations.

Our gene therapy manufacturing capabilities continue to benefit from partnerships with Aldevron and Catalent. We utilize a hybrid development and manufacturing strategy in which we rely on experienced contract manufacturing partners to develop, manufacture and commercialize our gene therapy programs in partnership with our internal expertise relative to AAV-based development and manufacturing. Catalent supports our clinical and commercial manufacturing demand for ELEVIDYS and our SRP-9003 LGMD program, while also acting as a potential manufacturing partner for potential future gene therapy programs. Aldevron provides plasmid for ELEVIDYS and is expected to provide plasmid source material for any future gene therapy programs. The collaboration integrates process development, clinical and commercial production and testing.

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 or in which we are seeking approval for our products.

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 the siRNA programs in our portfolio, we collaborate with our partner Arrowhead to maintain continuity of manufacturing and testing services for ongoing and future clinical trials. We believe Arrowhead's cGMP manufacturing facility has the capability to manufacture drug substance at multiple scales available and can expand capacity as needed to support future clinical and potential commercial-scale needs. We plan to leverage existing contract partners, which have technical expertise and experience working with siRNA therapies, for the manufacture of drug product and finished goods as well as for distribution. As appropriate, we will evaluate additional relationships with supply partners for specific products, taking into account production volume, logistics, business continuity and other routine business considerations.

Cash, Cash Equivalents, Restricted Cash and Investments

As of September 30, 2026, we had $998.7 million of cash, cash equivalents, restricted cash and investments, consisting of $472.8 million of cash and cash equivalents, $514.8 million of investments and $11.1 million of non-current restricted cash and investments. We believe that our balance of cash, cash equivalents and investments, along with cash inflows from operations and availability under our Revolving Credit Facility (defined below), is sufficient to fund our current operational plan for at least the next twelve months.

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, 2025.

Results of Operations for the Three and Nine Months Ended September 30, 2026 and 2025

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

	For the Three Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 323,763	$ 370,043	$ (46,280)	(13)%
Other revenues	57,588	29,313	28,275	96%
Total revenues	381,351	399,356	(18,005)	(5)%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	188,512	150,775	37,737	25%
Research and development	82,549	218,890	(136,341)	(62)%
Selling, general and administrative	118,612	91,893	26,719	29%
Restructuring charge	—	40,510	(40,510)	(100)%
Amortization of in-licensed rights	718	677	41	6%
Total cost and expenses	390,391	502,745	(112,354)	(22)%
Operating loss	(9,040)	(103,389)	94,349	(91)%
Other (loss) income, net:				
Other (expense) income, net	(16,726)	34,958	(51,684)	*
Gain on debt extinguishment	—	3,761	(3,761)	(100)%
Total other (loss) income, net	(16,726)	38,719	(55,445)	*
Loss before income tax expense (benefit)	(25,766)	(64,670)	38,904	(60)%
Income tax expense (benefit)	2,695	(14,102)	16,797	*
Net loss	$ (28,461)	$ (50,568)	$ 22,107	(44)%
Loss per share				
Basic	$ (0.27)	$ (0.50)	$ 0.23	(46)%
Diluted	$ (0.27)	$ (0.50)	$ 0.23	(46)%

*Not meaningful

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 982,967	$ 1,494,689	$ (511,722)	(34)%
Collaboration and other	530,438	260,614	269,824	104%
Total revenues	1,513,405	1,755,303	(241,898)	(14)%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	446,665	440,897	5,768	1%
Research and development	327,767	1,196,730	(868,963)	(73)%
Selling, general and administrative	335,163	363,419	(28,256)	(8)%
Litigation settlement charge	39,000	—	39,000	*
Restructuring charge	—	40,510	(40,510)	(100)%
Amortization of in-licensed rights	2,126	1,945	181	9%
Total cost and expenses	1,150,721	2,043,501	(892,780)	(44)%
Operating income (loss)	362,684	(288,198)	650,882	*
Other loss, net				
Other expense, net	(47,025)	(10,113)	(36,912)	365%
Gain on debt extinguishment	—	3,761	(3,761)	(100)%
Total other loss, net	(47,025)	(6,352)	(40,673)	*
Income (loss) before income tax expense	315,659	(294,550)	610,209	*
Income tax expense	18,051	6,634	11,417	172%
Net income (loss)	$ 297,608	$ (301,184)	$ 598,792	*
Earnings (loss) per share				
Basic	$ 2.82	$ (3.06)	$ 5.88	*
Diluted	$ 2.78	$ (3.06)	$ 5.84	*

*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 ("PHS") 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 group, for each of the periods indicated:

	For the Three Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
PMO Products	$ 240,313	$ 238,545	$ 1,768	1%
ELEVIDYS	83,450	131,498	(48,048)	(37)%
Products, net	$ 323,763	$ 370,043	$ (46,280)	(13)%

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
PMO Products	$ 699,427	$ 706,355	$ (6,928)	(1)%
ELEVIDYS	283,540	788,334	(504,794)	(64)%
Products, net	$ 982,967	$ 1,494,689	$ (511,722)	(34)%

Net product revenues for the three and nine months ended September 30, 2026 decreased by $46.3 million and $511.7 million compared with the same periods in 2025. The decreases were primarily driven by lower ELEVIDYS sales volume, reflecting changes in demand following the safety events that occurred in 2025 and subsequent label update for ELEVIDYS which included only the ambulatory patient population for treatment.

Collaboration and other revenues primarily relate to our collaboration agreement (the "Roche Collaboration Agreement") with F. Hoffmann-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 certain approvals for ELEVIDYS. We are eligible to receive royalties on these sales. While the Roche Supply Agreement is in the process of being negotiated, we delivered commercial ELEVIDYS supply to Roche that was agreed upon on a purchase order-by-purchase order basis. The transaction price of the contract manufacturing revenue is estimated at contract inception and reassessed annually and on an as-needed basis when additional information becomes available.

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

	For the Three Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Contract manufacturing	$ 50,100	$ 18,705	$ 31,395	168%
Royalty revenue	7,488	10,608	(3,120)	(29)%
Total other revenues	$ 57,588	$ 29,313	$ 28,275	96%

Other revenues for the three months ended September 30, 2026 increased by $28.3 million, or 96%, compared with the three months ended September 30, 2025. The increase was primarily driven by the following:

- $31.4 million increase in contract manufacturing revenue primarily due to increased deliveries of ELEVIDYS to Roche and increases in our manufacturing costs which impact the selling price of ELEVIDYS delivered to Roche; and
- $3.1 million decrease in royalty revenue primarily due to a lower royalty rate resulting from increases in our manufacturing costs.

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Collaboration revenue	$ 365,000	$ 175,500	$ 189,500	108%
Contract manufacturing	135,604	63,107	72,497	115%
Royalty revenue	19,834	22,007	(2,173)	(10)%
License revenue	10,000	—	10,000	*
Total collaboration and other	$ 530,438	$ 260,614	$ 269,824	104%

*Not meaningful

Collaboration and other revenues for the nine months ended September 30, 2026 increased by $269.8 million, or 104%, compared with the nine months ended September 30, 2025. The increase was primarily driven by the following:

- $189.5 million increase in collaboration revenue due to $365.0 million of revenue recognized in the nine months ended September 30, 2026 related to (1) $325.0 million for Roche's declined option to acquire certain program rights previously recorded as deferred revenue and (2) $40.0 million from a milestone recognized under the Roche Collaboration Agreement for the first commercial dosing of ELEVIDYS in Japan, as compared to $175.5 million of

collaboration revenue recognized in the same period of 2025 related to the expiration of an option for a certain program and $63.5 million for the regulatory approval of ELEVIDYS in Japan under the Roche Collaboration Agreement;

- $72.5 million increase in contract manufacturing revenue primarily driven by increased deliveries of ELEVIDYS to Roche and increases in our manufacturing costs which impact the selling price of ELEVIDYS delivered to Roche; and
- $10.0 million increase in license revenue related to the grant of intellectual property rights under a certain license agreement executed during the nine months ended September 30, 2026, with no similar activity during the same period in 2025.

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 the related overhead costs and royalty payments primarily to University of Western Australia ("UWA") for our PMO Products and to Nationwide Children's Hospital ("Nationwide") for ELEVIDYS. Cost of sales also include charges for inventory valuation for excess or obsolete inventory on hand and write-offs of batches of our products not meeting our quality specifications, including any associated costs expected to be reimbursed by Roche. 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 by December 2022. For ELEVIDYS sold in the three and nine months ended September 30, 2026 and September 30, 2025, a portion of related manufacturing costs incurred had previously been expensed as research and development expenses. If certain 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 $2.5 million and $7.9 million higher for the three and nine months ended September 30, 2026, respectively, as compared to $2.0 million and $20.2 million for the three and nine months ended September 30, 2025, 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 September 30,			
	2026	2025	Change $	Change %
	(in thousands)			
Product cost of sales (excluding Roche)	$ 131,506	$ 122,589	$ 8,917	7%
Roche product cost of sales**	47,581	17,527	30,054	171%
Royalty payments	9,425	10,659	(1,234)	(12)%
Total cost of sales (excluding amortization of in-licensed rights)	$ 188,512	$ 150,775	$ 37,737	25%

**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 September 30, 2026 increased by $37.7 million, or 25%, compared with the same period in 2025. The increase was primarily driven by the following:

- $8.9 million increase in product cost of sales (excluding Roche) primarily due to a take-or-pay charge of $62.0 million recorded during the three months ended September 30, 2026 related to non-cancelable manufacturing obligations with Catalent for certain inventories that we elected not to manufacture, as well as increased manufacturing costs and volume of deliveries for the PMO Products. The increase is partially offset by a decrease in the write-offs of certain batches of ELEVIDYS not meeting our quality specifications, an impairment of prepaid manufacturing deposits recorded during the three months ended September 30, 2025 and a decreased volume of deliveries of ELEVIDYS;
- $30.1 million increase in Roche product cost of sales primarily due to increases in manufacturing costs, the volume of ELEVIDYS shipments under the Roche Collaboration Agreement, the timing of allocation of costs to Roche associated with write-offs of certain batches of ELEVIDYS not meeting our quality specifications and scrapped and expired materials; and
- $1.2 million decrease in royalty payments primarily corresponding to the decreased ELEVIDYS sales volume in the U.S.

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Product cost of sales (excluding Roche)	$ 273,384	$ 349,090	$ (75,706)	(22)%
Roche product cost of sales**	145,437	51,488	93,949	182%
Royalty payments	27,844	40,319	(12,475)	(31)%
Total cost of sales (excluding amortization of in-licensed rights)	$ 446,665	$ 440,897	$ 5,768	1%

**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 nine months ended September 30, 2026 increased by $5.8 million, or 1%, compared with the same period in 2025. The increase was primarily driven by the following:

- $75.7 million decrease in product cost of sales (excluding Roche) primarily due to decreased ELEVIDYS sales volume, reflecting changes in demand following the safety events that occurred in 2025 and subsequent label update for ELEVIDYS which included only the ambulatory patient population for treatment as well as decreased write-offs of certain batches of ELEVIDYS not meeting our quality specifications and an impairment of prepaid manufacturing deposits recorded during the nine months ended September 30, 2025. The decrease was partially offset by a take-or-pay charge of $62.0 million recorded during the nine months ended September 30, 2026 related to non-cancelable manufacturing obligations with Catalent for certain inventories that we elected not to manufacture, as well as increased manufacturing costs for the PMO Products;
- $93.9 million increase in Roche product cost of sales primarily due to the timing of allocation of costs to Roche associated with write-offs of certain batches of ELEVIDYS not meeting our quality specifications, scrapped and expired materials and increases in the volume and manufacturing costs of ELEVIDYS shipments under the Roche Collaboration Agreement; and
- $12.5 million decrease in royalty payments primarily corresponding to the decreased ELEVIDYS sales volume in the U.S.

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 and collaboration license 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 September 30,			
	2026	2025	Change $	Change %
	(in thousands)			
SRP-9001	$ 16,439	$ 47,641	$ (31,202)	(65)%
siRNA platform	13,086	7,601	5,485	72%
Eteplirsen (exon 51)	3,819	10,933	(7,114)	(65)%
LGMD platform	3,127	11,150	(8,023)	(72)%
Other gene therapies	3,086	7,064	(3,978)	(56)%
Other projects	1,614	4,203	(2,589)	(62)%
Up-front and collaboration license fees	—	100,000	(100,000)	(100)%
Internal research and development expenses	48,311	42,227	6,084	14%
Roche collaboration reimbursement	(6,933)	(11,929)	4,996	(42)%
Total research and development expenses	$ 82,549	$ 218,890	$ (136,341)	(62)%

	For the Nine Months Ended September 30,			
	2026	2025	Change $	Change %
	(in thousands)			
SRP-9001	$ 52,705	$ 179,730	$ (127,025)	(71)%
Up-front and collaboration license fees	50,000	683,787	(633,787)	(93)%
siRNA platform	42,935	16,406	26,529	162%
Eteplirsen (exon 51)	14,232	32,376	(18,144)	(56)%
LGMD platform	11,879	60,299	(48,420)	(80)%
Other gene therapies	10,725	30,510	(19,785)	(65)%
Other projects	5,374	27,272	(21,898)	(80)%
Internal research and development expenses	161,747	232,090	(70,343)	(30)%
Roche collaboration reimbursement	(21,830)	(65,740)	43,910	(67)%
Total research and development expenses	$ 327,767	$ 1,196,730	$ (868,963)	(73)%

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

	For the Three Months Ended September 30,			
	2026	2025	Change $	Change %
	(in thousands)			
Compensation and other personnel expenses	$ 26,208	$ 20,300	$ 5,908	29%
Clinical trial expenses	20,511	28,613	(8,102)	(28)%
Facility- and technology-related expenses	14,762	22,987	(8,225)	(36)%
Stock-based compensation	8,509	4,139	4,370	106%
Professional services	3,808	7,108	(3,300)	(46)%
Manufacturing expenses	2,251	33,897	(31,646)	(93)%
Pre-clinical expenses	1,845	1,017	828	81%
Up-front and collaboration license fees	—	100,000	(100,000)	(100)%
Research and other	11,588	12,758	(1,170)	(9)%
Roche collaboration reimbursement	(6,933)	(11,929)	4,996	(42)%
Total research and development expenses	$ 82,549	$ 218,890	$ (136,341)	(62)%

Research and development expenses for the three months ended September 30, 2026 decreased by $136.3 million, or 62%, compared with the three months ended September 30, 2025. The decrease was primarily driven by the following:

- $5.9 million increase in compensation and other personnel expenses primarily due to incentive compensation accrual reversals in connection with the Restructuring during the three months ended September 30, 2025, partially offset by reduced headcount;

- $8.1 million decrease in clinical trial expenses primarily related to the completion of certain PMO studies as well as the pause of several studies for our LGMD programs as a result of our decision to reprioritize our pipeline announced in July 2025 (the "Pipeline Reprioritization"), partially offset by increased activity in our siRNA programs;
- $8.2 million decrease in facility- and technology-related expenses primarily due to lower depreciation expense resulting from certain assets becoming fully depreciated during 2025;
- $4.4 million increase in stock-based compensation primarily due to the reversal of previously recognized expense related to the forfeiture of certain awards in connection with the Restructuring and our decision to suspend our Employee Stock Purchase Plan ("ESPP Suspension") during the three months ended September 30, 2025, with no similar activity during the three months ended September 30, 2026, and grants of RSUs to employees in September 2025 that vest over two years;
- $3.3 million decrease in professional services primarily related to fewer third-party contractors used during the three months ended September 30, 2026 due to the Pipeline Reprioritization and the completion of certain SRP-9001 studies in 2025;
- $31.6 million decrease in manufacturing expenses primarily due to fewer clinical batches released for our SRP-9001 program;
- $100.0 million decrease in up-front and collaboration license fees due to our $100.0 million milestone payment to Arrowhead, 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 the DM1 program (the "First Arrowhead DM1 Milestone"), during the three months ended September 30, 2025, which did not recur in the three months ended September 30, 2026; and
- $5.0 million decrease in the offset to expense associated with a collaboration reimbursement from Roche primarily due to reduced SRP-9001 clinical supply.

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 78,117	$ 111,181	$ (33,064)	(30)%
Clinical trial expenses	61,917	95,271	(33,354)	(35)%
Facility- and technology-related expenses	52,663	72,254	(19,591)	(27)%
Up-front and collaboration license fees	50,000	683,787	(633,787)	(93)%
Stock-based compensation	27,295	36,733	(9,438)	(26)%
Manufacturing expenses	23,846	182,047	(158,201)	(87)%
Professional services	14,268	25,722	(11,454)	(45)%
Pre-clinical expenses	3,482	4,210	(728)	(17)%
Research and other	38,009	51,265	(13,256)	(26)%
Roche collaboration reimbursement	(21,830)	(65,740)	43,910	(67)%
Total research and development expenses	$ 327,767	$ 1,196,730	$ (868,963)	(73)%

Research and development expenses for the nine months ended September 30, 2026 decreased by $869.0 million, or 73%, compared with the nine months ended September 30, 2025. The decrease was primarily driven by the following:

- $33.1 million decrease in compensation and other personnel expenses primarily due to reduced headcount pursuant to the Restructuring;
- $33.4 million decrease in clinical trial expenses primarily related to the completion of certain PMO studies and the pause of several studies for our SRP-9001 and LGMD programs, the latter of which as a result of the Pipeline Reprioritization, partially offset by increased activity in our siRNA programs;
- $19.6 million decrease in facility- and technology-related expenses primarily as a result of the Restructuring and lower depreciation expense resulting from certain assets becoming fully depreciated during 2025;

- $633.8 million decrease in up-front and collaboration license fees related to our exclusive global licensing and collaboration agreement and stock purchase agreement (collectively, the "Arrowhead Collaboration Agreement") with Arrowhead. For the nine months ended September 30, 2025, we recorded (1) $583.6 million in payments allocated to the up-front license fee and (2) a $100.0 million milestone payment to Arrowhead for the First Arrowhead DM1 Milestone, neither of which recurred in the nine months ended September 30, 2026. For the nine months ended September 30, 2026, we recorded $50.0 million in annual collaboration license fee incurred and paid to Arrowhead;
- $9.4 million decrease in stock-based compensation primarily related to reduced headcount pursuant to the Restructuring and the vesting of certain restricted stock units with performance conditions ("PSUs") in March 2025 and March 2026, partially offset by the reversal of previously recognized expense related to the forfeiture of certain awards in connection with the Restructuring during the nine months ended September 30, 2025, with no similar activity during the nine months ended September 30, 2026;
- $158.2 million decrease in manufacturing expenses primarily due to fewer clinical batches released for our SRP-9001 and LGMD programs, the latter of which as a result of the Pipeline Reprioritization, as well as the Brammer Settlement during the nine months ended September 30, 2025, with no similar activity in 2026;
- $11.5 million decrease in professional services primarily related to fewer third-party contractors used during the nine months ended September 30, 2026 due to the completion of certain SRP-9001 and PMO studies in 2025 and the Pipeline Reprioritization;
- $13.3 million decrease in research and other expenses primarily due to ongoing cost reduction efforts and the Pipeline Reprioritization; and
- $43.9 million decrease in the offset to expense associated with a collaboration reimbursement from Roche primarily due to reduced SRP-9001 clinical supply as well as reduced headcount pursuant to the Restructuring resulting in lower reimbursable costs.

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 an allocation of our facility- and technology-related costs and professional fees for legal, consulting and accounting services.

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

	For the Three Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Professional services	$ 42,714	$ 35,103	$ 7,611	22%
Compensation and other personnel expenses	36,436	24,128	12,308	51%
Stock-based compensation	18,234	10,788	7,446	69%
Facility- and technology-related expenses	13,360	15,363	(2,003)	(13)%
Other	8,073	6,780	1,293	19%
Roche collaboration reimbursement	(205)	(269)	64	(24)%
Total selling, general and administrative expenses	$ 118,612	$ 91,893	$ 26,719	29%

Selling, general and administrative expenses for the three months ended September 30, 2026 increased by $26.7 million, or 29% compared with the three months ended September 30, 2025. This increase was primarily driven by the following:

- $7.6 million increase in professional service expenses primarily related to increased ELEVIDYS commercialization spending and increased legal spending related to certain intellectual property matters;
- $12.3 million increase in compensation and other personnel expenses primarily due to incentive compensation accrual reversals in connection with the Restructuring during the three months ended September 30, 2025; and
- $7.4 million increase in stock-based compensation primarily related to the reversal of previously recognized expense related to the forfeiture of certain awards in connection with the Restructuring during the three months ended September 30, 2025, with no similar activity during the three months ended September 30, 2026, awards granted to our

former CEO in December 2025 and our current CEO in July 2026, grants of RSUs to employees in September 2025 that vest over two years and the reversal of previously recognized expense related to our ESPP Suspension during the three months ended September 30, 2025.

	For the Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Professional services	$ 114,760	$ 127,848	$ (13,088)	(10)%
Compensation and other personnel expenses	107,182	116,170	(8,988)	(8)%
Stock-based compensation	53,319	56,647	(3,328)	(6)%
Facility- and technology-related expenses	41,886	41,883	3	(—)%
Other	18,682	21,499	(2,817)	(13)%
Roche collaboration reimbursement	(666)	(628)	(38)	6%
Total selling, general and administrative expenses	$ 335,163	$ 363,419	$ (28,256)	(8)%

Selling, general and administrative expenses for the nine months ended September 30, 2026 decreased by $28.3 million, or 8%, compared with the nine months ended September 30, 2025. This decrease was primarily driven by the following:

- $13.1 million decrease in professional service expenses primarily related to reduced ELEVIDYS commercialization spending following the November 2025 label update for ELEVIDYS which included only the ambulatory patient population for treatment, reduced medical affairs spending related to the Pipeline Reprioritization and lower facilities-related professional services spending following the opening of our Bedford, Massachusetts facility in 2025;
- $9.0 million decrease in compensation and other personnel expenses primarily due to reduced headcount pursuant to the Restructuring; and
- $3.3 million decrease in stock-based compensation primarily related to reduced headcount pursuant to the Restructuring and the vesting of certain PSUs in March 2025 and March 2026, partially offset by expense associated with awards granted to our former CEO in December 2025 and our current CEO in July 2026 and the reversal of previously recognized expense related to the forfeiture of certain awards in connection with the Restructuring during the nine months ended September 30, 2025, with no similar activity during the nine months ended September 30, 2026.

Litigation settlement charge

During the nine months ended September 30, 2026, we recorded a litigation settlement charge of $39.0 million related to a settlement agreement entered into in September 2026 between Sarepta Therapeutics, Inc., Sarepta Therapeutics Three, LLC, REGENXBIO, INC, the Trustees of the University of Pennsylvania and Catalent regarding, among other matters, the resolution of certain patent infringement litigations and an appeal from inter partes review proceedings relating to the '274 Patent and the '617 Patent (collectively, the “Litigations”). Refer to *Note 16, Commitments and Contingencies* for further discussion regarding the Litigations.

Restructuring charge

The Restructuring, announced in July 2025, was designed to reduce operating expenses and align our cost structure with strategic priorities, aiming to enhance financial flexibility and meet our 2027 financial obligations. We recorded a restructuring charge of $40.5 million during the three and nine months ended September 30, 2025, primarily related to employee termination benefits, including severance, along with accelerated depreciation for assets impacted by the restructuring plan, with no similar activity during the three and nine months ended September 30, 2026.

Amortization of in-licensed rights

Amortization of in-licensed rights relates to the agreements we entered into with UWA, Nationwide, BioMarin Pharmaceutical Inc. ("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 nine months ended September 30, 2026, we recorded amortization of in-licensed rights of approximately $0.7 million and $2.1 million, respectively. For the three and nine months ended September 30, 2025, we recorded amortization of in-licensed rights of approximately $0.7 million and $1.9 million, respectively.

Other (expense) income, net

Other (expense) income, 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 September 15, 2027 (the "2027 Notes") and our 4.875% convertible senior notes due September 1, 2030 (the "2030 Notes"), interest income on our cash, cash equivalents and investments and accretion of investment discount. Our cash equivalents and investments consist of money market funds, government and government agency bonds, corporate bonds, commercial paper and certificates of deposit.

For the three months ended September 30, 2026, other expense, net increased by $51.7 million compared with the three months ended September 30, 2025. This was primarily due to a $37.3 million decrease in the gain on strategic investments, primarily related to our investment in Arrowhead during the three months ended September 30, 2025, which we sold in August 2025, and a $12.2 million increase in interest expense due to the 2030 Notes carrying a higher interest rate than the 2027 Notes.

For the nine months ended September 30, 2026, other expense, net increased by $36.9 million compared with the nine months ended September 30, 2025. The change was primarily due to a $45.7 million increase in interest expense due to the 2030 Notes carrying a higher interest rate than the 2027 Notes as well as a decrease in interest income as a result of reduced investment balances during the nine months ended September 30, 2026, as compared to the nine months ended September 30, 2025. This was partially offset by a $14.6 million decrease in the loss on our strategic investments, primarily related to our investment in Arrowhead during the nine months ended September 30, 2025.

Gain on debt extinguishment

In August 2025, we completed a partial refinancing of the 2027 Notes (the "August 2025 Exchange") and exchanged $700.0 million in aggregate principal amount of 2027 Notes for $602.0 million in aggregate principal amount of 2030 Notes, cash payments of $127.3 million, including $4.0 million of accrued interest of the 2027 Notes and 5.9 million shares of our common stock with a fair market value of approximately $104.9 million, net of issuance costs of $2.4 million.

As a result of the August 2025 Exchange, we recognized a gain on debt extinguishment for the three and nine months ended September 30, 2025 of $3.8 million. No similar activity occurred during the three and nine months ended September 30, 2026.

Income tax expense (benefit)

Income tax expense for the three and nine months ended September 30, 2026 was $2.7 million and $18.1 million, respectively. Income tax (benefit) expense for the three and nine months ended September 30, 2025 was $(14.1) million and $6.6 million, respectively. Income tax expense for all periods presented primarily relates to state income taxes as a result of taxable profits in certain states requiring the capitalization of research and development costs and states which have suspended or limited the utilization of net operating loss carryforwards. As of September 30, 2026, 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.

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, 2025, except for the partial draw downs and repayments of the five-year $600.0 million senior secured revolving credit facility entered into in February 2025 (the "Revolving Credit Facility"), as discussed in *Note 13, Revolving Credit Facility*.

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

	As of September 30, 2026	As of December 31, 2025	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 472,760	$ 801,282	$ (328,522)	(41)%
Short-term investments	406,016	138,368	267,648	193%
Non-current investments	108,815	1,048	107,767	*
Restricted cash and investments	11,125	13,125	(2,000)	(15)%
Total cash, cash equivalents, restricted cash and investments	$ 998,716	$ 953,823	$ 44,893	5%
Borrowings:				
Convertible debt	$ 857,364	$ 828,974	$ 28,390	3%
Total borrowings	$ 857,364	$ 828,974	$ 28,390	3%
Working capital:				
Current assets	$ 2,272,889	$ 2,537,938	$ (265,049)	(10)%
Current liabilities	656,353	1,095,290	(438,937)	(40)%
Total working capital	$ 1,616,536	$ 1,442,648	$ 173,888	12%

*Not meaningful

For the nine months ended September 30, 2026, our principal sources of liquidity were primarily derived from the sales of our products, our collaboration arrangement with Roche, borrowings from the Revolving Credit Facility and the maturities and sales of investments. Please refer to *Note 13, Revolving Credit Facility*, for further discussion of our borrowing activity. Our principal uses of cash for the nine months ended September 30, 2026 were our repayments of borrowings from the Revolving Credit Facility, the payment of $200.0 million for the second of two DM1 milestones achieved by Arrowhead in 2025 (the "Second Arrowhead DM1 Milestone"), the $50.0 million annual license fee payment to Arrowhead, inventory commitments, research and development expenses, manufacturing costs, selling, general and administrative expenses, investments, capital expenditures and other working capital requirements.

For the year ended December 31, 2025, our principal sources of liquidity were primarily derived from the sales of our products, our collaboration arrangement with Roche, the maturities and sales of investments and proceeds from the exercise of stock options. Our principal uses of cash for 2025 were our $583.6 million up-front payment to Arrowhead, $241.4 million equity investment in Arrowhead's common stock and the $50.0 million cash component of the $100.0 million of the First Arrowhead DM1 Milestone, costs incurred as a result of the partial refinancings of the 2027 Notes in August and December 2025, inventory commitments, research and development expenses, manufacturing costs, selling, general and administrative expenses, investments, capital expenditures, 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 our Annual Report on Form 10-K for the year ended December 31, 2025 for further discussion of share repurchases under the 2024 Repurchase Program.

As of September 30, 2026, we had $987.6 million of cash, cash equivalents and investments. Our material cash requirements over the next 12 months include the repayment of the remaining $158.6 million aggregate principal amount of the 2027 Notes due September 2027, the second $50.0 million annual license fee payable to Arrowhead in February 2027 and the remaining $22.1 million payable under the manufacturing and supply agreements with Catalent related to the take-or-pay charge recognized during the three and nine months ended September 30, 2026. Please refer to *Note 16, Commitments and Contingencies* to the unaudited condensed consolidated financial statements contained in Item 1 for further discussion of our manufacturing obligations. We believe that existing cash and cash equivalents and investments, along with future cash generated from operations and availability under our Revolving Credit Facility will be sufficient to fund these requirements and the capital requirements of our operations for the next 12 months. Additional information regarding our Revolving Credit Facility is provided in *Note 13, Revolving Credit Facility* to the unaudited condensed consolidated financial statements contained in Item 1.

Beyond September 30, 2027, 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 commercial shipments of ELEVIDYS for non-ambulatory patients in the U.S.;
- the risk that the Restructuring and Pipeline Reprioritization may not generate their intended benefits to the extent or as quickly as anticipated;
- the impact of potential regulatory actions from the FDA including changes to our drug labels or revocation of accelerated approvals and directives to remove products from the market relating to the topline results of our ESSENCE trial that failed to meet statistical significance on its primary endpoint;
- 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 pre-clinical and clinical trials;
- the attainment of milestones and our obligations to make milestone and royalty 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;
- the obligations to holders of our 2027 Notes and 2030 Notes; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights, as well as costs related to securities and other matters.

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.

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 16, Commitments and Contingencies* to the unaudited condensed consolidated financial statements contained in Item 1.

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 $12.0 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 September 30, 2026, 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 Nine Months Ended September 30,			
	2026	2025	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ 54,353	$ (336,685)	$ 391,038	(116)%
Investing activities	(386,116)	(26,566)	(359,550)	*
Financing activities	241	(129,136)	129,377	(100)%
Decrease in cash, cash equivalents and restricted cash	$ (331,522)	$ (492,387)	$ 160,865	(33)%

*Not meaningful

Operating Activities

Cash provided by (used in) operating activities, which consists of our net income (loss) adjusted for non-cash items and changes in net operating assets and liabilities, totaled $54.4 million and $336.7 million for the nine months ended September 30, 2026 and 2025, respectively. Cash provided by operating activities for the nine months ended September 30, 2026 was primarily driven by the net income of $297.6 million, adjusted for the following:

- $80.6 million in stock-based compensation expense;
- $26.9 million in depreciation and amortization expense;
- $28.9 million in non-cash interest expense;
- $11.6 million in write-downs for obsolete inventory;
- $9.6 million reduction in the carrying amount of the right of use assets; and
- $2.7 million loss on strategic investments.

These non-cash charges were partially offset by $2.5 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:

- $338.5 million decrease in deferred revenue primarily related to the recognition of $325.0 million associated with Roche's declined option to acquire certain program rights previously recorded as deferred revenue and delivery of ELEVIDYS to Roche;
- $236.6 million decrease in accounts payable primarily related to timing of payments, including the payment of the Second Arrowhead DM1 Milestone during the nine months ended September 30, 2026;
- $26.8 million decrease in accrued expenses, lease liabilities and other liabilities primarily due to timing of payments to CMOs related to manufacturing commitments and accrued compensation, partially offset by the litigation settlement charge;
- $29.1 million decrease in accounts receivable primarily due to a decrease in demand for our products from year-end;
- $29.8 million decrease in inventory primarily due to the sales of our products and write-off of batches of our products not meeting our quality specifications, partially offset by the continued manufacturing of our products to meet contractual commitments with CMOs;
- $53.4 million decrease in manufacturing-related deposits and prepaids primarily due to delivery of materials and services by CMOs, including Catalent; and
- $87.2 million decrease in other assets primarily driven by the timing of payment on receivables from Roche.

Cash used in operating activities for the nine months ended September 30, 2025 was primarily driven by the net loss of $301.2 million, adjusted for the following:

- $93.4 million in stock-based compensation expense;
- $50.0 million in-kind milestone payment to Arrowhead as a result of an agreement with Arrowhead to transfer approximately 2.7 million shares of Arrowhead common stock pursuant to the achievement of the First Arrowhead DM1 Milestone;
- $39.3 million in depreciation and amortization expense;
- $17.3 million loss on strategic investments, primarily as a result of our disposition of the remainder of Arrowhead common stock previously classified as strategic investments;
- $17.0 million in impairments associated with prepaid deposits associated with our manufacturing collaboration agreement and manufacturing and supply agreement with Catalent;
- $11.3 million reduction in the carrying amount of the right of use assets; and
- $5.8 million in non-cash interest expense.

These non-cash charges were partially offset by the $4.9 million in accretion of investment discount, net and a $3.8 million gain on debt extinguishment.

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

- $319.6 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;
- $100.1 million increase in other assets primarily due to an increase in receivables from Roche associated with contract manufacturing revenue;
- $89.9 million decrease in accounts payable primarily due to the timing and invoicing of payments with our contract manufacturing organizations and contract research organizations as well as the timing and invoicing of payments related to certain capital expenditure projects;
- $55.5 million decrease in accrued expenses, lease liabilities and other liabilities primarily due to a reduction in accrued employee compensation costs pursuant to the Restructuring and payments on accrued employee compensation costs, partially offset by an increase in our reserve associated with product revenue and reimbursements received for build-out of our Bedford facility;
- $30.2 million increase in deferred revenue primarily related to our collaboration with Roche and contract manufacturing activities with Roche;
- $67.1 million decrease in manufacturing-related deposits and prepaids primarily due to timing of payments to Catalent; and
- $206.2 million decrease in accounts receivable, net primarily due to a decrease in demand for ELEVIDYS from year-end.

Investing Activities

Cash used in investing activities was $386.1 million and $26.6 million for the nine months ended September 30, 2026 and 2025, respectively. Cash used in investing activities for the nine months ended September 30, 2026 primarily consisted of $557.3 million, $5.6 million and $4.2 million of purchases of investments, purchases of property and equipment and intangible assets, partially offset by $181.0 million from the maturity and sale of investments.

Cash used in investing activities for the nine months ended September 30, 2025 primarily consisted of $245.8 million in the acquisition of strategic investments primarily related to Arrowhead, $98.4 million of purchases of property and equipment and $45.3 million of purchases of investments, partially offset by $195.9 million from the maturity and sale of investments and $174.1 million in proceeds from the sale of the Arrowhead investment.

Financing Activities

Cash provided by financing activities was $0.2 million for the nine months ended September 30, 2026, compared to $129.1 million of cash used in financing activities for the nine months ended September 30, 2025. Cash provided by financing activities for the nine months ended September 30, 2026 consisted of $0.2 million in proceeds from exercise of options. During the nine months ended September 30, 2026, we also drew down, and subsequently repaid, $425.0 million on our Revolving Credit Facility.

Cash used in financing activities for the nine months ended September 30, 2025 primarily consisted of $123.3 million for the repurchase of a portion of our 2027 Notes, $25.0 million of purchases of our common stock under our 2024 Repurchase Program and $12.8 million of debt issuance costs associated with our 2030 Notes. These amounts were partially offset by $20.0 million in proceeds from the private placement of approximately 1.1 million shares of our common stock pursuant to a privately negotiated subscription agreement with J. Wood Capital Advisors LLC and $18.4 million in proceeds from exercise of options and purchases under our Employee Stock Purchase Plan.

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

September 30, 2026, we had $998.7 million of cash, cash equivalents, restricted cash and investments, comprised of $472.8 million of cash and cash equivalents, $514.8 million of investments and $11.1 million non-current restricted cash and investments. Our debt securities are classified as available-for-sale. We also hold bank time deposits. The fair value of cash equivalents and investments is subject to change as a result of fluctuations 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 September 30, 2026, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.2 million to our interest rate sensitive instruments.

The $158.6 million aggregate principal amount outstanding 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. The $893.4 million aggregate principal amount outstanding of our 2030 Notes has a fixed interest rate of 4.875% per annum, payable semi-annually in cash on each March 1 and September 1. Therefore, no outstanding debt is subject to fluctuations in market interest rates. However, to the extent that we borrow funds pursuant to our Revolving Credit Facility in the future, indebtedness incurred under our Revolving Credit Facility would bear interest at a variable rate, which would make us vulnerable to increases in interest rates.

Market-Price-Sensitive Financial Instruments

Our strategic investment portfolio includes an investment in equity securities of a certain publicly traded biotechnology company as a result of a certain business development transaction. 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 our strategic equity investment. Changes in the fair value of this strategic equity investment 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 September 30, 2026, we estimate that such hypothetical adverse 10.0% movement would result in a hypothetical loss in fair value of approximately $0.1 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 September 30, 2026, 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 September 30, 2026, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended September 30, 2026, there were no changes in our internal control 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 16, 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 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;
- the effectiveness of our ongoing commercialization activities, including maintaining and 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, including our MIS51ON trial for EXONDYS 51, and our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45, since continued approval of accelerated approval products or transition to traditional approval for such 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 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 disease areas we aim to treat or their 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., including following the topline results of our ESSENCE trial, a confirmatory trial to verify the clinical benefits of AMONDYS 45 and VYONDYS 53, and any future results from our MIS51ON trial; and
- 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, payor and reimbursement discussions, negotiations and decisions could impact timing and may lead to delays in infusion. Delays in the process prior to infusion could negatively impact the sales of our products, including any future gene therapy products.

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 certain of our products 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.

EXONDYS 51, VYONDYS 53, and AMONDYS 45 are currently 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. The accelerated approvals for our PMO Products 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. In November 2025, we announced that the FDA approved an update to ELEVIDYS' Prescribing Information to include a boxed warning for risk of ALI and ALF and the removal of the non-ambulatory population from the Indication and Usage section.

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 one or more of our products that have received accelerated approval. 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. For example, on November 3, 2025, we announced topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45, which primary endpoint did not meet statistical significance. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026 with, as of the date of this Quarterly Report, an expected PDUFA target action date by February 28, 2027. However, these results could lead to regulatory actions from the FDA, including changes to our drug labels, revocation of accelerated approvals and directives to remove these products from the market altogether. For example, as part of an FDA postmarketing study requirement for EXONDYS 51, we are conducting a Phase 3 clinical trial, MIS51ON, comparing the safety and efficacy of higher doses of eteplirsen to the approved dose. Future results of this study could similarly lead to regulatory actions from the FDA that are difficult to predict.

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, including any government shutdown, 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 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.

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 requirements 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, the FDA proposed, and we agreed to, 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 LGMD trial for SRP-9004. The Company announced in November 2025 the conclusion of the label supplement for ELEVIDYS, including the addition of a boxed warning for risk of ALI and ALF and the removal of the non-ambulatory population from the Indication and Usage section of ELEVIDYS' Prescribing Information. We are in the process of conducting various clinical trials for ELEVIDYS, including a study to evaluate the use of an enhanced immunosuppressive regimen as part of treatment with ELEVIDYS for non-ambulant individuals living with Duchenne. We intend to discuss with the FDA the results of this study and a potential pathway forward to resume commercial dosing in the non-ambulatory population. Regardless of the outcome of the study, however, it is unclear if or when we will be able to resume shipments to non-ambulatory patients.

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 have and may be required in the future to provide discounts or rebates on our products in order to obtain or maintain adequate coverage.

We expect that third party payors, including private insurers and government health benefit programs, 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 payors may make coverage decisions that negatively impact sales of our products. For example, following the

ELEVIDYS Suspension, certain third-party payors have restricted coverage for ELEVIDYS for certain segments of the ambulatory patient population, 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. As a further example, while the New York Drug Utilization Review Board recommended in October 2025 that Medicaid pause coverage of ELEVIDYS, New York Department of Health did not pause coverage, but instead restricted coverage based on age. 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 or reductions in funding government health care programs such as Medicaid or a general increase in unemployment rates or shift from commercial payor coverage to government payor coverage may disrupt access to our products or result in 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 continue 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, regulatory or executive initiatives. 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 have, and may continue to, call for new levels of innovation in 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, continued development of long-term efficacy and safety data and fees associated with complex administration, dosing and patient monitoring requirements. In some cases, we have entered into, and it may be necessary in the future to enter into, unique approaches to payment, pricing strategies, rebates, 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. Recent years have seen a number of reform initiatives focused on drug pricing and payment. For example, the Inflation Reduction Act ("IRA"), passed in 2022, has had and will likely continue to have a significant impact on the pharmaceutical industry. In 2025, the current presidential administration issued two executive orders with multiple directives aimed at lowering drug prices. In the wake of these executive orders and related executive initiatives, a number of pharmaceutical manufacturers have announced direct-to-consumer offerings with

discounted prices and/or reached agreement with the federal government regarding pricing for drugs, including prices for Medicaid drugs and newly launched products. In January 2026, the current presidential administration announced a healthcare plan calling for codification of most-favored-nation pricing arrangements with pharmaceutical manufacturers, and in April 2026, the administration issued a further executive order establishing tariffs on certain imported patented pharmaceuticals while offering relief from such tariffs to manufacturers that commit to most-favored-nation pricing and to onshoring research and manufacturing operations. The federal government has also launched a website that offers access to pharmaceutical direct-to-consumer discount drug offers and discloses generic drug pricing. Federal agencies are also developing and implementing new drug pricing pilot programs for both Medicaid (authorizing the federal government to negotiate Medicaid supplemental rebates with participating manufacturers on behalf of state Medicaid programs in exchange for standardized coverage criteria for the manufacturer's drugs) and Medicare (providing for rebates determined on the basis of international prices for drugs rather than existing inflation-based Medicare rebates). Many of these reform initiatives would require additional legal and/or administrative action to implement and may be subject to legal challenge. See the Company's Annual Report on Form 10-K for the year ended December 31, 2025, "*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. Cost containment initiatives might include, among other possible actions, implementation or modification of:

- price controls or other challenges to current pricing;
- controls on government funded reimbursement for drugs;
- mandatory discount requirements under certain government sponsored programs;
- caps on drug reimbursement under commercial insurance;
- negotiation of direct-to-consumer pricing;
- increases in, or elimination of caps on, rebates paid on products under government healthcare programs;
- waivers from Medicaid drug rebate law requirements;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of coverage and reimbursement requirements;
- requirements for substitution of generic products for branded prescription drugs;
- mechanisms utilized by managed care organizations to control utilization of drugs and other health care; or
- prohibition on direct-to-consumer advertising or drug marketing practices.

Workforce reductions in and restructuring of the U.S. Department of Health and Human Services, including at the FDA, may also create regulatory uncertainty, potentially impacting drug and biologic development programs and approvals.

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 (the "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.

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 due to competition or other reasons, 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;
- the frequency of administering our products, including weekly dosing of our PMO Products; and
- the efficacy, safety and convenience of receiving treatment of our other product candidates and third parties' competitive therapies.

For example, in March and June 2025, we announced two reported cases of ALF resulting in death in non-ambulatory patients following treatment with ELEVIDYS. Following these announcements, the FDA proposed, and we agreed to, 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 our Phase 1 LGMD trial for SRP-9004. In November 2025, the Company announced the conclusion of the label supplement for ELEVIDYS, including the addition of a boxed warning for risk of ALI and ALF and the removal of the non-ambulatory population from the Indication and Usage section of ELEVIDYS' Prescribing Information. 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 continue to experience hesitation from patients, payers and healthcare providers, which could adversely impact our business. The degree to which such hesitation continues, and the degree to which it could adversely impact our business, is uncertain and difficult to predict.

Additionally, in November 2025, we announced topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45, which primary endpoint did not meet statistical significance. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026 with, as of the date of this Quarterly Report, an expected PDUFA target action date by February 28, 2027. However, these results could lead to regulatory actions from the FDA, including changes to our drug labels or revocation of accelerated approvals and directives to remove these products from the market altogether, negatively impact patient demand for these products or result in changes to reimbursement and coverage of these products by payors. Such outcomes could adversely impact our business, financial condition, results of operations, financial guidance, ability to accurately forecast key financial metrics, and prospects. In addition, as part of an FDA postmarketing study requirement for EXONDYS 51, we are conducting a Phase 3 clinical trial, MIS51ON, comparing the safety and efficacy of higher doses of eteplirsen to the approved dose. Depending on the outcome, the MIS51ON trial results may negatively impact patient demand, physician prescribing patterns, or payor coverage for EXONDYS 51 or the PMO Products generally, which could adversely impact our business and financial condition, including potential negative impacts on the costs of goods sold.

Further, the potential commercial success of our product candidates as well as continued commercialization of the PMO Products and 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 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, including other of our own products.

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 for 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, in March and June 2025 we 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 our Phase 1 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 previously granted on June 2, 2025. 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. 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 EC 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 for eteplirsen.

Our partner for ELEVIDYS, Roche, has received certain approvals for ELEVIDYS in territories outside of the U.S. On September 24, 2025, the EC refused marketing authorization under Regulation (EC) No 726/2004 of the European Parliament and of the Council for ELEVIDYS for ambulatory individuals aged three to seven years with Duchenne. We also announced in June 2025 that we paused our ENVISION study for ELEVIDYS, which study was subsequently placed and remains on clinical hold. In November 2026, we announced we would not resume enrollment.

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.

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, especially in light of current geopolitical issues. 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 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 our EAP, the patient may not be able to obtain access to our products if funding for the drug is not secured. Also geo-political and distribution changes and challenges might negatively impinge upon future revenue generated through our EAP.

To date, our business and financial results have not yet been materially adversely affected by the ongoing conflict between Russia and Ukraine, recent events in Venezuela, 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, Venezuela, the Middle East, or other territories potentially impacted by the current geopolitical issues, may be adversely affected in the future. Even though, the supply of healthcare related products have generally been exempted from global sanctions in the past, the U.S. and other nations have raised the possibility of sanctions on companies that do business with Russia or its allies, including

Belarus, including healthcare companies. 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. In addition, economic sanctions imposed on the U.S. could disrupt operations and have a negative impact on our business.

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 distribution or geo-political challenges, including those potentially resulting from the ongoing conflict between Russia and Ukraine or the instability in the Middle-East or Venezuela, 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. While 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, orphan drug exclusivity qualifies such drug for market exclusivity—meaning that FDA is unable to approve any other marketing application for the same chemical or biological product—for seven years from the time of approval of the applicable marketing authorization. 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 U.S. for seven years from the time of approval of the applicable market authorization, 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, which received initial FDA approval in September 2016, ended in September 2023.

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, gene therapy product candidates or our siRNA programs. 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. For example, a number of commercial personnel departed from the Company in connection with and following our Restructuring, which has resulted in the need for additional hiring and training efforts, which may continue in the future;
- 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 populations living with the diseases we target are small and have 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. 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. FSHD is a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 16,000. DM1 is also a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 40,000. Our estimates of the size of these patient populations and the patient populations of our other product candidates 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 has resulted, and may continue to 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 have approved products or are developing or testing product candidates that now, or may in the future, compete directly with our products or product candidates. Several of these competing product candidates have recently reached, or are approaching,

advanced stages of U.S. regulatory review, and, in some cases, could receive approval in the near term. For example, we face competition in the fields of Duchenne, gene therapy and siRNA by third parties who are developing or who had once developed:

(i) exon skipping product candidates, such as Wave (targeting various exons, including 53); Nippon (targeting various exons, including 51 and 44, 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 (for which the FDA has accepted for review a BLA), 53, and 55; Novartis (formerly Avidity Biosciences ("Avidity")) pursuing antibody-oligonucleotide conjugates for exons 44, 45 and 51, SQY Therapeutics and BioMarin (for exon 51); and Entrada (notably for exon 44, 45, 50, and 51);

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

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

(iv) other disease modifying approaches, such as PTC 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 (approved product vamorolone), Capricor Therapeutics (in partnership with Nippon), BioPhytis, Italfarmaco (approved product Givinostat), Dystrogen and Edgewise Therapeutics.

Certain of these competing product candidates have received FDA designations intended to expedite development and review and their sponsors have publicly disclosed near-term regulatory submission or decision timelines, in some cases in 2026 and 2027. If one or more of these competing product candidates is approved, particularly in disease areas or exon populations that overlap with our products, such approvals could occur before we are able to expand or defend our current market position, which could result in a rapid loss of market share, increased pricing pressure, and reduced revenues for our affected products.

In the siRNA field, we face competition by third parties who are also developing product candidates targeting the same disease states as our product candidates, including but not limited to Novartis (formerly Avidity) (DM1, FSHD), Dyne (DM1, FSHD), Arthex Biotech (DM1), PepGen (DM1), Vertex Pharmaceuticals ("Vertex") and Entrada (DM1), Celularity (FSHD), EpiCrispr Biotechnologies (FSHD), Biohaven (SCA), Vico Therapeutics (SCA, Huntington’s), Skyhawk Therapeutics (SCA, Huntington’s), uniQure (Huntington’s), Wave (Huntington’s), and PTC (Huntington’s). Some, but not all, of these entities’ product candidates use RNA technologies. Several of these companies’ product candidates are further along in development, and, in a number of cases, are in active regulatory review with the FDA or have publicly announced imminent regulatory filings, and may obtain regulatory approval in advance of our product candidates. These and other competitors may have greater financial, scientific, and commercial resources than us, which may impact our ability to secure the technologies we desire or to otherwise effectively compete in these disease states.

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. ("Alnylam"), Arbutus (formerly Tekmira Pharmaceuticals Corp.), Deciphera Pharmaceuticals (now Ono Pharmaceuticals), Ionis Pharmaceuticals, Inc., Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Shire plc (now Takeda), Biogen Inc. ("Biogen"), Moderna Therapeutics ("Moderna"), Stoke Therapeutics, Ultragenyx, Sanofi, Arrakis Therapeutics, Altay Therapeutics, Life Edit, VectorY Therapeutics, Arvinas, and Design Therapeutics. 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 (CRISPR 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, Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire (now Takeda), Eli Lilly, Alnylam, Moderna, Akashi, Capricor (in partnership with Nippon), Oxford University, Exonics Therapeutics (acquired by Vertex), and Editas Medicine. Because many of our products are in various stages of preclinical and clinical development, and given the unpredictability inherent in drug development, it is difficult to predict which third parties may provide the most competition.

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 or gain or maintain market share in the Duchenne, DM1, FSHD, SCA, and Huntington's therapeutic areas or other diseases targeted by our platform technologies, products and product candidate pipeline. The competitive pressure in Duchenne has intensified as multiple third-party product candidates have advanced through, or are approaching, key regulatory milestones, and we expect this trend to continue as additional competitors report clinical data and pursue accelerated or traditional approval pathways.

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 of our products and product candidates, are more effective than our products or product candidates or would render our technologies obsolete or noncompetitive. Conversely, our competitors have, and may in the future, announce data from their clinical trials that has a negative impact on the market perception of our product candidates and related clinical trials. 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 product candidates, may compete with or supersede our current approved products, which may impact future revenues from sales of our current approved products. Our product candidates are being developed for potential treatment of overlapping patient populations with our current approved products, and we have not determined if our product candidates will be used in patients in combination with our existing approved products or in separate treatment regimens.

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 some cases, physicians have prescribed ELEVIDYS as a separate treatment regimen to patients who were previously receiving treatment with our PMO Products. Accordingly, ELEVIDYS has, and in the future, 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, including with Roche and Arrowhead, and we 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, 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 or strategic partner that cause the delay or termination of the research, development, manufacturing, 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. 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. As a further example, Arrowhead has conducted the Phase 1/2 ascending dose studies for SRP-1001 (FSHD1) and SRP-1003 (DM1) to date, for which we announced early and limited results in March 2026. Failure by Roche or Arrowhead to meet their obligations under the Roche Collaboration Agreement and Arrowhead Collaboration 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 (loss) income. 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 Products and Product Candidates

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

Identifying and qualifying patients to participate in clinical trials of our product candidates and products 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 products or 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 could negatively impact commercialization efforts; result in increased costs; and delay, prevent or limit our ability to gain or maintain regulatory approval 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 our LGMD programs, including SRP-9003, on clinical hold following a case of ALF resulting in the death of a patient in our Phase 1 LGMD clinical trial for SRP-9004, which has impacted 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 the FDA's workforce could delay or materially impact the FDA's feedback on our development programs, including through meetings and other informal interactions, and affect the FDA's review and oversight of our product candidates. Additionally, changes in the FDA personnel under the current 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. In addition, as a result of any government shutdown, the FDA staff may be unable to process and review regulatory submissions in a timely manner or at all.

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 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 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 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 our LGMD programs on clinical hold. There is no assurance that any current hold or future hold would not have a material adverse effect on our development timelines. 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.

For example, in November 2026, we announced that we would not resume enrollment of our ENVISION study of ELEVIDYS.

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, SRP-1001 and SRP-1003 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. In September 2025 we presented 18 month functional data compared to external control for 6 ambulant patients from the SRP-9003-101 study and 5 non-ambulant patients from the SRP-9003-102 study, and 5 year safety data from the SRP-9003-101 study. In addition, in March 2026, we announced early results from Phase 1/2 ascending dose studies of SRP-1001 for FSHD1 and SRP-1003 for DM1. The results announced so far for SRP-1001 and SRP-1003 are predominantly from single ascending dose cohorts, which may differ from the results of upcoming multiple ascending dose cohorts. These data are based on small patient samples, and, given the heterogeneity of LGMD, FSHD and DM1 patients and potential lot-to-lot variability, the data may not be predictive and may differ from 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 seen, and may continue to see, new safety signals for our products or product candidates. For example, we reported new safety signals in the non-ambulatory population for ELEVIDYS and for our LGMD product candidate SRP-9004 in 2025. On March 18, 2025, Sarepta announced that a non-ambulatory patient 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 non-ambulatory patient following treatment with ELEVIDYS. Such adverse events have resulted in the FDA's proposal of, and our agreement to, 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 our Phase 1 LGMD trial for SRP-9004. Occurrences of undesirable side effects and new safety signals could impact the adoption of our products and may harm our business, financial

condition, prospects and ability to accurately forecast revenues. In addition to side effects caused by our product candidates or products, the administration process or related procedures also can cause adverse side effects.

We are currently evaluating an enhanced immunosuppressive regimen incorporating sirolimus intended to mitigate the risk of ALI and ALF in non-ambulatory patients, including through Cohort 8 of our ENDEAVOR study. We cannot be certain that this approach will succeed or that it will be free of its own adverse effects. Sirolimus is itself associated with known and potentially serious adverse reactions, any of which could occur in patients receiving sirolimus in combination with ELEVIDYS and could delay treatment or result in discontinuation of the use of sirolimus. Further, resumption of commercial dosing in the non-ambulatory population will depend in part on the FDA's analysis of whether sirolimus data positively changes ELEVIDYS' risk/benefit profile. If our sirolimus-based approach does not perform as anticipated, or if it introduces new safety signals of its own, our ability to resume commercial dosing in the non-ambulatory population, and the perception of ELEVIDYS more generally, could be further adversely affected. Further, such an outcome could also negatively impact the development and commercialization of our other product candidates.

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 the INDs for SRP-9003, SRP-9004, SRP-6004 and SRP-9005 on clinical hold following the reported death in a patient following dosing in the Company's Phase 1 LGMD trial of SRP-9004. As a further example, in June 2025 we announced that we paused our ENVISION study for ELEVIDYS, which study was subsequently placed and remains on clinical hold. In November 2026, we announced we would not resume enrollment.

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 products and 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 current presidential administration, 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 Phase 1 LGMD 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., we suspended shipment of ELEVIDYS in the U.S. to ambulatory patients effective July 22, 2025. On July 28, 2025, the FDA informed us that it recommended the removal of the voluntary hold for ambulatory patients. In response, we resumed commercial shipments of ELEVIDYS for ambulatory patients in the U.S. Further, the Company has agreed with the FDA to a boxed

warning for ALI and ALF and the removal of non-ambulatory population from the Indication and Usage section of ELEVIDYS' Prescribing Information. In November 2025, we announced the FDA's approval of dosing in a clinical trial for ELEVIDYS to evaluate the use of an enhanced immunosuppressive regimen as part of treatment with ELEVIDYS for non-ambulatory patients. It is currently unclear whether the FDA will pursue further actions related to ELEVIDYS, such as additional studies, additional product modifications, label supplements or controls, in the future.

We are investing significant resources in the development of novel siRNA and gene therapy 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 has published guidance documents in recent years with respect to the regulation of gene therapy products. These guidance documents have generally been intended by FDA to promote greater transparency and increase efficiency in the development of gene therapy products. Nevertheless, there can be no assurance that these guidance documents will enable the accelerated development or approval of any particular gene therapy products.

These regulatory review agencies, committees and advisory groups, including changes in FDA leadership, 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, the FDA has approved dosing in a clinical trial of ELEVIDYS to evaluate the use of an enhanced immunosuppressive regimen as part of treatment with ELEVIDYS for non-ambulatory patients. FDA may also seek additional clinical trials or studies related to ELEVIDYS, which could adversely impact the Company.

If the anticipated or actual timing of marketing approvals for our 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 products 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 products or 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 products or 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.

For example, we are in the process of conducting various clinical trials for ELEVIDYS, including to evaluate the use of an enhanced immunosuppressive regimen as part of treatment with ELEVIDYS for non-ambulant individuals living with Duchenne. Resumption of dosing in the non-ambulatory population will depend in part on the FDA's analysis of whether this data positively changes ELEVIDYS's risk/benefit profile. We intend to discuss with the FDA the results of this study and a potential pathway forward to resume commercial dosing in the non-ambulatory population. The FDA may interpret the data from these trials differently than us and, regardless of the trial results, not permit us to resume shipments to non-ambulatory patients. In addition, in November 2025, we announced topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45, which primary endpoint did not meet statistical significance. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026 with, as of the date of this Quarterly Report, an expected PDUFA target action date by February 28, 2027. The FDA may interpret the results of the ESSENCE trial differently from us, which could lead to regulatory actions from the FDA, including changes to our drug labels, revocation of accelerated approvals and directives to remove these products from the market altogether.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our products or product candidates, the regulatory approvals for such products or 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 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, breakthrough therapies, or RMAT 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 designation includes all the benefits of fast track and breakthrough therapy designations, including early interactions with the FDA, and may also support approval based on surrogate or intermediate endpoints and satisfaction of post-approval requirements through the use of alternative or expedited means, such as reliance on data from a meaningfully sized patient population and postmarketing confirmatory studies, which may be conducted after approval.

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 restructuring in July 2025, we have paused a number of our programs, including those for LGMD (except LGMD2E) 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.

Interim, initial, "topline" and preliminary data from our clinical trials that we announce or publish from time to time are subject to audit and verification procedures and may differ materially from final data as more patient data become available.

Preliminary or topline data from our preclinical studies and clinical trials that we announce or publish from time to time are based on preliminary analyses of then-available data, and the results, related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular preclinical study or clinical trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline or preliminary results may differ from future results of the same studies or trials, or different conclusions or considerations may qualify such results once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data. As a result, topline data should be viewed with caution until the final data are available. For example, we announced topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026 with, as of the date of this Quarterly Report, an expected PDUFA target action date by February 28, 2027. As another example, as part of an

FDA postmarketing study requirement for EXONDYS 51, we are conducting a Phase 3 clinical trial, MIS51ON, comparing the safety and efficacy of higher doses of eteplirsen to the approved dose.

From time to time, we also may disclose interim data from our preclinical studies and clinical trials. For example, in March 2026, we announced early results from Phase 1/2 ascending dose studies of SRP-1001 for FSHD1 and SRP-1003 for DM1. Interim data from clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as participant enrollment continues and more participant data become available or as participants from our clinical trials continue other treatments for their disease.

Furthermore, third parties, including regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could delay or prevent regulatory approval of, or limit commercial prospects for, the particular product candidate. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and investors or others may not agree with what we determine to disclose.

If the interim, topline or preliminary data that we report differ from final results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, financial condition, results of operations and prospects. Further, disclosure of interim, topline or preliminary data by us or by our competitors could result in volatility in the price of our common stock.

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 third-party logistics companies 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, support, or distribute 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 maintain and continue building out our network for commercial distribution in jurisdictions in which our products are approved, as well as in jurisdictions in which our products are available through our EAP, all of which will 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 contractual obligations and 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, including in some cases our strategic partners, to conduct some aspects of our early stage research and pre-clinical and clinical development, and in some instances are transitioning pre-clinical and clinical development work, including some clinical trials, to be performed internally. The inadequate performance by or loss of any of these third parties, or issues arising from transitioning work to be performed internally, could affect the development and commercialization of our product candidates.

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, fail to carry out their contractual duties or obligations, or do not meet applicable standards with respect to study conduct or quality, our programs related to any particular product candidate could be delayed, terminated, or unsuccessful. For example, Arrowhead has conducted the Phase 1/2 ascending dose studies for SRP-1001 (for FSHD1) and SRP-1003 (for DM1) to date, for which we announced early and limited results in March 2026. In addition, we are in the process of transitioning certain pre-clinical and clinical development work, including some clinical trials, from Arrowhead to Sarepta. The transition process, including the transfer and validation of assays, and any resulting adjustments has, and may continue to, impact anticipated timelines and regulatory milestones for several of our siRNA programs. If we are not able to transition this pre-clinical and clinical development work effectively or there are issues arising from the transition process, development of our affected programs and product candidates could be adversely affected and costs associated with development could be impacted.

Furthermore, if we fail to make required payments to our 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 on which 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. legislation, such as the BIOSECURE Act, which was enacted in December 2025. The BIOSECURE Act, among other things, prohibits U.S. federal agencies from entering into or renewing any contract with, or providing any grant or loan to, any entity that uses biotechnology equipment or services produced or provided by a "biotechnology company of concern" to perform that contract, grant, or loan. Generally, a "biotechnology company of concern" is a biotechnology company that is subject to the jurisdiction, direction, control, or operates on behalf of a foreign adversary's government and poses a risk to the national security of the United States. Biotechnology companies of concern include entities listed on the Department of Defense Section 1260H list of "Chinese military companies" and additional entities to be designated through an interagency process led by the Office of Management and Budget. If one of our key China-based service providers is identified as a biotechnology company of concern, the BIOSECURE Act could cause us to seek to exit some or all of our arrangements with them and transition these services to alternative companies, which transition may be costly, time-consuming and could delay our research and pre-clinical development timelines.

Risks Related to Manufacturing and CMC

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, starting materials and subunits), API and drug product and to provide release testing and labeling and packaging of vials and storage of our products and product candidates. The limited number of third parties with facilities, expertise and the capability suited to manufacture 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 for all three of our PMO commercial products and EXONDYS 51 drug product, one active source for AMONDYS 45 and VYONDYS 53 drug product, and one source for ELEVIDYS drug substance and drug product manufacturing.

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 clinical, 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 and release testing 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.

We may also be impacted by development, operation, or production disruptions involving these third parties and our partners, the reasons for which also may be outside of their or our control. Several factors could cause production interruptions, disturbances or supply chain issues at our third party manufacturing, supply, testing, packaging, and distribution sites, including but not limited to talent acquisition/retention, equipment malfunctions, quality control and quality assurance issues, facility contamination, raw material shortages or contamination, man-made or natural disasters, public-health pandemics or epidemics, disruption in utility services, regulatory decisions and 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 facility or business, government shutdowns, economic sanctions, human error, or disruptions in the operations of suppliers and other partners, including those caused by geopolitical conflicts or tariffs. Newly imposed or increased tariffs on pharmaceutical products, active pharmaceutical ingredients or manufacturing equipment and materials sourced from outside the U.S., including in connection with the April 2026 executive order establishing tariffs on certain imported patented pharmaceuticals, could increase our manufacturing and supply chain costs or disrupt our supply arrangements, particularly if our third-party manufacturers or their suppliers are located outside the U.S. 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. Any interruption in the operation of our third-party manufacturing, supply, packaging, release testing, or distribution partners or any of their suppliers or partners 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. In turn, any delay or interruption in the supply of finished commercial or clinical products could hinder our ability to distribute our products to meet commercial or clinical demand or execute our commercialization or clinical trial plans on the timing that we expect, which could result in the loss of potential revenues, adversely affect our ability to gain regulatory or market acceptance, or otherwise adversely affect our business, financial condition and prospects.

In addition, several of the components used in our manufacturing processes and for 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.

From time to time, we may need to add new manufacturing or testing capacity to meet increased product demand. The process for adding new capacity is lengthy and often causes delays in development efforts. Further, there may be circumstances in which we cease or temporarily suspend manufacturing for our products or product candidates altogether, which could impair the development of our product candidates and ability to meet commercial demand. For example, in June 2025, we suspended shipments of ELEVIDYS to non-ambulatory patients, and in July 2025 we temporarily suspended the shipments of ELEVIDYS to ambulatory patients. While the Company has resumed shipments of ELEVIDYS to ambulatory patients, the non-ambulatory population has since been removed from the Indications and Usages section of the ELEVIDYS Prescribing Information. Generally, any interruption or delay of the development of manufacturing facilities or testing capabilities, or any suspension of manufacturing or testing at our existing third-party facilities, could result in our inability to meet commercial or clinical product demand, which could result in the loss of potential revenues, adversely affect our ability to meet product development or regulatory milestones on anticipated timelines, or otherwise adversely affect our business, financial conditions and prospects. Moreover, should shipments of ELEVIDYS to the non-ambulatory population resume in the future, our CMOs may experience delays associated with increasing manufacturing output, which could adversely impact our ability to predict production timelines and meet commercial demand.

If any of the third parties on which we rely 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.

Lastly, we may enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties; in doing so, we constrain our operational flexibility. 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.

Our products 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 our programs, limit the supply of our products, product candidates or future approved products or otherwise harm our business.

Our commercial products and product candidates in development—including our PMOs, gene therapies, and siRNA therapies—are novel and complex technologies that are or may be difficult to manufacture. The novelty of these therapies, in addition to the regulatory oversight and review they face, may lead to production issues or volatility in both supply and demand.

We may not be able to accurately estimate commercial demand for our products given their novelty and complexity. If commercial demand for our products or product candidates is greater than we estimate, we and our third-party partners may be unable to fulfill all orders in a timely manner, which may adversely affect our business, financial condition and prospects. Conversely, if commercial demand is less than we estimate, we may be required to reduce, suspend, or cease production, which has, and could in the future, result in significant accounting charges relating to write-off of inventory if such inventory becomes in excess, obsolete or unusable.

Our ability to accurately predict commercial demand may also be impacted by regulatory decisions. For instance, following a safety label update process, the non-ambulatory population has been removed from the Indications and Usages section of the ELEVIDYS Prescribing Information. This removal has impacted our previously forecasted demand for ELEVIDYS, which increases the risk of products and portions of our products' supply expiring before sale or having excess materials on hand, which could have a material impact on our financial operations. To date, this has resulted in recorded reserves related to excess inventory. These types of events may continue to cause volatility in demand for ELEVIDYS, which could result in our inability to accurately forecast commercial demand for ELEVIDYS. In addition, we announced in November 2025 topline results from our ESSENCE trial, a confirmatory trial intended to verify the clinical benefits of VYONDYS 53 and AMONDYS 45. We submitted sNDAs related to these products in April 2026, which the FDA accepted for filing in June 2026 with, as of the date of this Quarterly Report, an expected PDUFA by February 28, 2027. Any future regulatory decision or action related to VYONDYS 53 or AMONDYS 45, including our anticipated PDUFA, could impact our previously-forecasted demand for these products, which may increase the risk of products and portions of our products' supply expiring before sale or having excess materials on hand. Changes in demand also may lead to

changes in our commercial relationships, including pricing, that are difficult to predict and could have a negative impact on our business.

If our third-party manufacturers and testing sites are unable to satisfy requirements related to the manufacturing of our products and product candidates, 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 our products or product candidates, or otherwise adversely affect our business, financial condition and prospects.

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

Our contract partners are required to produce and test our materials, APIs and drug products under cGMP. We do not have direct operational control over a third-party's compliance with regulations and requirements. We and our contract partners 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 and test 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 and testing processes and facilities. 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.

Further, changes in cGMP could negatively impact the ability of us or our contract partners to complete the manufacturing and testing processes of our products and product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively.

Failure by us or our contract manufacturing and testing partners to achieve, maintain compliance with, or adhere to applicable cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated errors, or our contract partners experiencing problems, may result in significant negative consequences, including product seizures or recalls, postponement or cancellation of clinical trials, clinical holds, loss or delay of product approvals, patient injury or death, 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.

Finally, with respect to ELEVIDYS and our gene therapy programs in particular, the current capacity to produce and test our viral vectors or gene therapy product candidates at commercial levels is limited, and the availability of sufficient GMP compliant 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. We also rely on a third party to develop, manufacture, obtain and maintain regulatory approval for necessary diagnostic tests for ELEVIDYS. Any delay or failure by us or our collaborators to develop, obtain or maintain regulatory approval of the necessary diagnostic tests could harm our business, possibly materially.

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.

We have historically focused on optimizing manufacturing, including for our product candidates, gene therapy, PMOs, and other programs. For example, as our product candidates advance to later clinical stages, we have sought and may seek in the future to alter manufacturing and/or other product components and processes to optimize the products and product candidates for the scale-up necessary for later stage clinical trials, potential approval, and commercialization. We may not be able to successfully increase manufacturing capacity or optimize manufacturing processes 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 or optimize manufacturing capacity and scale up production. We experience such issues in connection with manufacturing, testing, 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, including by negatively impacting 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/or optimize manufacturing capacity and processes, they may make proprietary improvements in the manufacturing or testing 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 and testing processes 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.

Deviations from manufacturing, testing, packaging and distribution processes and procedures could result in manufacturing delays or delays in product release, which may harm our business.

Sarepta maintains strict adherence to applicable regulatory guidance and industry standards. We employ multiple steps to control our manufacturing and testing processes to ensure product quality, safety and compliance throughout drug development and commercial manufacturing.

Several factors could result in delays in and expenses relating to this manufacturing control process. We or our third-party partners 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. Problems with the manufacturing or testing processes, even minor deviations from the normal process, could result in delays 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, which may and has resulted in write-offs of batches of our products not meeting our quality specifications. We also face risk of damage during shipping and storage of the APIs or finished drug product. 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 our products, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

In addition, the FDA, 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 a product lot not be distributed until the applicable regulatory authority has authorized its release, or may prohibit its distribution altogether.

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, our gene therapy-based products and product candidates, and our siRNA 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 or term of patent, 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 products and product candidates 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 may subject us 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:

- the federal healthcare anti-kickback law, which prohibits, 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 calculate, certify and report certain complex 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 or requiring disclosure of financial interactions between pharmaceutical manufactures and healthcare providers (e.g., requiring pharmaceutical companies to comply with specific compliance standards that restrict financial interactions between pharmaceutical companies and healthcare companies providers), state laws requiring the registration of pharmaceutical sales representatives, 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. For example, in September 2025, the FDA stated that it intends to more aggressively enforce requirements for direct-to-consumer ("DTC") drug advertising and sent more than 100 warning or untitled letters to companies for allegedly deceptive prescription drug advertising, which represents a dramatic increase in FDA actions as compared to prior years. The FDA also announced plans to expand its oversight of digital and social media advertising and to initiate a rulemaking that would call for drug companies to disclose additional safety information in DTC broadcast advertisements. The nature and extent of changes to FDA regulations and enforcement approach is unclear but may impact pharmaceutical marketing efforts industry-wide, including for us, which could in turn impact our sales and operations. As another example, recent government communications indicate that the longstanding focus of federal enforcement agencies on pharmaceutical company activities will continue. In 2025, the U.S. Department of Justice (“DOJ”) issued a white collar enforcement plan identifying enforcement priorities for prosecuting corporate and white collar crime which includes health care fraud. Similarly, the DOJ-HHS False Claims Act Working Group enforcement priorities include drug pricing and kickbacks related to drugs paid for by federal healthcare programs. More broadly, in early 2026, the current presidential administration created a Task Force to Eliminate Fraud to coordinate and accelerate a comprehensive "whole-of-government" national strategy to stop fraud, waste, and abuse within federal benefit programs, and a new DOJ division was established for national fraud enforcement, focusing on fraud targeting federal government programs, federally funded benefits, businesses, nonprofits, and private citizens nationwide. In June 2026, the DOJ announced the results of the National Health Care Fraud Takedown, which included charges against 455 defendants for alleged participation in health care fraud and opioid abuse schemes. In October 2026, a DOJ directive identified health care fraud as one of four priorities for federal fraud prosecutors. If we were ever subject to such an investigation, we may face substantial penalties and our business, operations and financial condition could be adversely affected.

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 workforce reduction represented approximately 36% of our workforce at the time it was implemented. 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 disruptions in our day-to-day operations. The restructuring and reduction in force has resulted, and may continue to result, in an increase in the turnover of our employees. 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, over 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 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 alter 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. Any such changes in Medicare average sales price reporting could implicate those pricing calculations and the Medicare reimbursement rates for drugs. For example, beginning in 2026, manufacturers must now meet enhanced documentation and certification requirements to exclude certain service fees from pricing calculations under the Medicare 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. 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.

Recent drug pricing and payment reforms initiatives under the current presidential administration such as the call for "most favored nation pricing" for products covered under government programs, if implemented, could affect our obligations under government pricing and price reporting programs. See "*Risks Related to Our Business—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*." Several states have also 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 other jurisdictions in which we operate and future guidance could adversely affect our business and financial condition.

We are unable to predict what tax reform may be proposed or enacted in the future by the U.S. and other countries in which we currently or may in the future operate in or what effect such changes would have on our business and results of operations. Changes in tax rates, laws, practices, treaties, policies or regulations, or the change in interpretation thereof, could increase our effective tax rate or otherwise affect our financial position, results of operations and financial condition and/or increase the complexity, burden and cost of tax compliance.

In addition, the Inflation Reduction Act of 2022, among other things, implemented a corporate book minimum tax ("BMT") rate of 15% 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 our cash tax liability and ability to utilize tax attributes. The current proposal of the BMT may also result in increases in taxes imposed by non-U.S. jurisdictions. 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, which may adversely affect our tax provision, cash tax liability and effective tax rate.

Further, on July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. The OBBBA contains, among other provisions, changes to the U.S. corporate income tax system, including allowing immediate expensing of U.S. qualifying research and development expenses and allowing taxpayers an election to accelerate the deduction for previously capitalized U.S. research and development costs. The favorable U.S. research and development expenditure provisions will reduce taxable income but will not have a material impact on the Company's net deferred tax assets.

These and other tax law changes and anti-avoidance initiative increase uncertainty and may adversely affect our tax provision, cash tax liability and effective tax rate.

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. 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. The U.S. net operating losses have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits.

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. In addition, these actions may divert our management's attention away from our day-to-day operations and may be disruptive to our business.

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. Additionally, the departure of senior leaders, including the recent departure of our Chief Executive Officer, could cause disruptions or increase the likelihood of turnover in other key employees.

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 the disease areas we aim to treat. 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 talent. 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, Chief Customer Officer in July 2025 and Bilal Arif, Chief Technical Operations Officer in August 2025. Additionally, Doug Ingram, our former Chief Executive Officer, retired from the Company as of July 28, 2026, and Michael Severino, M.D., was appointed Chief Executive Officer. 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. Additionally, our restructuring and reduction in force in July 2025 has resulted in an increase in the turnover of employees. The restructuring and reduction in force could also lead to unforeseen disruptions to our business and may continue to impact the likelihood of turnover of additional employees, 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 generated operating income of $362.7 million for the nine months ended September 30, 2026. Our accumulated deficit was $4.6 billion as of September 30, 2026. 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 (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.

If we fail to maintain effective internal controls, we may not be able to accurately report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

We were required, pursuant to Section 404 of the Sarbanes-Oxley Act of 2002 to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting for the fiscal year ended December 31, 2025. In addition, our independent registered public accounting firm was required to attest to the effectiveness of our internal control over financial reporting for the fiscal year ended December 31, 2025.

We cannot assure you that the measures we have taken to date, and actions we may take in the future, will prevent or avoid potential material weaknesses in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting, including as a result of impacts to our financial reporting team in light of the Restructuring, could severely inhibit our ability to accurately report our financial condition or results of operations and may result in a restatement of our financial statements for prior periods. If we are unable to conclude that our internal control over financial reporting is effective, or if management or our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

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 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, income tax and the impacts of litigation, including expected settlement arrangements. 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, the adverse events observed in non-ambulatory patients receiving ELEVIDYS and in our Phase 1 LGMD trial for SRP-9004, and our ESSENCE readout. For example, over the last twelve months, as of the date of this report, our stock has increased as much as 35% in a single day or decreased as much as 14% 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, as well as regulatory interactions and decision-making, which could be impacted by changes in FDA priorities, practices or leadership;
- 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 and our products;
- 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 existing litigation in which we are a party and new lawsuits filed 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, recent events in Venezuela, or the conflict in the Middle East. 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. The Company faced a securities class action litigation filed on June 26, 2025, the dismissal of which is currently subject to an appeal. In addition, related derivative lawsuits have been filed in 2025 and 2026. 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, enrollment forms and delays in patient infusions;
- 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 September 30, 2026, there were approximately 107.2 million shares of common stock outstanding and outstanding awards to purchase 13.5 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2026, there were approximately 6.9 million shares of common stock available for future issuance under our 2026 Equity Incentive Plan and approximately 1.1 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 2026 Equity Incentive 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 2027 Notes and 2030 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. Additionally, on August 28, 2025, the Company completed privately negotiated exchanges of $700.0 million in aggregate principal amount of 2027 Notes for consideration consisting of (i) $602.0 million in aggregate principal amount of 2030 Notes, (ii) an aggregate of 5,851,693 shares of the Company's Common Stock, and (iii) an aggregate of approximately $127.3 million in cash. On December 18, 2025, the Company completed privately negotiated exchanges of approximately $291.4 million in aggregate principal amount of 2027 Notes for consideration consisting of (i) approximately $291.4 million in aggregate principal amount of 2030 Notes and (ii) an aggregate of approximately $31.6 million in

cash. As of September 30, 2026, the aggregate principal amount of 2027 Notes totaled approximately $158.6 million and the aggregate principal amount of 2030 Notes totaled approximately $893.4 million.

Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the 2027 Notes and 2030 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, or our 2030 Notes which mature in 2030, 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 or the 2030 Notes as required upon a fundamental change, and our future debt may contain limitations on our ability to repurchase the 2027 Notes or the 2030 Notes.

Holders of the 2027 Notes and 2030 Notes will have the right to require us to repurchase their 2027 Notes and 2030 Notes, respectively, 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 or the 2030 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 or 2030 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 or 2030 Notes upon a fundamental change. Our failure to repurchase the 2027 Notes or 2030 Notes upon a fundamental change when required would result in an event of default with respect to the 2027 Notes or 2030 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 or 2030 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 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 products, 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, including economic sanctions imposed by the U.S. or on the U.S., 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 the third-parties we rely upon, including 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.

Despite our workforce reduction, we may in the future seek to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.

Despite our workforce reduction in July 2025, we may in the future expand our full-time employee base, as well as our consultant and contractor base to support our business operations. 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, have been and may continue to 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 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. For example, we currently are involved in various intellectual property and securities litigations. Consequently, we expect to expend significant amounts of money and company resources in connection with these and other 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. In addition, defending these lawsuits, including any appeals, and other disputes may divert our management’s attention away from our day-to-day operations and may be disruptive to our business.

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, and after evaluation, we have begun using certain AI tools across our organization. We are evaluating, and will continue to evaluate, the use of AI tools throughout our organization, including tools used by our third-party vendors, collaborators and service providers that may process our proprietary and patient data. There are risks involved in developing and using AI in our operations, including related to enhanced governmental or regulatory scrutiny, potential intellectual property, data privacy, cybersecurity and quality risks associated with third-party AI tools, 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 intellectual property, 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.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

During the three months ended September 30, 2026, 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	Amended and Restated Bylaws.	8-K	001-14895	3.1	9/25/14	
3.5	Amendment No. 1 to the Amended and Restated Bylaws.	8-K	001-14895	3.1	1/13/20	
3.6	Second Amended and Restated Bylaws.	8-K	001-14895	3.1	12/13/22	
3.7	Amendment No. 1 to Second Amended and Restated Bylaws of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	9/16/24	
10.1†	Employment Agreement, dated July 24, 2026, by and between Michael Severino, M.D., and Sarepta Therapeutics, Inc.	8-K	001-14895	10.1	7/27/26	
10.2†	Consulting and Advisory Agreement, dated July 26, 2026, by and between Douglas Ingram and Sarepta Therapeutics, Inc.	8-K	001-14895	10.2	7/27/26	
10.3^	Settlement Agreement between REGENXBIO Inc., The Trustees of the University of Pennsylvania, Sarepta Therapeutics, Inc., Sarepta Therapeutics Three, LLC, and Catalent, Inc., entered into as of September 23, 2026.					X
31.1	Certification of the Company's Principal Executive Officer, Michael Severino, M.D., 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 H. Wong, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Principal Executive Officer, Michael Severino, M.D., 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 H. 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.
* 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.
^ Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) the type of information the registrant treats as private or confidential.

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: November 5, 2026

By: /s/ MICHAEL SEVERINO, M.D.
Michael Severino, M.D.
Chief Executive Officer
(Principal Executive Officer)

Date: November 5, 2026

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

Exhibit 10.3

[**] = Information has been excluded from the exhibit because it is both (i) not material and (ii) the type of information the registrant treats as private or confidential.

SETTLEMENT AGREEMENT

This Settlement Agreement ("Agreement") is entered into as of September 23, 2026 ("Effective Date"), by and among REGENXBIO Inc. ("REGENXBIO") and The Trustees of the University of Pennsylvania ("UPenn") (together with REGENXBIO, "Plaintiffs"), on the one hand, and Sarepta Therapeutics, Inc. ("Sarepta Therapeutics"), Sarepta Therapeutics Three, LLC ("Sarepta Three") (together with Sarepta Therapeutics, "Sarepta") and Catalent, Inc. ("Catalent") (together with Sarepta, "Defendants"), on the other hand. Defendants and Plaintiffs are each referred to herein individually as a "Party" and collectively as the "Parties."

RECITALS

WHEREAS, REGENXBIO and UPenn commenced an action against Sarepta, alleging infringement of U.S. Patent No. 10,526,617 ("the '617 Patent"), captioned *REGENXBIO Inc. et al. v. Sarepta Therapeutics, Inc. et al.*, C.A. No. 20-cv-01226-RGA ("the '617 Proceeding");

WHEREAS, REGENXBIO and UPenn commenced an action against Sarepta and Catalent, alleging infringement of U.S. Patent No. 11,680,274 ("the '274 Patent") (together with the '617 patent, "the Patents-in-Suit"), captioned *REGENXBIO Inc. et al. v. Sarepta Therapeutics, Inc. et al.*, C.A. No. 23-cv-00667-RGA ("the '274 Proceeding");

WHEREAS, Sarepta filed a Petition for *Inter Partes* Review of the '274 Patent with the Patent Trial and Appeal Board ("the PTAB") ("IPR Proceeding") and subsequently appealed from the PTAB's decision to the U.S. Court of Appeals for the Federal Circuit in a matter captioned *Sarepta Therapeutics, Inc. v. Trustees of the University of Pennsylvania, REGENXBIO Inc.*, 2026-1071 ("Appeal Proceeding") (the IPR Proceeding and the Appeal Proceeding, together with the '617 Proceeding and the '274 Proceeding, "the Litigations");

WHEREAS, the Parties have agreed to resolve the Litigations on the terms set forth in this Agreement;

WHEREAS, the Parties wish to avoid the cost, distraction, and uncertainty of continued litigation and desire to fully and finally resolve their disputes on the terms set forth herein;

NOW, THEREFORE, in consideration of the mutual covenants and agreements set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

1. **Payment.** Within ten (10) days of the Effective Date and receipt of invoice, Sarepta shall pay REGENXBIO the total sum of Thirty-Nine Million Dollars ($39,000,000) ("the Settlement Payment"), by wire transfer of immediately available funds pursuant to the wire instructions set forth below:

[**]

REGENXBIO shall email the invoice for the Settlement Payment and a completed W-9 form to **[**]**.

2. **Dismissal of Litigations.** Within 3 business days of Sarepta's payment of the Settlement Payment, the Parties shall cause to be filed such motions, notices, or stipulations to terminate, withdraw, or otherwise dispose of the Litigations with prejudice, substantially in the form of the stipulations attached hereto as Exhibits A-C. Except as otherwise provided in this Agreement, each Party shall bear its own costs, expenses, and attorneys' fees incurred in connection with the Litigations and the negotiation and execution of this Agreement.

3. **Release.**

3.1 Release With Prejudice. Plaintiffs, on behalf of themselves and their current affiliates, will release and forever discharge Defendants and their current affiliates from any and all claims, causes of action, and liabilities of any kind relating to any Sarepta gene therapy product that uses an AAVrh74-based capsid, including ELEVIDYS®, whether known or unknown, of any nature whatsoever, that are claims of infringement of the Patents-in-Suit; U.S. Patent No. 9,198,984 ("the '984 Patent"); and/or any patents or patent applications that claim priority to the Patents-in-Suit or the '984 Patent, or from which the Patents-in-Suit or the '984 Patent claims priority, including any continuation, divisional, continuation-in-part, reissue, or foreign counterpart of any of the foregoing (collectively, "the UPENN Patents"), arising at any time up to and including the Effective Date of this Agreement. Except as expressly stated in this Agreement, no authorization, authorized sale, or express or implied license or future release is granted by the Plaintiffs.

3.2 Exclusion as to Catalent. Nothing in the release in this Section 3 shall be construed to release, waive, or otherwise affect any claims, rights, or obligations arising under any pre-existing agreements between REGENXBIO and Catalent that are separate from Catalent's manufacture, supply, or other activities performed for or on behalf of Sarepta with respect to ELEVIDYS® or any Sarepta gene therapy that uses an AAVrh74-based capsid.

4. **Covenant Not to Sue.**

4.1 Plaintiffs, on behalf of themselves and their current affiliates, and their successors, and assigns, will covenant not to sue Defendants, their current affiliates, licensees, commercial partners, successors, and permitted assigns, with respect to any and all claims of patent infringement of the UPENN Patents, solely to the extent that such infringement arises from or relates to any Sarepta gene therapy product that uses an AAVrh74-based capsid, including ELEVIDYS®.

4.2 REGENXBIO on behalf of itself and current affiliates, and their successors, and assigns, will covenant not to sue Sarepta, their current affiliates, licensees, commercial partners, successors, and permitted assigns, with respect to any and all claims of patent infringement of any REGENXBIO controlled or owned patents or patent applications that cover the existing form of ELEVIDYS® or that cover the AAVrh74 capsid sequence ("the REGENXBIO Patents"). This covenant not to sue with respect to the REGENXBIO Patents is referred to herein as the "REGENXBIO-only CNS." For the avoidance of doubt, the REGENXBIO Patents do not include the UPENN Patents.

4.3 Notwithstanding any language to the contrary, the REGENXBIO-only CNS does not extend to patents and/or pate applications that cover other aspects of gene therapy products, regardless of whether those gene therapy products also use an AAVrh74-based capsid. For the

avoidance of doubt, the current form of ELEVIDYS® drug substance and drug product are fully covered by this REGENXBIO-only CNS, including any changes made in accordance with 21 C.F.R. § 601.12 or any equivalent foreign regulation, but only to the extent that such changes do not change the sequence of the ELEVIDYS® promoter, transgene, or capsid protein.

5. Assignment and Transfer.

5.1 By Plaintiffs. The releases and covenants set forth in this Agreement are binding upon Plaintiffs and their current affiliates, and their successors, and assigns, including in the event of a merger, acquisition, bankruptcy, insolvency, reorganization, liquidation, or change of control, and will inure to the benefit of Defendants, as specified above, and their current affiliates, licensees, commercial partners, successors, and permitted assigns solely to the extent that their activities involve a Sarepta gene therapy product that uses an AAVrh74-based capsid. Plaintiffs will ensure that any sale, transfer, assignment, or other disposition of the UPENN Patents, whether in the ordinary course or pursuant to a bankruptcy or insolvency proceeding, is made expressly subject to the releases and covenants set forth in this Agreement, and Plaintiffs will provide Defendants with prompt written notice of any such sale, transfer, assignment, or disposition.

Furthermore, Plaintiffs will not grant, and will ensure that its affiliates, successors and assigns do not grant, to any future affiliate or third party any rights to enforce the UPENN Patents against the Defendants, or their affiliates, existing licensees, commercial partners, successors, or assigns to the extent that their activities involve a Sarepta gene therapy product that uses an AAVrh74-based capsid.

5.2 By REGENXBIO. REGENXBIO will ensure that any sale, transfer, assignment, or other disposition of the REGENXBIO Patents, whether in the ordinary course or pursuant to a bankruptcy or insolvency proceeding, is made expressly subject to the releases and covenants set forth in this Agreement, and REGENXBIO will provide Defendants with prompt written notice of any such sale, transfer, assignment, or disposition.

Furthermore, REGENXBIO will not grant, and will ensure that its affiliates, successors and assigns do not grant, to any future affiliate or third party any rights to enforce the REGENXBIO Patents against the Defendants, or their affiliates, existing licensees, commercial partners, successors, or assigns to the extent that their activities involve a Sarepta gene therapy product that uses an AAVrh74-based capsid.

The provisions of this section are subject to the representations and warranties in Section 6.2.

5.3 By Defendants. The releases and covenants set forth in this Agreement are granted to Defendants and their affiliates only. Such releases and covenants are not transferrable to any third party without prior written consent of Plaintiffs, except in the event of (1) a sale, license or other transfer of all or substantially all the assets of Sarepta or (2) a sale, license, or other transfer of ELEVIDYS® or any Sarepta gene therapy that uses an AAVrh74-based capsid, including but not limited to SRP-9003 (**[**]**), SRP-9004 (**[**]**), SRP-9005 (**[**]**), SRP-9006 (**[**]**), MYO-201 (**[**]**), and Calpain 3 (**[**]**) to an unrelated third party. For the avoidance of doubt, the releases and covenants set forth in this Agreement do not extend to any third party that develops, manufactures, or commercializes a biosimilar of any gene therapy product.

6. Representations and Warranties.

6.1 General Representations. Each Party represents and warrants that:

(a) it has the full right, power, and authority to enter into this Agreement and to grant the releases and covenants set forth herein;
(b) the execution and performance of this Agreement have been duly authorized by all necessary corporate action;
(c) this Agreement constitutes a valid and binding obligation, enforceable against each Party in accordance with its terms; and
(d) it has read and understands this Agreement, has had the opportunity to review its terms with counsel of its choosing, and executes this Agreement voluntarily and without coercion.

6.2 Representations on Behalf of Plaintiffs.

(a) UPenn represents that, as of the Effective Date, (i) it is the sole and lawful owner of the UPENN Patents, and GlaxoSmithKline, LLC (formerly SmithKline Beecham Corporation, d/b/a GlaxoSmithKline, referred to as "GSK") and REGENXBIO are the only licensees under the UPENN Patents, other than any license held by the federal government pursuant to and required by the Bayh-Dole Act and/or its implementing regulations and any license held by non-commercial entities solely for educational and research purposes; (ii) there is no person or entity other than UPenn and/or REGENXBIO who has an independent right to bring an infringement action under the UPENN Patents against Defendants for any conduct arising at any time with respect to any Sarepta gene therapy product that uses an AAVrh74-based capsid; (iii) UPenn will not voluntarily join, assist, finance, or otherwise voluntarily participate in any claim or action brought by any third party against Defendants, their affiliates, successors, or assigns, asserting infringement of the UPENN Patents based on any Sarepta gene therapy product that uses an AAVrh74-based capsid, for conduct arising at any time.

(b) UPenn represents that, as of the Effective Date: (i) to the knowledge of the current staff of the Penn Center for Innovation, the center at UPenn that is responsible for technology protection, licensing and enforcement ("PCI"), without independent investigation or inquiry and without any duty to conduct any search, investigation, inquiry or other diligence, there are no issued or pending patents or patent applications owned or controlled by UPenn, other than the UPENN Patents, that cover or purport to cover the AAVrh74 capsid or ELEVIDYS®, and (ii) PCI has not received written notice from any third party licensee of any patents owned or controlled by UPenn, other than the UPENN Patents, asserting or alleging that the development, manufacture, commercialization or exploitation of any Sarepta gene therapy product that uses an AAVrh74-based capsid, including ELEVIDYS®, infringed, misappropriated, or otherwise violated any claim of any patent or patent application licensed to such third party by UPenn.

(c) REGENXBIO represents and warrants that, as of the Effective Date, (i) there are no unexpired patents or patent applications that constitute REGENXBIO Patents, based on REGENXBIO's current knowledge and understanding of ELEVIDYS®, which is based solely on publicly available information; (ii) to the extent that there are any expired patents that would constitute REGENXBIO Patents, there is no person or entity other than REGENXBIO who has an independent right to bring

an infringement action under the REGENXBIO Patents against Defendants for any conduct arising at any time with respect to any Sarepta gene therapy product that uses an AAVrh74-based capsid; (iii) to the extent any patent or patent application later owned or controlled by REGENXBIO covers the existing form of ELEVIDYS® or covers the AAVrh74 capsid sequence, such patent or patent application shall be subject to the provisions of Sections 4 and 5 of this Agreement; (iv) GSK has granted REGENXBIO all authority and rights necessary, to the extent that any such authority or rights from GSK are necessary, to settle all claims against Defendants relating to the Patents-in-Suit that were asserted in the Litigations and to enter into the Plaintiffs' covenant not to sue relating to the Patents-in-Suit and the release, none of which violate or conflict with or require consent under any agreement between UPenn, REGENXBIO and GSK relating to the Patents-in-Suit and (v) REGENXBIO will not voluntarily join, assist, finance, or otherwise voluntarily participate in any claim or action brought by any third party against Defendants, their affiliates, successors, or assigns, asserting infringement of the REGENXBIO Patents based on any Sarepta gene therapy product that uses an AAVrh74-based capsid, for conduct arising at any time.

6.3 Representations on Behalf of Defendants. Sarepta represents that it will not affirmatively challenge the validity or enforceability of any claim of the UPENN Patents or the REGENXBIO Patents in any context or forum, including but not limited to any court or United States Patent and Trademark Office proceeding (including, but not limited to, inter partes review, ex parte re-examination, derivation, or post-grant review proceedings), including, but not limited to, initiating or cooperating with any Third Party in instituting or prosecuting a declaratory judgment action with respect to any of the UPENN Patents or the REGENXBIO Patents. Notwithstanding the foregoing, nothing in this section 6.3 shall prevent Defendants, their current affiliates, licensees, commercial partners, successors, and permitted assigns from defending themselves in any legal action or from challenging the validity or enforceability of any patents, including the UPENN Patents and the REGENXBIO Patents, if those patents are asserted against them in the future by any party, including but not limited to by (1) seeking relief from the U.S. Patent and Trademark Office (including, but not limited to, inter partes review, ex parte re-examination, derivation, or post-grant review proceedings), or (2) asserting any argument, claim or defense in litigation brought against it in court.

7. **Confidentiality.** The terms of this Agreement shall be maintained in confidence by the Parties and shall not be disclosed to any third party, except that any Party may disclose the terms of this Agreement: (a) to its accountants, auditors, attorneys, and financial advisors, provided that such persons are informed of, and agree to be bound by, the confidentiality of this Agreement; (b) in connection with a potential or actual merger, acquisition, financing, change of control, sale, license or transfer of all or substantially all of the applicable business or assets of such Party or the sale, license or transfer of ELEVIDYS® or any Sarepta gene therapy that uses an AAVrh74-based capsid, including but not limited to SRP-9001, SRP-9003, SRP-9004, SRP-9005, SRP-9006, MYO-201, and Calpain 3, when subject to confidentiality obligations at least as protective as those set forth herein; (c) as required by law or the rules or regulations of any stock exchange or governmental or regulatory body, including any disclosure believed in good faith by counsel to be necessary to comply with SEC reporting or disclosure obligations; (d) pursuant to subpoena or other compulsory legal process, provided that the disclosing Party gives the other Parties prompt written notice and a reasonable opportunity to seek a protective order or otherwise contest such disclosure before making it; (e) to any upstream licensors of the Parties as required by any existing agreements, when subject to confidentiality obligations at least as protective as those set forth

herein; or (f) as mutually agreed by the Parties. Any disclosure made pursuant to clause (c) shall be limited to what is reasonably necessary for the specific purpose at issue.

8. **Notices.** Any notice under this Agreement shall be effective if in writing and (i) personally delivered, (ii) sent by certified or registered mail, return receipt requested, or (iii) sent by electronic mail with confirmation of receipt, addressed as follows:

If to Sarepta, to:

Attn: General Counsel
215 First Street
Cambridge, Massachusetts 02142
[**]

with a copy to:

Jim Baker
Quinn Emanuel Urquhart & Sullivan, LLP
295 5th Avenue, 9th Floor
New York, New York 10016
[**]

If to Catalent, to:

Catalent, Inc.
Attn: General Counsel
200 Crossing Blvd., Floor 7
Bridgewater, NJ 08807
Telephone: (732) 537-6154
[**]

with a copy to:

Jim Baker
Quinn Emanuel Urquhart & Sullivan, LLP
295 5th Avenue, 9th Floor
New York, New York 10016
[**]

If to REGENXBIO, to:

REGENXBIO Inc.
9804 Medical Center Drive
Rockville, MD 20850
Attn: Chief Legal Officer
[**]

with a copy to:

[**]

and with a copy to:

Susan Morrison
Fish & Richardson, LLC
222 Delaware Avenue, 17th Floor
Wilmington, Delaware 19801
[**]

If to UPenn, to:

University of Pennsylvania Office of the General Counsel
Attn: General Counsel
FMC Tower at Cira Centre South
2929 Walnut Street, Suite 400
Philadelphia, PA 19104

with a copy to:

Penn Center for Innovation
Attn: Managing Director
3600 Civic Center Blvd, 9th floor
Philadelphia, PA 19104

and with a copy to:

Julie Goldemberg
Morgan, Lewis & Bockius LLP
2222 Market St.
Philadelphia, Pennsylvania 19103
[**]

Any such notice shall be deemed to have been received on the date actually received. A Party may change its notice information by giving the other Parties written notice in accordance with this Section 8.

9. Miscellaneous.

9.1 No Admission (General). Neither this Agreement, the dismissal of the Litigations, nor the Settlement Payment shall constitute or be construed as an admission by any Party of liability, infringement, validity, or any other matter.

9.2 Governing Law; Forum Selection. This Agreement shall be governed by, and construed in accordance with, the laws of the State of Delaware, without regard to its conflict of laws principles. The Parties irrevocably agree that any dispute arising out of or relating to this Agreement shall be brought exclusively in the United States District Court for the District of Delaware, and each Party consents to the personal jurisdiction and venue of that court.

9.3 Cure Period; Remedies for Breach. In the event any Party believes another Party has breached this Agreement, the non-breaching Party shall provide the breaching Party with written notice describing the alleged breach in reasonable detail. The breaching Party shall have thirty (30)

days from receipt of such notice to cure the alleged breach. If the breach is not cured within such thirty (30)-day period, the non-breaching Party may pursue any remedy available at law or in equity, subject to Section 9.2, provided that no breach of this Agreement by any Party, whether or not cured, shall give rise to a right of rescission, and the releases and covenants set forth in Sections 3 and 4 shall remain in full force and effect regardless of any breach of this Agreement. The prevailing Party in any action to enforce this Agreement, or arising from a breach of this Agreement, shall be entitled to recover its reasonable attorneys' fees and costs incurred in connection with such action, in addition to any other relief to which it may be entitled.

9.4 Severability. If any provision of this Agreement is held invalid, illegal, or unenforceable, the Parties shall negotiate in good faith a substitute provision that most closely reflects the original intent, and the remaining provisions shall continue in full force and effect.

9.5 Waiver. No delay or failure to enforce any right under this Agreement, and no acquiescence in any default, shall constitute a waiver of that right, except pursuant to an express written waiver signed by the waiving Party.

9.6 Entire Agreement. This Agreement constitutes the entire agreement among the Parties with respect to its subject matter and supersedes all prior or contemporaneous agreements or understandings, oral or written, relating thereto. This Agreement may be amended only by a writing signed by all Parties.

9.7 Construction. This Agreement has been negotiated and drafted jointly by the Parties through their respective counsel, and no provision shall be construed against any Party on the basis that it, or the Agreement as a whole, was drafted by that Party.

9.8 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original, and all of which together shall constitute one instrument. Signatures delivered by electronic means shall be deemed original signatures.

9.9 Headings. Headings are for reference only and shall not affect the interpretation of this Agreement.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties have executed this Agreement as of the Effective Date.

SAREPTA THERAPEUTICS, INC.

By: _/s/ Michael Severino, M.D._____
Name: Michael Severino, M.D.
Title: Chief Executive Officer
Date: 9/23/2026

SAREPTA THERAPEUTICS THREE, LLC

By: _/s/ Adam Hopkin_____
Name: Adam Hopkin
Title: Manager
Date: 9/23/2026

CATALENT, INC.

By: _/s/ Joseph Ferraro_____
Name: Joseph Ferraro
Title: SVP, General Counsel
Date: 9/23/2026

REGENXBIO INC.

By: _/s/ Patrick Christmas_____
Name: Patrick Christmas
Title: Chief Legal Officer
Date: 9/23/2026

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: _/s/ Wendy White_____
Name: Wendy White
Title: Senior Vice President and General Counsel
Date: 9/23/2026

EXHIBIT A

IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF DELAWARE

REGENXBIO INC. and THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA, Plaintiffs, v. SAREPTA THERAPEUTICS, INC., SAREPTA THERAPEUTICS THREE, LLC, and CATALENT, INC., Defendants.	)	C.A. No. 23-667 (RGA)

STIPULATION OF DISMISSAL

IT IS HEREBY STIPULATED AND AGREED, by and between the parties and pursuant to Rule 41(a)(1) and (c) of the Federal Rules of Civil Procedure, Plaintiffs REGENXBIO Inc. and The Trustees of the University of Pennsylvania and Defendants Sarepta Therapeutics, Inc., Sarepta Therapeutics Three, LLC, and Catalent, Inc. hereby stipulate and agree that the above action, including all claims, counterclaims and affirmative defenses, are dismissed with prejudice. All parties shall bear their own costs and fees incurred in this action.

FISH & RICHARDSON P.C.
/s/

Susan E. Morrison (#4690)
Casey M. Kraning (#6298)
222 Delaware Avenue, 17th Floor
Wilmington, DE 19801
(302) 652-5070
morrison@fr.com
kraning@fr.com

Attorneys for Plaintiff REGENXBIO Inc.

MORGAN, LEWIS & BOCKIUS LLP
/s/

Amy M. Dudash (#5741)
1201 North Market Street, Suite 2201
Wilmington, DE 19801
(302) 574-3000
amy.dudash@morganlewis.com

Attorneys for Plaintiff The Trustees of the University of Pennsylvania

MORRIS, NICHOLS, ARSHT & TUNNELL LLP

/s/

_____
Derek J. Fahnestock (#4705)
1201 North Market Street
P.O. Box 1347
Wilmington, DE 19899 (302) 658-9200
dfahnestock@mnat.com

Attorneys for Defendants
September __, 2026

SO ORDERED, this ____ day of _____, 2026.

_____
United States District Judge

EXHIBIT B

IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF DELAWARE

REGENXBIO INC. and THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA, Plaintiffs, v. SAREPTA THERAPEUTICS, INC., and SAREPTA THERAPEUTICS THREE, LLC, Defendants.	)	C.A. No. 20-1226 (RGA)

STIPULATION OF DISMISSAL

IT IS HEREBY STIPULATED AND AGREED, by and between the parties and pursuant to Rule 41(a)(1) and (c) of the Federal Rules of Civil Procedure, Plaintiffs REGENXBIO Inc. and The Trustees of the University of Pennsylvania and Defendants Sarepta Therapeutics, Inc. and Sarepta Therapeutics Three, LLC hereby stipulate and agree that the above action, including all claims, counterclaims and affirmative defenses, are dismissed with prejudice. All parties shall bear their own costs and fees incurred in this action.

FISH & RICHARDSON P.C.
/s/

Susan E. Morrison (#4690)
Casey M. Kraning (#6298)
222 Delaware Avenue, 17th Floor
Wilmington, DE 19801
(302) 652-5070
morrison@fr.com
kraning@fr.com

Attorneys for Plaintiff REGENXBIO Inc.

MORGAN, LEWIS & BOCKIUS LLP
/s/

Amy M. Dudash (#5741)
1201 North Market Street, Suite 2201
Wilmington, DE 19801
(302) 574-3000
amy.dudash@morganlewis.com

Attorneys for Plaintiff The Trustees of the University of Pennsylvania

MORRIS, NICHOLS, ARSHT & TUNNELL LLP

/s/

_____
Derek J. Fahnestock (#4705)
1201 North Market Street
P.O. Box 1347
Wilmington, DE 19899 (302) 658-9200
dfahnestock@mnat.com

Attorneys for Defendants
September __, 2026

SO ORDERED, this ____ day of _____, 2026.

_____
United States District Judge

EXHIBIT C

IN THE UNITED STATES COURT OF APPEALS
FOR THE FEDERAL CIRCUIT

SAREPTA THERAPEUTICS, INC., Appellant, v. TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA and REGENXBIO INC., Appellees.	)	No. 2026-1071

JOINT STIPULATION OF VOLUNTARY DISMISSAL

Pursuant to Federal Rule of Appellate Procedure 42(b)(1), the parties hereby stipulate to the dismissal of the appeal filed by the Sarepta Therapeutics, Inc., docketed under Case No. 2026-1071, with each side to bear its own fees and costs associated with this appeal.

QUINN EMANUEL URQUHART & SULLIVAN, LLP

/s/

Robert B. Wilson
Anne S. Toker
James M. Glass
Anastasia M. Fernands
295 Fifth Avenue, 9th Floor
New York, NY 10016
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jimglass@quinnemanuel.com
anastasiafernands@quinnemanuel.com

Attorneys for Appellant

FISH & RICHARDSON P.C.

/s/

Susan E. Morrison
Casey M. Kraning
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Wilmington, DE 19801
(302) 652-5070
morrison@fr.com
kraning@fr.com

Attorneys for Appellee REGENXBIO Inc.

MORGAN, LEWIS & BOCKIUS LLP

/s/

_____
Amy M. Dudash
1201 North Market Street, Suite 2201
Wilmington, DE 19801
(302) 574-3000
amy.dudash@morganlewis.com

Attorneys for Appellee Trustees of the University of Pennsylvania

EXHIBIT 31.1

CERTIFICATION

I, Michael Severino, M.D., 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.

November 5, 2026

/s/ MICHAEL SEVERINO, M.D.
Michael Severino, M.D.
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.

November 5, 2026

/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, Michael Severino, M.D., 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 September 30, 2026, 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.

November 5, 2026

/s/ MICHAEL SEVERINO, M.D.
Michael Severino, M.D.
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 September 30, 2026, 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.

November 5, 2026

/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.