Patients can’t wait for the next breakthrough in medical research.

So neither will we.

Second Quarter 2026 Financial Results

Wednesday, August 5, 2026

SAREPTA THERAPEUTICS

DILLON
Living with Duchenne muscular dystrophy

Forward-looking statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, forward-looking statements will be made during this presentation. Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Forward-looking statements may be accompanied by words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions. These forward-looking statements include, without limitation, statements relating to expectations of senior management; our earnings, financial projections, and future operations, including our net product revenue guidance for 2026 and expectations with respect to revenue in 2027; our pipeline and priorities; the potential for our siRNA programs to be best-in-class and differentiated; ELEVIDYS; the strength and durability of our PMO franchise; our belief that we are well-funded to meet our medium-term liabilities and 2027 Notes; our ongoing and planned clinical trials, including data to date; the potential impacts of our initiatives; and our expected plans and milestones in 2026 and 2027, including for ELEVIDYS, fully enrolling Cohort 8 of SRP-9001-103 by end of 2026, sharing 12-week data and our plan to meet with FDA in the first quarter of 2027, data readouts from FSHD and DM1 programs in the second half of 2026, and expected regulatory decisions related to the sNDAs for VYONDYS and AMONDYS.

Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; we may not be able to comply with all FDA requests in a timely manner or at all; success in clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials are positive, these data may not be sufficient to support approval; we may not be able to reach alignment with FDA with respect to any next steps for our products and product candidates; our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our business; we may not be able to meet expectations with respect to sales of our products or maintain profitability; we rely on third parties, including in some cases our strategic partners, to conduct some aspects of our early stage research and development, and 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 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; we may experience delays in treating patients at infusion sites; the estimates and judgments the Company makes, or the assumptions on which it relies, in preparing its financial statements could prove inaccurate; failure to retain our key personnel or an inability to attract and retain additional qualified personnel could present a challenge to our business objectives; our existing and any future indebtedness could adversely affect our ability to operate our business; our revenues and operating results could fluctuate significantly, which may adversely affect our stock price and our ability to maintain profitability; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; we may incur substantial costs in connection with litigation and other disputes; and those risks identified under the heading "Risk Factors" in our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company, which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained herein. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.

Non-GAAP Financial Measures

This presentation includes both GAAP information and Non-GAAP information. Non-GAAP net income (loss) is defined as GAAP net (loss) income excluding interest expense/income, net, depreciation and amortization expense, stock-based compensation expense, loss (gain) on strategic investments, impairment of strategic investment, and litigation contingency charge as well as the estimated income tax impact of each pre-tax non-GAAP adjustment. Non-GAAP earnings per share is defined as non-GAAP net income divided by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding, adjusted for the inclusion of additional shares under both the treasury stock method and the "if-converted" method, if applicable and not anti-dilutive. Non-GAAP net loss per share is defined as non-GAAP net loss divided by the weighted-average number of shares of common stock as the inclusion of dilutive common stock equivalents outstanding is anti-dilutive. Non-GAAP operating income (loss) is defined as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and litigation contingency charge. Non-GAAP research and development expenses are defined as GAAP research and development expenses excluding depreciation and amortization expense and stock-based compensation expense. Non-GAAP selling, general and administrative expenses are defined as GAAP selling, general and administrative expenses excluding depreciation expense and stock-based compensation expense.

Sarepta regularly uses both GAAP and Non-GAAP results and expectations to assess its financial operating performance and cash requirement internally. Because Non-GAAP net income (loss), Non-GAAP earnings (loss) per share, Non-GAAP operating income (loss), Non-GAAP research and development expense and Non-GAAP selling, general and administrative expense are important internal measurements for Sarepta, the Company believes that providing this information in conjunction with Sarepta's GAAP information enhances investors' and analysts' ability to meaningfully compare the Company's results from period to period and to its forward-looking guidance, and to identify operating trends in the Company's principal business. Sarepta also uses Non-GAAP net income (loss) internally to understand, manage and evaluate its business and to make operating decisions.

Non-GAAP net income (loss) and its components are not meant to be considered in isolation or as a substitute for, or superior to, comparable GAAP measures and should be read in conjunction with the consolidated financial information prepared in accordance with GAAP. Investors should note that the Non-GAAP information is not prepared under any comprehensive set of accounting rules or principles and does not reflect all of the amounts associated with the Company's results of operations as determined in accordance with U.S. GAAP. Investors should also note that these Non-GAAP financial measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future, there may be other items that the Company may exclude for purposes of its Non-GAAP financial measures; likewise, the Company may in the future cease to exclude items that it has historically excluded for purposes of its Non-GAAP financial measures. Because of the non-standardized definitions, the Non-GAAP financial measures as used by Sarepta in this presentation may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by other companies.

The Company provides forward-looking statements in the form of guidance during its quarterly earnings conference calls. This guidance is provided on a Non-GAAP basis and cannot be reconciled to the closest GAAP measures without unreasonable effort because of the unpredictability of the amounts and timing of events affecting the items the Company excludes from Non-GAAP measures. For example, stock-based compensation is unpredictable for the Company's performance-based awards, which can fluctuate significantly based on current expectations of future achievement of performance-based targets. In addition, from time to time, the Company excludes certain items that occur infrequently, which are also inherently difficult to predict and estimate. As such, the costs that are being excluded from Non-GAAP guidance are difficult to predict and a reconciliation or a range of results could lead to disclosure that would be imprecise or potentially misleading.

Contents

CEO Transition Positions Sarepta for Continued Execution and Next Phase of Growth

Michael Severino, M.D.
Chief Executive Officer,
Sarepta Therapeutics

- ✓ **Proven Biopharma Leader with Broad Execution Experience**
- ✓ **Strong Strategic Fit with Sarepta's Precision Medicine Mission**
- ✓ **Structured Transition with Continuity and Momentum**

Q2 2026 and Recent Highlights

Corporate Highlights

- **New CEO Appointed:** Michael Severino, M.D. brings deep biopharma leadership, R&D expertise, and franchise-building experience
- **>1,500 patients*** treated with ELEVIDYS in commercial settings and clinical studies
- **Narrowed FY 2026 guidance**

**Total ambulatory and non-ambulatory patients treated as of July 31, 2026, including >1,300 ambulatory patients*

R&D Updates

- **Regulatory progress for PMOs:** sNDAs for AMONDYS 45 and VYONDYS 53 accepted by the FDA for review with PDUFA Feb. 28, 2027
- **ENDEAVOR Cohort 8:** Full enrollment expected by year-end 2026, with 12-week data expected in Q1 2027
- **DM1 and FSHD:** Phase 1/2 MAD cohorts with additional biomarker, safety and early functional data remain on track for readout in 2H 2026

Financial Results

- **Profitable and cash-generating:** Delivered another quarter of GAAP and Non-GAAP operating profit and generated $197M increase in cash and investments**, reflecting the strength of the base business
- **Net product revenues of $329M**
- **Disciplined execution:** Maintained a lean cost structure with Non-GAAP R&D and SG&A expenses of $165M, enabling continued investment in the next phase of growth opportunities

***Includes cash, cash equivalents, restricted cash and investments*

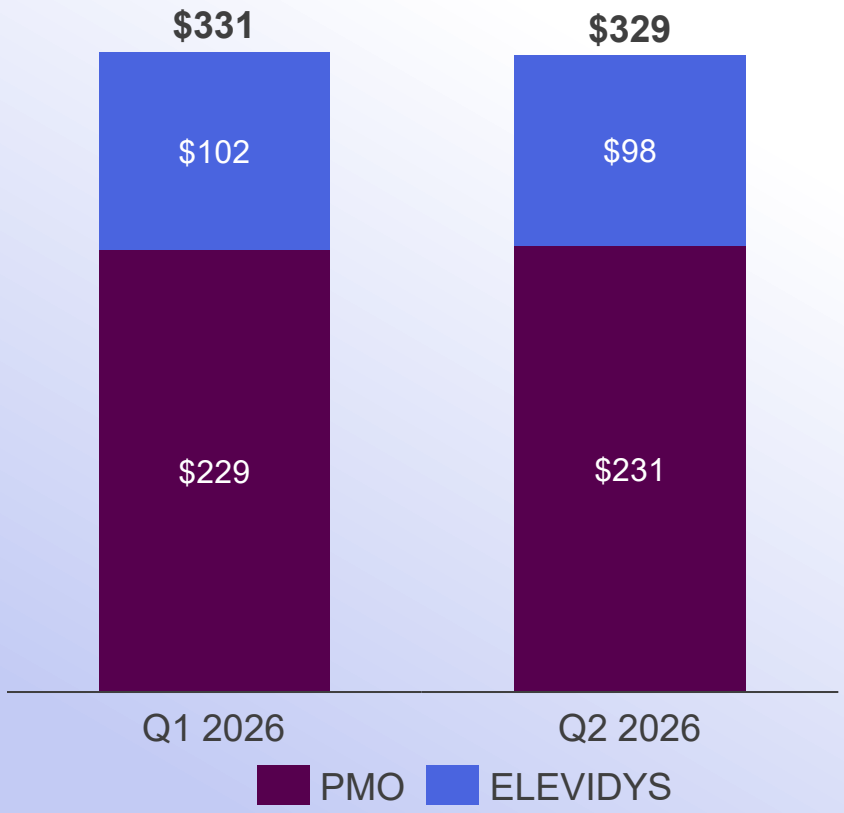
Commercial Performance

Patrick Moss, PharmD
Executive Vice President, Chief Commercial Officer

SAREPTA THERAPEUTICS

Commercial Initiatives Taking Hold as PMO Franchise Remains Durable

$ in Millions



Note: Charts may not foot due to rounding

Q2 2026 Performance

- **ELEVIDYS:** Sales remained relatively steady, with quarter-over-quarter growth in enrollment forms signaling increasing demand
- **PMOs:** Stable demand supported by sustained physician confidence, extensive real-world experience, and a well-established safety profile

ELEVIDYS Initiatives Are Taking Hold

- Record healthcare provider engagement in Q2
- Majority of Q2 enrollment forms linked to providers engaged within prior 90 days
- Growth in new and returning sites submitted enrollment forms versus Q1

Guidance & Outlook

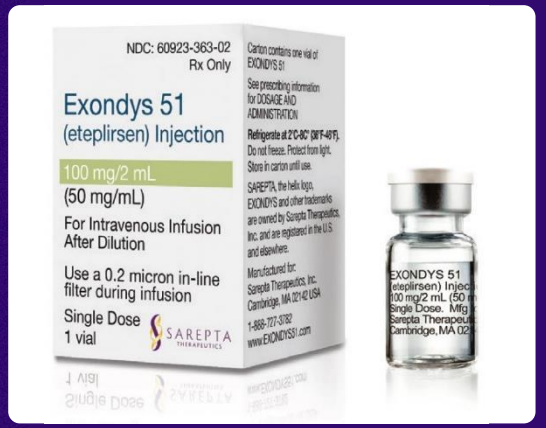
- **2026 Product net revenue guidance narrowed to $1.2B - $1.3B**, consistent with prior expectation to trend toward the lower end of the original range
- Recent ELEVIDYS enrollment gains are expected to contribute primarily to 2027 revenue given the ~6-month treatment journey from enrollment to infusion
- 2H26 Expected modestly below 1H26 and Q3 ELEVIDYS to trend lower than Q2
- Revenue expected to have quarter-to-quarter variability in the near-term; long-term opportunity intact

Exon-skipping franchise sets precedent in Duchenne

Sarepta's exon skippers have an established safety profile and have successfully treated >1,800 patients worldwide across many age groups

>1,800
patients worldwide ranging from infants to adults in their 30's and over

10+
years of clinical and commercial experience



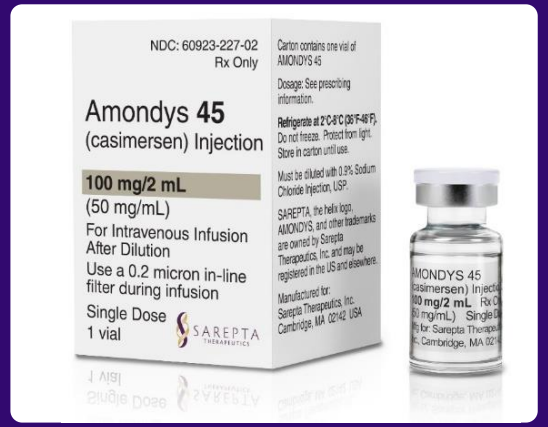
Established safety profile[1-4]

7.5 year
Delay in need for nighttime ventilation and significantly slower pulmonary decline[5]

Slowing disease progression[6-9]

- Delayed loss of ambulation
- Slowed pulmonary and cardiac decline
- Prolonged survival

Adherence rates exceeding
90%
underscoring clinical value[4]



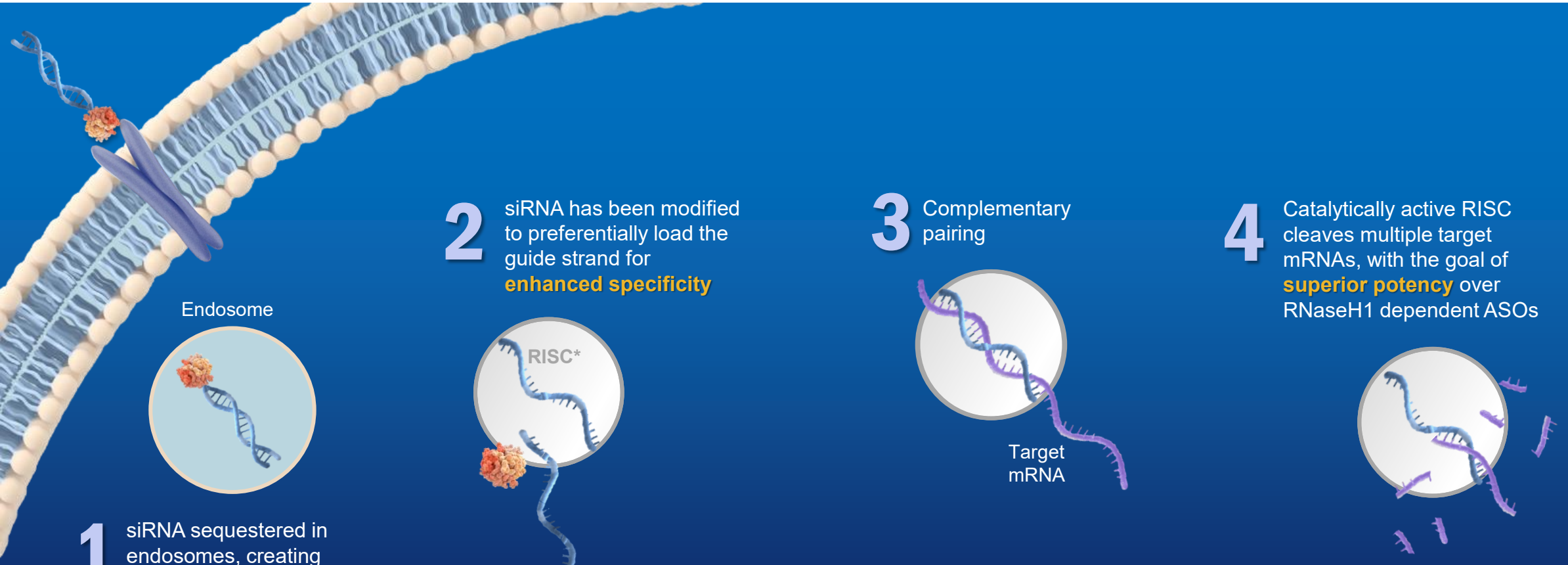
1. EXONDYS 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc. 2025.
2. VYONDYS 53 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc. 2024
3. AMONDYS 45 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc. 2026)
4. Tian C, et al. Presented at: Neuromuscular Study Group Annual Scientific Meeting; September 20-22, 2024; Tarrytown, NY.
5. https://www.mdaconference.org/abstract-library/delayed-pulmonary-progression-in-golodirsen-treated-patients-with-duchenne-muscular-dystrophy-vs-mutation-matched-external-controls/
6. Mathews K, et al. Presented at: Muscular Dystrophy Association Clinical and Scientific Conference; March 16-19, 2025; Dallas, TX;
7. Iff J, et al. Presented at: International Congress of the World Muscle Society; September 20-24, 2021; Virtual.
8. Iff J, et al. J Neuromuscul Dis. 2025:22143602251366721; Mitelman O, et al. J Neuromuscul Dis. 2022;9(1):39-52; Khan N, et al. J Neuromuscul Dis. 2019;6(2):213-225.
9. Iff J, et al. Muscle Nerve. 2024;70(1):60-70.

R&D Updates

Louise Rodino-Klapac, PhD
President, R&D and Technical Operations

SAREPTA THERAPEUTICS

A Biology-First Approach: siRNA-mediated mRNA Degradation Designed to Enable Specific Target Knockdown



1 siRNA sequestered in endosomes, creating drug depot that allows for **sustained durability**

2 siRNA has been modified to preferentially load the guide strand for **enhanced specificity**

Passenger strand is removed

3 Complementary pairing

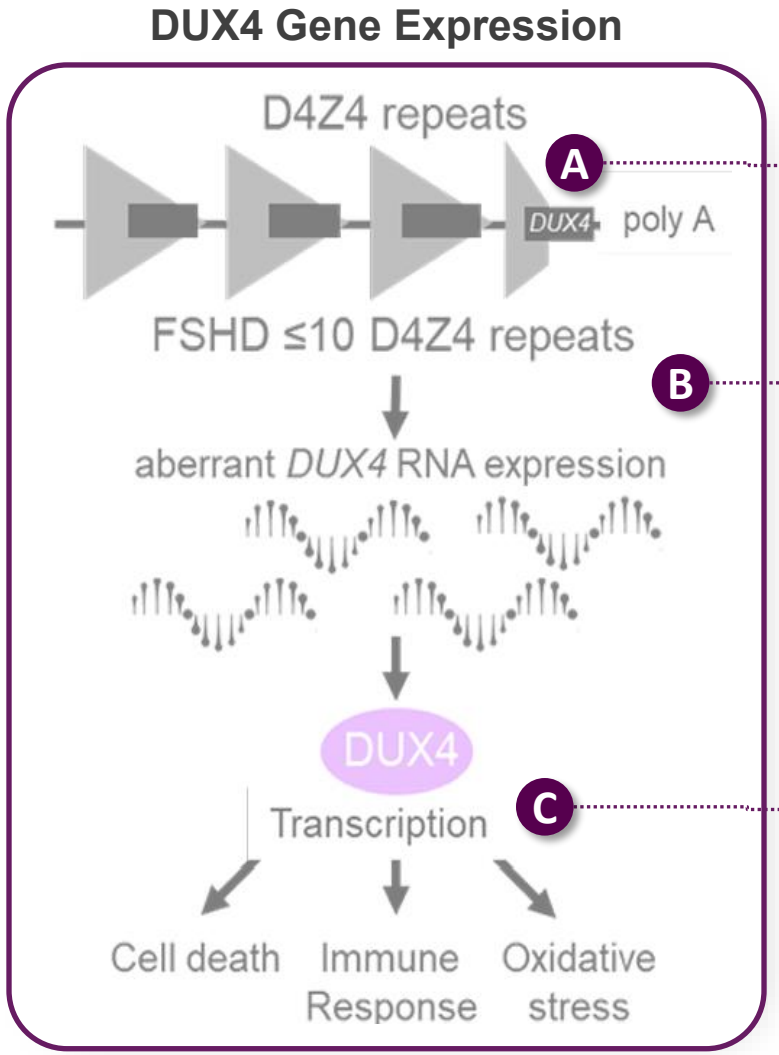
4 Catalytically active RISC cleaves multiple target mRNAs, with the goal of **superior potency** over RNaseH1 dependent ASOs

*RNA-induced Silencing Complex (RISC)

Early Clinical Data Strengthen Potential Best-in-Class Approach for SRP-1001 and SRP-1003 to Treat FSHD1 and DM1

- **Maximizing Therapeutic Delivery and Effect to Muscle**
  - Clinical experience to date matches preclinical data: The αvβ6 integrin-targeting ligand drives potentially greater construct muscle delivery than other approaches
  - No saturation of muscle siRNA uptake observed to date, with consistent dose-dependent increases in plasma and muscle drug exposures across clinical and nonclinical studies
  - Enhanced siRNA chemistry improves drug stability, potentially enabling less frequent and optimized clinical dosing regimen
- **Reaching our Target to Impact Disease**
  - Successful target engagement with emerging biomarker evidence of potentially meaningful treatment efficacy
- **No Indication of Dose-related Safety Signals that would Limit Continued Dose Escalation**
  - Favorable safety and tolerability profile to date

FSHD is Caused by the Contraction of the D4Z4 Repeat, Leading to the Abnormal Expression of DUX4, which is Typically Silenced Early in Life



DUX4 Transcription Factor

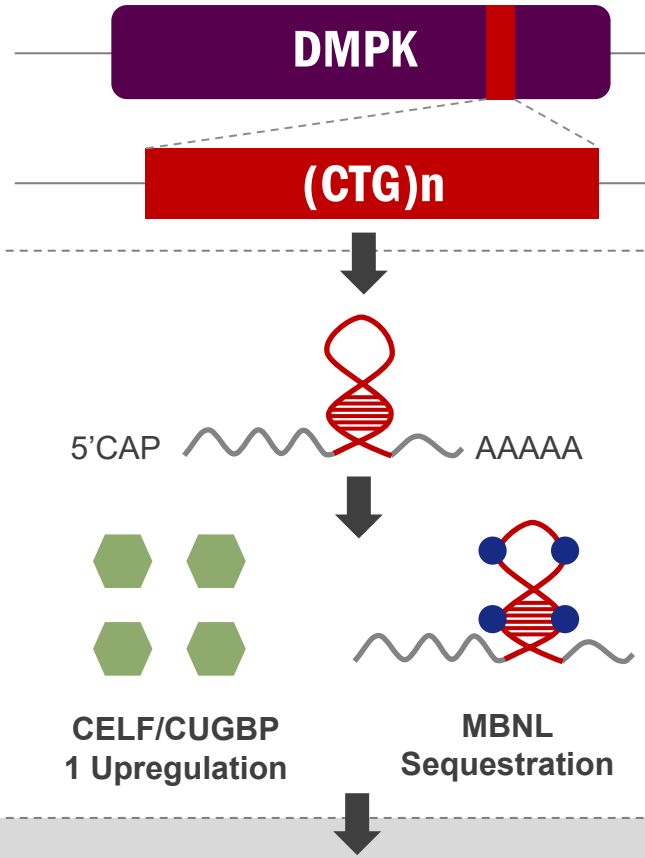
FSHD genotype leads to aberrant DUX4 expression

Downstream Cellular Consequences

DM1: Expanded DMPK Repeat, Leading to Multi-System Impact

Targeting and knockdown of DMPK leads to therapeutic benefit

DM1 Genetic Cause



DNA

Expanded tri-nucleotide repeat in the *DMPK* gene (50 – 1000 repeats)

RNA

Repeats accumulate in the nucleus and create 3D structures that bind MBNL and alter CUGBP1 expression

Downstream mis-splicing of proteins implicated in cognitive, skeletal, and cardiac muscle function

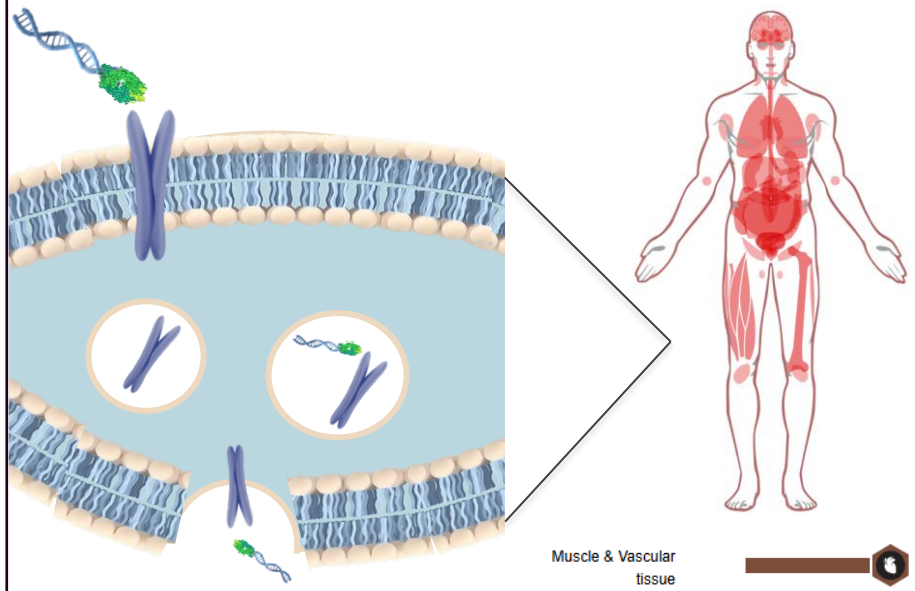
Driving Delivery Efficiency: Key Objectives

- Long-term exposure in muscle
- Superior muscle concentration
- Successful target engagement and knockdown

Targeting $\alpha_v\beta_6$ Integrin over TfR1 for Greater Muscle Uptake

High expression and surface availability of $\alpha_v\beta_6$ in muscle make it a preferred receptor for targeted siRNA delivery

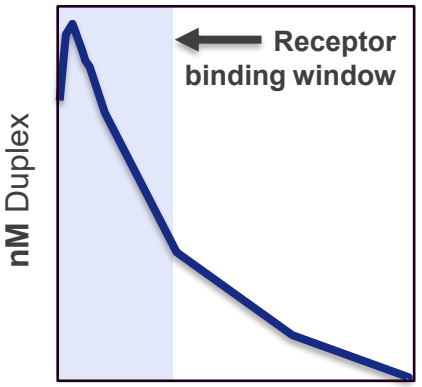
TfR1: 5%[1] of receptors available for binding on surface at any one time; majority are intracellular trafficking transferrin or being recycled to membrane



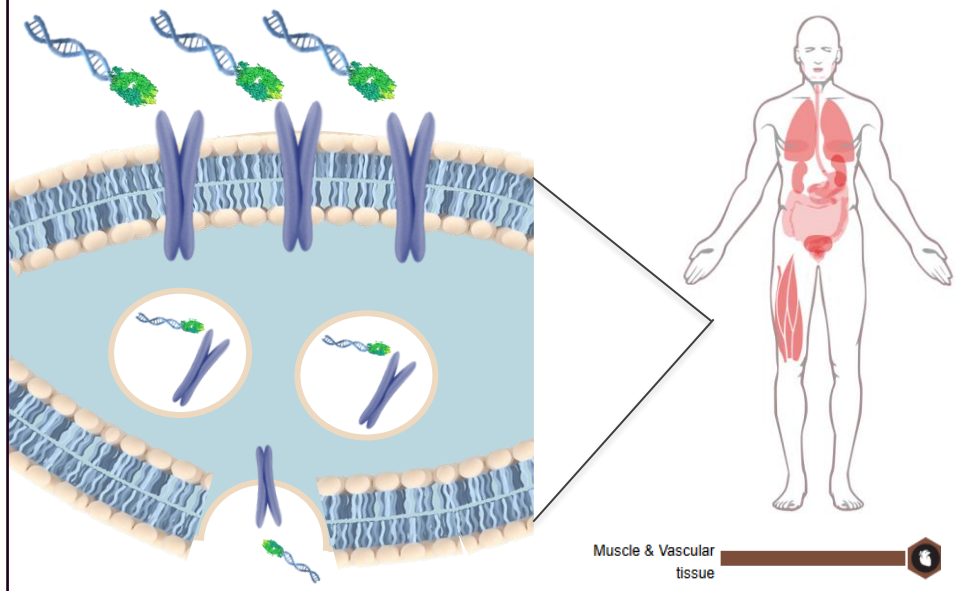
TFRC Protein Atlas

IV Delivery

Receptor recycling times range from 30 minutes to a few hours[1,2], creating short window for receptor-mediated endocytosis



$\alpha_v\beta_6$: ~40%[3] of receptors available for binding on surface at any one time; higher muscle and vascular expression than TfR1



ITGB6 Protein Atlas

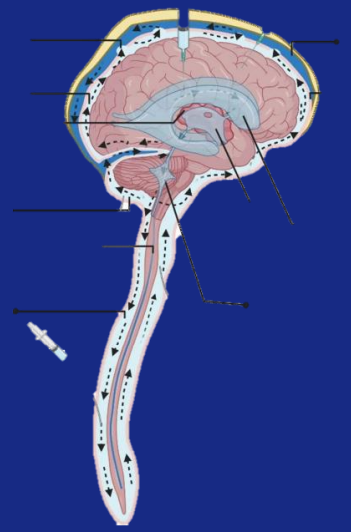
[1]https://doi.org/10.3390/ph14060535
[2]https://doi.org/10.3389/fcell.2022.920303
[3]http://dx.doi.org/10.1159/000443180

TfR1-Binding Designed to Optimize CNS Delivery in Huntington's Disease

CNS-SC platform demonstrates improved delivery to deep brain in NHP compared to direct CSF injection

SRP-1005 HD program employs a subcutaneous route of administration delivering siRNA across the BBB to the source of disease in the deep brain.

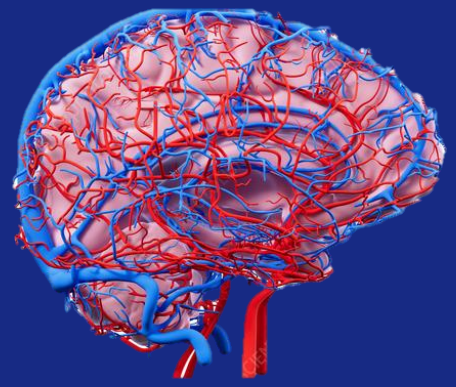
CSF delivery via IT injection



Accessing the brain via intrathecal injection (IT) is limited by CSF flow

J. Pers. Med. 2022, 12(12), 1979; https://doi.org/10.3390/jpm12121979

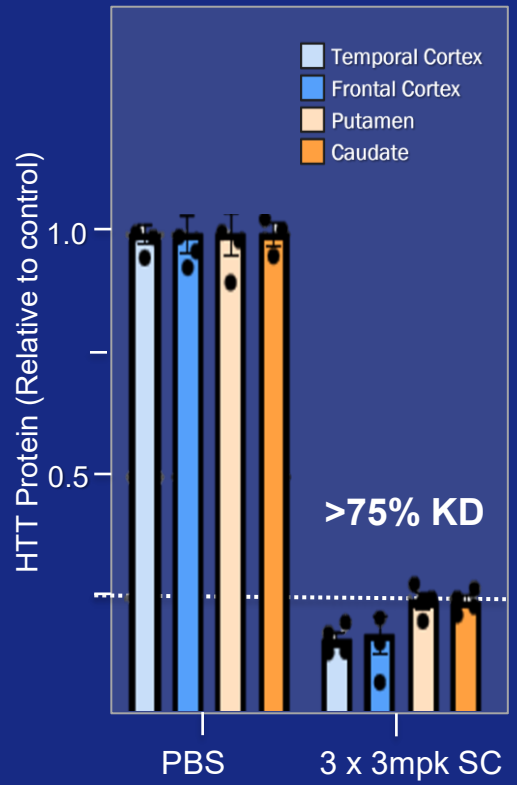
Systemic delivery via SC injection



Accessing the brain via the blood-brain barrier through TfR1-mediated crossing potentially leads to greater deep brain distribution

Thom Leach / Science Photo Library

SRP-1005 in NHP



Growing Body of Clinical Data Support ELEVIDYS, the Only Gene Therapy Approved to Treat Duchenne

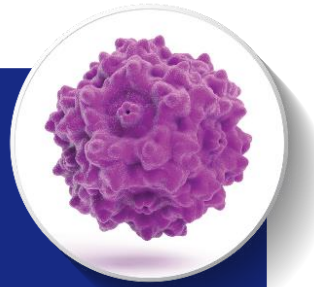
- Interim safety data from the Phase 4 ENDURE study to be presented at the Neuromuscular Study Group Meeting in September 2026 showing zero incidence of ALI in patients treated prophylactically with sirolimus
- Cohort 8, ENDEAVOR (Study SRP-9001-103): Enrollment completion expected by end of 2026; 12-week data from full cohort and engagement with FDA expected in Q1 ’27
- Robust presence at the World Muscle Society (WMS) Meeting 2026 expected: Supportive expression and safety data in ELEVIDYS-treated patients under 4, encore presentations of EMBARK 3-year outcomes, cardiac function data, pooled safety, and early-intervention preclinical data

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

Regulatory Progress: AMONDYS 45 and VYONDYS 53

- FDA accepted filing of supplemental New Drug Applications (sNDAs) for AMONDYS 45 and VYONDYS 53
- sNDA submissions seek conversion of the accelerated approvals of AMONDYS 45 and VYONDYS 53 to traditional approvals
- Target action date – February 28, 2027

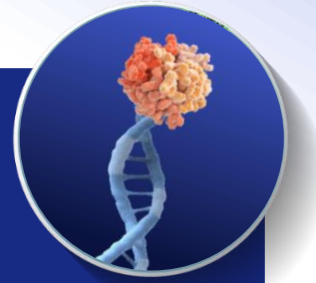
Key Upcoming Milestones



GENE THERAPY

ELEVIDYS
Duchenne
ENDEAVOR Cohort 8 enrollment complete – 2H 2026, and 12-wk data from full cohort in Q1 2027

SRP-9003
LGMD2E/R4
Review sirolimus data with FDA and align on path forward for BLA



siRNA

SRP-1001
FSHD1
MAD study data, incl. safety, PK, DUX4-regulated gene panel, circulating DUX4-related biomarkers, CK and preliminary functional assessments – 2H 2026

SRP-1005
Huntington's disease
Proof-of-biology data – 1H 2027

SRP-1003
DM1
MAD study data, incl. safety, serum & muscle PK, DMPK knock-down, CASI-22 splicing index and vHOT analyses – 2H 2026

Financial Results

Ryan Wong
Executive Vice President, Chief Financial Officer

SAREPTA THERAPEUTICS

Financial Highlights

Q2 2026 Financial Results

Product Revenue
$329 million

Total Revenues
$401 million

Operating Income GAAP / Non-GAAP[1]
$13 million / $86 million

Cash and Investments[2]
$945 million

Financial strength enables investing to maintain DMD leadership and future growth across neuromuscular and CNS diseases

- **Consistent Profitability:** Base business delivered another quarter of GAAP and Non-GAAP operating profit, supported by durable revenue performance and disciplined execution
- **Significant Cash Generation:** Generated approximately $197M of cash during Q2 2026 driven by strong execution and $40M receipt for commercial sale milestone from Roche
- **Disciplined Expense Management:** Maintained a lean cost structure with combined Non-GAAP R&D and SG&A expenses of $165M
- **Funding Future Growth**: Established commercial business generates meaningful revenue and cash flow to fund promising pipeline; medium-term liabilities are well-covered

Footnotes

1. *Non-GAAP operating income is defined by us as GAAP operating income excluding depreciation and amortization expense, stock-based compensation expense and litigation contingency charge. For reconciliation of this Non-GAAP financial measure to comparable GAAP measures, as well as additional information regarding our use of non-GAAP financial measures, please refer to the Appendix to this presentation and to our press release dated August 5, 2026, which is accessible in the Investors section of our website at www.sarepta.com.*
2. *Includes cash, cash equivalents, restricted cash and investments*

Q2 2026 Select Financial Data

$ In Millions, except percentages	Q2 2026	Q2 2025	YoY %	YTD Q2 2026	YTD Q2 2025	YoY %
Total Product Revenues	$329	$513	-36%	$659	$1,125	-41%
Collaboration and Other Revenues	$73	$98		$473	$231	
Total Revenues	$401	$611	-34%	$1,132	$1,356	-17%
Cost of Sales (excludes amortization of in-licensed rights)	$149	$153	-2%	$258	$290	-11%
Combined **GAAP** R&D and SG&A Expenses	$199	$342	-42%	$462	$1,249	-63%
Combined **Non-GAAP** R&D and SG&A Expenses[1]	$165	$295	-44%	$388	$1,151	-66%
GAAP Operating Income / (Loss)	$13	$116		$372	($185)	
Non-GAAP Operating Income / (Loss)[1]	$86	$163		$484	($87)	

YTD Q2 2025 GAAP and Non-GAAP R&D Expenses include Arrowhead collaboration transaction costs[2] of $584M

Q2 and YTD 2026 GAAP Operating Income includes impact of $39M litigation contingency charge

Note: Table may not foot due to rounding

Footnotes

1. *Non-GAAP research and development expenses are defined by us as GAAP research and development expenses excluding depreciation and amortization expense and stock-based compensation expense. Non-GAAP selling, general and administrative expenses are defined by us as GAAP selling, general and administrative expenses excluding depreciation expense and stock-based compensation expense. Non-GAAP operating income (loss) is defined by us as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and litigation contingency charge. For reconciliation of this Non-GAAP financial measure to comparable GAAP measures, as well as additional information regarding our use of non-GAAP financial measures, please refer to the Appendix to this presentation and to our press release dated August 5, 2026, which is accessible in the Investors section of our website at www.sarepta.com.*
2. *$584M R&D expense related to Arrowhead collaboration transaction costs ($500M upfront license fee and $83.6M premium related to equity investment)*

FY 2026 Guidance

	FY 2026 Guidance As of August 5, 2026	Previous Guidance	Assumptions
Total Net Product Revenues	$1,200-1,300M *Midpoint is an appropriate reference*	$1,200-1,400M *Anchored to lower end*	• At guidance midpoint, 2H26 revenue modestly lower than 1H26 • Quarter to quarterly variability due to one-time infusion gene therapy model
Total Collaboration, License, Contract Manufacturing and Royalty Revenues	$550-600M	$450-550M	• Increase of $75M from midpoint driven primarily by increase in contract manufacturing revenues • 2H 2026 and forward to consist primarily of contract manufacturing and royalty revenues
Combined GAAP R&D and SG&A Expenses	$940-1,010M	$925-1,075M	• Includes $140-160M of stock-based compensation and depreciation and amortization expenses
Combined Non-GAAP R&D and SG&A Expenses[1]	$800-850M	$800-900M	• Includes $50M annual collaboration license fee to Arrowhead

Footnotes

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Q&A

SAREPTA THERAPEUTICS

Appendix

Condensed Consolidated Statements of (Loss) Income

$ in Thousands, except for per share amounts
Note: Tables may not foot due to rounding

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
Revenues:				
Products, net	$ 328,689	$ 513,123	$ 659,204	$ 1,124,646
Collaboration and other	72,562	97,968	472,850	231,301
Total revenues	401,251	611,091	1,132,054	1,355,947
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	149,385	152,558	258,153	290,122
Research and development	91,258	204,392	245,218	977,840
Selling, general and administrative	107,600	137,897	216,551	271,526
Litigation contingency charge	39,000	—	39,000	—
Amortization of in-licensed rights	717	667	1,408	1,268
Total cost and expenses	387,960	495,514	760,330	1,540,756
Operating income (loss)	13,291	115,577	371,724	(184,809)
Other (loss) income, net:				
Other (expense) income, net	(15,040)	38,061	(30,299)	(45,071)
(Loss) income before income tax expense	(1,749)	153,638	341,425	(229,880)
Income tax expense (benefit)	3,141	(43,254)	15,356	20,736
Net (loss) income	$ (4,890)	$ 196,892	$ 326,069	$ (250,616)
(Loss) earnings per share:				
Basic	$ (0.05)	$ 2.01	$ 3.10	$ (2.57)
Diluted	$ (0.05)	$ 1.89	$ 2.99	$ (2.57)
Weighted average number of shares of common stock used in computing (loss) earnings per share:				
Basic	105,431	98,005	105,211	97,685
Diluted	105,431	106,623	122,260	97,685

Reconciliation of GAAP Reported Net (Loss) Income to Non-GAAP Net Income (Loss)

$ in Thousands, except for per share amounts
Note: Tables may not foot due to rounding

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
GAAP net (loss) income	$ (4,890)	$ 196,892	$ 326,069	$ (250,616)
Interest expense (income), net	13,341	(1,921)	26,293	(9,846)
Depreciation and amortization expense	9,692	10,173	19,595	19,550
Stock-based compensation expense	24,472	37,025	53,871	78,453
Loss (gain) on strategic investments	448	(36,721)	2,160	54,007
Impairment of strategic investment	1,000	—	1,000	—
Litigation contingency charge	39,000	—	39,000	—
Income tax effect of adjustments	(4,477)	9,728	(4,023)	(8,870)
Non-GAAP net income (loss)	$ 78,586	$ 215,176	$ 463,965	$ (117,322)
GAAP (loss) earnings per share - diluted:	$ (0.05)	$ 1.89	$ 2.99	$ (2.57)
Add: impact of GAAP to Non-GAAP adjustments	0.69	0.13	0.80	1.37
Non-GAAP earnings (loss) per share - diluted[1]	$ 0.64	$ 2.02	$ 3.79	$ (1.20)
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:				
GAAP	105,431	106,623	122,260	97,685
Non-GAAP	122,648	106,623	122,284	97,685

[1]GAAP and non-GAAP earnings per share is calculated using diluted shares whereas GAAP and non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive.

Reconciliation of GAAP to Non-GAAP Reported Operating Income, SG&A and R&D Expenses, and Total Effective Tax Rate

$ in Thousands, except for per share amounts
Note: Tables may not foot due to rounding

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
GAAP research and development expenses	$ 91,258	$ 204,392	$ 245,218	$ 977,840
Stock-based compensation expense	(8,509)	(15,277)	(18,786)	(32,594)
Depreciation and amortization expense	(6,023)	(7,397)	(12,229)	(14,374)
Non-GAAP research and development expenses	$ 76,726	$ 181,718	$ 214,203	$ 930,872

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
GAAP selling, general and administrative expenses	$ 107,600	$ 137,897	$ 216,551	$ 271,526
Stock-based compensation expense	(15,963)	(21,748)	(35,085)	(45,859)
Depreciation expense	(3,669)	(2,776)	(7,366)	(5,176)
Non-GAAP selling, general and administrative expenses	$ 87,968	$ 113,373	$ 174,100	$ 220,491

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
GAAP operating income (loss)	$ 13,291	$ 115,577	$ 371,724	$ (184,809)
Stock-based compensation expense	24,472	37,025	53,871	78,453
Depreciation and amortization expense	9,691	10,173	19,594	19,550
Litigation contingency charge	39,000	—	39,000	—
Non-GAAP operating income (loss)	$ 86,454	$ 162,775	$ 484,189	$ (86,806)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
Total effective tax rate, GAAP	(179.6) %	(28.2) %	4.5 %	(9.0) %
Less: impact of GAAP to Non-GAAP adjustments	188.4	(4.4)	(0.5)	(25.0)
Total effective tax rate, Non-GAAP	8.8 %	(32.6) %	4.0 %	(34.0) %

PMO Revenue Breakdown by Product

$ in Thousands	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
	(in thousands)			
Exondys 51	$ 119,826	$ 126,390	$ 243,252	$ 263,811
Vyondys 53	33,996	33,478	61,787	61,463
Amondys 45	76,742	71,404	154,075	142,536
Total PMO Product Revenues	$ 230,564	$ 231,272	$ 459,114	$ 467,810

Total Revenue and Cost of Sales Breakdown

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2026	2025	2026	2025
	(in thousands)			
Revenues:				
Total product revenues, net	$ 328,689	$ 513,123	$ 659,204	$ 1,124,646
Contract manufacturing revenue	54,399	27,023	85,504	44,402
License revenue	10,000	—	10,000	—
Royalty revenue	8,163	7,445	12,346	11,399
Collaboration revenue	—	63,500	365,000	175,500
Total collaboration and other revenues	72,562	97,968	472,850	231,301
Total revenues	$ 401,251	$ 611,091	$ 1,132,054	$ 1,355,947
Cost of sales (excluding amortization of in-licensed rights):				
Product cost of sales (excluding Roche)	$ 80,982	$ 116,743	$ 141,878	$ 226,501
Roche product cost of sales	58,203	21,818	97,856	33,961
Royalty payments	10,200	13,997	18,419	29,660
Cost of sales (excluding amortization of in-licensed rights)	$ 149,385	$ 152,558	$ 258,153	$ 290,122
Net product revenues gross profit ($)[1]	$ 247,707	$ 396,380	$ 517,326	$ 898,145
Net product revenues gross margin (%)[1]	75%	77%	78%	80%

[1] Net product revenues gross profit is calculated as total product revenues, net, less product cost of sales (excluding Roche). Net product revenues gross margin is calculated as net product revenues gross profit divided by total product revenues, net.