

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

So neither will we.

First Quarter 2025 Financial Results
Tuesday, May 6, 2025



MÉLANIE
Living with limb-girdle
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 conference call. Any statements made by Sarepta that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements relating to our future operations, financial performance, guidance and projections for 2025 and beyond, including our revised 2025 product revenue guidance; the potential for our action plan to help us achieve our ELEVIDYS financial guidance, including our expectation that we will be able to shorten lead times, optimize site capacity, deliver educational initiatives and launch promotional efforts; potential net cannibalization; business plans, market opportunities, priorities, research and development programs, and the potential benefits of our product candidates; the durability of ELEVIDYS and its potential to transform the trajectory of Duchenne; and our expected plans and milestones, including submitting a BLA in the second half of 2025 for SRP-9003, the timing and potential of any LGMD approvals, EMERGENCE expression data mid-year, initiating a phase 3 trial for SRP-9004 by end of year, preliminary results from SRP-1001 and SRP-1003, phase 1 safety data from SRP-1004, first-in-human study initiation for SRP-9010 and SRP-1005, and holding an investor R&D day in the second half of 2025.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. 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: we may not be able to meet expectations with respect to sales of our products or attain or maintain the anticipated net revenues, profitability or positive cash-flow from operations; our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects; we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations; because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate or forecast product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; our data for our programs, including those with strategic partners, may not be sufficient for obtaining regulatory approval; success in preclinical and 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 may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; we may experience delays in treating patients at infusion sites; we may observe adverse reactions in our clinical trials or in patients who receive our approved products; the commencement and completion of our clinical trials, including those in connection with our strategic partners, and announcement of results may be delayed or prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; 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 of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; if the actual number of patients living with the diseases we aim to treat is smaller than estimated, our revenue and ability to maintain profitability may be adversely affected; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, many of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; 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. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

Non-GAAP Financial Measures

This presentation includes both GAAP information and Non-GAAP information. Non-GAAP (loss) income is defined as GAAP net (loss) income excluding interest income, net, depreciation and amortization expense, stock-based compensation expense, loss (gain) on strategic investments, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items. Non-GAAP earnings per share is defined as non-GAAP net income, as defined previously, 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 the “if-converted” method, if applicable and not anti-dilutive. Non-GAAP net loss per share is defined as non-GAAP net loss, as defined above, 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 (loss) income is defined as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items.

Sarepta regularly uses both GAAP and Non-GAAP results and expectations to assess its financial operating performance and cash requirement internally. Because Non-GAAP (loss) income, Non-GAAP (loss) earnings per share, Non-GAAP operating (loss) income, 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 (loss) income internally to understand, manage and evaluate its business and to make operating decisions.

Non-GAAP (loss) income 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 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 measure 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. Amortization of intangible assets, acquisition-related costs and restructuring costs are all impacted by the timing and size of potential future actions, which are difficult to predict. 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.

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Opening Remarks

Doug Ingram

President & Chief Executive Officer



Q1 2025 and Recent Highlights

“Sarepta is well positioned, as we have a significant number of approved therapies and significant revenue, a strong P&L and balance sheet and the ability to continue to independently drive our portfolio of gene therapies and siRNA programs.”



- Revised FY 2025 Product Revenue Guidance to \$2.3B - \$2.6B
- ELEVIDYS fell short of expectations, despite growing 180% year-over-year and reporting another sequential quarter of organic patient growth
- PMOs remained steady contributor
- Excluding Arrowhead collaboration transaction closing costs¹, Company would have reported another sequential quarter of operating profit



- >800 Patients treated with ELEVIDYS in commercial settings and clinical studies
- U.S. FDA has confirmed that screening and dosing may proceed in Study SRP-9005-101 for LGMD2C/R5
- Enrollment and dosing completed in Study SRP-9004-102 for LGMD2D/R3
- On track for BLA submission for SRP-9003 for LGMD2E/R4 in 2H25



- Closed global licensing and collaboration agreement with ARWR and integration efforts underway for 4 clinical-stage, 3 preclinical-stage and up to 6 additional muscle/cardiac/CNS targets

Footnotes

1. \$584M R&D expense related to Arrowhead collaboration transaction costs (\$500M upfront license fee and \$83.6M premium related to equity investment)

ELEVIDYS Guidance Update Reflects Trends seen with Broad Label Population

2025 Total Product Revenue Guidance: \$2.3B - \$2.6B

Q1 2025 Factors driving ELEVIDYS performance

Severe flu season

Site specific administrative issues

Impact of recently reported safety event

Factors driving 2025 guidance update

Continued impact of recently reported safety event as broader physician community and families seek more information

Longer cadence from start form to infusion as seen with broader label population

Key Areas of Focus

Deliver comprehensive educational initiatives for treating clinicians and patient communities

Shorten turnaround times through engagement with broader site community

Increase support to treatment centers to improve overall site network productivity

Commercial Performance

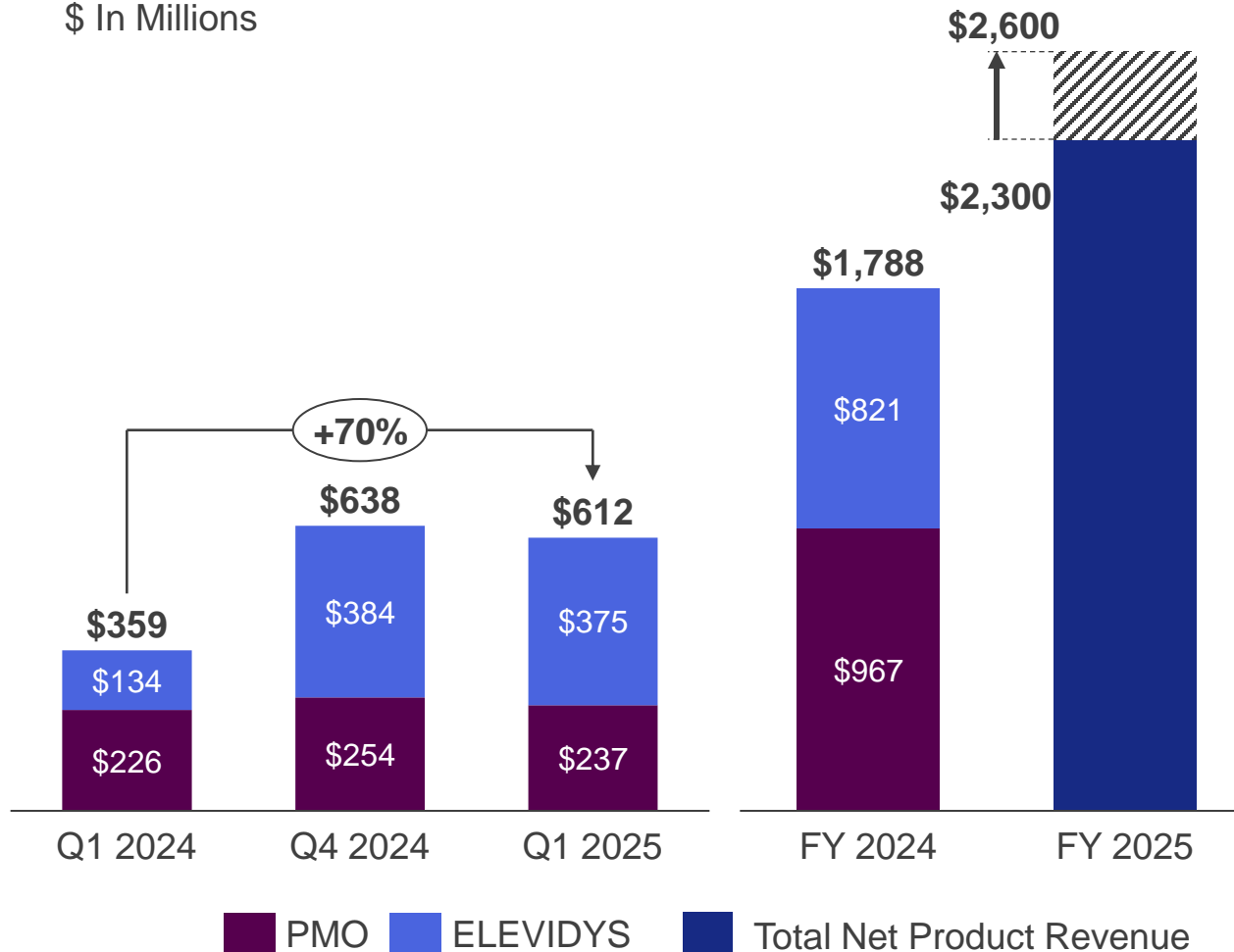
Dallan Murray

EVP, Chief Customer Officer



Commercial Update: Key Areas of Focus

\$ In Millions



Note: Graphs may not foot due to rounding

- **Education:** Disseminate biomarker safety data in conjunction with new 2yr EMBARK data across wider treating and referring physician landscape
- **Shorten turnaround times:** Work with individual sites to create more efficiencies and reduce delays
- **Optimize site capacity:** Proactively engage with treatment centers that have potential to increase patient volume to expand overall site network utilization
- **Promotional efforts:** Launch comprehensive HCP and patient promotional campaign including new ELEVIDYS.com website and digital DTC to drive awareness and understanding

R&D Highlights

Louise Rodino-Klapac, Ph.D.

EVP, Head of R&D, Chief Scientific Officer



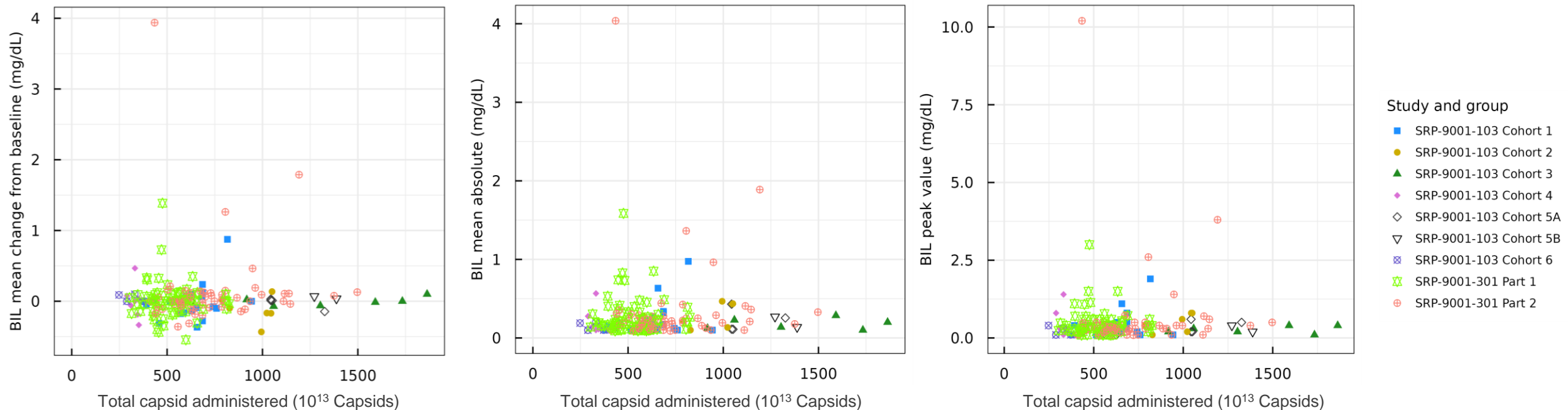
Q1 2025 R&D Highlights

- **Strengthening ELEVIDYS profile**
- **Advancing LGMD portfolio**
- **Building potential best-in-class siRNA therapies**

No Observed Correlation Between Liver Function and Age, Weight, Dose, and Total Viral Load

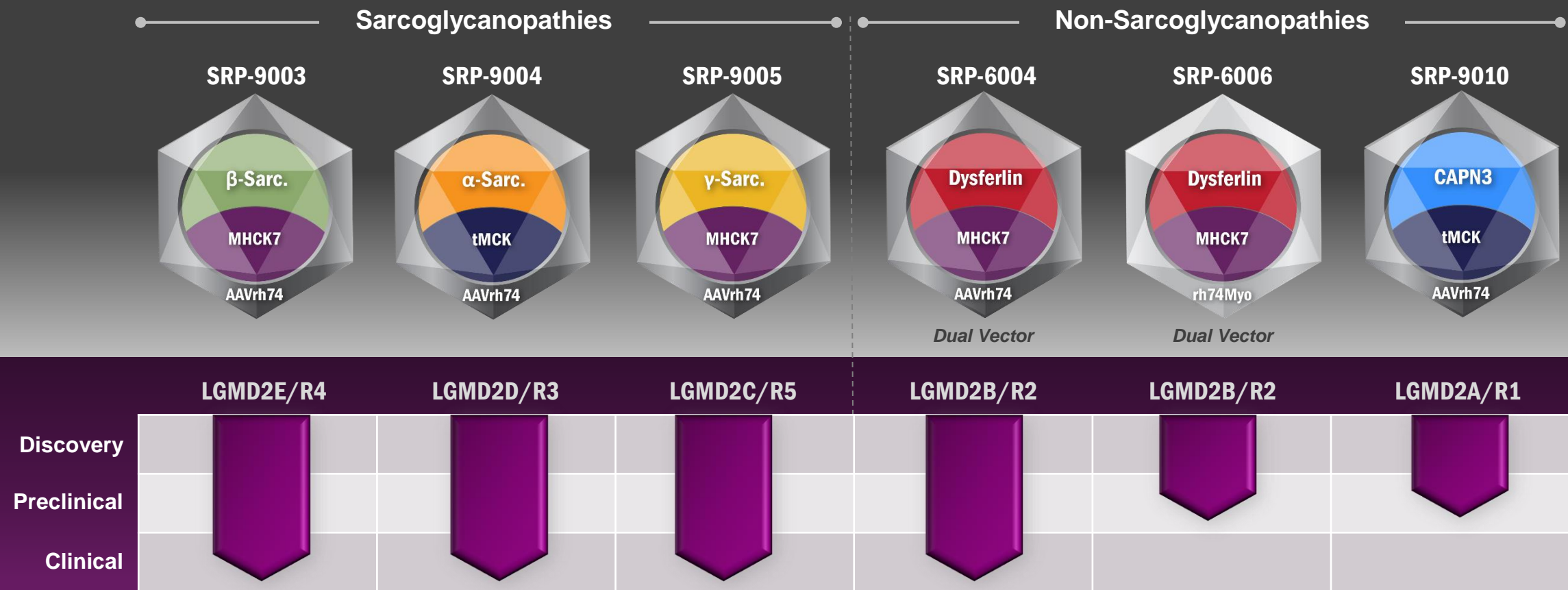
- No difference in the rates of adverse events relative to age or weight
- No relationships between liver safety biomarkers and total dose administered, patient age or patient weight
- Totality of data (over 800 patients) supports safety of ELEVIDYS weight-based dosing across the labelled population of Duchenne patients, regardless of ambulatory status

Bilirubin vs Total Capsid Administered



BIL=Bilirubin (mg/dL). BIL mean absolute: 178 of 178 subjects had observations for this combination of exposure and biomarker.

Market Leading Gene Therapy Portfolio in LGMD



Potential Best-in-Class siRNA Platform

siRNA Design

Effective and efficient target knock-down, improved durability, specificity, and reduction of off-target effects

Delivery

Superior tissue targeting

Dosing/Safety

Less frequent dosing; favorable safety profile



R&D Progress and a Look Ahead

MILESTONES ACHIEVED TO DATE (2025)

ELEVIDYS

- Positive EMBARK 2-year topline results
- Positive results from Part 2 of EMBARK study

SRP-9003, LGMD2E/R4

- EMERGE enrollment completed
- Positive pre-BLA meeting with FDA in February

SRP-9004-102, LGMD2D/R3

- Phase 1 study (DISCOVERY) dosing completed

SRP-9005, LGMD2C/R5

- Registrational Phase 1/3 study (COMPASS) cleared to proceed

SRP-1004 (formerly ARO-ATXN2), SCA2

- Phase 1 initiated

LOOKING AHEAD

ELEVIDYS

- ENVISION: Enrollment completion
- Study 104 (imlifidase) and Study 105 (plasmapheresis): Expression data
- ENDEAVOR: Cohort 6 expression data
- BLA supplement <4 years old

SRP-9003, LGMD2E/R4

- EMERGE: Expression data, mid-year
- BLA filing: 2H 2025
- VOYAGENE data: Expression, function, safety

SRP-9004, LGMD2D/R3

- Initiate Phase 3 before end of 2025

SRP-1001 (formerly ARO-DUX4), FSHD1

- Preliminary results from Phase 1

SRP-1003 (formerly ARO-DM1), DM1

- Preliminary results from Phase 1

SRP-1004 (formerly ARO-ATXN2), SCA2

- Phase 1 safety data

First-in-human study initiation expected in 2025

- SRP-9010, LGMD2A/R1
- SRP-1005, Huntington's Disease

Investor R&D Day – 2H 2025

Financial Results

Ian Estepan

EVP, Chief Financial Officer



Financial Highlights

Q1 2025 Financial Results

Total Revenues	Operating (Loss) GAAP / Non-GAAP ¹	Cash and Investments ³
\$745 million	(\$300) / (\$250) million	\$647 million

2025 On Track to Achieve Robust Revenue and Profit Growth and Maintain a Strong Balance Sheet Providing Significant Flexibility for Capital Allocation

- Excluding Arrowhead closing transaction costs² of \$584M, we would have reported an operating profit in Q1 2025
- Committed to disciplined expense management and continued operating expense leverage to drive profit expansion
- Focused on opportunistic capital allocation strategy to deliver shareholder value while preserving financial flexibility to invest in our long-term growth opportunities
 - Undrawn \$600M Revolving credit facility supplements balance sheet
 - Previously authorized \$500M share repurchase program

Footnotes

1. Non-GAAP operating income (loss) is defined by us as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP profit (loss) is defined by us as GAAP net profit (loss) excluding interest income, net, depreciation and amortization expense, stock-based compensation expense, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items. 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 in our press release dated May 6, 2025, 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)
3. Includes cash, cash equivalents, restricted cash and investments

Q1 2025 Select Financial Data

\$ In Millions, except percentages	Q1 2025	Q1 2024	YoY %
Total Product Revenue	\$612	\$359	70%
Collaboration and Other Revenues	\$133	\$54	
Total Revenues	\$745	\$413	80%
Cost of Sales (excludes amortization of in-licensed rights)	\$138	\$51	
Combined GAAP R&D and SG&A Expenses	\$907	\$327	
Combined Non-GAAP R&D and SG&A Expenses ¹	\$856	\$279	
GAAP Operating (Loss) / Income	(\$300)	\$35	
Non-GAAP Operating (Loss) / Income ¹	(\$250)	\$84	

GAAP and Non-GAAP R&D Expenses include Arrowhead collaboration transaction costs² of \$584M

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, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by us as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items. Non-GAAP operating income (loss) is defined by us as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items. 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 in our press release dated May 6, 2025, 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)

Updated 2025 Full Year Guidance

	Prior Guidance FY 2025 As of February 26, 2025	Updated Guidance FY 2025 As of May 6, 2025	Assumptions
Total Product Revenue	\$2,900 - \$3,100M	\$2,300 - \$2,600M	Reflects updated ELEVIDYS outlook <ul style="list-style-type: none"> Represents +37% YoY growth at the midpoint of revised range
Combined Non-GAAP R&D and SG&A Expenses ¹	\$1,200 - \$1,300M	\$1,784 - \$2,184M	Consistent opex outlook from prior guidance now includes Arrowhead collaboration: <ul style="list-style-type: none"> Closing transaction costs (\$584M) Potential DM1 development milestone payments (\$100-\$300M)

Footnotes

- Non-GAAP research and development expenses are defined by us as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by us as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items. 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 in our press release dated May 6, 2025, which is accessible in the Investors section of our website at www.sarepta.com.

Question & Answer Session



Appendix



Condensed Consolidated Statements of Income (Loss)

\$ In Thousands, except per share amounts

	For the Three Months Ended March 31,	
	2025	2024
Revenues:		
Products, net	\$ 611,523	\$ 359,484
Collaboration and other	133,333	53,980
Total revenues	744,856	413,464
Cost and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	137,564	50,559
Research and development	773,448	200,396
Selling, general and administrative	133,629	127,003
Amortization of in-licensed rights	601	601
Total cost and expenses	1,045,242	378,559
Operating (loss) income	(300,386)	34,905
Other (loss) income, net:		
Other (expense) income, net	(83,132)	6,543
(Loss) income before income tax expense	(383,518)	41,448
Income tax expense	63,990	5,329
Net (loss) income	<u>\$ (447,508)</u>	<u>\$ 36,119</u>
(Loss) earnings per share:		
Basic	\$ (4.60)	\$ 0.38
Diluted	\$ (4.60)	\$ 0.37
Weighted average number of shares of common stock used in computing (loss) earnings per share:		
Basic	97,362	93,991
Diluted	97,362	99,114

Note: Tables may not foot due to rounding

Reconciliation of GAAP Reported Net Income to Non-GAAP Net Income

\$ In Thousands, except per share amounts

	For the Three Months Ended March 31,	
	2025	2024
GAAP net (loss) income	\$ (447,508)	\$ 36,119
Interest income, net	(7,925)	(15,731)
Depreciation and amortization expense	9,377	8,143
Stock-based compensation expense	41,428	40,692
Change in fair value of derivatives	—	10,100
Loss (gain) on strategic investments*	90,728	(931)
Income tax effect of adjustments	(18,598)	(1,042)
Non-GAAP net (loss) income	<u>\$ (332,498)</u>	<u>\$ 77,350</u>
GAAP (loss) earnings per share - diluted:	\$ (4.60)	\$ 0.37
Add: impact of GAAP to Non-GAAP adjustments	1.18	0.35
Non-GAAP (loss) earnings per share - diluted**	<u>\$ (3.42)</u>	<u>\$ 0.72</u>
Weighted average number of shares of common stock used in computing diluted (loss) earnings per share:***		
GAAP	97,362	99,114
Non-GAAP	97,362	107,215

*Beginning in the first quarter of 2025, loss (gain) on strategic investments was included as a non-GAAP measurement to adjust our GAAP financial measures. Non-GAAP financial results for the first quarter 2024 have been updated to reflect this change for comparability. Please refer to the “Use of Non-GAAP Measures” section above for additional detail.

**Non-GAAP earnings per share is calculated using diluted shares whereas non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive.

***The difference between the weighted average number of shares of common stock used in computing diluted GAAP and non-GAAP earnings per share for the three months ended March 31, 2024, is a result of the exclusion of the potential share settlement of the 2027 Convertible Notes from the GAAP earnings per share as the inclusion of such shares was anti-dilutive.

Note: Tables may not foot due to rounding

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

\$ In Thousands

	For the Three Months Ended March 31,	
	2025	2024
GAAP research and development expenses	\$ 773,448	\$ 200,396
Stock-based compensation expense	(17,317)	(16,273)
Depreciation and amortization expense	(6,977)	(6,046)
Non-GAAP research and development expenses	<u>\$ 749,154</u>	<u>\$ 178,077</u>

	For the Three Months Ended March 31,	
	2025	2024
GAAP selling, general and administrative expenses	\$ 133,629	\$ 127,003
Stock-based compensation expense	(24,111)	(24,419)
Depreciation expense	(2,400)	(2,097)
Non-GAAP selling, general and administrative expenses	<u>\$ 107,118</u>	<u>\$ 100,487</u>

	For the Three Months Ended March 31,	
	2025	2024
GAAP operating (loss) income	\$ (300,386)	\$ 34,905
Stock-based compensation expense	41,428	40,692
Depreciation and amortization expense	9,377	8,143
Non-GAAP operating (loss) income	<u>\$ (249,581)</u>	<u>\$ 83,740</u>

Note: Tables may not foot due to rounding