Sarepta Therapeutics Announces Strategic Restructuring and Pipeline Prioritization Plan to Maintain Long-term, Sustainable Growth and Provides Update on ELEVIDYS Label

7/16/25

– ***After strategic review, Sarepta focuses pipeline on high-impact programs, prioritizing potentially best-in-class siRNA platform assets***

– ***Strategic restructuring includes reduced operating expenses, delivering approximately $400 million in anticipated annual cost savings, and implementing a 36% workforce reduction of approximately 500 employees***

– ***Duchenne portfolio continues to deliver stable and robust revenues, with preliminary quarterly results showing total net product revenue of $513 million for the second quarter 2025***

– ***U.S. FDA has requested and Sarepta has agreed to include a black box warning in the ELEVIDYS label, resolving any material issues with the ambulant population indication***

– ***Sarepta has completed an Expert Committee on a protocol for the use of additional prophylactic immunosuppression for non-ambulant patients, and will submit the protocol to FDA imminently and discuss the pathway to resume shipment of ELEVIDYS for non-ambulant patients***

– ***Company to host investor call on July 16, 2025, at 4:30 p.m. Eastern time***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 16, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced a strategic restructuring plan designed to prioritize high-value, high impact programs, meet its 2027 financial obligations, and support its long-term financial viability. This plan is expected to position the Company for long-term sustainable growth, with an emphasis on near- and mid-term opportunities from the siRNA platform. These decisive changes aim to ensure sustained profitability and preserve the Company's ability to deliver on its mission of advancing innovative medicines for those with rare genetic diseases.

"Faced with environmental changes, we have decided to act decisively, implementing a focused strategy to ensure Sarepta remains a vibrant, financially enduring, patient-centric organization dedicated to improving the lives of those with rare genetic diseases," said Doug Ingram, chief executive officer, Sarepta Therapeutics. "These changes will ensure we remain a financially strong and profitable organization built on a sharpened and focused strategy. We will continue to drive performance of ELEVIDYS and our three PMOs in service of the Duchenne community those therapies benefit, and with our financial performance, we will advance our high-value, focused pipeline of programs for rare genetic diseases, primarily relying on the siRNA platform, while ensuring we meet our financial obligations."

In addition to the restructuring plan, the Company provided an update on activities related to the label updates underway for ELEVIDYS (delandistrogene moxeparvovec), the first and only approved gene therapy for the treatment of Duchenne muscular dystrophy and reported preliminary financial results for the quarter ended June 30, 2025.

ELEVIDYS Label Update and Enhanced Safety Efforts
Following previously communicated steps being taken to strengthen the safety profile of ELEVIDYS, Sarepta is providing an update on on-going engagement with the U.S. Food and Drug Administration (FDA) regarding the ELEVIDYS (delandistrogene moxeparvovec) label. Consistent with other AAV-delivered gene therapies, the FDA has requested that the label include a black box warning for acute liver injury (ALI) and acute liver failure (ALF). Sarepta agrees with this change, which appears to resolve any material issues with the ambulant portion of the ELEVIDYS label.

Sarepta recently announced that it was pausing shipments of ELEVIDYS for non-ambulant patients while it explored the adoption of additional prophylactic immunosuppression. To that end, Sarepta convened an Expert Committee of neuromuscular specialists, hepatologists, hematologists, and immunologists to review cases of ALF and explore additional immunosuppression regimens. The Committee aligned on an enhanced immunosuppressive regimen with sirolimus for ELEVIDYS in non-ambulant patients. Sarepta will submit the finding of the expert panel and proposed protocol to the FDA imminently and will discuss a proposal to gather data on the regimen in a new cohort (Cohort 8) of the ENDEAVOR study (Study SRP-9001-103) as a pathway to re-establish dosing in the non-ambulant setting. Additionally, Sarepta is assessing real-world data generation opportunities for ambulant patients through investigator-initiated trials.

Strategic Restructuring to Bolster Financial Foundation
Sarepta has initiated immediate changes to reduce operating expenses and align its cost structure with strategic priorities, aiming to enhance financial flexibility and meet its 2027 financial obligations.

Collectively, these measures are projected to deliver approximately $400 million in annual cost reductions, significantly lowering the Company's average annual non-GAAP R&D and SG&A expenses to between $800 million and $900M starting in 2026.

These include:

- A 36% workforce reduction, impacting approximately 500 employees, projected to generate approximately $120 million in annual cash cost savings in 2026
- Pipeline reprioritization expected to deliver approximately $300 million in annual non-personnel cost savings starting in 2026
- Over $100 million in cost savings anticipated through the end of 2025, net of estimated severance and one-time charges

totaling $32-$37 million

This rigorous approach is designed to maintain access to Sarepta's $600 million revolving credit facility and generate robust cash flow to proactively manage liabilities, including the repayment of the 2027 convertible note.

Preliminary Second Quarter 2025 Financial Highlights
For the second quarter ended June 30, 2025, Sarepta reported preliminary financial results:

- Total net product revenue: $513 million
  - ELEVIDYS net product revenue: $282 million
  - RNA-based PMOs net product revenue: $231 million
  - Net product revenue does not include collaboration, contract manufacturing or royalty revenue
- Combined research and development expense and selling, general and administrative expense:
  - GAAP: $338 million
  - Non-GAAP: $294 million
  - The difference of $44 million consists of stock-based compensation expense of $34 million and depreciation and amortization expense of $10 million
- Cash, cash equivalents, restricted cash and investments: approximately $850 million as of June 30, 2025

These selected financial results are preliminary and subject to adjustment. The Company has not completed its financial closing procedures for the quarter ended June 30, 2025, and its actual results could be materially different from these preliminary financial results. Sarepta will report its final and complete second quarter 2025 financial results in early August 2025.

Refocused Pipeline on siRNA platform
Sarepta's dedication to advancing genetic medicine remains steadfast. The Company will continue to support its four on-market Duchenne therapies and all associated clinical trial commitments and evidence-generation activities. Revenues from this robust Duchenne portfolio are expected to continue driving profitability and funding a focused pipeline of high-impact development programs primarily leveraging Sarepta's potentially best-in-class siRNA platform. This strategic pivot emphasizes chronically administered therapies for neurodegenerative and pulmonary diseases.

As a result of this reprioritization, several programs, including most of the gene therapies in development for limb-girdle muscular dystrophy (LGMD), will be paused. Sarepta expects to submit the Biologics License Application for SRP-9003 for LGMD type 2E/R4 in the second half of this year. Sarepta intends to seek strategic alternatives, including partnering, for programs that it no longer intends to fund directly.

The siRNA programs include investigational treatments for:

- Facioscapulohumeral muscular dystrophy (FSHD)
- Myotonic dystrophy type 1 (DM1)
- Spinocerebellar ataxia type 2 (SCA2)
- Idiopathic Pulmonary Fibrosis (IPF)
- Huntington's disease

These programs offer tremendous near-term potential, addressing areas of significant unmet medical need with potentially best-in-class approaches. Sarepta is also pursuing preclinical programs for Spinocerebellar ataxia type 1 (SCA1) and Spinocerebellar ataxia type 3 (SCA3) and has an exclusive collaboration with Arrowhead Pharmaceuticals to develop therapies for skeletal muscle diseases, with plans to pursue up to six discovery targets in muscle or central nervous system disorders.

Executive Leadership Appointments
In conjunction with the restructuring, Sarepta announced key executive appointments:

- Ian Estepan named President and Chief Operating Officer
- Louise Rodino-Klapac, Ph.D., named President of Research & Development and Technical Operations
- Ryan Wong, named Chief Financial Officer, previously Senior Vice President of Strategic Finance, Treasury, and Investor Relations
- Rachael Potter, Ph.D., named Chief Scientific Officer, previously Senior Vice President, Head of Research Sciences
- Patrick Moss, Pharm.D., named Chief Commercial Officer, previously Senior Vice President of U.S. Market Access and Sales, succeeding Dallan Murray, Chief Customer Officer, who is leaving Sarepta. The Company thanks Mr. Murray for his long-time service and wishes him well.

Investor Webcast Details
Sarepta will be hosting a conference call and webcast to discuss these updates on Wednesday, July 16, 2025, at 4:30 p.m. Eastern time. The event will be webcast live under the investor relations section of Sarepta's website at: **https://investorrelations.sarepta.com/events-presentations** and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using **this online form**. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

Use of Non-GAAP Measures
In addition to the GAAP financial measures set forth in this press release, we have included the following non-GAAP measurements:

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.
2. 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.

The following components are used to adjust our GAAP financial measures into the previously defined non-GAAP measurements:

1. Depreciation and amortization - Depreciation expense can vary substantially from period to period as the purchases of property and equipment may vary significantly from period to period and without any direct correlation to our operating performance. Amortization expense primarily associated with patent costs are amortized over a period of several years after acquisition or patent application or renewal.
2. Stock-based compensation expenses - Stock-based compensation expenses represent non-cash charges related to equity awards we have granted. Although these are recurring charges to operations, we believe the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within our control. Therefore, we believe that excluding these charges facilitates comparisons of our operational performance in different periods.

We use these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. We believe these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating our performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of our financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses and non-GAAP selling, general and administrative expenses may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles.

About ELEVIDYS (delandistrogene moxeparvovec-rokl)
ELEVIDYS (delandistrogene moxeparvovec-rokl) is a single-dose, adeno-associated virus (AAV)-based gene transfer therapy for intravenous infusion designed to address the underlying genetic cause of Duchenne muscular dystrophy – mutations or changes in the DMD gene that result in the lack of dystrophin protein – through the delivery of a transgene that codes for the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle.

ELEVIDYS is indicated for the treatment of Duchenne muscular dystrophy (DMD) in individuals at least 4 years of age.

- For patients who are ambulatory and have a confirmed mutation in the DMD gene
- For patients who are non-ambulatory and have a confirmed mutation in the DMD gene.

The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin in skeletal muscle. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

IMPORTANT SAFETY INFORMATION

CONTRAINDICATION: ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene.

WARNINGS AND PRECAUTIONS:
Infusion-related Reactions:

- Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred during or up to several hours following ELEVIDYS administration. Closely monitor patients during administration and for at least 3 hours after the end of infusion. If symptoms of infusion-related reactions occur, slow, or stop the infusion and give appropriate treatment. Once symptoms resolve, the infusion may be restarted at a lower rate.
- ELEVIDYS should be administered in a setting where treatment for infusion-related reactions is immediately available.
- Discontinue infusion for anaphylaxis.

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS, and administration may result in elevations of liver enzymes (such as GGT, GLDH, ALT, AST) or total bilirubin, typically seen within 8 weeks.
- Patients with preexisting liver impairment, chronic hepatic condition, or acute liver disease (e.g., acute hepatic viral infection) may be at higher risk of acute serious liver injury. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.
- Prior to ELEVIDYS administration, perform liver enzyme test and monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT, and total bilirubin levels return to near baseline levels).
- Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated. If acute serious liver injury is suspected, consultation with a specialist is recommended.

Immune-mediated Myositis:

- In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the DMD gene. Symptoms of severe muscle weakness,

including dysphagia, dyspnea, and hypophonia, were observed.
- Limited data are available for ELEVIDYS treatment in patients with mutations in the DMD gene in exons 1 to 17 and/or exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.
- Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea, or hypophonia, as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

Myocarditis:

- Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.
- If a patient experiences myocarditis, those with pre-existing left ventricle ejection fraction (LVEF) impairment may be at higher risk of adverse outcomes. Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion and continue monitoring if clinically indicated. More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.
- Advise patients to contact a physician immediately if they experience cardiac symptoms.

Preexisting Immunity against AAVrh74:

- In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all patients developed anti-AAVrh74 antibodies.
- Perform baseline testing for presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration.
- ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers greater than or equal to 1:400.

Adverse Reactions:

- The most common adverse reactions (incidence ≥5%) reported in clinical studies were vomiting, nausea, liver injury, pyrexia, and thrombocytopenia.

Report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics
Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (Duchenne) and limb-girdle muscular dystrophies (LGMDs) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases. For more information, please visit **www.sarepta.com** or follow us on **LinkedIn**, **X**, **Instagram** and **Facebook**.

Internet Posting of Information
We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Forward-Looking Statements
This press release contains "forward-looking statements." Any statements 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 financial results and projections and future operations; our pipeline and priorities; ELEVIDYS and the potential benefits of our proposed enhanced regimen; our ongoing and planned clinical trials; the reduction in force and our revised cost structure; the potential for our restructuring activities to help us meet our 2027 financial obligations, sustain profitability and position us for long-term sustainable growth; our expectation that the label for ELEVIDYS will include a black box warning for acute liver injury and acute liver failure; and expected plans and milestones, including our intention to seek alignment with the FDA to test our enhanced regimen in a new cohort of the ENDEAVOR study, submitting the BLA for SRP-9003 later this year, potentially seeking additional strategic alternatives for programs no longer directly funded, and near-term opportunities from the siRNA platform.

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; the reduction in force may take longer or result in more significant charges or cash expenditures than anticipated or otherwise negatively impact the Company and its business plans during and after the period during which the reduction in force is being executed; we may not be able to meet expectations with respect to sales of our products or maintain profitability; the estimates and judgments the Company makes, or the assumptions on which it relies, in preparing its financial statements could prove inaccurate; 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; 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; 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; 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; 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250716134932/en/

Investor Contact:
Ian Estepan
617-274-4052
iestepan@sarepta.com

Media Contacts:
Tracy Sorrentino
617-301-8566
tsorrentino@sarepta.com

Kara Hoeger
617-710-3898
KHoeger@sarepta.com

Source: Sarepta Therapeutics, Inc.