Sarepta Therapeutics Announces First Quarter 2025 Financial Results and Recent Corporate Developments

5/6/25

- ***Net product revenues for the first quarter 2025 totaled $611.5 million, a 70% increase over the same quarter of the prior year***
- ***ELEVIDYS net product revenue for the first quarter totaled $375.0 million; Royalty revenue from the sales of ELEVIDYS by Roche for the quarter totaled $4.0 million***
- ***Revised 2025 total net product revenues guidance to $2.3 to $2.6 billion***
- ***Meaningfully advanced multiple clinical candidates in limb-girdle muscular dystrophy portfolio***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 6, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the first quarter 2025.

“In the first quarter, we achieved net product revenue of $611.5 million, a 70% increase over the same quarter prior year; our PMO franchise performed well at $236.5 million; and ELEVIDYS achieved $375.0 million, growing at 180% over the same quarter prior year. However, we also faced headwinds in the quarter. While we are taking a variety of actions to address and resolve these challenges, we have adjusted our guidance for 2025 to $2.3 billion to $2.6 billion,” said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. “The broader biotech market has also faced significant pressure in the first quarter of 2025. Fortunately, as an organization with four life-changing therapies, significant revenue, and a deep pipeline, Sarepta is well-positioned to navigate these chaotic times. To that end, in addition to driving the launch of ELEVIDYS and the continued performance of our three approved PMO therapies, we continue to invest in our pipeline and look forward to a significant number of LGMD gene therapy approvals in the coming few years. We are also advancing our siRNA platform and look forward to proof of concept and proof of biology readouts later this year in both our DM1 and FSHD1 programs.”

Corporate Highlights:

- In the first quarter Sarepta closed its global licensing and collaboration agreement with Arrowhead Pharmaceuticals, Inc. (“Arrowhead”). The agreement includes global rights to four clinical-stage and three preclinical-stage programs in muscle, central nervous system, and rare pulmonary disorders, including potential best-in-class siRNA-based treatments for DM1 and FSHD1. The agreement adds meaningfully to Sarepta’s mid- and early-stage pipeline, complementing the Company’s existing leadership in Duchenne muscular dystrophy and limb-girdle muscular dystrophies and gene therapy, while adding new indications and expanding into adjacent therapeutic areas. Later this year the Company plans to share data from the candidates in development for FSHD1 and DM1.
- **Pipeline progress for multiple of Sarepta's limb-girdle muscular dystrophy programs:**
  - **SRP-9005 for LGMD type 2C/R5:** Following input from the FDA’s Office of Therapeutic Products (OTP), the Company is cleared to proceed with dosing in Study SRP-9005-101 (COMPASS) in the U.S. COMPASS is Sarepta’s first-in-human clinical study of SRP-9005, an investigational gene therapy for LGMD type 2C/R5, or gamma-sarcoglycanopathy.
  - **SRP-9004 for LGMD type 2D/R3:** Enrollment and dosing is complete in Study SRP-9004-102 (DISCOVERY). DISCOVERY is a phase 1, proof-of-concept study evaluating safety and expression of the alpha-sarcoglycan protein after treatment with SRP-9004, an investigational gene therapy for the treatment of LGMD type 2D/R3, or alpha-sarcoglycanopathy.
  - **SRP-9003 for LGMD type 2E/R4:** Enrollment and dosing is complete in Study SRP-9003-301 (EMERGENE). EMERGENE is a phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of LGMD type 2E/R4, or beta-sarcoglycanopathy. EMERGENE is a global study and the primary endpoint is the biomarker expression of beta-sarcoglycan protein. A pre-Biologics License Application (BLA) meeting has occurred and the OTP has confirmed eligibility for the accelerated approval pathway for the program. Sarepta remains on track to submit a BLA to the U.S. FDA in the second half of 2025.
- **2025 MDA Clinical and Scientific Conference:** Sarepta showcased progress across its portfolio of approved and pipeline therapies for Duchenne and limb-girdle muscular dystrophy with 11 abstracts and two oral presentations. The oral presentations included:
  - Results from the 2-year EMBARK and 3-year pooled analyses indicating stabilization or slowing of disease progression compared with well-matched external control assessed by functional outcomes predictive for delaying loss of ambulation.
  - Two-year muscle MRI data, from a subset of patients in EMBARK Part 1, showing stabilization or minimal progression of underlying muscle pathology which remains consistent with functional benefits.

All of the posters and presentations are available on the Company's website, here.

- Sarepta remains on track to achieve robust revenue growth in 2025 and maintain a strong balance sheet.

Conference Call

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.

Q1 2025 Financial Highlights[1]

	For the Three Months Ended March 31,			
	2025	2024	Change	Change
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 744.9	$ 413.5	$ 331.4	80%
Operating (loss) income:				
GAAP	$ (300.4)	$ 34.9	$ (335.3)	NM*
Non-GAAP	$ (249.6)	$ 83.7	$ (333.3)	NM*
Net (loss) income:				
GAAP	$ (447.5)	$ 36.1	$ (483.6)	NM*
Non-GAAP	$ (332.5)	$ 77.4	$ (409.9)	NM*
Diluted (loss) earnings per share				
GAAP	$ (4.60)	$ 0.37	$ (4.97)	NM*
Non-GAAP	$ (3.42)	$ 0.72	$ (4.14)	NM*

*NM: not meaningful

[1] For an explanation of our use of non-GAAP financial measures, please refer to the "Use of Non-GAAP Financial Measures" section later in this press release, and for a reconciliation of each non-GAAP financial measure to the most comparable GAAP measures, see the table at the end of this press release.

	As of March 31, 2025	As of December 31, 2024
	(in millions)	
Cash, cash equivalents, and investments	$ 647.5	$ 1,503.5

Revenues

Total revenues were $744.9 million for the three months ended March 31, 2025, as compared to $413.5 million for the same period of 2024, an increase of $331.4 million. The increase primarily reflects an increase of $241.0 million in net product revenue of ELEVIDYS as a result of its expanded label approval in June 2024. In addition, collaboration revenues increased $64.0 million related to the $112.0 million of collaboration revenue recognized related to F. Hoffman-La Roche Ltd.'s ("Roche") expiration of an option for a program during the first quarter of 2025, as compared to $48.0 million of collaboration revenue in 2024 related to Roche's declined option to acquire the ex-US rights to an external, early-stage Duchenne development program. Furthermore, contract manufacturing revenues and royalty revenues increased $11.6 million and $3.8 million, respectively, associated with an increase in commercial ELEVIDYS supply delivered to Roche as well as royalty revenue from sales of ELEVIDYS by Roche, respectively.

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

Cost of sales (excluding amortization of in-license rights) were $137.6 million for the three months ended March 31, 2025, as compared to $50.6 million for the same period of 2024, an increase of $87.0 million. This increase primarily reflects an increase in cost of sales related to ELEVIDYS as we consume previously expensed inventory, an increased demand for ELEVIDYS following the expanded label approval in June 2024, as well as an increase in cost of sales related to products sold to Roche under the Roche collaboration agreement, partially offset by a decrease in the write-offs of certain batches of products not meeting quality specifications for the three months ended March 31, 2025, as compared to the same period of 2024.

Operating expenses and others

Research and development expenses were $773.4 million for the three months ended March 31, 2025, as compared to $200.4 million for the same period of 2024, an increase of $573.0 million. The increase in research and development expenses primarily reflects an increase in up-front and milestone expense associated with the licensing and collaboration agreement and stock purchase agreement with Arrowhead, partially offset by a decrease in clinical trial expenses primarily due to the discontinuation of the PPMO programs in 2024. For the three months ended March 31, 2025, non-GAAP research and development expenses were $749.2 million, as compared to $178.1 million for the same period of 2024, an increase of $571.1 million.

Selling, general and administrative expenses were $133.6 million for the three months ended March 31, 2025, as compared to $127.0 million for the same period of 2024, an increase of $6.6 million. The increase is primarily driven by an increase in compensation and other personnel expenses primarily due to changes in headcount as well as an increase in professional services used for the continuing efforts to commercialize ELEVIDYS. For the three months ended March 31, 2025, non-GAAP selling, general and administrative expenses were $107.1 million, as compared to $100.5 million for the same period of 2024, an increase of $6.6 million.

Other (expense) income, net for the three months ended March 31, 2025 and 2024, was approximately $(83.1) million and $6.5 million, respectively. The change primarily reflects the loss on strategic investments, which includes the recurring fair value adjustments of our investments in publicly-traded companies, including Arrowhead, during the three months ended March 31, 2025.

Income tax expense for the three months ended March 31, 2025 and 2024, was approximately $64.0 million and $5.3 million, respectively. Income tax expense for all periods presented primarily relates to state, federal and foreign income taxes for which available tax losses or credits were not available to offset.

2025 Financial Guidance

	Updated Guidance	Prior Guidance	Assumptions
	As of May 6, 2025	As of February 26, 2025	
Total net product revenues	$2,300 - $2,600M	$2,900 - $3,100M	Reflects updated ELEVIDYS outlook • Represents +37% YoY growth at the midpoint of the revised range
Combined non-GAAP R&D and SG&A expenses	$1,784 - $2,184M	$1,200 - $1,300M	Consistent operating expense outlook from prior guidance, now includes Arrowhead collaboration: • Closing transaction costs ($584M) • Potential DM1 development milestone payments ($100-$300M)

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 net (loss) income is defined by us as GAAP net (loss) income excluding interest income (expense), 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.
2. Non-GAAP net loss per share is defined by us as non-GAAP net loss, as defined above, divided by the weighted-average number of shares of common stock outstanding as the inclusion of dilutive common stock equivalents outstanding is anti-dilutive. Non-GAAP earnings per share is defined by us 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.
3. Non-GAAP operating (loss) income is defined by us as GAAP operating (loss) income excluding depreciation and amortization expense, stock-based compensation expense and other items.
4. 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.
5. 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.

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

1. Interest, depreciation and amortization - Interest income (expense), net amounts can vary substantially from period to period due to changes in cash and debt balances and interest rates driven by market conditions outside of our operations. 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.
3. Other items - We evaluate other items of expense and income on an individual basis. We take into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to our ongoing business operations, and (c) whether we expect the items to continue or occur on a regular basis. These other items include the loss (gain) on strategic investments and changes in the fair value of derivatives and may include other items that fit the above characteristics in the future. We exclude from our non-GAAP results:

a) The loss (gain) on strategic investments as it is a non-cash item and the results of such gains and losses are not representative of our normal business operations, which accordingly, would make it difficult to compare our results to peer companies that also provide non-GAAP disclosures. We are making this change beginning in 2025 because, as our strategic investments have increased, we recognized that the resulting variability can impede comparability between periods of our financial performance for our ongoing business operations.

b) The change in fair value of derivatives related to 1.) regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to an academic institution under a separate license agreement and 2.) the derivative asset associated with capped call options for our $570.0 million aggregate principal amount of senior convertible notes that were due on November 15, 2024, as these are non-cash items and are not considered to be normal operating expenses due to the variability of amounts and lack of predictability as to occurrence and/or timing.

We use these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. We also 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, non-GAAP selling, general and administrative expenses, non-GAAP other income and loss adjustments, non-GAAP operating (loss) income, non-GAAP net (loss) income, and non-GAAP diluted (loss) earnings per share 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. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table “Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures.”

About EXONDYS 51

EXONDYS 51 uses Sarepta’s proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion, or “skipping”, of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

EXONDYS 51 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including bronchospasm, chest pain, cough, tachycardia, and urticaria have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 mg or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

The most common adverse reactions from observational clinical studies (N=163) seen in greater than 10% of patients were headache, cough, rash, and vomiting.

Other adverse events may occur.

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For further information, please see the full Prescribing Information.

About VYONDYS 53

VYONDYS 53 (golodirsen) uses Sarepta’s proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or “skipping,” of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

VYONDYS 53 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for VYONDYS 53

CONTRAINDICATIONS: VYONDYS 53 is contraindicated in patients with a serious hypersensitivity reaction to golodirsen or to any of the inactive ingredients in VYONDYS 53. Anaphylaxis has occurred in patients receiving VYONDYS 53.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions: Hypersensitivity reactions, including anaphylaxis, rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion, interrupting, or discontinuing the VYONDYS 53 therapy and monitor until the condition resolves. VYONDYS 53 is contraindicated in patients with a history of a serious hypersensitivity reaction to golodirsen or to any of the inactive ingredients in VYONDYS 53.

Kidney Toxicity: Kidney toxicity was observed in animals who received golodirsen. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients.

Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

ADVERSE REACTIONS: Adverse reactions observed in at least 20% of treated patients and greater than placebo were (VYONDYS 53, placebo): headache (41%, 10%), pyrexia (41%, 14%), fall (29%, 19%), abdominal pain (27%, 10%), nasopharyngitis (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

Other adverse events may occur.

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For further information, please see the full Prescribing Information.

About AMONDYS 45
AMONDYS 45 (casimersen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

AMONDYS 45 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 45 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

AMONDYS 45 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for AMONDYS 45

CONTRAINDICATIONS: AMONDYS 45 is contraindicated in patients with a known serious hypersensitivity to casimersen or any of the inactive ingredients in AMONDYS 45. Instances of hypersensitivity including angioedema and anaphylaxis have occurred.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions: Hypersensitivity reactions, including angioedema and anaphylaxis, have occurred in patients who were treated with AMONDYS 45. If a hypersensitivity reaction occurs, institute appropriate medical treatment, and consider slowing the infusion, interrupting, or discontinuing the AMONDYS 45 infusion and monitor until the condition resolves. AMONDYS 45 is contraindicated in patients with known serious hypersensitivity to casimersen or to any of the inactive ingredients in AMONDYS 45.

Kidney Toxicity: Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse Reactions: Adverse reactions occurring in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were (AMONDYS 45, placebo): upper respiratory infections (65%, 55%), cough (33%, 26%), pyrexia (33%, 23%), headache (32%, 19%), arthralgia (21%, 10%), and oropharyngeal pain (21%, 7%).

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were: ear pain, nausea, ear infection, post-traumatic pain, and dizziness and light-headedness.

Other adverse events may occur.

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For further information, please see the full Prescribing Information.

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 (noted hereafter as “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 for ELEVIDYS

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 of ELEVIDYS 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.

Pre-existing 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 (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on LinkedIn, X (formerly Twitter), Instagram and Facebook.

Forward-Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, financial performance, guidance and financial projections for 2025 and beyond, including our revised 2025 product revenue guidance; business plans, market opportunities, priorities, research and development programs, and the potential benefits of our product candidates; and our expected plans and milestones, including submitting a BLA in the second half of 2025 for SRP-9003, sharing data from candidates in development for FSHD1 and DM1 later this year, and the potential to receive a significant number of LGMD gene therapy approvals in the coming years.

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 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; 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; 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; 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.

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.

Sarepta Therapeutics, Inc.
Condensed Consolidated Statements of (Loss) Income
(unaudited, 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	$ (447,508)	$ 36,119
(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

Sarepta Therapeutics, Inc.
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures
(unaudited, 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	$ (332,498)	$ 77,350
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**	$ (3.42)	$ 0.72
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.

	For the Three Months Ended March 31,	
	2025	2024
Total effective tax rate, GAAP	(16.7)%	14.2%
Less: impact of GAAP to Non-GAAP adjustments	(16.4)	(5.4)
Total effective tax rate, Non-GAAP	(33.1)%	8.8%

Sarepta Therapeutics, Inc.
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures
(unaudited, 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	$ 749,154	$ 178,077

	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	$ 107,118	$ 100,487

	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	$ (249,581)	$ 83,740

Sarepta Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(unaudited, in thousands, except share and per share data)

	As of March 31, 2025	As of December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 240,867	$ 1,103,010
Short-term investments	281,895	251,782
Accounts receivable, net	659,371	601,988
Inventory	941,432	749,960
Manufacturing-related deposits and prepaids	203,490	276,262
Other current assets	105,478	90,461
Total current assets	2,432,533	3,073,463
Property and equipment, net	360,587	340,336
Right of use assets	147,063	148,310
Non-current inventory	181,158	187,986
Strategic investments	154,370	3,710
Non-current investments	109,125	133,163
Other non-current assets	80,551	76,205
Total assets	$ 3,465,387	$ 3,963,173
Liabilities and Stockholders' Equity		

Current liabilities:		
Accounts payable	$ 156,105	$ 214,442
Accrued expenses	391,650	373,513
Deferred revenue, current portion	44,859	130,256
Other current liabilities	12,974	13,473
Total current liabilities	605,588	731,684
Long-term debt	1,138,289	1,137,124
Lease liabilities, net of current portion	205,460	192,473
Deferred revenue, net of current portion	325,000	325,000
Contingent consideration	47,400	47,400
Other non-current liabilities	927	1,750
Total liabilities	2,322,664	2,435,431
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 98,254,898 and 96,900,496 issued and outstanding at March 31, 2025, and December 31, 2024, respectively	10	10
Additional paid-in capital	5,801,161	5,738,924
Accumulated other comprehensive income (loss), net of tax	34	(218)
Accumulated deficit	(4,658,482)	(4,210,974)
Total stockholders' equity	1,142,723	1,527,742
Total liabilities and stockholders' equity	$ 3,465,387	$ 3,963,173

Source: Sarepta Therapeutics, Inc.

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