Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2024 Financial Results and Recent Corporate Developments

2/26/25

– ***Net product revenues for the fourth quarter 2024 totaled $638.2 million, a 75% increase over the same quarter of the prior year***

– ***ELEVIDYS net product revenue for the quarter totaled $384.2 million; Royalty revenue from the sales of ELEVIDYS by Roche for the quarter totaled $4.9 million***

– ***Achieved GAAP and non-GAAP net income of $159.0 million and $206.0 million for the fourth quarter of 2024, respectively***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 26, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the fourth quarter and full-year 2024.

"2024 performance represented the fruition of our multi-year strategy to become a self-sustaining profitable biotech dedicated to improving the lives of patients with rare genetic disease. After obtaining a broad label for our gene therapy ELEVIDYS covering the vast majority of Duchenne patients, we had the most successful gene therapy launch in history, even as we continued to serve the community with our PMOs, EXONDYS 51, VYONDYS 53 and AMONDYS 45. And as we advanced our internal gene therapy pipeline, we also continued our diversification and secured our future by in-licensing a broad platform of siRNA programs, with potential blockbuster opportunities that could reach the market in 2028 and 2029," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "In 2025, we intend to capitalize on our 2024 achievements. In addition to 2025 net product revenue guidance of $2.9 billion to $3.1 billion, representing 70% year-over-year growth and 162% yearly growth for ELEVIDYS, we expect to reach multiple important milestones this year, including the proof of biology readouts in our myotonic dystrophy type 1 (DM1) and facioscapulohumeral muscular dystrophy type 1 (FSHD) programs and the Biologics License Application (BLA) submission for SRP-9003 which, if successful, would lead to our first approval in our LGMD pipeline."

Fourth Quarter 2024 and Recent Developments:

- **Established inaugural $600 million senior secured revolving credit facility:** This instrument, available to Sarepta because of the Company's financial strength and positive business outlook, provides flexibility to use non-dilutive financing to supplement a strong balance sheet and offer contingent liquidity in execution of the Company's strategic plan.
- **Announced positive results from Part 2 of the EMBARK study:** In February, Sarepta reported topline results from Part 2 of EMBARK (Study SRP-9001-301), a Phase 3 clinical study of ELEVIDYS (delandistrogene moxeparvovec-rokl), the only approved gene therapy for Duchenne muscular dystrophy. The study showed that crossover-treated patients, who received ELEVIDYS after initially receiving a placebo, improved 2.34 points from baseline compared to matched external controls on the North Star Ambulatory Assessment (NSAA) 52 weeks after treatment ($P<0.0001$), during which time the study remained blinded. Despite being one year older (average age 7.18 years) than those treated in Part 1 (average age 5.98 years), crossover-treated patients showed clinically meaningful and statistically significant functional benefit for NSAA, Time to Rise (TTR), and 10-meter walk/run (10MWR) function tests compared with a pre-specified, propensity-weighted external control group (EC).

  At two years the Part 1 patients showed clinically meaningful and statistically significant functional benefit in NSAA, TTR and 10MWR compared with EC. Patients treated in Part 1 of the study had biopsies taken at 64 weeks after dosing and showed consistent and sustained expression of ELEVIDYS micro-dystrophin compared to week 12 biopsies, as measured by western blot, and provide biological support for observed functional outcomes. Additionally, skeletal MRI conducted on Part 1 patients indicated minimal muscle pathology progression, aligning with observed functional benefits.

  These results contribute to the growing body of clinical evidence supporting both the durability of ELEVIDYS treatment and the importance of intervening early to preserve muscle. The safety profile of ELEVIDYS remained consistent with previous findings.
- **Closed global licensing and collaboration agreement with Arrowhead Pharmaceuticals:** Sarepta has obtained exclusive 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 FSHD. The agreement also encompasses a discovery collaboration for up to six additional muscle, cardiac and or CNS targets, using Arrowhead's novel delivery technologies. 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. In addition, Doug Ingram, president and chief executive officer, Sarepta, has been appointed to Arrowhead's Board of Directors.
  - The clinical-stage programs covered under the agreement are:
    - **SRP-1001 (formerly ARO-DUX4):** designed to reduce the production of human double homeobox 4 (DUX4)

protein in skeletal muscle; currently in a Phase 1/2 clinical study for the treatment of facioscapulohumeral muscular dystrophy (FSHD)
    - **SRP-1003 (formerly ARO-DM1):** designed to target and suppress myotonic dystrophy protein kinase (DMPK) in skeletal muscle; Phase 1/2 clinical study for myotonic dystrophy type 1 (DM1)
    - **SRP-1002 (formerly ARO-MMP7):** designed to reduce expression of matrix metalloproteinase 7 (MMP7) in pulmonary epithelial cells; Phase 1/2 clinical study for idiopathic pulmonary fibrosis (IPF)
    - **SRP-1004 (formerly ARO-ATXN2):** designed to target the ataxin-2 protein (ATXN2) in the CNS; Phase 1/2 clinical study for spinocerebellar ataxia 2 (SCA2) commenced at the end of 2024
- **Enrollment and dosing completed in EMERGENE (Study SRP-9003-301) for LGMD2E/R4:** EMERGENE is a Phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec), an investigational gene therapy for the treatment of limb-girdle muscular dystrophy Type 2E/R4 (LGMD2E/R4), or beta-sarcoglycanopathy. EMERGENE is a global study, and the primary endpoint is the biomarker expression of beta-sarcoglycan protein, the absence of which is the sole cause of LGMD2E/R4. The design of the trial is notable as it sets an important precedent informing development plans for Sarepta's other LGMD pipeline programs, including LGMD2D and LGMD2C for which clinical trials are underway. Data from EMERGENE are expected in the first half of 2025. Following a positive pre-BLA meeting with FDA, the Company remains on track to submit a BLA filing later this year seeking accelerated approval for SRP-9003.

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.

Q4 2024 Financial Highlights[1]

	For the Three Months Ended December 31,			
	2024	2023	QTD Change	
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 658.4	$ 396.8	261.6	66%
Operating income:				
GAAP	$ 161.7	$ 24.6	137.1	NM*
Non-GAAP	$ 221.2	$ 81.1	140.1	173%
Net income:				
GAAP	$ 159.0	$ 45.7	113.3	NM*
Non-GAAP	$ 206.0	$ 86.6	119.4	138%
Diluted earnings per share:				
GAAP	$ 1.50	$ 0.47	1.03	NM*
Non-GAAP	$ 1.90	$ 0.82	1.08	132%

	For the Twelve Months Ended December 31,			
	2024	2023	YTD Change	
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 1,902.0	$ 1,243.3	658.7	53%
Operating income (loss):				
GAAP	$ 218.1	$ (267.8)	485.9	NM*
Non-GAAP	$ 437.7	$ (42.5)	480.2	NM*
Net income (loss):				
GAAP	$ 235.2	$ (536.0)	771.2	NM*
Non-GAAP	$ 397.9	$ (59.5)	457.4	NM*
Diluted earnings (loss) per share:				
GAAP	$ 2.34	$ (5.80)	8.14	NM*
Non-GAAP	$ 3.69	$ (0.64)	4.33	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 tables at the end of this press release.

	As of December 31, 2024	As of December 31, 2023
	(in millions)	
Cash, cash equivalents, restricted cash and investments	$ 1,503.5	$ 1,691.8

Revenues

Total revenues increased by $261.6 million and $658.7 million for the three and twelve months ended December 31, 2024, respectively, compared to the same periods of 2023. The increases primarily reflect the initial product launch of ELEVIDYS in June 2023 and subsequent expanded label approval in June 2024.

Additionally, included in total revenues for the three and twelve months ended December 31, 2024, is $20.3 million and $66.0 million, respectively, of contract manufacturing and other revenues associated with commercial ELEVIDYS supply delivered to Roche and royalty revenue received from Roche, as compared to $9.2 million of contract manufacturing and other revenues for the three and twelve months ended December 31, 2023.

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

Cost of sales (excluding amortization of in-license rights) increased by $88.1 million and $168.8 million for the three and twelve months ended December 31, 2024, respectively, compared with the same periods of 2023. The increases in both periods primarily reflect the initial product launch of ELEVIDYS in June 2023 and subsequent expanded label approval in June 2024, as well as cost of sales related to products sold to Roche under our collaboration agreement increasing by $5.1 million and $20.4 million for the three and twelve months ended December 31, 2024, respectively, compared with the same periods of 2023.

Operating expenses and others

Research and development expenses increased by $4.4 million for the three months ended December 31, 2024, compared with the same period of 2023, primarily as a result of an increase in manufacturing expense related to a ramp up of batches produced for our Limb-girdle muscular dystrophy (“LGMD”) programs, partially offset by a decrease in clinical trial expenses primarily due to our decision to discontinue our PPMO program during 2024.

Research and development expenses decreased by $72.9 million for the twelve months ended December 31, 2024, compared with the same period of 2023. The decrease in research and development expense primarily reflects capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023, partially offset by costs associated with the termination of the development, commercial manufacturing and supply agreement (the “Thermo Agreement”) related to Brammer Bio MA, LLC, an affiliate of Thermo Fisher Scientific, Inc. in August 2024, net of the reimbursable termination costs by Roche.

Non-GAAP research and development expenses increased by $7.6 million for the three months ended December 31, 2024, compared with the same period of 2023. Non-GAAP research and development expenses decreased by $57.4 million for the twelve months ended December 31, 2024, compared with the same period of 2023.

Selling, general and administrative expenses increased by $32.2 million and $76.0 million for the three and twelve months ended December 31, 2024, compared with the same periods of 2023. The increase in selling, general and administrative expenses for both periods is primarily driven by professional services used to support the continued efforts to commercialize ELEVIDYS and ongoing litigation matters, the timing of charitable contributions, and compensation-related expenses, including stock-based compensation, partially due to changes in headcount. Non-GAAP selling, general and administrative expenses increased by $25.9 million and $66.3 million for the three and twelve months ended December 31, 2024, respectively, compared with the same periods of 2023.

For the three months ended December 31, 2024, other income, net decreased by $5.7 million, compared with the same period of 2023, which primarily reflects a decrease in interest income and accretion of investment discount, net as a result of lower interest rates and the investment mix of our investment portfolio during the three months ended December 31, 2024. For the twelve months ended December 31, 2024, other income (loss), net increased by $295.0 million compared with the same period of 2023, which primarily reflects a $387.3 million loss on debt extinguishment, partially offset by a $102.0 million gain on the sale of a Priority Review Voucher (“PRV”) during the twelve months ended December 31, 2023, with no similar activities in 2024.

Income tax expense for the three months ended December 31, 2024, was approximately $12.7 million. Income tax benefit for the three months ended December 31, 2023, was approximately $5.3 million. Income tax expense for the twelve months ended December 31, 2024 and 2023, was approximately $25.5 million and $15.9 million, respectively. Income tax expense (benefit) 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.

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 income (loss) is defined by us as GAAP net income (loss) excluding interest income (expense), net, depreciation and amortization expense, stock-based compensation expense, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items.

2. 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. 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 as the inclusion of dilutive common stock equivalents outstanding is anti-dilutive.
3. 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.
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 impairment of strategic investments, change in fair value of derivatives, gain from sale of the PRV and loss on debt extinguishment and may include other items that fit the above characteristics in the future. We exclude from our non-GAAP results:

a. The impairment of any strategic investments as it is a non-cash item and is not considered to be a normal operating expense due to the variability of amount and lack of predictability as to the occurrence and/or timing of such impairments.
b. The loss on debt extinguishment, which is considered to be an infrequent and non-cash event as it is associated with a distinct financing decision and is not indicative of the performance of our core operations, which accordingly, would make it difficult to compare our results to peer companies that also provide non-GAAP disclosures.
c. The gain from sale of the PRV obtained as a result of the Food and Drug Administration's (“FDA”) accelerated approval of ELEVIDYS in June 2023 as it is a non-recurring event and is not indicative of our core operations.
d. The change in fair value of derivatives related to 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 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 income (loss), non-GAAP net income (loss), and non-GAAP diluted earnings (loss) 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: 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

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 (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 and projections, business plans, market opportunities, priorities and research and development programs and technologies; the potential benefits of our technologies, scientific approaches and strategic partnerships; and expected milestones and plans, including the potential for siRNA programs to reach the market in 2028 and 2029, our expectation to disclose proof of biology readouts in our myotonic dystrophy type 1 (DM1) and facioscapulohumeral muscular dystrophy type 1 (FSHD) programs in 2025, and announcing data from EMERGENE in the first half of 2025 with a potential Biologics License Application (BLA) submission for SRP-9003 in 2025.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's 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: 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; the expected benefits and opportunities related to our agreements with strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations, challenges and uncertainties inherent in product research and development and manufacturing limitations; 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; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve 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, some 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, and 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 for the quarter ended September 30, 2024 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 '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 Income (Loss)
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2024	2023	2024	2023
Revenues:				
Products, net	$ 638,157	$ 365,071	$ 1,787,960	$ 1,144,876
Collaboration and other	20,255	31,710	114,019	98,460
Total revenues	658,412	396,781	1,901,979	1,243,336
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	132,304	44,176	319,099	150,343
Research and development	199,953	195,517	804,522	877,387
Selling, general and administrative	163,873	131,700	557,872	481,871
Amortization of in-licensed rights	601	763	2,405	1,559
Total cost and expenses	496,731	372,156	1,683,898	1,511,160
Operating income (loss)	161,681	24,625	218,081	(267,824)
Other income (loss), net:				
Other income, net	10,062	15,746	42,693	33,055
Gain from sale of Priority Review Voucher	—	—	—	102,000
Loss on debt extinguishment	—	—	—	(387,329)
Total other income (loss), net	10,062	15,746	42,693	(252,274)
Income (loss) before income tax expense (benefit)	171,743	40,371	260,774	(520,098)
Income tax expense (benefit)	12,694	(5,284)	25,535	15,879
Net income (loss)	$ 159,049	$ 45,655	$ 235,239	$ (535,977)
Earnings (loss) per share:				
Basic	$ 1.65	$ 0.49	$ 2.47	$ (5.80)
Diluted	$ 1.50	$ 0.47	$ 2.34	$ (5.80)
Weighted average number of shares of common stock used in computing earnings (loss) per share:				

Basic	96,283	93,617	95,075	92,398
Diluted	108,474	97,494	107,875	92,398

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 December 31,		For the Twelve Months Ended December 31,	
	2024	2023	2024	2023
GAAP net income (loss)	$ 159,049	$ 45,655	$ 235,239	$ (535,977)
Interest income, net	(10,753)	(17,469)	(53,909)	(64,034)
Depreciation and amortization expense	9,854	10,609	35,319	42,838
Stock-based compensation expense	49,676	45,826	184,300	182,514
Change in fair value of derivatives	(727)	—	7,838	1,200
Gain from sale of Priority Review Voucher	—	—	—	(102,000)
Loss on debt extinguishment	—	—	—	387,329
Impairment of strategic investments	—	2,500	—	30,321
Income tax effect of adjustments	(1,092)	(541)	(10,864)	(1,738)
Non-GAAP net income (loss)	$ 206,007	$ 86,580	$ 397,923	$ (59,547)
GAAP earnings (loss) per share - diluted:	$ 1.50	$ 0.47	$ 2.34	$ (5.80)
Add: impact of GAAP to Non-GAAP adjustments	$ 0.40	$ 0.35	$ 1.35	$ 5.16
Non-GAAP earnings (loss) per share - diluted*	$ 1.90	$ 0.82	$ 3.69	$ (0.64)
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:**				
GAAP	108,474	97,494	107,875	92,398
Non-GAAP	108,474	105,594	107,875	92,398

*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 December 31, 2023, 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 December 31,		For the Twelve Months Ended December 31,	
	2024	2023	2024	2023
Total effective tax rate, GAAP	7.4 %	(12.0) %	9.8 %	(3.1) %
Less: impact of GAAP to Non-GAAP adjustments	(1.1)	(0.7)	(1.4)	(39.0)
Total effective tax rate, Non-GAAP	6.3 %	(12.7) %	8.4 %	(42.1) %

Sarepta Therapeutics, Inc.
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures
(unaudited, in thousands)

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2024	2023	2024	2023
GAAP research and development expenses	$ 199,953	$ 195,517	$ 804,522	$ 877,387
Stock-based compensation expense	(19,897)	(22,174)	(74,010)	(82,489)
Depreciation and amortization expense	(7,356)	(8,217)	(26,048)	(33,011)
Non-GAAP research and development expenses	$ 172,700	$ 165,126	$ 704,464	$ 761,887

	For the Three Months Ended December 31,	For the Twelve Months Ended December 31,

	2024	2023	2024	2023
GAAP selling, general and administrative expenses	$ 163,873	$ 131,700	$ 557,872	$ 481,871
Stock-based compensation expense	(29,779)	(23,652)	(110,290)	(100,025)
Depreciation expense	(2,498)	(2,392)	(9,271)	(9,827)
Non-GAAP selling, general and administrative expenses	$ 131,596	$ 105,656	$ 438,311	$ 372,019

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2024	2023	2024	2023
GAAP operating income (loss)	$ 161,681	$ 24,625	$ 218,081	$ (267,824)
Stock-based compensation expense	49,676	45,826	184,300	182,514
Depreciation and amortization expense	9,854	10,609	35,319	42,838
Non-GAAP operating income (loss)	$ 221,211	$ 81,060	$ 437,700	$ (42,472)

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

	As of December 31,	
	2024	2023
Assets		
Current assets:		
Cash and cash equivalents	$ 1,103,010	$ 428,430
Short-term investments	251,782	1,247,820
Accounts receivable, net	601,988	400,327
Inventory	749,960	322,859
Manufacturing-related deposits and prepaids	276,262	102,181
Other current assets	90,461	77,714
Total current assets	3,073,463	2,579,331
Property and equipment, net	340,336	227,154
Right of use assets	148,310	129,952
Non-current inventory	187,986	191,368
Non-current investments	133,163	—
Other non-current assets	79,915	136,771
Total assets	$ 3,963,173	$ 3,264,576
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 214,442	$ 164,918
Accrued expenses	373,513	314,997
Deferred revenue, current portion	130,256	50,416
Current portion of long-term debt	—	105,483
Other current liabilities	13,473	17,845
Total current liabilities	731,684	653,659
Long-term debt	1,137,124	1,132,515
Lease liabilities, net of current portion	192,473	140,965
Deferred revenue, net of current portion	325,000	437,000
Contingent consideration	47,400	38,100
Other non-current liabilities	1,750	3,000
Total liabilities	2,435,431	2,405,239
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; 96,900,496 and 93,731,831 issued and outstanding at December 31, 2024 and 2023, respectively	10	9
Additional paid-in capital	5,738,924	5,304,623
Accumulated other comprehensive (loss) income, net of tax	(218)	918
Accumulated deficit	(4,210,974)	(4,446,213)

Total stockholders' equity	1,527,742	859,337
Total liabilities and stockholders' equity	$ 3,963,173	$ 3,264,576

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