



Sarepta Therapeutics Announces Third Quarter 2024 Financial Results and Recent Corporate Developments

11/6/24

- **Net product revenues for the third quarter 2024 totaled \$429.8 million, a 39% increase over the same quarter of the prior year**
- **ELEVIDYS net product revenue for the quarter totaled \$181.0 million; Royalty revenue from the sales of ELEVIDYS by Roche for the quarter totaled \$9.5 million**
- **Achieved GAAP and non-GAAP net income of \$33.6 million and \$67.0 million for the third quarter 2024, respectively**

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 6, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the third quarter 2024.

"I am pleased to report another strong quarter of performance, as we posted \$429.8 million in net product revenue for the third quarter, a 39% increase over the same quarter of the prior year. Reflecting our detailed preparation and track record of commercial execution, the launch of ELEVIDYS is proceeding to plan. ELEVIDYS net product revenue was \$181.0 million in the quarter, exceeding prior guidance. If one includes royalty revenue generated on Roche's ex-US sales of ELEVIDYS, total ELEVIDYS performance was \$190.5 million for the quarter. Likewise, net product revenue for our three PMOs -- EXONDYS 51, VYONDYS 53, and AMONDYS 45 -- performed well, achieving approximately \$248.8 million in the quarter and, as anticipated, reflecting a lack of near-term cannibalization from the launch of ELEVIDYS," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "At the same time, we have made important decisions regarding portfolio prioritization and have made great progress on our pipeline in the quarter. As examples, by mid-2025 we will have submitted a Biologics License Application for one and will be in clinical trials for two others of our Limb-girdle muscular dystrophy programs. Assuming success, these three therapeutic candidates will deliver our next wave of approved therapies as our multi-program platform advances productively."

Third Quarter 2024 and Recent Developments:

- Sarepta has made the decision to discontinue the SRP-5051 (vesleteplirsen) development program. This decision was informed by information available to date, including the risk-benefit of the program, feedback from the FDA, and the evolving therapeutic landscape for Duchenne.
- **Presented new data from its neuromuscular portfolio at 2024 World Muscle Society Congress (WMS 2024):** Among the multiple presentations from Sarepta at WMS 2024 were new safety and efficacy results from several studies in the SRP-9001 clinical development program. These data included:
 - Skeletal muscle MRI data from Study 9001-301 EMBARK where multiple muscles from a subset of patients (n=39) were evaluated using MRI/MRS and MRI T2 signal prior to treatment and at 52 weeks post treatment. Across all measures, patients treated with SRP-9001 showed improvement over the placebo group with less accumulation of fat and fibrosis. The finding correlates with the functional outcomes in Part 1 of EMBARK which showed stabilization or slowing of disease progression in patients treated with SRP-9001 compared to placebo.
 - Cardiac MRI data from EMBARK where an assessment of a subgroup of patients (n=19), found no negative effects on cardiac safety compared to placebo at 52 weeks and no differences in cardiac measures in SRP-9001-treated patients compared to the placebo group at one year. Sarepta plans for future longitudinal cardiac MRI studies to evaluate the long-term protection of cardiac muscle.
 - Five-year functional results from Study SRP-9001-101, the longest-term data to date for a gene therapy in Duchenne. These patients, with an average age of 10 years at the time of the assessment, were stable or showed a slowing of disease progression with an increase in divergence from natural history over time as shown by an external control analysis. Notably this evaluation was conducted at an age when many Duchenne patients are entering the steep decline phase of the disease. The five-year results showed:
 - A 9.8 point (p=0.0127) increase in change from baseline in the North Star Ambulatory Assessment (NSAA) total score to five years.
 - At year five the sustained increase in NSAA total score was statistically significant and clinically meaningful when compared to an external control cohort.
 - An 8.8 second decrease (p=0.0198) in time to rise from floor, change from baseline to five years. The improvement was statistically significant and clinically meaningful when compared to an external control cohort.
 - Patients maintained their 10-meter walk test time throughout the five years.
 - No new safety signals.
- **Results from EMBARK study of delandistrogene moxeparvovec published in *Nature Medicine*:** In early October, efficacy and safety results from Part 1 of the EMBARK study of delandistrogene moxeparvovec-rokl for the treatment of Duchenne muscular dystrophy were published in *Nature Medicine*. These published results demonstrate a treatment

benefit with delandistrogene moxeparvovec that is clinically meaningful and similar regardless of age and a favorable risk-benefit profile. EMBARK, also known as Study SRP-9001-301, is a global, randomized, double-blind, placebo-controlled, Phase 3 clinical study of delandistrogene moxeparvovec in patients with Duchenne muscular dystrophy between the ages of 4 through 7 years.

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.

Q3 2024 Financial Highlights¹

	For the Three Months Ended September 30,		QTD Change	
	2024	2023		
	(in millions, except for per share amounts)		\$	%
Total Revenues	\$ 467.2	\$ 331.8	135.4	41%
Operating income (loss):				
GAAP	\$ 22.2	\$ (20.8)	43.0	NM*
Non-GAAP	\$ 74.9	\$ 37.7	37.2	99%
Net income (loss):				
GAAP	\$ 33.6	\$ (40.9)	74.5	NM*
Non-GAAP	\$ 67.0	\$ 31.5	35.5	113%
Diluted earnings (loss) per share:				
GAAP	\$ 0.34	\$ (0.46)	0.80	NM*
Non-GAAP	\$ 0.62	\$ 0.31	0.31	99%

	For the Nine Months Ended September 30,		YTD Change	
	2024	2023		
	(in millions, except for per share amounts)		\$	%
Total Revenues	\$ 1,243.6	\$ 846.6	397.0	47%
Operating income (loss):				
GAAP	\$ 56.4	\$ (292.4)	348.8	NM*
Non-GAAP	\$ 216.5	\$ (123.5)	340.0	NM*
Net income (loss):				
GAAP	\$ 76.2	\$ (581.6)	657.8	NM*
Non-GAAP	\$ 191.9	\$ (146.1)	338.0	NM*
Diluted earnings (loss) per share:				
GAAP	\$ 0.78	\$ (6.56)	7.34	NM*
Non-GAAP	\$ 1.78	\$ (1.65)	3.43	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 September 30, 2024	As of December 31, 2023
	(in millions)	
Cash, cash equivalents, restricted cash and investments	\$ 1,395.8	\$ 1,691.8

Revenues

Total revenues increased by \$135.4 million for the three months ended September 30, 2024, compared to the same period of 2023. The increase primarily reflects the initial product launch of ELEVIDYS in June 2023 and expanded label in June 2024, partially offset by a \$22.5 million decrease in

the amortization of the single, combined performance obligation under our collaboration agreement with F. Hoffman-La Roche Ltd. ("Roche"), which was fully amortized as of December 31, 2023.

Total revenues increased by \$397.0 million for the nine months ended September 30, 2024, compared to the same period of 2023. The increase primarily reflects the initial product launch of ELEVIDYS in June 2023 and expanded label in June 2024, as well as the \$48.0 million of collaboration revenue recognized related to an option declined by Roche to acquire the ex-US rights to a certain external, early stage Duchenne-specific program, partially offset by a \$66.8 million decrease in the amortization of the single, combined performance obligation under our collaboration agreement with Roche, which was fully amortized as of December 31, 2023.

Additionally, included in total revenues for the three and nine months ended September 30, 2024, is \$37.4 million and \$45.8 million, respectively, of contract manufacturing and other revenues associated with commercial ELEVIDYS supply delivered to Roche and royalty revenue received from Roche, with no similar activity for the same periods of 2023.

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

Cost of sales (excluding amortization of in-license rights) increased by \$54.7 million and \$80.6 million for the three and nine months ended September 30, 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 expanded label in June 2024. For the three and nine months ended September 30, 2024, we recognized \$13.7 million and \$15.4 million of cost of sales related to products sold to Roche under our collaboration agreement, with no similar activity for the same periods of 2023.

Operating expenses and others

Research and development expenses increased by \$30.2 million for the three months ended September 30, 2024, compared with the same period of 2023. The increase in research and development expense primarily reflects \$55.4 million of 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, partially offset by a decrease in clinical and manufacturing activity for our PPMO platform and Eteplirsen program.

Research and development expenses decreased by \$77.3 million for the nine months ended September 30, 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 \$55.4 million of costs associated with the termination of the Thermo Agreement, net of the reimbursable termination costs by Roche.

Non-GAAP research and development expenses increased by \$35.9 million for the three months ended September 30, 2024, compared with the same period of 2023. Non-GAAP research and development expenses decreased by \$65.0 million for the nine months ended September 30, 2024, compared with the same period of 2023.

Selling, general and administrative expenses increased by \$7.3 million and \$43.8 million for the three and nine months ended September 30, 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, compensation due to changes in headcount, as well as the achievement of performance conditions related to certain Performance Stock Units. Non-GAAP selling, general and administrative expenses increased by \$7.4 million and \$40.3 million for the three and nine months ended September 30, 2024, respectively, compared with the same periods of 2023.

For the three months ended September 30, 2024, other income (loss), net increased by \$24.1 million, compared with the same period of 2023, which primarily reflects an impairment of a strategic investment during the three months ended September 30, 2023, with no similar activity in 2024. For the nine months ended September 30, 2024, other income (loss), net increased by \$300.6 million compared with the same period of 2023, which primarily reflects a \$387.3 million loss on debt extinguishment offset by a \$102.0 million gain on the sale of a Priority Review Voucher ("PRV") during the nine months ended September 30, 2023, with no similar activities in 2024.

Income tax expense for the three months ended September 30, 2024 and 2023, was approximately \$0.4 million and \$7.8 million, respectively. Income tax expense for the nine months ended September 30, 2024 and 2023, was approximately \$12.8 million and \$21.2 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.

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 income (loss) is defined by us as GAAP net income (loss) excluding interest income, net, depreciation and amortization expense, stock-based compensation expense, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items.
2. Non-GAAP earnings (loss) per share is defined by us as non-GAAP net income (loss), as defined previously, divided by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. The non-GAAP earnings per share is calculated using diluted shares whereas the non-GAAP net loss per share is calculated using basic shares as all other instruments are 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, 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:
 - 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.
 - 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.
 - The gain from sale of the PRV obtained as a result of the FDA accelerated approval of ELEVIDYS in June 2023 as it is a non-recurring event.
 - 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 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.
4. Beginning in the fourth quarter of 2023, amortization of in-licensed rights (formerly included within depreciation and amortization expense) and income tax (benefit) expense are no longer excluded from the non-GAAP results. We now include the income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable effective income tax rate. Non-GAAP financial results for the for the three and nine months ended September 30, 2023 have been updated to reflect this change for comparability.

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 net 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 $\geq 5\%$) 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 and scientific approaches, including the potential benefits of SRP-9003; and expected milestones and plans, including our expectation that by mid-2025 we will have submitted a Biologics License Application for one, and will be in clinical trials for two others, of our Limb-girdle muscular dystrophy programs.

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; 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 Annual Report on Form 10-K for the year ended December 31, 2023, and 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 '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		For the Nine Months Ended	
	September 30,		September 30,	
	2024	2023	2024	2023
Revenues:				
Products, net	\$ 429,771	\$ 309,322	\$ 1,149,803	\$ 779,805
Collaboration and other	37,401	22,495	93,764	66,750
Total revenues	<u>467,172</u>	<u>331,817</u>	<u>1,243,567</u>	<u>846,555</u>
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	91,691	37,026	186,795	106,167
Research and development	224,483	194,301	604,569	681,870
Selling, general and administrative	128,200	120,893	393,999	350,171
Amortization of in-licensed rights	602	439	1,804	796
Total cost and expenses	<u>444,976</u>	<u>352,659</u>	<u>1,187,167</u>	<u>1,139,004</u>
Operating income (loss)	<u>22,196</u>	<u>(20,842)</u>	<u>56,400</u>	<u>(292,449)</u>
Other income (loss), net:				
Other income (expense), net	11,810	(12,332)	32,631	17,309
Gain from sale of Priority Review Voucher	—	—	—	102,000
Loss on debt extinguishment	—	—	—	(387,329)
Total other income (loss), net	<u>11,810</u>	<u>(12,332)</u>	<u>32,631</u>	<u>(268,020)</u>
Income (loss) before income tax expense	34,006	(33,174)	89,031	(560,469)
Income tax expense	395	7,763	12,841	21,163
Net income (loss)	<u>\$ 33,611</u>	<u>\$ (40,937)</u>	<u>\$ 76,190</u>	<u>\$ (581,632)</u>
Earnings (loss) per share:				
Basic	\$ 0.35	\$ (0.46)	\$ 0.80	\$ (6.56)
Diluted	\$ 0.34	\$ (0.46)	\$ 0.78	\$ (6.56)
Weighted average number of shares of common stock used in computing earnings (loss) per share:				
Basic	95,390	88,889	94,669	88,609
Diluted	100,448	88,889	99,572	88,609

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 September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
GAAP net income (loss)	\$ 33,611	\$ (40,937)	\$ 76,190	\$ (581,632)
Interest income, net	(13,415)	(17,593)	(43,156)	(46,565)
Depreciation and amortization expense*	9,204	10,489	25,465	32,229
Stock-based compensation expense	43,450	48,061	134,624	136,688
Change in fair value of derivatives	(1,535)	2,000	8,565	1,200
Gain from sale of Priority Review Voucher	—	—	—	(102,000)
Loss on debt extinguishment	—	—	—	387,329
Impairment of strategic investments	—	27,500	—	27,821
Income tax effect of adjustments**	(4,300)	1,992	(9,772)	(1,197)
Non-GAAP net income (loss)**	<u>\$ 67,015</u>	<u>\$ 31,512</u>	<u>\$ 191,916</u>	<u>\$ (146,127)</u>
GAAP net earnings (loss) per share - diluted:	\$ 0.34	\$ (0.46)	\$ 0.78	\$ (6.56)
Add: impact of GAAP to Non-GAAP adjustments	\$ 0.28	\$ 0.77	\$ 1.00	\$ 4.91
Non-GAAP net earnings (loss) per share - diluted***	<u>\$ 0.62</u>	<u>\$ 0.31</u>	<u>\$ 1.78</u>	<u>\$ (1.65)</u>
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:****				
GAAP	100,448	88,889	99,572	88,609
Non-GAAP	108,548	101,722	107,672	88,609

*Beginning in the fourth quarter of 2023, depreciation and amortization excludes amortization of in-licensed rights. Non-GAAP financial results for the three and nine months ended September 30, 2023, have been updated to reflect this change for comparability.

**Beginning in the fourth quarter of 2023, income tax (benefit) expense is no longer excluded from the non-GAAP results. We have replaced this metric with income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable statutory income tax rate. Refer below for a reconciliation of effective tax rates. Non-GAAP financial results for the three and nine months ended September 30, 2023, have been updated to reflect this change for comparability.

***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. There was a \$0.04 impact to the calculation of non-GAAP net earnings per share as a result of the inclusion of diluted shares for the three months ended September 30, 2023.

****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 and nine months ended September 30, 2024, is a result of the exclusion of the potential share settlement of the 2027 Notes from the GAAP earnings per share as the inclusion of such shares was anti-dilutive.

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
Total effective tax rate, GAAP	1.2 %	(23.4) %	14.4 %	(3.8) %
Less: impact of GAAP to Non-GAAP adjustments	6.2	(16.7)	(3.8)	(24.4)
Total effective tax rate, Non-GAAP	<u>7.4 %</u>	<u>(40.1) %</u>	<u>10.6 %</u>	<u>(28.2) %</u>

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
GAAP research and development expenses	\$ 224,483	\$ 194,301	\$ 604,569	\$ 681,870
Stock-based compensation expense	(18,034)	(22,325)	(54,113)	(60,315)
Depreciation and amortization expense	(6,664)	(8,109)	(18,692)	(24,794)
Non-GAAP research and development expenses	<u>\$ 199,785</u>	<u>\$ 163,867</u>	<u>\$ 531,764</u>	<u>\$ 596,761</u>
	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
GAAP selling, general and administrative expenses	\$ 128,200	\$ 120,893	\$ 393,999	\$ 350,171

Stock-based compensation expense	(25,416)	(25,736)	(80,511)	(76,373)
Depreciation expense	(2,540)	(2,380)	(6,773)	(7,435)
Non-GAAP selling, general and administrative expenses	<u>\$ 100,244</u>	<u>\$ 92,777</u>	<u>\$ 306,715</u>	<u>\$ 266,363</u>
	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2024	2023	2024	2023
GAAP operating income (loss)	\$ 22,196	\$ (20,842)	\$ 56,400	\$ (292,449)
Stock-based compensation expense	43,450	48,061	134,624	136,688
Depreciation and amortization expense	9,204	10,489	25,465	32,229
Non-GAAP operating income (loss)	<u>\$ 74,850</u>	<u>\$ 37,708</u>	<u>\$ 216,489</u>	<u>\$ (123,532)</u>

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

	<u>As of</u> <u>September 30, 2024</u>	<u>As of</u> <u>December 31, 2023</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 197,855	\$ 428,430
Short-term investments	1,000,534	1,247,820
Accounts receivable, net	434,524	400,327
Inventory	565,924	322,859
Manufacturing-related deposits and prepaids	321,055	102,181
Other current assets	165,477	77,714
Total current assets	<u>2,685,369</u>	<u>2,579,331</u>
Property and equipment, net	305,788	227,154
Right of use assets	140,898	129,952
Non-current inventory	202,550	191,368
Non-current investments	181,770	—
Other non-current assets	83,559	136,771
Total assets	<u>\$ 3,599,934</u>	<u>\$ 3,264,576</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 118,774	\$ 164,918
Accrued expenses	344,830	314,997
Deferred revenue, current portion	127,001	50,416
Current portion of long-term debt	91,595	105,483
Other current liabilities	17,289	17,845
Total current liabilities	<u>699,489</u>	<u>653,659</u>
Long-term debt	1,135,965	1,132,515
Lease liabilities, net of current portion	170,009	140,965
Deferred revenue, net of current portion	325,000	437,000
Contingent consideration	47,400	38,100
Other non-current liabilities	1,000	3,000
Total liabilities	<u>2,378,863</u>	<u>2,405,239</u>
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; 95,493,005 and 93,731,831 issued and outstanding at September 30, 2024, and December 31, 2023, respectively	10	9
Additional paid-in capital	5,588,839	5,304,623
Accumulated other comprehensive income, net of tax	2,245	918
Accumulated deficit	<u>(4,370,023)</u>	<u>(4,446,213)</u>
Total stockholders' equity	<u>1,221,071</u>	<u>859,337</u>
Total liabilities and stockholders' equity	<u>\$ 3,599,934</u>	<u>\$ 3,264,576</u>

Source: Sarepta Therapeutics, Inc.

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Source: Sarepta Therapeutics, Inc.