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

8/7/24

- ***Net product revenues for the second quarter 2024 totaled $360.5 million, a 51% increase over the same quarter of the prior year***
- ***ELEVIDYS net product revenue for the quarter totaled $121.7 million; Royalty revenue from the sales of ELEVIDYS by Roche for the quarter totaled $2.4 million***
- ***Achieved GAAP net income of $6.5 million for the second quarter 2024 and non-GAAP net income of $46.7 million for the same period***
- ***Sarepta provides net product revenues guidance of $2.9 billion to $3.1 billion for 2025***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 7, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the second quarter 2024.

"The second quarter of 2024 represents the most significant achievement in the advancement of medicine for Duchenne since researchers identified that lack of dystrophin was the underlying cause of Duchenne in 1986. Based on a wealth of compelling clinical evidence, the FDA broadened access to our gene therapy ELEVIDYS for all patients at least 4 years of age, with a traditional approval for ambulatory patients and accelerated approval for all non-ambulatory patients. We look forward to reviewing the comprehensive data supporting the safety and efficacy of ELEVIDYS at the 29th Annual Congress of the World Muscle Society taking place in October, including muscle and cardiac MRI data and other biomarker results showing improvement in muscle health of treated patients," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "Sarepta is second to no other organization in the world in its ability to launch Duchenne therapies and support the community with education, access and reimbursement. We were well prepared for all aspects of this broadened launch and all signals thus far exceed even our optimistic expectations. To that point, second quarter total net product revenues across our four approved therapies were $360.5 million, a 51% increase over the same quarter last year. Our PMO products - EXONDYS 51, VYONDYS 53 and AMONDYS 45 - continue to perform, contributing $238.8 million in net product revenues. ELEVIDYS achieved $121.7 million in net product revenue, which positions us well for significant growth in the latter half of 2024 as families complete the 3-to-5-month process from enrollment form to infusion. Tracking our launch beyond 2024, based on our early launch signals and continuing performance of our PMOs, we anticipate that net product revenues in 2025 will be in the range of $2.9 to $3.1 billion."

Second Quarter 2024 and Recent Developments:

- **U.S. FDA expands labeled indication for ELEVIDYS**: On June 20, 2024, Sarepta announced U.S. Food and Drug Administration (FDA) approval of an expansion to the labeled indication for ELEVIDYS (delandistrogene moxeparvovec-rokl) to include individuals with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene who are at least 4 years of age. Confirming the functional benefits, the FDA granted traditional approval for ambulatory patients. The FDA granted accelerated approval for non-ambulatory patients. Continued approval for non-ambulatory Duchenne patients may be contingent upon verification of clinical benefit in a confirmatory trial. Consistent with the accelerated approval pathway, Sarepta has committed to conduct and submit the results of a randomized, controlled trial to verify and confirm the clinical benefit of ELEVIDYS in patients with Duchenne who are non-ambulatory. ENVISION (Study SRP-9001-303), a global, randomized, double-blind, placebo-controlled Phase 3 study of ELEVIDYS in non-ambulatory and older ambulatory individuals with Duchenne, is underway and intended to serve as this postmarketing requirement.
- **Sarepta's partner Roche announced that the European Medicines Agency (EMA) has initiated review of the ELEVIDYS marketing authorization application (MAA) for the treatment of Duchenne:** On June 24, 2024, Roche announced that the EMA has initiated the review of the MAA for ELEVIDYS for the treatment of ambulatory patients ages 3 to 7 years and is expecting approval in 2025. Roche is responsible for commercialization of ELEVIDYS outside of the United States.
- **Sarepta to present data from its SRP-9001 program at the 29th Annual Congress of the World Muscle Society (WMS 2024):** In October, the Company will share data from its SRP-9001 program at WMS 2024, taking place Oct. 8-12, 2024, in Prague, Czechia. The full program is available at: https://www.wms2024.com/page/programme. This list does not include Sarepta's encore or pipeline presentations nor does it include late-breaker submissions. The following have been accepted as regular submissions for SRP-9001:

Presentation #	Title	Date, Time
19O	Muscle MRI outcomes in patients with Duchenne Muscular Dystrophy treated with delandistrogene moxeparvovec: Findings from EMBARK Part 1	Oct. 12, 2024 1:45-2:00 AM EDT 7:45-8:00 AM CET

428P	Cardiac MRI outcomes in patients with Duchenne Muscular Dystrophy treated with delandistrogene moxeparvovec: Findings from EMBARK Part 1	Oct. 9, 2024 11:15 AM-12:15 PM EDT 5:15-6:15 PM CET
424P	Micro-dystrophin expression and safety with delandistrogene moxeparvovec gene therapy for DMD in a broad population: Phase 1b trial (ENDEAVOR)	
425P	Five-year outcomes with delandistrogene moxeparvovec in patients with Duchenne Muscular Dystrophy (DMD): a phase 1/2a study	

- **Execution on the ELEVIDYS long-term follow-up studies:** The long-term follow-up studies for ELEVIDYS include ENDURE, a Phase 4 observational study that will follow individuals treated with ELEVIDYS for up to 10 years, and EXPEDITION, a Phase 3 study enrolling approximately 400 patients who were previously enrolled in ELEVIDYS clinical trials and followed for consistent safety and efficacy measures for up to 5 years.
- **U.S. FDA grants Fast Track designation to SRP-9003 (bidridistrogene xeboparvovec):** The Company announced that they received Fast Track designation for SRP-9003 an investigational gene therapy being developed for the treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E/R4), or beta sarcoglycanopathy. SRP-9003 is intended to deliver a full-length beta-sarcoglycan transgene and uses the MHCK7 promoter, chosen for its ability to robustly express in the heart, which is critically important for patients with LGMD2E/R4, many of whom die from pulmonary or cardiac complications. The Fast Track designation is a process designed to facilitate the development and expedited review of drugs that treat serious conditions and fill unmet medical needs.
- **Jerry R. Mendell, M.D., named to TIME100 Health inaugural list of 2024 most influential people in global health:** TIME named renown neuromuscular researcher, physician and gene therapy pioneer Jerry R. Mendell, M.D., to the inaugural 2024 TIME100 Health, a list of 100 individuals who most influenced global health this year. Dr. Mendell was recognized for his lifetime commitment to neuromuscular disease and achievements in genetic medicine that propelled a new age of treatment for several genetic diseases, including Duchenne muscular dystrophy. The TIME100 Health list recognizes the impact, innovation, and achievement of the world's most influential individuals in health. TIME selected a community of leaders across industries dedicated to creating tangible and credible change for a healthier population, including pioneering scientists who are steering the evolution of global health in 2024.

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.

Q2 2024 Financial Highlights[1]

	For the Three Months Ended June 30,		QTD Change	
	2024	2023		
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 362.9	$ 261.2	101.7	39%
Operating (loss) income:				
GAAP	$ (0.7)	$ (133.5)	132.8	(99%)
Non-GAAP	$ 57.9	$ (75.5)	133.4	NM*
Net income (loss):				
GAAP	$ 6.5	$ (23.9)	30.4	NM*
Non-GAAP	$ 46.7	$ (89.9)	136.6	NM*
Diluted earnings (loss) per share:				
GAAP	$ 0.07	$ (0.27)	0.34	NM*
Non-GAAP	$ 0.44	$ (1.01)	1.45	NM*

	For the Six Months Ended June 30,		YTD Change	
	2024	2023		
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 776.4	$ 514.7	261.7	51%
Operating income (loss):				
GAAP	$ 34.2	$ (271.6)	305.8	NM*
Non-GAAP	$ 141.6	$ (161.2)	302.8	NM*
Net income (loss):				

GAAP	$ 42.6	$ (540.7)	583.3	NM*
Non-GAAP	$ 124.9	$ (177.6)	302.5	NM*
Diluted earnings (loss) per share:				
GAAP	$ 0.44	$ (6.11)	6.55	NM*
Non-GAAP	$ 1.16	$ (2.01)	3.17	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 June 30, 2024	As of December 31, 2023
	(in millions)	
Cash, cash equivalents, restricted cash and investments	$ 1,476.1	$ 1,691.8

Revenues
Total revenues increased by $101.7 million for the three months ended June 30, 2024, compared to the same period of 2023. The increase primarily reflects the product launch of ELEVIDYS in June 2023, partially offset by a $22.3 million decrease in the amortization of the single, combined performance obligation under our collaboration agreement with F. Hoffman-La Roche Ltd.'s (“Roche”), which was fully amortized as of December 31, 2023.

Total revenues increased by $261.7 million for the six months ended June 30, 2024, compared to the same period of 2023. The increase primarily reflects the product launch of ELEVIDYS in June 2023, 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.

Additionally, for the three and six months ended June 30, 2024, we recognized $2.4 million and $8.4 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 $10.4 million and $26.0 million for the three and six months ended June 30, 2024, compared with the same periods of 2023. The increase in both periods primarily reflects the product launch of ELEVIDYS in June 2023.

Operating expenses and others
Research and development expenses decreased by $62.2 million and $107.5 million for the three and six months ended June 30, 2024, compared with the same periods of 2023. The decreases in research and development expenses for both periods primarily reflect capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023. Non-GAAP research and development expenses decreased by $58.3 million and $100.9 million for the three and six months ended June 30, 2024, compared with the same periods of 2023.

Selling, general and administrative expenses increased by $20.2 million and $36.5 million for the three and six months ended June 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 for the launch of ELEVIDYS and ongoing litigation matters, the timing of charitable contributions, as well as the achievement of performance conditions related to certain Performance Stock Units. Non-GAAP selling, general and administrative expenses increased by $15.7 million and $32.9 million for the three and six months ended June 30, 2024, compared with the same periods of 2023.

For the three months ended June 30, 2024, other income, net decreased by $104.6 million, compared with the same period of 2023, which primarily reflects a $102.0 million decrease in the gain on sale of a Priority Review Voucher (“PRV”) period over period. For the six months ended June 30, 2024, other income (loss), net increased by $276.5 million compared with the same period of 2023, which primarily reflects a $387.3 million loss on debt extinguishment that occurred during the six months ended June 30, 2023, offset by a $102.0 million gain on sale of a PRV, with no similar activities in 2024.

Income tax expense for the three months ended June 30, 2024 and 2023, was approximately $7.1 million and $9.4 million, respectively. Income tax expense for the six months ended June 30, 2024 and 2023, was approximately $12.4 million and $13.4 million, respectively. Income tax expense for all periods presented primarily relates to state, federal and foreign income taxes.

Use of Non-GAAP Measures
In addition to the GAAP financial measures set forth in this press release, we have included certain non-GAAP measurements. The 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. The non-GAAP earnings (loss) per share is defined by us as non-GAAP 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. The non-GAAP operating income (loss) is defined by us as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined by us as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by us as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items.

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 contingent consideration, net, 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.
- We exclude from our non-GAAP results 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.
- We exclude from our non-GAAP results 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.
- We exclude from our non-GAAP results the change in fair value of contingent consideration, net related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to academic institutions under separate license agreements as it is a non-cash item and is not considered to be normal operating expenses due to its variability of amounts and lack of predictability as to occurrence and/or timing.

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 six months ended June 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 (loss) income, 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.

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, products and technologies; the potential benefits of our technologies and scientific approaches; our expectation that net product revenues in 2025 will be in the range of $2.9 to $3.1 billion; the potential for our partner Roche to receive approval from the EMA in 2025 for ELEVIDYS for the treatment of ambulatory patients ages 3 to 7 years; and expected milestones and plans, including reviewing the comprehensive data supporting the safety and efficacy of ELEVIDYS at the 29th Annual Congress of the World Muscle Society taking place in October.

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: 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; 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; 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, changes in coverage and reimbursement policies of health plans and health insurers, 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 June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
Revenues:				
Products, net	$ 360,548	$ 238,988	$ 720,032	$ 470,483
Collaboration and other	2,383	22,250	56,363	44,255
Total revenues	362,931	261,238	776,395	514,738
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	44,545	34,124	95,104	69,141
Research and development	179,690	241,890	380,086	487,569
Selling, general and administrative	138,796	118,564	265,799	229,278
Amortization of in-licensed rights	601	179	1,202	357
Total cost and expenses	363,632	394,757	742,191	786,345
Operating (loss) income	(701)	(133,519)	34,204	(271,607)
Other income (loss), net:				
Other income, net	14,278	16,934	20,821	29,641
Gain from sale of Priority Review Voucher	—	102,000	—	102,000
Loss on debt extinguishment	—	—	—	(387,329)
Total other income (loss), net	14,278	118,934	20,821	(255,688)
Income (loss) before income tax expense	13,577	(14,585)	55,025	(527,295)
Income tax expense	7,117	9,355	12,446	13,400
Net income (loss)	$ 6,460	$ (23,940)	$ 42,579	$ (540,695)
Earnings (loss) per share:				
Basic	$ 0.07	$ (0.27)	$ 0.45	$ (6.11)
Diluted	$ 0.07	$ (0.27)	$ 0.44	$ (6.11)
Weighted average number of shares of common stock used in computing earnings (loss) per share:				
Basic	94,618	88,743	94,305	88,466
Diluted	99,144	88,743	99,129	88,466

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 June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
GAAP net income (loss)	$ 6,460	$ (23,940)	$ 42,579	$ (540,695)
Interest income, net	(14,010)	(15,980)	(29,741)	(28,972)
Depreciation and amortization expense*	8,118	10,613	16,261	21,740
Stock-based compensation expense	50,482	47,377	91,174	88,627
Change in fair value on contingent consideration	—	(800)	10,100	(800)
Gain from sale of Priority Review Voucher	—	(102,000)	—	(102,000)
Loss on debt extinguishment	—	—	—	387,329
Impairment of strategic investments	—	—	—	321
Income tax effect of adjustments**	(4,389)	(5,172)	(5,472)	(3,189)
Non-GAAP net income (loss)**	$ 46,661	$ (89,902)	$ 124,901	$ (177,639)
GAAP net earnings (loss) per share - diluted:	$ 0.07	$ (0.27)	$ 0.44	$ (6.11)

Add: impact of GAAP to Non-GAAP adjustments	$ 0.37	$ (0.74)	$ 0.73	$ 4.10
Non-GAAP net earnings (loss) per share - diluted***	$ 0.44	$ (1.01)	$ 1.16	$ (2.01)
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:****				
GAAP	99,144	88,743	99,129	88,466
Non-GAAP	107,245	88,743	107,230	88,466

*Beginning in the fourth quarter of 2023, depreciation and amortization excludes amortization of in-licensed rights. Non-GAAP financial results for the three and six months ended June 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 six months ended June 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.
****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 six months ended June 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 June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
Total effective tax rate, GAAP	8.4%	(1.7)%	22.6%	(2.5)%
Less: impact of GAAP to Non-GAAP adjustments	(4.6)	(6.2)	(10.0)	(7.8)
Total effective tax rate, Non-GAAP	3.8%	(7.9)%	12.6%	(10.3)%

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

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
GAAP research and development expenses	$ 179,690	$ 241,890	$ 380,086	$ 487,569
Stock-based compensation expense	(19,806)	(21,577)	(36,079)	(37,990)
Depreciation and amortization expense	(5,982)	(8,134)	(12,028)	(16,685)
Non-GAAP research and development expenses	$ 153,902	$ 212,179	$ 331,979	$ 432,894

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
GAAP selling, general and administrative expenses	$ 138,796	$ 118,564	$ 265,799	$ 229,278
Stock-based compensation expense	(30,676)	(25,800)	(55,095)	(50,637)
Depreciation expense	(2,136)	(2,479)	(4,233)	(5,055)
Non-GAAP selling, general and administrative expenses	$ 105,984	$ 90,285	$ 206,471	$ 173,586

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
GAAP operating (loss) income	$ (701)	$ (133,519)	$ 34,204	$ (271,607)
Stock-based compensation expense	50,482	47,377	91,174	88,627
Depreciation and amortization expense	8,118	10,613	16,261	21,740
Non-GAAP operating income (loss)	$ 57,899	$ (75,529)	$ 141,639	$ (161,240)

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

	As of June 30, 2024	As of December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 383,622	$ 428,430
Short-term investments	1,076,852	1,247,820
Accounts receivable, net	359,997	400,327
Inventory	485,795	322,859
Manufacturing-related deposits and prepaids	302,627	102,181
Other current assets	74,743	77,714
Total current assets	2,683,636	2,579,331
Property and equipment, net	276,200	227,154
Right of use assets	124,001	129,952
Non-current inventory	204,691	191,368
Other non-current assets	135,729	136,771
Total assets	$ 3,424,257	$ 3,264,576
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 107,417	$ 164,918
Accrued expenses	350,404	314,997
Deferred revenue, current portion	122,036	50,416
Current portion of long-term debt	91,505	105,483
Other current liabilities	17,128	17,845
Total current liabilities	688,490	653,659
Long-term debt	1,134,810	1,132,515
Lease liabilities, net of current portion	143,601	140,965
Deferred revenue, net of current portion	325,000	437,000
Contingent consideration	48,200	38,100
Other non-current liabilities	7,087	3,000
Total liabilities	2,347,188	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; 95,282,600 and 93,731,831 issued and outstanding at June 30, 2024, and December 31, 2023, respectively	10	9
Additional paid-in capital	5,481,723	5,304,623
Accumulated other comprehensive (loss) income, net of tax	(1,030)	918
Accumulated deficit	(4,403,634)	(4,446,213)
Total stockholders' equity	1,077,069	859,337
Total liabilities and stockholders' equity	$ 3,424,257	$ 3,264,576

View source version on businesswire.com: https://www.businesswire.com/news/home/20240807747128/en/

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

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

Source: Sarepta Therapeutics, Inc.