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

8/6/25

- ***ELEVIDYS shipments have resumed for ambulatory individuals living with Duchenne following notification from the U.S. Food and Drug Administration (FDA); continuing to work with FDA on safety labeling process and risk-mitigation approach for non-ambulatory individuals***
- ***Net product revenues for the second quarter 2025 totaled $513.1 million, a 42% increase over the same quarter of the prior year***
- ***Previously announced restructuring advancing as planned; on track to realize over $100 million in cost savings through the end of 2025***
- ***Multiple clinical data readouts and milestones expected in 2025 and into early 2026 to support siRNA franchise - FSHD, DM1, SCA2, and Huntington's Disease***

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

"We are very pleased that following a rapid review of the safety data, the FDA swiftly recommended that we take the ambulatory patient population off shipment pause and, following that, we have already resumed deliveries. Infusions are taking place for the ambulatory community. We will continue to work with the FDA to define the path and develop the risk mitigation necessary to bring ELEVIDYS back to the non-ambulatory community as well," said Doug Ingram, chief executive officer, Sarepta. "With the temporary pause lifted, the execution of our restructuring, cost savings and plans designed to meet our financial obligations, and with important siRNA readouts expected later this year, we are well positioned to achieve our strategic objective to remain a patient-centric, financially disciplined organization into the next decade and beyond."

Corporate Highlights

ELEVIDYS Updates

- On July 28, 2025, the FDA recommended that Sarepta remove its voluntary pause and resume shipments of ELEVIDYS (delandistrogene moxeparvovec) for ambulatory individuals living with Duchenne. The notification came after the FDA completed its review of ELEVIDYS safety data.
- Shipments have resumed so that patient infusions can continue.
- Discussions between FDA and Sarepta are underway on next steps in the safety labeling process for acute liver injury/acute liver failure (ALI/ALF) and risk-mitigation approach for non-ambulatory individuals living with Duchenne.

Financial Updates

- In the second quarter of 2025, the Company delivered strong financial results, reporting both GAAP and non-GAAP operating profit. The quarter was cash flow positive and total cash, cash equivalents and short- and long-term investments increased by $202.8 million from the previous quarter.
- The Company is taking steps to further strengthen its financial foundation and is focused on proactive liability management. Among many things, it is evaluating opportunities to enhance operational efficiency and adjust manufacturing commitments based on latest demand to further strengthen our liquidity position.
- The strategic restructuring announced on July 16, 2025, will sharpen the Company's focus on high-impact programs, particularly its siRNA platform, while reducing expenses by approximately $400 million annually starting in 2026. These strategic cost reductions and pipeline prioritization are designed to position Sarepta to repay its 2027 convertible notes and continue delivering transformative therapies to patients with rare genetic diseases.

Expected Near-Term Milestones (2025/2026)

- **Facioscapulohumeral muscular dystrophy (FSHD)**
  - Study Status: Phase 1/2 study of SRP-1001 FSHD1 patients underway; Cohorts 1, 2, and 3 in part 1 are fully enrolled in the single ascending dose (SAD) study
  - Next Milestone: Preliminary data in second half of 2025 focused on safety, DUX4 mRNA knockdown, DUX4 regulated gene expression, and functional assessments
- **Myotonic dystrophy type 1 (DM1)**
  - Study Status: Phase 1/2 study of SRP-1003 in patients with DM1 underway with recent authorization to dose escalate; Cohorts 1 and 2 in the SAD arm of the study fully enrolled

- Recently Achieved Collaboration Milestone: In July 2025, the program reached the first of two pre-specified enrollment targets, and the safety review warranted dose escalation in the Phase 1/2 study, triggering $100.0 million milestone payment to Arrowhead Pharmaceuticals, Inc. (Arrowhead)
- Next Milestone: Preliminary Phase 1 data in second half of 2025; primary endpoint is safety, with key secondary endpoints including DMPK knockdown, DMPK-mediated splice indices, and functional assessments such as Video Hand Opening Time (VHOT)

- **Spinocerebellar ataxia type 2 (SCA2)**
  - Study Status: Phase 1 study of SRP-1004 in patients with SCA2 underway as a randomized, placebo-controlled, SAD study, and Cohort 1 in this arm of the study has been fully enrolled
  - Next Milestone: On track for First Participant In (FPI), Cohort 2 in the third quarter of 2025
- **Huntington's Disease (HD)**
  - Sarepta intends to file its Clinical Trial Application (CTA) for SRP-1005 in HD by the end of 2025 and initiate its clinical trial in the first half of 2026

2025 ASGCT Annual Conference: Sarepta showcased data spanning different age groups including statistically significant functional outcomes for 8- and 9-year-old patients, and new protein expression and safety results in participants 2 years old at the time of treatment.

- Part 2 analysis of EMBARK study of 8- and 9-year-olds (n=14) demonstrated statistically significant differences across all key endpoints including 4.75 points (P=0.0026) on North Star Ambulatory Assessment (NSAA), 6.87 seconds in time-to-rise (TTR) from the floor (P=0.0010), and 4.76 seconds in 10-meter walk/run (10MWR) (P=0.0097) compared to a well-matched external control cohort
- Results from ENDEAVOR study show treatment in 2-year-old participants with ELEVIDYS for Duchenne muscular dystrophy resulted in mean protein expression of 93.87% as measured by western blot in study participants (n=6)

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

As previously announced, there will be no conference call for this quarterly earnings release.

Q2 2025 Financial Highlights[1]

	For the Three Months Ended June 30,			
	2025	2024	QTD Change	
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 611.1	$ 362.9	248.2	68%
Operating income (loss):				
GAAP	$ 115.6	$ (0.7)	116.3	NM*
Non-GAAP	$ 162.8	$ 57.9	104.9	181%
Net income:				
GAAP	$ 196.9	$ 6.5	190.4	NM*
Non-GAAP	$ 215.2	$ 46.5	168.7	NM*
Diluted earnings per share:				
GAAP	$ 1.89	$ 0.07	1.82	NM*
Non-GAAP	$ 2.02	$ 0.43	1.59	NM*

	For the Six Months Ended June 30,			
	2025	2024	YTD Change	
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 1,355.9	$ 776.4	579.5	75%
Operating (loss) income:				
GAAP	$ (184.8)	$ 34.2	(219.0)	NM*
Non-GAAP	$ (86.8)	$ 141.6	(228.4)	NM*
Net (loss) income:				
GAAP	$ (250.6)	$ 42.6	(293.2)	NM*
Non-GAAP	$ (117.3)	$ 123.8	(241.1)	NM*
Diluted (loss) earnings per share:				
GAAP	$ (2.57)	$ 0.44	(3.01)	NM*
Non-GAAP	$ (1.20)	$ 1.15	(2.35)	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 GAAP financial measure to the most comparable non-GAAP measures, see the tables at the end of this press release.

	As of June 30, 2025	As of December 31, 2024
	(in millions)	
Cash, cash equivalents, restricted cash and short- and long-term investments	$ 850.3	$ 1,503.5

Revenues

Total revenues were $611.1 million for the three months ended June 30, 2025, as compared to $362.9 million for the same period of 2024, an increase of $248.2 million. The increase primarily reflects an increase of $160.1 million in net product revenue of ELEVIDYS as a result of its expanded label approval in June 2024. In addition, collaboration and other revenues increased $95.6 million primarily due to $63.5 million of collaboration revenue recognized related to a milestone payment received from F. Hoffman-La Roche Ltd.'s (“Roche”) for the regulatory approval of ELEVIDYS in Japan (the “Japan Approval Milestone”). Furthermore, contract manufacturing revenues and royalty revenues increased $27.0 million and $5.1 million, respectively, associated with an increase in commercial ELEVIDYS supply delivered to Roche as well as royalty revenue from sales of ELEVIDYS by Roche, respectively.

Total revenues were $1,355.9 million for the six months ended June 30, 2025, as compared to $776.4 million for the same period of 2024, an increase of $579.5 million. The increase primarily reflects an increase of $401.2 million in net product revenue of ELEVIDYS as a result of its expanded label approval in June 2024. In addition, collaboration and other revenues increased $174.9 million primarily related to the $63.5 million of collaboration revenue recognized from the Japan Approval Milestone and the $112.0 million of collaboration revenue recognized related to Roche's expiration of an option for a program during the six months ended June 30, 2025, as compared to $48.0 million of collaboration revenue recognized in the same period of 2024 related to Roche’s declined option to acquire certain ex-US rights to an external, early-stage Duchenne development program. Furthermore, contract manufacturing revenues and royalty revenues increased $38.6 million and $8.8 million, respectively, associated with an increase in commercial ELEVIDYS supply delivered to Roche as well as royalty revenue from sales of ELEVIDYS by Roche, respectively.

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

Cost of sales (excluding amortization of in-license rights) were $152.6 million for the three months ended June 30, 2025, as compared to $44.5 million for the same period of 2024, an increase of approximately $108.1 million. Cost of sales (excluding amortization of in-license rights) were $290.1 million for the six months ended June 30, 2025, as compared to $95.1 million for the same period of 2024, an increase of $195.0 million. The increases in both periods primarily reflect the depletion of previously expensed ELEVIDYS inventory, an increased demand for ELEVIDYS following the expanded label approval in June 2024, an increase in products sold to Roche under the Roche collaboration agreement, as well as an increase in the write-offs of certain batches of products not meeting quality specifications for the periods ended June 30, 2025, as compared to the same periods of 2024.

Operating expenses and others

Research and development expenses were $204.4 million for the three months ended June 30, 2025, as compared to $179.7 million for the same period of 2024, an increase of $24.7 million. The increase is primarily due to an increase in ELEVIDYS clinical material expense as well as $13.0 million of costs associated with our settlement agreement with Brammer Bio MA, LLC (“Brammer”), an affiliate of Thermo Fisher Scientific, Inc., to resolve outstanding claims related to the termination of the development, commercial manufacturing, and supply agreement with Brammer (the “Brammer Settlement”). For the three months ended June 30, 2025, non-GAAP research and development expenses were $181.7 million, as compared to $153.9 million for the same period of 2024, an increase of $27.8 million.

Research and development expenses were $977.8 million for the six months ended June 30, 2025, as compared to $380.1 million for the same period of 2024, an increase of approximately $597.7 million. The increase primarily reflects a $583.6 million increase in up-front and milestone expense associated with the licensing and collaboration agreement and stock purchase agreement with Arrowhead, as well as $13.0 million of costs associated with the Brammer Settlement. For the six months ended June 30, 2025, non-GAAP research and development expenses were $930.9 million, as compared to $332.0 million for the same period of 2024, an increase of $598.9 million.

Selling, general and administrative expenses were $137.9 million for the three months ended June 30, 2025, as compared to $138.8 million for the same period of 2024, a decrease of $0.9 million. The decrease is primarily driven by a net decrease in stock-based compensation primarily due to the achievement of performance conditions related to certain PSUs during the three months ended June 30, 2024, partially offset by an increase in professional services used for the commercialization of ELEVIDYS. For the three months ended June 30, 2025, non-GAAP selling, general and administrative expenses were $113.4 million, as compared to $106.0 million for the same period of 2024, an increase of $7.4 million.

Selling, general and administrative expenses were $271.5 million for the six months ended June 30, 2025, as compared to $265.8 million for the same period of 2024, an increase of $5.7 million. The increase is primarily driven by an increase in compensation and other personnel expenses as well as an increase in professional services used for the commercialization of ELEVIDYS, partially offset by a net decrease in stock-based compensation primarily due to the achievement of performance conditions related to certain PSUs during the six months ended June 30, 2024. For the six months ended June 30, 2025, non-GAAP selling, general and administrative expenses were $220.5 million, as compared to $206.5 million for the same period of 2024, an increase of $14.0 million.

Other income (expense), net for the three months ended June 30, 2025 and 2024, was $38.1 million and $14.3 million, respectively. Other (expense) income, net for the six months ended June 30, 2025 and 2024, was $(45.1) million and $20.8 million, respectively. The changes in each period primarily reflect the fair value adjustments of our investments in publicly-traded companies, including Arrowhead, during the periods ended June 30, 2025.

Income tax (benefit) expense for the three months ended June 30, 2025 and 2024, was $(43.3) million and $7.1 million, respectively. Income tax expense for the six months ended June 30, 2025 and 2024, was $20.7 million and $12.4 million, respectively. Income tax (benefit) 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 net income (loss) is defined by us as GAAP net income (loss) excluding interest income/expense, net, depreciation and amortization expense, stock-based compensation expense, gain/loss on strategic investments, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items.
2. Non-GAAP earnings per share is defined by us as non-GAAP net income, as defined previously, divided by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding, adjusted for the inclusion of additional shares under the “if-converted” method, if applicable and not anti-dilutive. Non-GAAP net loss per share is defined by us as non-GAAP net loss, as defined above, divided by the weighted-average number of shares of common stock outstanding as the inclusion of dilutive common stock equivalents outstanding is anti-dilutive.
3. Non-GAAP operating income (loss) is defined by us as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items.
4. Non-GAAP research and development expenses are defined by us as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items.
5. Non-GAAP selling, general and administrative expenses are defined by us as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items.

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

1. Interest, depreciation and amortization - Interest income (expense), net amounts can vary substantially from period to period due to changes in cash and debt balances and interest rates driven by market conditions outside of our operations. Depreciation expense can vary substantially from period to period as the purchases of property and equipment may vary significantly from period to period and without any direct correlation to our operating performance. Amortization expense primarily associated with patent costs are amortized over a period of several years after acquisition or patent application or renewal.
2. Stock-based compensation expenses - Stock-based compensation expenses represent non-cash charges related to equity awards we have granted. Although these are recurring charges to operations, we believe the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within our control. Therefore, we believe that excluding these charges facilitates comparisons of our operational performance in different periods.
3. Other items - We evaluate other items of expense and income on an individual basis. We take into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to our ongoing business operations, and (c) whether we expect the items to continue or occur on a regular basis. These other items include the gain/loss on strategic investments and changes in the fair value of derivatives and may include other items that fit the above characteristics in the future. We exclude from our non-GAAP results:

    a) The (gain) loss on strategic investments as it is a non-cash item and the results of such gains and losses are not representative of our normal business operations, which accordingly, would make it difficult to compare our results to peer companies that also provide non-GAAP disclosures. We are making this change beginning in 2025 because, as our strategic investments have increased, we recognized that the resulting variability can impede comparability between periods of our financial performance for our ongoing business operations.
    b) The change in fair value of derivatives related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus Therapeutics, Inc. selling shareholders as well as to an academic institution under a separate license agreement as these are non-cash items and are not considered to be normal operating expenses due to the variability of amounts and lack of predictability as to occurrence and/or timing.

We use these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. We also believe these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating our performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of our financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP operating (loss) income, non-GAAP net (loss) income, and non-GAAP diluted (loss) earnings per share may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table “Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures.”

About EXONDYS 51

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

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

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

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

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

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

Other adverse events may occur.

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

For further information, please see the full US Prescribing Information for EXONDYS 51 (eteplirsen).

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 US Prescribing Information for VYONDYS 53 (golodirsen).

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

CONTRAINDICATION: 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 US Prescribing Information for AMONDYS 45 (casimersen).

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

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

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

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

Important Safety Information for ELEVIDYS

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

WARNINGS AND PRECAUTIONS:

Infusion-related Reactions:

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

- ELEVIDYS should be administered in a setting where treatment for infusion-related reactions is immediately available.
- Discontinue infusion for anaphylaxis.

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS, and administration of ELEVIDYS may result in elevations of liver enzymes (e.g., 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, a 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 between exons 1 to 17 and exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.
- Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea or hypophonia as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

Myocarditis:

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

Pre-existing Immunity against AAVrh74:

- In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all subjects developed anti-AAVrh74 antibodies.
- Perform baseline testing for the 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 function test increased, 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 for ELEVIDYS (delandistrogene moxeparvovec-rokl).

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

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; our clinical trials; the potential benefits of our technologies and scientific approaches; ongoing discussions with FDA related to

ELEVIDYS, including the safety labeling process and risk-mitigation approach for non-ambulatory individuals; and expected plans and milestones, including multiple clinical data readouts and milestones expected in 2025 and into early 2026 for our siRNA franchise and working with FDA to resume shipments of ELEVIDYS to non-ambulatory individuals.

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; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; 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, the COVID-19 pandemic 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, 2024 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 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Sarepta Therapeutics, Inc.
Condensed Consolidated Statements of Income (Loss)
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Products, net	$ 513,123	$ 360,548	$ 1,124,646	$ 720,032
Collaboration and other	97,968	2,383	231,301	56,363
Total revenues	611,091	362,931	1,355,947	776,395
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	152,558	44,545	290,122	95,104
Research and development	204,392	179,690	977,840	380,086
Selling, general and administrative	137,897	138,796	271,526	265,799
Amortization of in-licensed rights	667	601	1,268	1,202
Total cost and expenses	495,514	363,632	1,540,756	742,191
Operating income (loss)	115,577	(701)	(184,809)	34,204
Other income (loss), net:				
Other income (expense), net	38,061	14,278	(45,071)	20,821
Total other income (loss), net	38,061	14,278	(45,071)	20,821
Income (loss) before income tax expense	153,638	13,577	(229,880)	55,025
Income tax (benefit) expense	(43,254)	7,117	20,736	12,446
Net income (loss)	$ 196,892	$ 6,460	$ (250,616)	$ 42,579
Earnings (loss) per share:				
Basic	$ 2.01	$ 0.07	$ (2.57)	$ 0.45
Diluted	$ 1.89	$ 0.07	$ (2.57)	$ 0.44
Weighted average number of shares of common stock used in computing earnings (loss) per share:				
Basic	98,005	94,618	97,685	94,305
Diluted	106,623	99,144	97,685	99,129

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,	
	2025	2024	2025	2024
GAAP net income (loss)	$ 196,892	$ 6,460	$ (250,616)	$ 42,579
Interest income, net	(1,921)	(14,010)	(9,846)	(29,741)
Depreciation and amortization expense	10,173	8,118	19,550	16,261
Stock-based compensation expense	37,025	50,482	78,453	91,174
Change in fair value of derivatives	—	—	—	10,100
(Gain) loss on strategic investments*	(36,721)	(148)	54,007	(1,079)
Income tax effect of adjustments	9,728	(4,389)	(8,870)	(5,472)
Non-GAAP net income (loss)	$ 215,176	$ 46,513	$ (117,322)	$ 123,822
GAAP net earnings (loss) per share - diluted:	$ 1.89	$ 0.07	$ (2.57)	$ 0.44
Add: impact of GAAP to Non-GAAP adjustments	$ 0.13	$ 0.36	$ 1.37	$ 0.71
Non-GAAP net earnings (loss) per share - diluted**	$ 2.02	$ 0.43	$ (1.20)	$ 1.15
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:***				
GAAP	106,623	99,144	97,685	99,129
Non-GAAP	106,623	107,245	97,685	107,230

*Beginning in the first quarter of 2025, (gain) loss on strategic investments was included as a non-GAAP measurement to adjust our GAAP financial measures. Non-GAAP financial results for the three and six months ended June 30, 2024, have been updated to reflect this change for comparability. Please refer to the “Use of Non-GAAP Measures” section above for additional detail.

**Non-GAAP earnings per share is calculated using diluted shares whereas non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive.

***The difference between the weighted average number of shares of common stock used in computing diluted GAAP and non-GAAP earnings per share for the three and six months June 30, 2024, is a result of the exclusion of the potential share settlement of the 2027 Convertible Notes from the GAAP earnings per share as the inclusion of such shares was anti-dilutive during those periods.

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
Total effective tax rate, GAAP	(28.2)%	8.4%	(9.0)%	22.6%
Less: impact of GAAP to Non-GAAP adjustments	(4.4)	(4.6)	(25.0)	(10.0)
Total effective tax rate, Non-GAAP	(32.6)%	3.8%	(34.0)%	12.6%

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,	
	2025	2024	2025	2024
GAAP research and development expenses	$ 204,392	$ 179,690	$ 977,840	$ 380,086
Stock-based compensation expense	(15,277)	(19,806)	(32,594)	(36,079)
Depreciation and amortization expense	(7,397)	(5,982)	(14,374)	(12,028)
Non-GAAP research and development expenses	$ 181,718	$ 153,902	$ 930,872	$ 331,979

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
GAAP selling, general and administrative expenses	$ 137,897	$ 138,796	$ 271,526	$ 265,799
Stock-based compensation expense	(21,748)	(30,676)	(45,859)	(55,095)
Depreciation expense	(2,776)	(2,136)	(5,176)	(4,233)
Non-GAAP selling, general and administrative expenses	$ 113,373	$ 105,984	$ 220,491	$ 206,471

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
GAAP operating income (loss)	$ 115,577	$ (701)	$ (184,809)	$ 34,204
Stock-based compensation expense	37,025	50,482	78,453	91,174
Depreciation and amortization expense	10,173	8,118	19,550	16,261
Non-GAAP operating income (loss)	$ 162,775	$ 57,899	$ (86,806)	$ 141,639

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

	As of June 30, 2025	As of December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 510,598	$ 1,103,010
Short-term investments	289,541	251,782
Accounts receivable, net	527,295	601,988
Inventory	994,036	749,960
Manufacturing-related deposits and prepaids	207,921	276,262
Other current assets	127,594	90,461
Total current assets	2,656,985	3,073,463
Property and equipment, net	371,857	340,336
Right of use assets	143,041	148,310
Non-current inventory	194,668	187,986
Strategic investments	195,522	3,710
Non-current investments	34,604	133,163
Other non-current assets	83,137	76,205
Total assets	$ 3,679,814	$ 3,963,173
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 136,702	$ 214,442
Accrued expenses	377,399	373,513
Deferred revenue, current portion	395,431	130,256
Other current liabilities	10,416	13,473
Total current liabilities	919,948	731,684
Long-term debt	1,139,458	1,137,124
Lease liabilities, net of current portion	214,419	192,473
Deferred revenue, net of current portion	—	325,000
Contingent consideration	47,400	47,400
Other non-current liabilities	1,204	1,750
Total liabilities	2,322,429	2,435,431
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 98,356,950 and 97,706,074 issued and outstanding, respectively, at June 30, 2025 and 96,900,496 issued and outstanding at December 31, 2024	10	10
Treasury stock, at cost, 650,876 and 0 shares at June 30, 2025 and December 31, 2024, respectively	(25,263)	—
Additional paid-in capital	5,844,279	5,738,924
Accumulated other comprehensive loss, net of tax	(51)	(218)
Accumulated deficit	(4,461,590)	(4,210,974)
Total stockholders' equity	1,357,385	1,527,742
Total liabilities and stockholders' equity	$ 3,679,814	$ 3,963,173

Source: Sarepta Therapeutics, Inc.

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