



Sarepta Therapeutics Announces Third Quarter 2025 Financial Results and Recent Corporate Developments, Including Completion of Its Confirmatory Study, ESSENCE

11/3/25

- **Sarepta announces completion of the confirmatory trial commitment for its ultra-rare disease PMO therapies AMONDYS 45 and VYONDYS 53:**
 - **While the ESSENCE study did not achieve statistical significance on its primary endpoint, results indicate positive and encouraging trends favoring therapy at 96 weeks**
 - **Sarepta reports that the study was impacted by the COVID-19 pandemic and, when COVID-impacted data is excluded, meaningful treatment effect is seen on the primary endpoint**
 - **ESSENCE supported favorable safety profile of AMONDYS 45 and VYONDYS 53**
 - **The Company intends to schedule a meeting with FDA to discuss path to a traditional approval based on the positive risk-benefits of the therapies from the results of the ESSENCE study and significant positive multi-year real-world evidence**
- **Net product revenues for the third quarter 2025 totaled \$370.0 million, consisting of \$238.5M of PMO and \$131.5M of ELEVIDYS**
- **Refinancing of a majority portion of 2027 Notes and cost restructuring initiatives strengthened overall financial position**
- **ELEVIDYS labeling discussions progressing and expected to be concluded soon**
- **Presentations at 2025 World Muscle Society congress added to the body of evidence regarding ELEVIDYS safety and efficacy**

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 3, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the third quarter of 2025 and the completion of ESSENCE, its global, Phase 3 randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of AMONDYS 45 (casimersen) and VYONDYS 53 (golodirsen) compared to placebo in 225 patients, ages 6-13 years old, with Duchenne muscular dystrophy (Duchenne) amenable to exon 45 or 53 skipping.

Topline Results from ESSENCE

Topline results found that numerical trends favored treatment versus placebo; however, the observed difference of 0.05 steps/second in least square means (LSM), did not reach statistical significance (P=0.309) on the primary endpoint, the 4-step ascend velocity at 96 weeks.

The ESSENCE study was conducted over a nine-year period that included the COVID-19 pandemic, which impacted study participants and outcomes. An analysis that excludes data from participants whose double-blind period overlapped with the COVID-19 pandemic (n=57), shows a 30% reduction (LSM 0.11 steps/second, P=0.09) in disease progression over 2 years on the 4-step ascend velocity in non-COVID impacted treated participants versus placebo (n=168). This is a clinically meaningful change.

There were no new safety signals in the ESSENCE study, reinforcing the favorable and stable safety profile observed with exon-skipping therapies over years. Adverse events were mostly mild (88%) or moderate (10.3%) and comparable between treatment and placebo groups. The most common treatment-emergent adverse events (≥10%) were vomiting, nasopharyngitis, pyrexia, headache, cough, fall, and upper respiratory infections.

For more than a decade, Sarepta's PMO (phosphorodiamidate morpholino oligomer) therapies have been used to treat over 1,800 amenable patients worldwide, from infants as young as 7 months to adults well into their 30s. The results from ESSENCE add to the available evidence for VYONDYS 53 and AMONDYS 45, including real-world studies demonstrating, for instance, that treatment with VYONDYS 53 is associated with a 7.5 year delay in the need for nighttime ventilation¹ and treatment with AMONDYS 45 is associated with a statistically significant slowing of lung function decline and a potentially meaningful benefit in the predicted time to use of a cough assist device². Across our PMO portfolio, real-world evidence indicates a multi-year benefit on mortality^{3,4}, delays in time to loss of ambulation of 3 and 4 years^{5,6}, a substantial reduction in risk of reaching a left ventricular ejection fraction (LVEF) of less than 55%⁷, and a significant reduction in emergency room and other hospital visits⁸.

Based on the encouraging trends seen in ESSENCE, the substantial real-world evidence, and the positive safety profile of AMONDYS 45 and VYONDYS 53, Sarepta intends to schedule a meeting with the U.S. Food and Drug Administration (FDA) to discuss the possibility of converting from accelerated to traditional approval.

Sarepta continues to analyze the results from ESSENCE and, together with real-world evidence, full results will be submitted to the FDA as part of the planned sNDA filings for these two exon-skipping therapies. The completion of the ESSENCE study is expected to fulfill the primary postmarketing requirement. Additionally, results from ESSENCE will be shared at future medical meetings, and publication will be pursued in a medical journal.

"While the ESSENCE study did not meet statistical significance on its primary endpoint, we believe the results demonstrated a clear treatment effect, showing clinically meaningful functional outcomes for people with Duchenne who have mutations amenable to skipping exons 45 or 53. These topline findings reinforce the potential impact of these therapies to slow muscle weakness and other symptoms. The results of the study are consistent with the growing body of real-world evidence accumulated over several years. These data, which we have shared with the FDA, strengthen our confidence in the benefit of added dystrophin over time," said Louise Rodino-Klapac, Ph.D., president of research & development and technical

operations, Sarepta.

Dr. Rodino-Klapac continued, “This trial enrolled an ultra-rare subset of eligible Duchenne patients. The complexity of Duchenne, combined with the heterogeneity of the population and the impact of the COVID pandemic on participation, made this an extraordinary undertaking. We are deeply grateful to the families and investigators whose dedication made this possible, and we remain committed to advancing care for the Duchenne community by delivering options that can change the course of Duchenne.”

“In the trial and my clinical practice, I’ve followed boys and young men treated with casimersen and golodirsen since their initial approvals and, in my opinion, these therapies can help preserve critical functions like walking, stair climbing and feeding themselves. Over time, these gains can translate into a delayed loss of ambulation and even slower respiratory decline, potentially offering these individuals a meaningful path to maintaining quality of life,” said Craig McDonald, M.D., professor and chair of the UC Davis Health Department of Physical Medicine and Rehabilitation, and an investigator in the ESSENCE study.

Business Highlights for the Quarter Ended Sept. 30, 2025

“We are pleased to have met our primary post-marketing obligation with the completion of ESSENCE, a particularly challenging trial to execute in the context of these ultra-rare diseases that heterogeneously degenerate over the course of decades. We look forward to discussing the ESSENCE results and the real-world evidence for AMONDYS 45 and VYONDYS 53 with the FDA,” stated Doug Ingram, chief executive officer, Sarepta.

Mr. Ingram continued, “We are also pleased to report solid performance in the quarter from our gene therapy, ELEVIDYS, and our three PMOs, EXONDYS 51, VYONDYS 53 and AMONDYS 45. Our net product revenue stood at \$370.0 million for the quarter. Additionally, having taken steps to bolster our financial position, including the refinancing of our convertible debt and a significant financial restructuring, I am pleased to report positive cash flow in the quarter. Looking forward, we have a strong financial position from which to continue to serve our community as we advance a very exciting siRNA portfolio.”

- **ELEVIDYS (delandistrogene moxeparvovec-rokl) Regulatory Discussions:** Discussions between FDA and Sarepta in the safety labeling process are expected to be finalized in the near-term with an outcome that includes a box warning and the removal of non-ambulatory indication from the Indication and Usages section of the Prescribing Information. Discussions with FDA are also ongoing regarding Sarepta’s proposed study to evaluate an additional immunosuppression regimen toward re-including non-ambulatory in the label.
- **Pipeline progress for multiple siRNA programs:** Readouts of FSHD and DM1 phase 1/2 studies from both SAD and MAD cohorts expected in early 2026.
 - **Facioscapulohumeral muscular dystrophy (FSHD):** Phase 1/2 study of SRP-1001 enrollment of the SAD is complete and cohort 6 of MAD is ongoing.
 - **Myotonic dystrophy type 1 (DM1):** Phase 1/2 study of SRP-1003 enrollment of the SAD is complete and cohort 4 of the MAD is ongoing.
 - **Huntington’s Disease (HD):** On track to initiate clinical trial for SRP-1005 by end of 2025 utilizing subcutaneous route of administration.
 - **Research target selection:** Three new research targets have been selected in addition to second-generation DM1 candidate selected at close of Arrowhead deal.
- **2025 World Muscle Society (WMS):** At the WMS meeting, Sarepta presented new data on ELEVIDYS, as well as updates from our PMO and LGMD 2E programs. Additionally, multiple independent studies were presented, including preliminary analysis of safety, tolerability and efficacy of a prophylactic sirolimus protocol for patients receiving delandistrogene moxeparvovec-rokl gene therapy. All posters and presentations from the WMS meeting are available on the Company’s website [here](#).
- **Financial Foundation:** The Company has taken steps this quarter to further strengthen its financial foundation by extending the maturity of a meaningful portion of its convertible notes to 2030, enhancing liquidity with the disposition of its Arrowhead equity investment, and achieving expense savings above stated cost restructuring targets.

Conference Call Information

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. This event can be accessed using [this link](#).

Q3 2025 Financial Highlights¹

	For the Three Months Ended September 30,		QTD Change	
	2025	2024		
	(in millions, except for per share amounts)		\$	%
Total Revenues	\$ 399.4	\$ 467.2	(67.8)	(15%)
Operating (loss) income:				
GAAP	\$ (103.4)	\$ 22.2	(125.6)	NM*
Non-GAAP	\$ (35.7)	\$ 74.9	(110.6)	NM*
Net (loss) income:				
GAAP	\$ (179.9)	\$ 33.6	(213.5)	NM*

Non-GAAP	\$	(12.9)	\$	69.9	(82.8)	NM*
Diluted (loss) earnings per share:						
GAAP	\$	(1.80)	\$	0.34	(2.14)	NM*
Non-GAAP	\$	(0.13)	\$	0.64	(0.77)	NM*

	For the Nine Months Ended					
	September 30,					
	2025	2024	YTD Change			
	(in millions, except for per share amounts)		\$	%		
Total Revenues	\$	1,755.3	\$	1,243.6	511.7	41%
Operating (loss) income:						
GAAP	\$	(288.2)	\$	56.4	(344.6)	NM*
Non-GAAP	\$	(122.5)	\$	216.5	(339.0)	NM*
Net (loss) income:						
GAAP	\$	(430.6)	\$	76.2	(506.8)	NM*
Non-GAAP	\$	(130.2)	\$	193.7	(323.9)	NM*
Diluted (loss) earnings per share:						
GAAP	\$	(4.37)	\$	0.78	(5.15)	NM*
Non-GAAP	\$	(1.32)	\$	1.80	(3.12)	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 September 30, 2025	As of December 31, 2024
	(in millions)	
Cash, cash equivalents, restricted cash and short- and long-term investments	\$ 865.2	\$ 1,503.5

Revenues

Total revenues were \$399.4 million for the three months ended September 30, 2025, as compared to \$467.2 million for the same period of 2024, a decrease of \$67.8 million. The decrease primarily reflects \$49.5 million less in net product revenue of ELEVIDYS as a result of lower volume following our decision to suspend shipments of ELEVIDYS to non-ambulatory patients in the U.S. in June 2025. In addition, other revenues decreased \$8.1 million primarily due to contract manufacturing revenues decreasing \$9.2 million driven by a lower volume of shipments of ELEVIDYS to Roche.

Total revenues were \$1,755.3 million for the nine months ended September 30, 2025, as compared to \$1,243.6 million for the same period of 2024, an increase of \$511.7 million. The increase primarily reflects \$351.7 million more in net product revenue of ELEVIDYS as a result of its expanded label approval in June 2024. In addition, collaboration and other revenues increased approximately \$166.9 million primarily related to the \$63.5 million of collaboration revenue recognized as a result of regulatory approval in Japan and the \$112.0 million of collaboration revenue recognized related to Roche's expiration of an option for a program during the nine months ended September 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 \$29.4 million and \$9.9 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 \$150.8 million for the three months ended September 30, 2025, as compared to \$91.7 million for the same period of 2024, an increase of approximately \$59.1 million. The increase primarily reflects the depletion of previously expensed ELEVIDYS inventory, the impairment of prepaid manufacturing deposits and an increase in the write-offs of certain batches of our products not meeting our quality specifications for the three months ended September 30, 2025, as compared to the same period of 2024. Cost of sales (excluding amortization of in-license rights) were \$440.9 million for the nine months ended September 30, 2025, as compared to \$186.8 million for the same period of 2024, an increase of \$254.1 million. The increase primarily reflects an increased demand for ELEVIDYS following the expanded label approval in June 2024, the depletion of previously expensed ELEVIDYS inventory, an increase in the write-offs of certain batches of our products not meeting our quality specifications, an increase in products sold to Roche under the Roche collaboration agreement and the impairment of prepaid manufacturing deposits for the nine months ended September 30, 2025, as compared to the same period of 2024.

Operating expenses and others

Research and development expenses were \$218.9 million for the three months ended September 30, 2025, as compared to \$224.5 million for the same period of 2024, a decrease of \$5.6 million. The decrease is primarily due to a \$102.7 million decrease in manufacturing expenses predominantly as a result of costs associated with the termination of our development, commercial manufacturing and supply agreement with Brammer Bio MA, LLC, an affiliate of Thermo Fisher Scientific, Inc. (Thermo Agreement) during the three months ended September 30, 2024, with no similar activity for the three months ended September 30, 2025, as well as a decrease in compensation, other personnel, and stock-based compensation expenses as a result of our restructuring plan announced during the three months ended September 30, 2025. This decrease was partially offset by a \$100.0

million increase in up-front and milestone expenses due to our milestone payment to Arrowhead, triggered by Arrowhead's achievement of the first of two predetermined enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 study for the DM1 program, with no similar activity for the three months ended September 30, 2024. For the three months ended September 30, 2025, non-GAAP research and development expenses were \$206.5 million, as compared to \$199.8 million for the same period of 2024, an increase of \$6.7 million.

Research and development expenses were \$1,196.7 million for the nine months ended September 30, 2025, as compared to \$604.6 million for the same period of 2024, an increase of approximately \$592.1 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 the \$100.0 million milestone payment made to Arrowhead for the nine months ended September 30, 2025, partially offset by a decrease of \$74.9 million in manufacturing expenses primarily due to the termination of the Thermo Agreement during the nine months ended September 30, 2024, as well as a decrease in compensation, other personnel, and stock-based compensation expenses as a result of our restructuring plan in July 2025. For the nine months ended September 30, 2025, non-GAAP research and development expenses were \$1,137.4 million, as compared to \$531.8 million for the same period of 2024, an increase of \$605.6 million.

Selling, general and administrative expenses were \$91.9 million for the three months ended September 30, 2025, as compared to \$128.2 million for the same period of 2024, a decrease of \$36.3 million. The decrease is primarily driven by lower compensation and other personnel expenses due to reduced headcount, as well as a decrease in stock-based compensation due to the reversal of previously recognized expense related to unvested awards, all as a result of our restructuring plan announced in July 2025. For the three months ended September 30, 2025, non-GAAP selling, general and administrative expenses were \$77.1 million, as compared to \$100.2 million for the same period of 2024, a decrease of \$23.1 million.

Selling, general and administrative expenses were \$363.4 million for the nine months ended September 30, 2025, as compared to \$394.0 million for the same period of 2024, a decrease of \$30.6 million. The decrease is primarily driven by reduced headcount as a result of our restructuring plan for the nine months ended September 30, 2025 and a net decrease in stock-based compensation primarily due to the achievement of performance conditions related to certain PSUs during the nine months ended September 30, 2024, and the reversal of previously recognized expense related to unvested awards as a result of our restructuring plan announced in July 2025, partially offset by the fulfillment of remaining service conditions associated with certain PSUs in March 2025. For the nine months ended September 30, 2025, non-GAAP selling, general and administrative expenses were \$297.6 million, as compared to \$306.7 million for the same period of 2024, a decrease of \$9.1 million.

Restructuring charges were \$40.5 million for the three and nine months ended September 30, 2025, with no similar activity for the same periods of 2024. These charges were primarily related to employee termination benefits, including severance, along with accelerated depreciation for assets impacted by our restructuring plan, announced in July 2025, that was designed to reduce operating expenses and align our cost structure with strategic priorities, aiming to enhance financial flexibility and meet our 2027 financial obligations.

Loss on debt extinguishment was \$138.6 million for the three and nine months ended September 30, 2025, with no similar activity for the same periods of 2024. The loss on debt extinguishment is a result of our partial refinancing of the 2027 Notes through a privately negotiated exchange transaction where we exchanged \$700.0 million in aggregate principal amount of 2027 Notes for (1) \$602.0 million in aggregate principal amount of new convertible senior notes due on September 1, 2030 (the "2030 Notes"), net of issuance costs of \$13.4 million, (2) cash payments of \$127.3 million, including \$4.0 million of accrued interests, and (3) the issuance of 5.9 million shares of our common stock with fair market value of approximately \$107.3 million, net of issuance costs of \$2.4 million.

Other income, net for the three months ended September 30, 2025 and 2024, was \$48.0 million and \$11.8 million, respectively. The change primarily reflects the fair value adjustments of our investments, including Arrowhead, during the three months ended September 30, 2025. Other income, net for the nine months ended September 30, 2025 and 2024, was \$2.9 million and \$32.6 million, respectively. The change primarily reflects the decrease in interest income due to changes in the investment mix of our investment portfolio and the fair value adjustments of our investments in Arrowhead during the nine months ended September 30, 2025.

Income tax (benefit) expense for the three months ended September 30, 2025 and 2024, was \$(14.1) million and \$0.4 million, respectively. Income tax expense for the nine months ended September 30, 2025 and 2024, was \$6.6 million and \$12.8 million, respectively. Income tax benefit for the three months ended September 30, 2025, relates to the tax benefit recorded on the quarter to date loss which offsets tax expense recorded on profits in prior interim periods. Income tax expense for the nine months ended September 30, 2025, as well as for the three and nine months ended September 30, 2024, 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 (loss) income is defined by us as GAAP net (loss) income excluding interest income/expense, net, depreciation and amortization expense, stock-based compensation expense, restructuring charges, other items and the estimated income tax impact of each pre-tax non-GAAP adjustment.
2. Non-GAAP net loss per share is defined by us as non-GAAP net loss, as defined above, divided by the weighted-average number of shares of common stock outstanding as the inclusion of dilutive common stock equivalents outstanding is anti-dilutive. Non-GAAP earnings per share is defined by us as non-GAAP net income, as defined previously, divided by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding, adjusted for the inclusion of additional shares under the "if-converted" method, if applicable and not anti-dilutive.
3. Non-GAAP operating (loss) income is defined by us as GAAP operating (loss) income excluding depreciation and amortization expense, stock-based compensation expense, and restructuring charges.
4. Non-GAAP research and development expenses are defined by us as GAAP research and development expenses excluding depreciation and amortization expense, and stock-based compensation expense.
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. Restructuring charges do not have a direct correlation to future business operations, nor do the resulting charges recorded reflect the performance of our ongoing operations for the period in which such charges are recorded. In addition, restructuring charges are not considered to be normal operating expenses due to the variability of amounts and lack of predictability as to occurrence and/or timing.
4. 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, changes in the fair value of derivatives, restructuring charges, and loss on debt extinguishment and may include other items that fit the above characteristics in the future. We exclude from our non-GAAP results:
 - a. The (gain) loss on strategic investments as 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.
 - c. The loss on debt extinguishment is considered to be an infrequent 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 the our results to peer companies that also provide non-GAAP disclosures.

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 (eteplisen).

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 \geq 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 moxeparvec-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 a leadership position 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 ongoing and planned clinical trials; expected plans and milestones, including our intention to seek alignment with FDA regarding the results of our ESSENCE confirmatory trial and a path to traditional approval of casimersen and golodirsen and our expectations regarding the label for ELEVIDYS.

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; topline data is based on preliminary analysis and could differ materially from final data; we may not be able to reach alignment with the FDA regarding a path to traditional approval for casimersen and golodirsen, including due to any limitations on the FDA's reliance of real-world evidence; the reduction in force may take longer or result in more significant charges or cash expenditures than anticipated or otherwise negatively impact the Company and its business plans during and after the period during which the reduction in force is being executed; 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, 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; the impact of the federal government shutdown on the FDA; 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 (Loss) Income
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024
	Revenues:			
Products, net	\$ 370,043	\$ 429,771	\$ 1,494,689	\$ 1,149,803
Collaboration and other	29,313	37,401	260,614	93,764
Total revenues	<u>399,356</u>	<u>467,172</u>	<u>1,755,303</u>	<u>1,243,567</u>
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	150,775	91,691	440,897	186,795
Research and development	218,890	224,483	1,196,730	604,569
Selling, general and administrative	91,893	128,200	363,419	393,999
Restructuring charge	40,510	—	40,510	—
Amortization of in-licensed rights	677	602	1,945	1,804
Total cost and expenses	<u>502,745</u>	<u>444,976</u>	<u>2,043,501</u>	<u>1,187,167</u>
Operating (loss) income	<u>(103,389)</u>	<u>22,196</u>	<u>(288,198)</u>	<u>56,400</u>
Other (loss) income, net:				
Loss on debt extinguishment	(138,613)	—	(138,613)	—
Other income, net	47,953	11,810	2,882	32,631
Total other (loss) income, net	<u>(90,660)</u>	<u>11,810</u>	<u>(135,731)</u>	<u>32,631</u>
(Loss) income before income tax (benefit) expense	(194,049)	34,006	(423,929)	89,031
Income tax (benefit) expense	(14,102)	395	6,634	12,841
Net (loss) income	<u>\$ (179,947)</u>	<u>\$ 33,611</u>	<u>\$ (430,563)</u>	<u>\$ 76,190</u>
(Loss) earnings per share:				
Basic	\$ (1.80)	\$ 0.35	\$ (4.37)	\$ 0.80
Diluted	\$ (1.80)	\$ 0.34	\$ (4.37)	\$ 0.78
Weighted average number of shares of common stock used in computing (loss) earnings per share:				
Basic	100,237	95,390	98,545	94,669
Diluted	100,237	100,448	98,545	99,572

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024

GAAP net (loss) income	\$ (179,947)	\$ 33,611	\$ (430,563)	\$ 76,190
Interest income, net	(817)	(13,415)	(10,663)	(43,156)
Depreciation and amortization expense	12,221	9,204	31,771	25,465
Stock-based compensation expense	14,927	43,450	93,380	134,624
Change in fair value of derivatives	(11,100)	(1,535)	(11,100)	8,565
(Gain) loss on strategic investments*	(36,739)	2,883	17,268	1,804
Restructuring charge	40,510	—	40,510	—
Loss on debt extinguishment	138,613	—	138,613	—
Income tax effect of adjustments	9,477	(4,300)	607	(9,772)
Non-GAAP net (loss) income	<u>\$ (12,855)</u>	<u>\$ 69,898</u>	<u>\$ (130,177)</u>	<u>\$ 193,720</u>
GAAP net (loss) earnings per share - diluted:	\$ (1.80)	\$ 0.34	\$ (4.37)	\$ 0.78
Add: impact of GAAP to Non-GAAP adjustments	\$ 1.67	\$ 0.30	\$ 3.05	\$ 1.02
Non-GAAP net (loss) earnings per share - diluted**	<u>\$ (0.13)</u>	<u>\$ 0.64</u>	<u>\$ (1.32)</u>	<u>\$ 1.80</u>

Weighted average number of shares of common stock used in computing diluted (loss) earnings per share:***

GAAP	100,237	100,448	98,545	99,572
Non-GAAP	100,237	108,548	98,545	107,672

*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 nine months ended September 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 nine months September 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 September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024
Total effective tax rate, GAAP	7.3%	1.2%	(1.6)%	14.4%
Less: impact of GAAP to Non-GAAP adjustments	57.4	6.2	(3.3)	(3.8)
Total effective tax rate, Non-GAAP	<u>64.7%</u>	<u>7.4%</u>	<u>(4.9)%</u>	<u>10.6%</u>

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP research and development expenses	\$ 218,890	\$ 224,483	\$ 1,196,730	\$ 604,569
Stock-based compensation expense	(4,139)	(18,034)	(36,733)	(54,113)
Depreciation and amortization expense	(8,227)	(6,664)	(22,601)	(18,692)
Non-GAAP research and development expenses	<u>\$ 206,524</u>	<u>\$ 199,785</u>	<u>\$ 1,137,396</u>	<u>\$ 531,764</u>

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP selling, general and administrative expenses	\$ 91,893	\$ 128,200	\$ 363,419	\$ 393,999
Stock-based compensation expense	(10,788)	(25,416)	(56,647)	(80,511)
Depreciation expense	(3,994)	(2,540)	(9,170)	(6,773)
Non-GAAP selling, general and administrative expenses	<u>\$ 77,111</u>	<u>\$ 100,244</u>	<u>\$ 297,602</u>	<u>\$ 306,715</u>

For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
2025	2024	2025	2024

GAAP operating (loss) income	\$ (103,389)	\$ 22,196	\$ (288,198)	\$ 56,400
Stock-based compensation expense	14,927	43,450	93,380	134,624
Depreciation and amortization expense	12,221	9,204	31,771	25,465
Restructuring charge	40,510	—	40,510	—
Non-GAAP operating (loss) income	<u>\$ (35,731)</u>	<u>\$ 74,850</u>	<u>\$ (122,537)</u>	<u>\$ 216,489</u>

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

	As of September 30, 2025	As of December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 613,077	\$ 1,103,010
Short-term investments	237,916	251,782
Accounts receivable, net	395,739	601,988
Inventory	1,077,166	749,960
Manufacturing-related deposits and prepaids	206,062	276,262
Other current assets	192,349	90,461
Total current assets	<u>2,722,309</u>	<u>3,073,463</u>
Property and equipment, net	358,936	340,336
Right of use assets	139,167	148,310
Non-current inventory	198,601	187,986
Non-current investments	1,040	133,163
Other non-current assets	73,384	79,915
Total assets	<u>\$ 3,493,437</u>	<u>\$ 3,963,173</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 94,015	\$ 214,442
Accrued expenses	295,793	373,513
Deferred revenue, current portion	485,449	130,256
Contingent consideration, current portion	36,000	—
Other current liabilities	10,168	13,473
Total current liabilities	<u>921,425</u>	<u>731,684</u>
Long-term debt	1,035,146	1,137,124
Lease liabilities, net of current portion	215,042	192,473
Deferred revenue, net of current portion	—	325,000
Contingent consideration, net of current portion	300	47,400
Other non-current liabilities	1,404	1,750
Total liabilities	<u>2,173,317</u>	<u>2,435,431</u>
Stockholders' equity:		
Preferred stock, \$0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, \$0.0001 par value, 198,000,000 shares authorized; 105,418,987 and 104,768,111 issued and outstanding, respectively, at September 30, 2025 and 96,900,496 issued and outstanding at December 31, 2024	11	10
Treasury stock, at cost, 650,876 and 0 shares at September 30, 2025 and December 31, 2024, respectively	(25,263)	—
Additional paid-in capital	5,986,689	5,738,924
Accumulated other comprehensive income (loss), net of tax	220	(218)
Accumulated deficit	<u>(4,641,537)</u>	<u>(4,210,974)</u>
Total stockholders' equity	<u>1,320,120</u>	<u>1,527,742</u>
Total liabilities and stockholders' equity	<u>\$ 3,493,437</u>	<u>\$ 3,963,173</u>

Source: Sarepta Therapeutics, Inc.

¹ Iff J, et al. Delayed Pulmonary Progression in Golodirsén-Treated Patients With Duchenne Muscular Dystrophy vs Mutation-Matched External Controls. Presented at MDA 2024.

² Kuntz N, et al. Pulmonary Function in Advanced-Stage Patients With Duchenne Muscular Dystrophy Treated With Casimersen. Presented at WMS 2025.

³ Iff J, , et al. Survival among patients receiving eteplirsen for up to 8 years for the treatment of Duchenne muscular dystrophy and contextualization with natural history controls. *Muscle & Nerve*. 2024; 70(1): 60-70. doi:10.1002/mus.28075.

⁴ Data on file.

⁵ Mathews K, et al. Comparative Analysis of Loss of Ambulation in Eteplirsen-Treated Patients With DMD in the EVOLVE Study and Propensity Score-Weighted External Controls. Presented at MDA 2025.

⁶ Muntoni F, et al. Comparing Ambulatory Outcomes of Golodirsen-Treated Patients vs Mutation-Matched External Controls. Presented at CNS 2025.

⁷ Iff J, et al. Association Between Exon-Skipping Therapy With Eteplirsen and Cardiac Outcomes in Duchenne Muscular Dystrophy. Presented at MDA 2025.

⁸ Iff J, et al. *Journal Comp Eff Res*. 2023 Sep;12(9):e230086. doi: 10.57264/ceer-2023-0086.

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Source: Sarepta Therapeutics, Inc.