Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2023 Financial Results and Recent Corporate Developments

2/28/24

– ***Net product revenues for the fourth quarter 2023 totaled $365.1 million, a 55% increase over the same quarter of the prior year; 2023 net product revenues for the full-year 2023 totaled $1.1 billion, an increase of approximately 36% over the prior year***

– ***ELEVIDYS revenues for the quarter totaled $131.2 million and for full-year totaled $200.4 million***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 28, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the fourth quarter and full-year 2023.

"We are pleased to report another strong quarter and year of performance serving the patient community. With our three approved PMO therapies, EXONDYS 51, VYONDYS 53 and AMONDYS 45, and supported by the successful launch of our gene therapy, ELEVIDYS, Sarepta's net product revenue grew 55% in the fourth quarter and 36% for the full year when compared to 2022, as we posted $1.14 billion in full-year net product revenue and we achieved GAAP profitability in the fourth quarter 2023 after achieving profitability on a non-GAAP basis in the third quarter 2023," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "2023 was arguably Sarepta's most significant year in service of our mission to improve the lives of Duchenne patients. With the acceptance for review of our BLA supplement to expand the ELEVIDYS label, continued performance of our four approved therapies, and as we continue to advance our pipeline, 2024 offers the potential of being the most important year yet for families living with Duchenne and those invested in the improvement of patients' lives."

Fourth Quarter 2023 and Recent Developments:

- ***Announced U.S. FDA acceptance of an efficacy supplement to expand the ELEVIDYS indication:*** The U.S. Food and Drug Administration (FDA) has accepted and filed Sarepta's efficacy supplement to the Biologics License Application (BLA) for ELEVIDYS (delandistrogene moxeparvovec-rokl). The goals of the efficacy supplement are twofold:
    - To expand the labeled indication for ELEVIDYS as follows: "[ELEVIDYS is indicated for] ***the treatment of Duchenne muscular dystrophy (DMD) patients with a confirmed mutation in the DMD gene."***
    - To convert the ELEVIDYS accelerated approval to a traditional approval.

  The FDA has granted the efficacy supplement a Priority Review with a review goal date of June 21, 2024. The Agency has also confirmed they are not planning to hold an advisory committee meeting to discuss the supplement.

- ***Submitted efficacy supplement to expand the ELEVIDYS label to include Duchenne muscular dystrophy patients without restriction to age or ambulatory status:*** Submitted an efficacy supplement to the BLA for ELEVIDYS (delandistrogene moxeparvovec-rokl) to expand its labeled indication as follows "[ELEVIDYS is indicated for] ***the treatment of DMD patients with a confirmed mutation in the DMD gene."*** The efficacy supplement is supported by results from EMBARK (Study SRP-9001-301), a global, randomized, double-blind, placebo-controlled, Phase 3 clinical study in patients with Duchenne between the ages of 4 through 7 years and data from ENDEAVOR (Study SRP-9001-103), an open label clinical study in patients with Duchenne, that is enrolling patients ages 2 years and older. The supplement was submitted to the U.S. Food and Drug Administration (FDA) with a request for Priority Review. Sarepta has also completed the EMBARK postmarketing requirement (PMR) and submitted the PMR to FDA requesting conversion from accelerated approval to traditional approval.

- ***Announced positive data from Part B of MOMENTUM, a phase 2 study of SRP-5051 in patients with Duchenne muscular dystrophy amenable to skipping exon 51:*** Announced positive data from Part B of the MOMENTUM study (Study SRP-5051-201), a global, Phase 2, multi-ascending dose clinical trial of SRP-5051 (vesleteplirsen) that enrolled patients aged 8 to 21 years. SRP-5051 is a next-generation peptide phosphorodiamidate morpholino oligomer (PPMO) treatment for patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping. Data from Part B of MOMENTUM found that at the higher, target dose, approximately 30 mg/kg dosed every four weeks, SRP-5051 resulted in mean dystrophin expression of 5.17%, and mean exon skipping of 11.11% at 28 weeks (n=20). Consistent dystrophin expression was seen in ambulatory (4.76%, n=11) and non-ambulatory (5.67%, n=9) participants at 28 weeks. Hypomagnesemia has previously been identified in patients taking SRP-5051 and was managed and monitored through prophylactic magnesium supplementation as part of the study protocol.

- ***Initiated screening in EMERGENE, a phase 3 clinical study of SRP-9003 for the treatment of limb-girdle muscular dystrophy type 2E/R4*****:** Announced that screening is underway in Study SRP-9003-301, also known as EMERGENE. Study 9003-301 is a Phase 3, multi-national, open-label study of SRP-9003 (bidridistrogene xeboparvovec) for the

treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E/R4), or beta sarcoglycanopathy. EMERGENE will enroll 15 participants (ambulatory and non-ambulatory), aged 4 and older, and uses commercially representative process SRP-9003 material. The EMERGENE study design incorporates a six-month natural history lead-in. The primary endpoint is expression of beta-sarcoglycan 60 days after dosing. Other endpoints include functional measures through month 60 and safety.

Conference Call

The event will be webcast live under the investor relations section of Sarepta's website at https://investorrelations.sarepta.com/events-presentations and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using **this online form.** After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

Financial Results

For the three months ended December 31, 2023, the Company reported a GAAP net income of $45.7 million, or $0.49 per basic and $0.47 per diluted share, compared to a GAAP net loss of $109.2 million reported for the same period of 2022, or $1.24 per basic and diluted share. The non-GAAP net income for the three months ended December 31, 2023 was $86.6 million, or $0.82 per diluted share, compared to a non-GAAP net loss of $53.6 million, or $0.61 per diluted share for the same period of 2022.

For the twelve months ended December 31, 2023, the Company reported a GAAP net loss of $536.0 million, or $5.80 per basic and diluted share, compared to a GAAP net loss of $703.5 million reported for the same period of 2022, or $8.03 per basic and diluted share. The non-GAAP net loss for the twelve months ended December 31, 2023 was $59.5 million, or $0.64 per diluted share, compared to a non-GAAP net loss of $290.4 million, or $3.32 per diluted share for the same period of 2022.

Revenues

For the three months ended December 31, 2023, the Company recorded total revenues of $396.8 million, which consist of net product revenues and collaboration and other revenues, compared to total revenues of $258.4 million for the same period of 2022, an increase of $138.4 million. For the twelve months ended December 31, 2023, the Company recorded total revenues of $1,243.3 million, compared to total revenues of $933.0 million for the same period of 2022, an increase of $310.3 million. The increases primarily reflect increasing demand for EXONDYS 51, AMONDYS 45 and VYONDYS 53 (collectively, the “PMO Products”), as well as $131.2 million and $200.4 million of net product revenues associated with sales of ELEVIDYS during the three and twelve months ended December 31, 2023, respectively, after its approval in June 2023.

For the three months ended December 31, 2023, the Company recorded net product revenues of $365.1 million, compared to net product revenues of $235.9 million for the same period of 2022, an increase of $129.2 million. For the twelve months ended December 31, 2023, the Company recorded net product revenues of $1,144.9 million, compared to net product revenues of $843.8 million for the same period of 2022, an increase of $301.1 million. The increases primarily reflect increasing demand for PMO Products, as well as $131.2 million and $200.4 million of net product revenues associated with sales of ELEVIDYS during the three and twelve months ended December 31, 2023, respectively.

For the three and twelve months ended December 31, 2023, the Company recognized $31.7 million and $98.5 million of collaboration and other revenues, respectively. For the three and twelve months ended December 31, 2022, the Company recognized $22.5 million and $89.2 million of collaboration revenue, respectively. For all periods presented, collaboration and other revenues primarily relate to the F. Hoffman-La Roche Ltd. (Roche) collaboration arrangement. For the three and twelve months ended December 31, 2023, the Company recognized $9.2 million of contract manufacturing collaboration revenue associated with multiple batches of commercial ELEVIDYS supply delivered to Roche, with no similar activity for the three and twelve months ended December 31, 2022.

Cost and Expenses

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

For the three months ended December 31, 2023, cost of sales (excluding amortization of in-licensed rights) was $44.2 million, compared to $30.8 million for the same period of 2022, an increase of $13.4 million. The increase in the three months ended December 31, 2023 primarily reflects increasing demand for the Company's PMO Products, an increase in royalty payments due to ELEVIDYS sales in 2023 with no similar activity in 2022 and write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the three months ended December 31, 2023, with no similar activity for the three months ended December 31, 2022.

For the twelve months ended December 31, 2023, cost of sales (excluding amortization of in-licensed rights) was $150.3 million, compared to $140.0 million for the same period of 2022, an increase of $10.3 million. The change for the twelve months ended December 31, 2023 primarily reflects increasing demand for the Company's PMO Products, partially offset by a decrease in write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the twelve months ended December 31, 2023, as compared to the same period of 2022, as well as a decrease in royalty payments due to changes in the BioMarin Pharmaceuticals, Inc. (BioMarin) royalty terms.

Research and development

Research and development expenses were $195.5 million for the three months ended December 31, 2023, compared to $213.8 million for the same period of 2022, a decrease of $18.3 million. The decrease in research and development expenses primarily reflects the following:

- $57.6 million decrease in manufacturing expenses primarily due to an increase in capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023;
- $16.9 million decrease in up-front, milestone and other expenses primarily due to timing and costs related to the execution of certain research and license agreements and achievement of certain milestones year over year;
- $3.5 million decrease in research and other expenses primarily driven by a decrease in collaboration cost-sharing expenses related to Genethon's micro-dystrophin drug candidate;
- $1.6 million increase in pre-clinical expenses primarily due to an increase in toxicology study activity across multiple gene therapy and PPMO platforms;
- $1.9 million increase in professional service expenses primarily due to clinical consulting fees for various clinical trials

sponsored by the Company;
- $3.2 million increase in stock-based compensation expense primarily due to the achievement of performance conditions related to certain shares with performance conditions (PSUs) during the twelve months ended December 31, 2023 with continuing vesting requirements related to a service condition, as well as changes in headcount and the value of stock awards;
- $4.8 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $7.9 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $12.0 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MOMENTUM and EXPEDITION programs, as well as additional PPMO clinical trials; and
- $28.2 million decrease in the offset to expense associated with a collaboration reimbursement from Roche primarily due to a decrease in reimbursed cost related to the minimum purchase requirements under the gene therapy manufacturing and supply agreement (the Thermo Agreement) with Thermo Fisher Scientific, Inc. (Thermo) for the three months ended December 31, 2022, with no similar activity in 2023, partially offset by the continuing development of the Company's SRP-9001 gene therapy programs.

Research and development expenses were $877.4 million for the twelve months ended December 31, 2023, compared to $877.1 million for the same period of 2022, a slight increase of $0.3 million. The increase in research and development expenses primarily reflects the following:

- $51.5 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MIS51ON, MOMENTUM, ENVISION, EMERGENE and EXPEDITION programs, as well as additional PPMO clinical trials;
- $38.8 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $21.2 million increase in stock-based compensation expense primarily due to changes in headcount and the value of stock awards, as well as the achievement of performance conditions related to certain PSUs during the twelve months ended December 31, 2023 with continuing vesting requirements related to a service condition;
- $18.3 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $12.0 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the twelve months ended December 31, 2023 and an increase in collaboration cost-sharing expenses related to Genethon's micro-dystrophin drug candidate;
- $9.7 million increase in professional service expenses primarily related to the launch of ELEVIDYS prior to its regulatory approval in June 2023;
- $3.1 million increase in pre-clinical expenses primarily due to an increase in toxicology study activity across multiple gene therapy and PPMO platforms;
- $21.9 million decrease in up-front, milestone and other expenses, primarily due to timing and costs related to the execution of certain research and license agreements and achievement of certain milestones year over year;
- $143.7 million decrease in manufacturing expenses primarily due to the capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023 and a decrease of $54.0 million related to the minimum purchase requirements under the Thermo Agreement for the twelve months ended December 31, 2022, with no similar activity in the same period of 2023; and
- $11.3 million decrease in the offset to expense associated with a collaboration reimbursement from Roche primarily due to a decrease in reimbursed cost related to the minimum purchase requirements under the Thermo Agreement for the twelve months ended December 31, 2022, with no similar activity in the same period of 2023, partially offset by the continuing development of the Company's SRP-9001 gene therapy programs.

Non-GAAP research and development expenses were $165.1 million and $186.8 million for the three months ended December 31, 2023 and 2022, respectively, a decrease of $21.7 million. Non-GAAP research and development expenses were $761.9 million and $784.1 million for the twelve months ended December 31, 2023 and 2022, respectively, a decrease of $22.2 million.

Selling, general and administrative

Selling, general and administrative expenses were $131.7 million for the three months ended December 31, 2023 compared to $120.5 million for the same period in 2022, an increase of $11.2 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $18.4 million increase in professional service expenses primarily related to the launch of ELEVIDYS and ongoing litigation matters;
- $7.9 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $3.6 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $7.9 million decrease in stock-based compensation expense primarily related to the execution of the Chief Executive Officer (CEO) grant modification agreement in 2022, partially offset by the achievement of performance conditions related to certain PSUs during the twelve months ended December 31, 2023 with continuing vesting requirements related to a

service condition, as well as changes in headcount and the value of stock awards; and
- $10.7 million decrease in other expenses primarily due to timing of charitable contributions.

Selling, general and administrative expenses were $481.9 million for the twelve months ended December 31, 2023, compared to $451.4 million for the same period in 2022, an increase of $30.5 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $60.9 million increase in professional service expenses primarily related to the launch of ELEVIDYS and ongoing litigation matters;
- $35.2 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $10.9 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $4.6 million decrease in other expenses primarily related to timing of charitable contributions; and
- $71.7 million decrease in stock-based compensation expense primarily related to the CEO grant modification agreement in 2022, partially offset by the achievement of performance conditions related to certain PSUs during the twelve months ended December 31, 2023 with continuing vesting requirements related to a service condition, as well as changes in headcount and the value of stock awards.

Non-GAAP selling, general and administrative expenses were $105.7 million and $86.6 million for the three months ended December 31, 2023 and 2022, respectively, an increase of $19.1 million. Non-GAAP selling, general and administrative expenses were $372.0 million and $270.3 million for the twelve months ended December 31, 2023 and 2022, respectively, an increase of $101.7 million.

Amortization of in-licensed rights

For the three and twelve months ended December 31, 2023, the Company recorded amortization of in-licensed rights of approximately $0.8 million and $1.6 million, respectively. For the three and twelve months ended December 31, 2022, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $0.7 million, respectively. This is related to the amortization of the in-licensed right assets recognized as a result of agreements the Company entered into with the University of Western Australia, Nationwide Children's Hospital, BioMarin and Parent Project Muscular Dystrophy in April 2013, December 2016, July 2017 and May 2018, respectively.

Loss on debt extinguishment

On November 14, 2017, the Company issued $570.0 million aggregate principal amount of senior convertible notes due on November 15, 2024 (2024 Notes). On March 2, 2023, the Company entered into separate, privately negotiated exchange agreements with certain holders of the outstanding 2024 Notes (Exchange Agreements). The Exchange Agreements resulted in a conversion of $313.5 million in aggregate principal value of the 2024 Notes held by the holders (2024 Notes Conversion). In connection with the 2024 Notes Conversion, the Company issued approximately 4.5 million shares of its common stock representing the agreed upon contractual conversion rate under the Exchange Agreements. The conversion was not pursuant to the conversion privileges included in the terms of the debt at issuance and therefore was accounted for as a debt extinguishment. The Company accounted for the debt extinguishment by recognizing the difference between the fair value of the shares of common stock transferred on the conversion date and the net carrying amount of the extinguished debt as a loss on debt extinguishment. The loss incurred on the extinguishment for the twelve months ended December 31, 2023 was $387.3 million, inclusive of $6.9 million in third-party debt conversion costs.

Gain from Sale of Priority Review Voucher

In June 2023, the Company entered into an agreement to sell the rare pediatric disease Priority Review Voucher (ELEVIDYS PRV) it received from the FDA in connection with the approval of ELEVIDYS for consideration of $102.0 million, with no commission costs. The transaction was not subject to the conditions set forth under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and closed during June 2023. The net proceeds were recorded as a gain from sale of the ELEVIDYS PRV during the twelve months ended December 31, 2023 as it did not have a carrying value at the time of the sale.

Other income (expense), net

For the three months ended December 31, 2023 and 2022, other income, net was $15.7 million and $5.5 million, respectively. The increase was primarily due to an increase in accretion of investment discount, net due to an increase in interest rates. For the twelve months ended December 31, 2023 and 2022, other income, net was $33.1 million and other expense, net was $28.3 million, respectively. The change is primarily due to an increase in accretion of investment discount, net and an increase in interest income due to the investment mix of the Company's investment portfolio and an increase in interest rates, as well as a reduction of interest expense incurred as a result of the repayment of the Company's December 2019 Term Loan in 2022, partially offset by the impairment of strategic investments and a decrease in gain (loss) on contingent consideration, net.

Income tax (benefit) expense

Income tax benefit for the three months ended December 31, 2023 was approximately $5.3 million. Income tax expense for the twelve months ended December 31, 2023 was $15.9 million. Income tax expense for the three and twelve months ended December 31, 2022 was $7.9 million and $13.5 million, respectively. Income tax (benefit) expense for the three and twelve months ended December 31, 2023 relates to state, foreign and federal income taxes, while income tax expense for the three and twelve months ended December 31, 2022 relates to state and foreign income taxes.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had approximately $1.7 billion in cash, cash equivalents, investments and long-term restricted cash as of December 31, 2023, compared to $2.0 billion as of December 31, 2022. This decrease is driven by cash used to fund the Company's ongoing operations during 2023.

Use of Non-GAAP Measures

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements. The non-GAAP income (loss) is defined by the Company as GAAP net income (loss) excluding interest (income) expense, net, depreciation and amortization expense, stock-based compensation expense, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items. Non-GAAP research and development expenses are defined by the Company as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by the Company as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and

other items.

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 the Company's 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 the Company's 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 granted by the Company. Although these are recurring charges to operations, the Company believes 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 the Company's control. Therefore, the Company believes that excluding these charges facilitates comparisons of the Company's operational performance in different periods.

3. Other items
The Company evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to the Company's ongoing business operations, and (c) whether the Company expects the items to continue or occur on a regular basis. These other items include impairment of strategic investments, loss (gain) on contingent consideration, net, gain from sale of the PRV and loss on debt extinguishment and may include other items that fit the above characteristics in the future.

- The Company excludes from its non-GAAP results the impairment of any strategic investments as it is a non-cash item and is not considered to be a normal operating expense due to the variability of amount and lack of predictability as to the occurrence and/or timing of such impairments.
- The Company excludes from its non-GAAP results the loss on debt extinguishment, which is considered to be an infrequent and non-cash event as it is associated with a distinct financing decision and is not indicative of the performance of the Company's core operations, which accordingly, would make it difficult to compare the Company's results to peer companies that also provide non-GAAP disclosures.
- The Company excludes from its non-GAAP results the loss (gain) on contingent consideration, net related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to academic institutions under separate license agreements as it is a non-cash item and is not considered to be normal operating expenses due to its variability of amounts and lack of predictability as to occurrence and/or timing.
- The Company excludes from its non-GAAP results the gain from sale of the PRV obtained as a result of the FDA approval of ELEVIDYS in June 2023 as it is an infrequent event and is not indicative of the performance of the Company's core operations, which accordingly would make it difficult to compare the Company's results to peer companies that also provide non-GAAP disclosures.

Beginning in the fourth quarter of 2023, amortization of in-licensed rights (formerly included within depreciation and amortization expense) and income tax (benefit) expense are no longer excluded from the non-GAAP results. The Company now includes the income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable effective income tax rate. Non-GAAP financial results for the fourth quarter and full-year 2022 have been updated to reflect this change for comparability.

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

About EXONDYS 51
EXONDYS 51 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 production 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.

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

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

not recommended.

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

For further information, please see the full Prescribing Information.

About VYONDYS 53
VYONDYS 53 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.

Important Safety Information for VYONDYS 53
Hypersensitivity reactions, including 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 or interrupting the VYONDYS 53 therapy.

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 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.

For further information, please see the full Prescribing Information.

About AMONDYS 45
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.

Important Safety Information for AMONDYS 45

CONTRAINDICATION:

Known hypersensitivity to casimersen or any of the inactive ingredients. 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.

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.

For further information, please see the full Prescribing Information.

About ELEVIDYS (delandistrogene moxeparvovec-rokl)
ELEVIDYS is indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. This indication is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS. 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:

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS. 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.
- 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.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (Duchenne) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on X (formerly Twitter), LinkedIn, Instagram and Facebook.

Forward-Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs and technologies; the potential benefits of our technologies, product candidates and scientific approaches; the potential for the ELEVIDYS efficacy supplement to expand the approved label for ELEVIDYS and convert the ELEVIDYS accelerated approval to a traditional approval; the review goal date of June 21, 2024 for the ELEVIDYS efficacy supplement; our understanding that FDA does not plan to hold an advisory committee to discuss the ELEVIDYS supplement; the potential for 2024 being the most important year yet for families living with Duchenne and those invested in the improvement of patients' lives; and expected plans and milestones.

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; because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze; 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; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 2023 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 Operations
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2023	2022	2023	2022
Revenues:				
Products, net	$ 365,071	$ 235,933	$ 1,144,876	$ 843,769
Collaboration and other	31,710	22,494	98,460	89,244
Total revenues	396,781	258,427	1,243,336	933,013
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	44,176	30,799	150,343	139,989
Research and development	195,517	213,804	877,387	877,090
Selling, general and administrative	131,700	120,478	481,871	451,421
Amortization of in-licensed rights	763	179	1,559	714
Total cost and expenses	372,156	365,260	1,511,160	1,469,214
Operating income (loss)	24,625	(106,833)	(267,824)	(536,201)
Other income (loss), net:				
Other income (expense), net	15,746	5,527	33,055	(28,321)
Loss on debt extinguishment	—	—	(387,329)	(125,441)

Gain from sale of Priority Review Voucher	—	—	102,000	—
Total other income (loss), net	15,746	5,527	(252,274)	(153,762)
Income (loss) before income tax (benefit) expense	40,371	(101,306)	(520,098)	(689,963)
Income tax (benefit) expense	(5,284)	7,938	15,879	13,525
Net income (loss)	$ 45,655	$ (109,244)	$ (535,977)	$ (703,488)
Net earnings (loss) per share:				
Basic	$ 0.49	$ (1.24)	$ (5.80)	$ (8.03)
Diluted	$ 0.47	$ (1.24)	$ (5.80)	$ (8.03)
Weighted-average number of shares of common stock used in computing basic net earnings (loss) per share	93,617	87,838	92,398	87,559
Weighted-average number of shares of common stock used in computing diluted net earnings (loss) per share	105,594	87,838	92,398	87,559

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2023	2022	2023	2022
GAAP net income (loss)	$ 45,655	$ (109,244)	$ (535,977)	$ (703,488)
Interest (income) expense, net	(17,469)	(8,865)	(64,034)	25,525
Depreciation and amortization expense*	10,609	10,374	42,838	41,150
Stock-based compensation expense	45,826	50,510	182,514	233,018
Impairment of strategic investments	2,500	2,575	30,321	2,575
Loss on debt extinguishment	—	—	387,329	125,441
Loss (gain) on contingent consideration, net	—	—	1,200	(6,700)
Gain from sale of Priority Review Voucher	—	—	(102,000)	—
Income tax effect of adjustments**	(541)	1,075	(1,738)	(7,915)
Non-GAAP net income (loss)**	$ 86,580	$ (53,575)	$ (59,547)	$ (290,394)
Non-GAAP net earnings (loss) per share:				
Diluted***	$ 0.82	$ (0.61)	$ (0.64)	$ (3.32)
Weighted-average number of shares of common stock used in computing earnings per share:				
Diluted	105,594	87,838	92,398	87,559

*Beginning in the fourth quarter of 2023, depreciation and amortization excludes amortization of in-licensed rights. Non-GAAP financial results for the fourth quarter and full-year 2022 have been updated to reflect this change for comparability.
**Beginning in the fourth quarter of 2023, income tax (benefit) expense is no longer excluded from the non-GAAP results. The Company has replaced this metric with income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable effective income tax rate. Refer below for a reconciliation of effective tax rates. Non-GAAP financial results for the fourth quarter and full-year 2022 have been updated to reflect this change for comparability.
***Non-GAAP earnings per share is calculated using diluted shares whereas non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive.

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2023	2022	2023	2022
Total effective tax rate, GAAP	(12.0)%	5.5%	(3.1)%	(2.0)%
Less: impact of GAAP to Non-GAAP adjustments	(0.7)	(1.8)	(39.0)	(6.0)
Total effective tax rate, Non-GAAP	(12.7)%	3.7%	(42.1)%	(8.0)%

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2023	2022	2023	2022
GAAP research and development expenses	$ 195,517	$ 213,804	$ 877,387	$ 877,090
Stock-based compensation expense	(22,174)	(18,963)	(82,489)	(61,293)
Depreciation and amortization expense	(8,217)	(8,013)	(33,011)	(31,713)
Non-GAAP research and development expenses	$ 165,126	$ 186,828	$ 761,887	$ 784,084

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2023	2022	2023	2022
GAAP selling, general and administrative expenses	$ 131,700	$ 120,478	$ 481,871	$ 451,421
Stock-based compensation expense	(23,652)	(31,547)	(100,025)	(171,725)
Depreciation expense	(2,392)	(2,361)	(9,827)	(9,437)
Non-GAAP selling, general and administrative expenses	$ 105,656	$ 86,570	$ 372,019	$ 270,259

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

	As of December 31,	
	2023	2022
Assets		
Current assets:		
Cash and cash equivalents	$ 428,430	$ 966,777
Short-term investments	1,247,820	1,022,597
Accounts receivable, net	400,327	214,628
Inventory	322,859	203,968
Other current assets	179,895	149,891
Total current assets	2,579,331	2,557,861
Property and equipment, net	227,154	180,037
Right of use assets	129,952	64,954
Non-current inventory	191,368	162,545
Other non-current assets	136,771	162,969
Total assets	$ 3,264,576	$ 3,128,366
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 164,918	$ 95,875
Accrued expenses	314,997	418,996
Deferred revenue, current portion	50,416	89,244
Current portion of long-term debt	105,483	—
Current portion of lease liabilities	17,845	15,489
Total current liabilities	653,659	619,604
Long-term debt	1,132,515	1,544,292
Lease liabilities, net of current portion	140,965	57,578
Deferred revenue, net of current portion	437,000	485,000
Contingent consideration	38,100	36,900
Other non-current liabilities	3,000	42
Total liabilities	2,405,239	2,743,416
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; 93,731,831 and 87,950,117 issued and outstanding at December 31, 2023 and 2022, respectively	9	9
Additional paid-in capital	5,304,623	4,296,841
Accumulated other comprehensive income (loss), net of tax	918	(1,664)

Accumulated deficit	(4,446,213)	(3,910,236)
Total stockholders' equity	859,337	384,950
Total liabilities and stockholders' equity	$ 3,264,576	$ 3,128,366

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