Sarepta Therapeutics Announces Third Quarter 2023 Financial Results and Recent Corporate Developments

11/1/23

- ***ELEVIDYS net product revenues for the quarter totaled $69.1 million***
- ***Total revenues, which consist of net product revenues and collaboration revenues, for the third quarter 2023 totaled $331.8 million***
- ***Net product revenues for the third quarter 2023 totaled $309.3 million, a 49% increase over the same quarter of prior year***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 1, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the third quarter 2023.

“The third quarter was a defining moment for Sarepta. We launched ELEVIDYS, our fourth therapy and the first gene therapy for boys with Duchenne muscular dystrophy, we continued to drive great performance of our three PMOs and importantly, on a non-GAAP basis we have achieved profitability, placing us in ever more rarified territory in biotech,” said Doug Ingram, president and CEO, Sarepta Therapeutics. “Reflecting a superb launch, ELEVIDYS net product revenue came in at $69.1 million. Total net product revenue stands at $309.3 million, growing 49 percent over the same quarter last year. And non-GAAP earnings stood at approximately $38.0 million in the quarter, a major milestone for Sarepta.”

Third Quarter 2023 and Recent Development:

- ***Announced topline results from EMBARK, a global pivotal study of ELEVIDYS gene therapy for Duchenne muscular dystrophy:*** The topline results from EMBARK support the conclusion that ELEVIDYS modifies the course of the disease in patients with Duchenne. ELEVIDYS-treated patients showed an increase on the North Star Ambulatory Assessment, a measure of motor function, compared to placebo-treated patients at 52 weeks, although the primary endpoint was not met. Robust, statistically significant results on all key functional pre-specified secondary endpoints, including time to rise (p=0.0025), and 10-meter walk test (p=0.0048), demonstrated evidence of a clinically meaningful treatment benefit that was similar in magnitude and statistical significance across all age groups. No new safety signals were observed. Importantly, we have shared the EMBARK topline results with Food and Drug Administration (FDA) leadership and they have confirmed that, based on the totality of the evidence, they are open to such label expansion if supported by review of the data, and that they intend to proceed rapidly with consideration of the submission.

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 September 30, 2023, the Company reported a GAAP net loss of $40.9 million, or $0.46 per basic and diluted share, compared to a GAAP net loss of $257.7 million reported for the same period of 2022, or $2.94 per basic and diluted share. The non-GAAP net income for the three months ended September 30, 2023 was $37.7 million, or $0.37 per diluted share, compared to a non-GAAP net loss of $70.0 million, or $0.80 per diluted share for the same period of 2022.

For the nine months ended September 30, 2023, the Company reported a GAAP net loss of $581.6 million, or $6.56 per basic and diluted share, compared to a GAAP net loss of $594.2 million reported for the same period of 2022, or $6.79 per basic and diluted share. The non-GAAP net loss for the nine months ended September 30, 2023 was $123.0 million, or $1.39 per diluted share, compared to a non-GAAP net loss of $221.7 million, or $2.53 per diluted share for the same period of 2022.

Revenues

For the three months ended September 30, 2023, the Company recorded total revenues of $331.8 million, which consist of net product revenues and collaboration revenues, compared to total revenues of $230.3 million for the same period of 2022, an increase of $101.5 million. For the nine months ended September 30, 2023, the Company recorded total revenues of $846.6 million, compared to total revenues of $674.6 million for the same period of 2022, an increase of $172.0 million. The increase primarily reflects increasing demand for EXONDYS 51, AMONDYS 45 and VYONDYS 53 (collectively, the “PMO Products”), as well as $69.1 million of net product revenues associated with sales of ELEVIDYS during the three and nine months ended September 30, 2023.

For the three months ended September 30, 2023, the Company recorded net product revenues of $309.3 million, compared to net product revenues of $207.8 million for the same period of 2022, an increase of $101.5 million. For the nine months ended September 30, 2023, the Company recorded net product revenues of $779.8 million, compared to net product revenues of $607.8 million for the same period of 2022, an increase of $172.0 million.

The increase primarily reflects increasing demand for the PMO Products, as well as $69.1 million of net product revenues associated with sales of ELEVIDYS during the three and nine months ended September 30, 2023.

For both the three and nine months ended September 30, 2023 and 2022, the Company recognized $22.5 million and $66.8 million of collaboration revenue, respectively. For all periods presented, collaboration revenue primarily relates to the F. Hoffman-La Roche Ltd. (Roche) collaboration arrangement.

Cost and Expenses

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

For the three months ended September 30, 2023, cost of sales (excluding amortization of in-licensed rights) was $37.0 million, compared to $40.0 million for the same period of 2022, a decrease of $3.0 million. For the nine months ended September 30, 2023, cost of sales (excluding amortization of in-licensed rights) was $106.2 million, compared to $109.2 million for the same period of 2022, a decrease of $3.0 million. The decrease in the three months ended September 30, 2023 primarily reflects write-offs of certain batches of the Company's products not meeting the Company's quality specifications in the three months ended September 30, 2022, with no similar activity for the same period of 2023, partially offset by an increasing demand for the Company's PMO Products. The change for the nine months ended September 30, 2023 primarily reflects a decrease in royalty payments during the nine months ended September 30, 2023 due to changes in the BioMarin Pharmaceuticals, Inc. (BioMarin) royalty terms and a decrease in write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the nine months ended September 30, 2023, as compared to the same period of 2022, partially offset by an increasing demand for the Company's PMO Products.

Research and development

Research and development expenses were $194.3 million for the three months ended September 30, 2023, compared to $216.7 million for the same period of 2022, a decrease of $22.4 million. The decrease in research and development expenses primarily reflects the following:

- $50.5 million decrease in manufacturing expenses primarily due to lower purchases of raw material consumables in the three months ended September 30, 2023, compared to the same period of 2022, as well as the capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023;
- $1.7 million decrease in up-front, milestone and other expenses primarily due to $5.0 million of up-front payments as a result of the execution of certain research and license agreements and $0.5 million of expense incurred as a result of milestone achievements in certain research and license agreements in the three months ended September 30, 2022, partially offset by $3.8 million of expense incurred as a result of the milestone achievements in certain research and license agreements in the same period of 2023;
- $1.2 million increase in pre-clinical expenses primarily due to an increase in toxicology study activity across multiple gene therapy and RNA platforms;
- $1.9 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the three months ended September 30, 2023 and an increase in lab-related expenses as a result of changes in headcount;
- $2.4 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors for clinical programs;
- $2.4 million increase in collaboration cost-sharing expenses primarily due to the timing of expense incurred related to Genethon's micro-dystrophin drug candidate;
- $5.7 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $6.3 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $7.5 million increase in stock-based compensation expense primarily due to the achievement of performance conditions related to certain shares with performance conditions (PSUs), as well as changes in headcount and the value of stock awards;
- $15.3 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MIS51ON, MOMENTUM and ENVISION programs; and
- $12.9 million increase in the offset to expense associated with a collaboration reimbursement from Roche due to continuing development of the Company’s SRP-9001 gene therapy programs.

Research and development expenses were $681.9 million for the nine months ended September 30, 2023, compared to $663.3 million for the same period of 2022, an increase of $18.6 million. The increase in research and development expenses primarily reflects the following:

- $39.4 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MIS51ON, MOMENTUM, ENVISION programs, as well as additional PPMO and LGMD clinical trials;
- $31.0 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $18.0 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 as of the nine months ended September 30, 2023;
- $13.5 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 nine months ended September 30, 2023 and an increase in lab-related expenses as a result of changes in headcount;
- $7.8 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors for the launch of ELEVIDYS prior to the ELEVIDYS approval in June 2023 and for clinical programs;
- $3.5 million increase in collaboration cost-sharing expenses primarily due to the timing of expense incurred related to Genethon's micro-dystrophin drug candidate;
- $1.5 million increase in pre-clinical expenses primarily due to an increase in toxicology study activity across multiple gene therapy and PPMO platforms;
- $4.9 million decrease in up-front, milestone and other expenses primarily due to $7.8 million of up-front payments as a result of the execution of certain research and license agreements, $4.5 million of expense incurred as a result of milestone achievements in certain research and license agreements and $4.5 million of option and termination expenses in the nine months ended September 30, 2022, partially offset by $7.8 million of up-front payments as a result of the execution of certain research and license agreements and $3.9 million of expense incurred as a result of the milestone achievements in certain research and license agreements in the same period of 2023;
- $86.2 million decrease in manufacturing expenses primarily due to lower purchases of raw material consumables in the nine months ended September 30, 2023, compared to the same period of 2022, as well as the capitalization of commercial batches of ELEVIDYS, the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo Fisher Scientific, Inc. and the $17.1 million termination charge related to the manufacturing and supply agreement with Henogen SA, both of which occurred in the nine months ended September 30, 2022, with no similar activity in the same period of 2023. These amounts were partially offset by a continuing ramp-up of SRP-9001 manufacturing prior to the ELEVIDYS approval in June 2023 and continued manufacturing of clinical batches of SRP-9001; and
- $17.0 million increase in the offset to expense associated with a collaboration reimbursement from Roche due to continuing development of the Company’s SRP-9001 gene therapy programs.

Non-GAAP research and development expenses were $163.9 million and $193.7 million for the three months ended September 30, 2023 and 2022, respectively, a decrease of $29.8 million. Non-GAAP research and development expenses were $596.8 million and $597.3 million for the nine months ended September 30, 2023 and 2022, respectively, a decrease of $0.5 million.

Selling, general and administrative

Selling, general and administrative expenses were $120.9 million for the three months ended September 30, 2023, compared to $104.8 million for the same period in 2022, an increase of $16.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $12.8 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors for the launch of ELEVIDYS and ongoing litigation matters;
- $6.6 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $3.4 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $3.2 million increase in other expenses primarily due to changes in charitable contribution activity; and
- $9.9 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by the achievement of performance conditions related to certain PSUs, as well as changes in headcount and the value of stock awards.

Selling, general and administrative expenses were $350.2 million for the nine months ended September 30, 2023, compared to $330.9 million for the same period in 2022, an increase of $19.3 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $42.6 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors for the launch of ELEVIDYS and ongoing litigation matters;
- $27.3 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $7.3 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $6.1 million increase in other expenses primarily due to changes in charitable contribution activity; and
- $63.8 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by the achievement of performance conditions related to certain PSUs as of the nine months ended September 30, 2023, as well as changes in headcount and the value of stock awards.

Non-GAAP selling, general and administrative expenses were $92.8 million and $66.8 million for the three months ended September 30, 2023 and 2022, respectively, an increase of $26.0 million. Non-GAAP selling, general and administrative expenses were $266.4 million and $183.7 million for the nine months ended September 30, 2023 and 2022, respectively, an increase of $82.7 million.

Amortization of in-licensed rights

For the three and nine months ended September 30, 2023, the Company recorded amortization of in-licensed rights of approximately $0.4 million and $0.8 million, respectively. For the three and nine months ended September 30, 2022, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $0.5 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.

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 closing of 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 nine months ended September 30, 2023 as it did not have a carrying value at the time of the sale.

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 nine months ended September 30, 2023 was $387.3 million, inclusive of $6.9 million in third-party debt conversion costs.

Other income (expense), net

For the three months ended September 30, 2023 and 2022, other expense, net was $12.3 million and other income, net was $0.4 million, respectively. The change was primarily due to the impairment of equity investment and a loss on contingent consideration, net, partially offset by increases in accretion of investment discount, net and increases in interest income due to the investment mix of the Company's investment portfolio, as well as a reductions of interest expense incurred as a result of the repayment of the Company's December 2019 Term Loan in 2022. For the nine months ended September 30, 2023 and 2022, other income, net was $17.3 million and other expense, net was $33.8 million, respectively. The change was primarily due to increases in accretion of investment discount, net and interest income due to the investment mix of the Company's investment portfolio, as well as a reductions 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 equity investment and a loss on contingent consideration, net incurred in the nine months ended September 30, 2023.

Income tax expense

Income tax expense for the three and nine months ended September 30, 2023 was approximately $7.8 million and $21.2 million, respectively. Income tax expense for the three and nine months ended September 30, 2022 was $1.3 million and $5.6 million, respectively. Income tax expense for the three and nine months ended September 30, 2023 relates to state, foreign and federal income taxes, while income tax expense for the three and nine months ended September 30, 2022 relates to state and foreign income taxes.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had approximately $1.8 billion in cash, cash equivalents, investments and long-term restricted cash as of September 30, 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 loss excluding interest (income) expense, net, income tax expense, depreciation and amortization expense, stock-based compensation expense 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 and amortization expense, stock-based compensation expense and other items.

1. Interest, tax, 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. Tax amounts can vary substantially from period to period due to tax adjustments that are not directly related to underlying operating performance. 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 in-licensed rights as well as patent costs are amortized over a period of several years after acquisition or patent application or renewal and generally cannot be changed or influenced by management.

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 on a regular basis. These other items include impairment of equity investments, loss (gain) on contingent consideration, net, gain from sale of the PRV and loss on debt extinguishment.

- The Company excludes from its non-GAAP results the impairment of any equity 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 (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 a non-recurring 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.
- The Company excludes from its non-GAAP results the loss on debt extinguishment, which is considered to be a non-recurring 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 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 (eteplirsen) 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 in patients who have a confirmed mutation of the dystrophin 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 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, 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 Duchenne 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 following adverse reactions have been identified during observational studies that were conducted as part of the clinical development program and continued post approval.

In open-label observational studies, 163 patients received at least one intravenous dose of EXONDYS 51, with doses ranging between 0.5 mg/kg (0.017 times the recommended dosage) and 50 mg/kg (1.7 times the recommended dosage). All patients were male and had genetically confirmed Duchenne muscular dystrophy. Age at study entry was 6 months to 19 years. Most (85%) patients were Caucasian.

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

For further information, please see the full Prescribing Information.

About VYONDYS 53

VYONDYS 53 (golodirsen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to

bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or “skipping,” of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the dystrophin 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 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

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 (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 in patients who have a confirmed mutation of the dystrophin 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 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:

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).

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

IMPORTANT SAFETY INFORMATION

CONTRAINDICATION:

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

WARNINGS AND PRECAUTIONS:

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.

Sarepta is responsible for global development and manufacturing for ELEVIDYS, and distribution within the U.S. will commence immediately. In December 2019, Sarepta partnered with Roche to accelerate access to ELEVIDYS for patients outside the United States.

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 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 and scientific approaches; the potential benefits of ELEVIDYS, including its potential to modify the course of Duchenne; our understanding that FDA, based on the totality of the evidence, is open to a label expansion if supported by the review of the data and intend to proceed rapidly with consideration of the submission for ELEVIDYS; 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; 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; 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; 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, 2022 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 Operations
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2023	2022	2023	2022
Revenues:				
Products, net	$ 309,322	$ 207,774	$ 779,805	$ 607,836
Collaboration	22,495	22,495	66,750	66,750
Total revenues	331,817	230,269	846,555	674,586
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	37,026	39,952	106,167	109,190
Research and development	194,301	216,707	681,870	663,286
Selling, general and administrative	120,893	104,787	350,171	330,943
Amortization of in-licensed rights	439	178	796	535
Total cost and expenses	352,659	361,624	1,139,004	1,103,954

Operating loss	(20,842)	(131,355)	(292,449)	(429,368)
Other loss, net:				
Gain from sale of Priority Review Voucher	—	—	102,000	—
Loss on debt extinguishment	—	(125,441)	(387,329)	(125,441)
Other (expense) income, net	(12,332)	378	17,309	(33,848)
Total other loss, net	(12,332)	(125,063)	(268,020)	(159,289)
Loss before income tax expense	(33,174)	(256,418)	(560,469)	(588,657)
Income tax expense	7,763	1,320	21,163	5,587
Net loss	$ (40,937)	$ (257,738)	$ (581,632)	$ (594,244)
Net loss per share — basic and diluted	$ (0.46)	$ (2.94)	$ (6.56)	$ (6.79)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	88,889	87,628	88,609	87,465

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,	
	2023	2022	2023	2022
GAAP net loss	$ (40,937)	$ (257,738)	$ (581,632)	$ (594,244)
Interest (income) expense, net	(17,593)	6,521	(46,565)	34,390
Income tax expense	7,763	1,320	21,163	5,587
Impairment of equity investments	27,500	—	27,821	—
Loss (gain) on contingent consideration, net	2,000	(6,700)	1,200	(6,700)
Gain from sale of Priority Review Voucher	—	—	(102,000)	—
Loss on debt extinguishment	—	125,441	387,329	125,441
Depreciation and amortization expense	10,928	10,703	33,025	31,311
Stock-based compensation expense	48,061	50,418	136,688	182,508
Non-GAAP net income (loss)	$ 37,722	$ (70,035)	$ (122,971)	$ (221,707)
Non-GAAP net earnings (loss) per share:				
Diluted Non-GAAP net earnings (loss) per share*	$ 0.37	$ (0.80)	$ (1.39)	$ (2.53)
Weighted average number of shares of common stock used in computing earnings per share:				
Diluted	101,722	87,628	88,609	87,465

*Non-GAAP earnings per share is calculated using diluted shares whereas non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive. There was a $0.05 impact to the calculation of non-GAAP net earnings per share as a result of the inclusion of diluted shares for the three months ended September 30, 2023.

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2023	2022	2023	2022
GAAP research and development expenses	$ 194,301	$ 216,707	$ 681,870	$ 663,286
Stock-based compensation expense	(22,325)	(14,795)	(60,315)	(42,330)
Depreciation and amortization expense	(8,109)	(8,166)	(24,794)	(23,700)
Non-GAAP research and development expenses	$ 163,867	$ 193,746	$ 596,761	$ 597,256

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2023	2022	2023	2022
GAAP selling, general and administrative expenses	$ 120,893	$ 104,787	$ 350,171	$ 330,943

Stock-based compensation expense	(25,736)	(35,623)	(76,373)	(140,178)
Depreciation and amortization expense	(2,380)	(2,359)	(7,435)	(7,076)
Non-GAAP selling, general and administrative expenses	$ 92,777	$ 66,805	$ 266,363	$ 183,689

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

	As of September 30, 2023	As of December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 541,932	$ 966,777
Short-term investments	1,191,610	1,022,597
Accounts receivable	318,855	214,628
Inventory	244,011	203,968
Other current assets	154,441	149,891
Total current assets	2,450,849	2,557,861
Property and equipment, net	212,367	180,037
Right of use assets	133,454	64,954
Non-current inventory	176,112	162,545
Other non-current assets	136,925	162,969
Total assets	$ 3,109,707	$ 3,128,366
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 87,948	$ 95,875
Accrued expenses	322,350	418,996
Deferred revenue, current portion	22,494	89,244
Other current liabilities	17,951	15,489
Total current liabilities	450,743	619,604
Long-term debt	1,236,755	1,544,292
Lease liabilities, net of current portion	134,752	57,578
Deferred revenue, net of current portion	485,000	485,000
Contingent consideration	38,100	36,900
Other non-current liabilities	—	42
Total liabilities	2,345,350	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; 93,537,355 and 87,950,117 issued and outstanding at September 30, 2023, and December 31, 2022, respectively	9	9
Additional paid-in capital	5,256,854	4,296,841
Accumulated other comprehensive loss, net of tax	(638)	(1,664)
Accumulated deficit	(4,491,868)	(3,910,236)
Total stockholders' equity	764,357	384,950
Total liabilities and stockholders' equity	$ 3,109,707	$ 3,128,366

View source version on businesswire.com: https://www.businesswire.com/news/home/20231101211984/en/

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