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

8/2/23

– ***Following approval on June 22, the first patient received commercially reimbursed ELEVIDYS (delandistrogene moxeparvovec-rokl), earlier today***

– ***Total revenues, which consist of net product revenues and collaboration revenues, for the second quarter 2023 totaled $261.2 million***

– ***Net product revenues for the second quarter 2023 totaled $239.0 million, a 13% increase over the same quarter of prior year***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 2, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the second quarter 2023.

"With a positive Advisory Committee vote followed by the approval of ELEVIDYS in June, this quarter marks an historic milestone in the treatment of Duchenne muscular dystrophy. The launch of ELEVIDYS is off to a great start, with our first reimbursed infusion today, ahead of plan. In addition to making this launch a success, our paramount goal is to translate a positive result in our confirmatory trial, EMBARK, later this year to a broad label as rapidly as possible," said Doug Ingram, president and chief executive officer, Sarepta. "Even as we focus on the launch of our fourth approved therapy, we have remained committed to serving the community with our three approved PMO therapies. Achieving net product revenue in the second quarter of $239.0 million on combined sales of EXONDYS 51, AMONDYS 45, and VYONDYS 53, I am pleased to report that we continued to serve the Duchenne community."

Second Quarter 2023 and Recent Developments:

- ***First ELEVIDYS commercial patient received treatment today:*** Following approval on June 22, the first patient received commercially reimbursed ELEVIDYS (delandistrogene moxeparvovec-rokl), earlier today.
- ***Announced FDA approval of ELEVIDYS, the first gene therapy to treat Duchenne muscular dystrophy:*** The company received FDA accelerated approval of ELEVIDYS (delandistrogene moxeparvovec-rokl), an adeno-associated virus based gene therapy 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 the protein produced by ELEVIDYS in skeletal muscle observed in patients treated with ELEVIDYS. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene. ELEVIDYS is supported by biologic and empirical evidence, in addition to efficacy data from two clinical studies: SRP-9001-102 and SRP-9001-103 and safety data from SRP-9001-101, SRP-9001-102 and SRP-9001-103. Consistent with the accelerated approval pathway, Sarepta has committed to the completion of a confirmatory trial. EMBARK, the global, randomized, double-blind, placebo-controlled Phase 3 trial for ELEVIDYS, will serve as the post-marketing confirmatory trial and is fully enrolled with top-line results expected in late 2023.
- ***Completed the sale of Rare Pediatric Disease Priority Review Voucher (PRV):*** Sarepta received a payment of $102.0 million upon completion of the sale of its PRV. Sarepta was awarded the PRV following U.S. Food and Drug Administration (FDA) accelerated approval of ELEVIDYS (delandistrogene moxeparvovec-rokl). The company will invest proceeds from the sale of the PRV into R&D efforts to support the development of additional potentially transformative therapies.
- ***Commenced study SRP-9001-303 or ENVISION in non-ambulatory and ambulatory individuals with Duchenne:*** The ENVISION study is a global, randomized, double-blind, placebo-controlled 2-part study evaluating the safety and efficacy of delandistrogene moxeparvovec gene therapy in non-ambulatory and ambulatory individuals with Duchenne. The primary outcome for Part 1 is change from baseline at Week 72 in the total score of PUL (performance of upper limb). Participants will be in the study for approximately 128 weeks. All participants will have the opportunity to receive intravenous (IV) delandistrogene moxeparvovec in either Part 1 or Part 2. The trial design was selected in order to satisfy ex-U.S. regulatory requirements. The study is designed to maximize probability of success in this population and is intended to make treatment available to the broadest population of individuals living with Duchenne by providing robust studies to global regulatory agencies.
- ***Dosed first patient in the proof-of-concept trial SRP-6004-102 or NAVIGENE study:*** In the second quarter, Sarepta successfully dosed the first patient in the NAVIGENE study; a Phase 1 open-label trial evaluating the safety, tolerability, and efficacy of SRP-6004 administered by systemic infusion in ambulatory individuals living with LGMD2B/R2 (dysferlinopathy). The learnings from this study will be used to inform the development program which would include studies with a larger number of participants. SRP-6004 is Sarepta's investigational dual-vector gene therapy approach for treating dysferlinopathy and is designed to express the full-length protein dysferlin that is missing in individuals with LGMD2B/R2.

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 June 30, 2023, the Company reported a GAAP net loss of $23.9 million, or $0.27 per basic and diluted share, compared to a GAAP net loss of $231.5 million reported for the same period of 2022, or $2.65 per basic and diluted share. The non-GAAP net loss for the three months ended June 30, 2023 was $75.2 million, or $0.85 per basic and diluted share, compared to a non-GAAP net loss of $103.0 million, or $1.18 per basic and diluted share for the same period of 2022.

For the six months ended June 30, 2023, the Company reported a GAAP net loss of $540.7 million, or $6.11 per basic and diluted share, compared to a GAAP net loss of $336.5 million reported for the same period of 2022, or $3.85 per basic and diluted share. The non-GAAP net loss for the six months ended June 30, 2023 was $160.7 million, or $1.82 per basic and diluted share, compared to a non-GAAP net loss of $151.7 million, or $1.74 per basic and diluted share for the same period of 2022.

Revenues

For the three months ended June 30, 2023, the Company recorded total revenues of $261.2 million, which consist of net product revenues and collaboration revenues, compared to total revenues of $233.5 million for the same period of 2022, an increase of $27.7 million. For the six months ended June 30, 2023, the Company recorded total revenues of $514.7 million, compared to total revenues of $444.3 million for the same period of 2022, an increase of $70.4 million.

For the three months ended June 30, 2023, the Company recorded net product revenues of $239.0 million, compared to net product revenues of $211.2 million for the same period of 2022, an increase of $27.8 million. For the six months ended June 30, 2023, the Company recorded net product revenues of $470.5 million, compared to net product revenues of $400.1 million for the same period of 2022, an increase of $70.4 million. The increase primarily reflects increasing demand for EXONDYS 51, AMONDYS 45 and VYONDYS 53 (collectively, “PMO Products”). There were no product sales related to ELEVIDYS during the three or six months ended June 30, 2023.

For both the three and six months ended June 30, 2023 and 2022, the Company recognized $22.3 million and $44.3 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 June 30, 2023, cost of sales (excluding amortization of in-licensed rights) was $34.1 million, compared to $37.8 million for the same period of 2022, a decrease of $3.7 million. For the six months ended June 30, 2023, cost of sales (excluding amortization of in-licensed rights) was $69.1 million, compared to $69.2 million for the same period of 2022, a decrease of $0.1 million. The decrease in the three months ended June 30, 2023 primarily reflects a decrease in royalty payments during the three months ended June 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, as compared to the same period of 2022, partially offset by an increasing demand for the Company's PMO Products. The change for the six months ended June 30, 2023 primarily reflects a decrease in royalty payments during the six months ended June 30, 2023 due to changes in the BioMarin royalty terms, offset by an increasing demand for the Company's PMO Products and an increase in write-offs of certain batches of the Company's products not meeting the Company's quality specifications, as compared to the same period of 2022.

Research and development

Research and development expenses were $241.9 million for the three months ended June 30, 2023, compared to $252.3 million for the same period of 2022, a decrease of $10.4 million. The decrease in research and development expenses primarily reflects the following:

- $51.3 million decrease in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo Fisher Scientific Inc. (Thermo) and the $17.1 million termination charge related to the manufacturing and supply agreement with Henogen SA (Henogen), both of which occurred in the three months ended June 30, 2022, with no similar activity in the same period of 2023, partially offset by a continuing ramp-up of SRP-9001 manufacturing prior to the ELEVIDYS approval in June 2023;
- $3.7 million decrease in up-front, milestone and other expenses primarily due to $2.8 million of up-front payments as a result of the execution of certain research and license agreements, $4.0 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 three months ended June 30, 2022, partially offset by $7.5 million of up-front payments as a result of the execution of certain research and license agreements in the same period of 2023;
- $1.8 million increase in pre-clinical expenses primarily due to a decrease in toxicology study activity across multiple gene therapy and RNA platforms;
- $2.7 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;
- $4.1 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the three months ended June 30, 2023 and an increase in lab-related expenses primarily due to changes in headcount;
- $5.0 million increase in facility- and technology-related expenses primarily due to the Company’s continuing expansion efforts;
- $7.1 million increase in stock-based compensation expense primarily due to the achievement of one performance condition related to certain restricted stock units with performance conditions (PSUs) during the three months ended June 30, 2023, as well as changes in headcount and the value of stock awards;

- $10.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $13.7 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MOMENTUM, ENVISION and MIS51ON programs; and
- $1.6 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 $487.6 million for the six months ended June 30, 2023, compared to $446.6 million for the same period of 2022, an increase of $41.0 million. The increase in research and development expenses primarily reflects the following:

- $24.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $24.1 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for the Company's MOMENTUM and MIS51ON programs;
- $10.5 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 one performance condition related to certain PSUs during the three months ended June 30, 2023;
- $10.1 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the six months ended June 30, 2023 and an increase in lab-related expenses;
- $7.8 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $5.4 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;
- $3.2 million decrease in up-front, milestone and other expenses primarily due to $2.8 million of up-front payments as a result of the execution of certain research and license agreements, $4.0 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 six months ended June 30, 2022, partially offset by $7.8 million of up-front payments as a result of the execution of certain research and license agreements in the same period of 2023;
- $35.6 million decrease in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo and the $17.1 million termination charge related to the manufacturing and supply agreement with Henogen, both of which occurred in the three months ended June 30, 2022, with no similar activity in the same period of 2023, partially offset by a continuing ramp-up of SRP-9001 manufacturing prior to the ELEVIDYS approval in June 2023; and
- $4.1 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 $212.2 million and $230.4 million for the three months ended June 30, 2023 and 2022, respectively, a decrease of $18.2 million. Non-GAAP research and development expenses were $432.9 million and $403.5 million for the six months ended June 30, 2023 and 2022, respectively, an increase of $29.4 million.

Selling, general and administrative

Selling, general and administrative expenses were $118.6 million for the three months ended June 30, 2023, compared to $154.3 million for the same period in 2022, a decrease of $35.7 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $62.6 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by changes in headcount and the value of stock awards and the achievement of one performance condition related to certain PSUs during the three months ended June 30, 2023;
- $2.2 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;
- $9.7 million increase in compensation and other personnel expenses primarily due to changes in headcount; and
- $12.0 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.

Selling, general and administrative expenses were $229.3 million for the six months ended June 30, 2023, compared to $226.2 million for the same period in 2022, an increase of $3.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $29.8 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;
- $20.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $3.9 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $2.9 million increase in other expenses primarily due to changes in charitable contribution activity; and
- $53.9 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by changes in headcount and the value of stock awards and the

achievement of one performance condition related to certain PSUs during the six months ended June 30, 2023.

Non-GAAP selling, general and administrative expenses were $90.3 million and $63.7 million for the three months ended June 30, 2023 and 2022, respectively, an increase of $26.6 million. Non-GAAP selling, general and administrative expenses were $173.6 million and $116.9 million for the six months ended June 30, 2023 and 2022, respectively, an increase of $56.7 million.

Amortization of in-licensed rights

For both the three months ended June 30, 2023 and 2022, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For both the six months ended June 30, 2023 and 2022, the Company recorded amortization of in-licensed rights of approximately $0.4 million. 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 as it did not have a carrying value at the time of the sale. There was no such gain recognized during the same period in 2022.

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 six months ended June 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 June 30, 2023 and 2022, other income, net was $16.9 million and other expense, net was $17.0 million, respectively. For the six months ended June 30, 2023 and 2022, other income, net was $29.6 million and other expense, net was $34.2 million, respectively. The changes are primarily due to 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 reduction of interest expense incurred as a result of the repayment of the December 2019 Term Loan in 2022.

Income tax expense

Income tax expense for the three and six months ended June 30, 2023 was approximately $9.4 million and $13.4 million, respectively. Income tax expense for the three and six months ended June 30, 2022 was $3.4 million and $4.3 million, respectively. Income tax expense for the three and six months ended June 30, 2023 relates to state, foreign and federal income taxes, while income tax expense for the three and six months ended June 30, 2022 relates to state and foreign income taxes.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had approximately $1.9 billion in cash, cash equivalents, investments and long-term restricted cash as of June 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 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 loss on debt extinguishment, impairment of equity investments,

gain from sale of the PRV and gain on contingent consideration.

- 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 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 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.
- The Company excludes from its non-GAAP results the gain on contingent consideration 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 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 income tax expense, non-GAAP net loss, and non-GAAP basic and diluted net 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 Duchenne 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. Urinei 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
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 Duchenne 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 observed 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 tract 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 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 (delandistrogene moxeparvovec-rokl) is a single-dose gene transfer therapy for intravenous infusion designed to address the underlying cause of Duchenne muscular dystrophy through the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle. ELEVIDYS has been evaluated in three on-going clinical studies: SRP-9001-101, SRP-9001-102 and SRP-9001-103. Accelerated approval was primarily based on data from SRP-9001-102 and SRP-9001-103. More than 80 treated patients across the three studies contributed to the safety profile of ELEVIDYS. ELEVIDYS is also being studied in Study SRP-9001-301 (also known as EMBARK), a global, randomized, double-blind, placebo-controlled Phase 3 clinical trial in 126 participants with Duchenne between the ages of 4 to 7 years.

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.

ELEVIDYS is approved under accelerated review based on expression of ELEVIDYS micro-dystrophin in skeletal muscle. Continued approval for this indication in this and other age groups will be contingent upon verification of a clinical benefit in confirmatory trials. ELEVIDYS has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

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, including our ENVISION and NAVIGENE studies; and expected plans and milestones, including receiving top-line results from EMBARK in late 2023, our paramount goal to translate a positive result in our confirmatory trial, EMBARK, later this year to a broad label as rapidly as possible, and investing proceeds from the sale of the PRV into R&D efforts to support the development of more transformative therapies.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, some of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, the COVID-19 pandemic and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading “Risk Factors” in our most recent Annual Report on Form 10-K for the year ended December 31, 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 June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
Revenues:				
Products, net	$ 238,988	$ 211,237	$ 470,483	$ 400,062
Collaboration	22,250	22,250	44,255	44,255
Total revenues	261,238	233,487	514,738	444,317
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	34,124	37,795	69,141	69,238
Research and development	241,890	252,329	487,569	446,579
Selling, general and administrative	118,564	154,316	229,278	226,156
Amortization of in-licensed rights	179	179	357	357
Total cost and expenses	394,757	444,619	786,345	742,330
Operating loss	(133,519)	(211,132)	(271,607)	(298,013)
Other income (loss), net:				
Gain from sale of Priority Review Voucher	102,000	—	102,000	—
Loss on debt extinguishment	—	—	(387,329)	—
Other income (expense), net	16,934	(16,961)	29,641	(34,226)
Total other income (loss), net	118,934	(16,961)	(255,688)	(34,226)
Loss before income tax expense	(14,585)	(228,093)	(527,295)	(332,239)
Income tax expense	9,355	3,388	13,400	4,267
Net loss	$ (23,940)	$ (231,481)	$ (540,695)	$ (336,506)
Net loss per share — basic and diluted	$ (0.27)	$ (2.65)	$ (6.11)	$ (3.85)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	88,743	87,511	88,466	87,383

Sarepta Therapeutics, Inc.
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
GAAP net loss	$ (23,940)	$ (231,481)	$ (540,695)	$ (336,506)
Interest (income) expense, net	(15,980)	12,288	(28,972)	27,869
Income tax expense	9,355	3,388	13,400	4,267
Impairment of equity investment	—	—	321	—
Gain on contingent consideration	(800)	—	(800)	—
Gain from sale of Priority Review Voucher	(102,000)	—	(102,000)	—
Loss on debt extinguishment	—	—	387,329	—
Depreciation and amortization expense	10,792	9,889	22,097	20,608
Stock-based compensation expense	47,377	102,892	88,627	132,090
Non-GAAP net loss	$ (75,196)	$ (103,024)	$ (160,693)	$ (151,672)
Non-GAAP net loss per share:				
Basic and diluted	$ (0.85)	$ (1.18)	$ (1.82)	$ (1.74)
Weighted average number of shares of common stock used in computing earnings per share:				
Basic and diluted	88,743	87,511	88,466	87,383

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
GAAP research and development expenses	$ 241,890	$ 252,329	$ 487,569	$ 446,579
Stock-based compensation expense	(21,577)	(14,467)	(37,990)	(27,535)
Depreciation and amortization expense	(8,134)	(7,512)	(16,685)	(15,534)
Non-GAAP research and development expenses	$ 212,179	$ 230,350	$ 432,894	$ 403,510

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
GAAP selling, general and administrative expenses	$ 118,564	$ 154,316	$ 229,278	$ 226,156
Stock-based compensation expense	(25,800)	(88,425)	(50,637)	(104,555)
Depreciation and amortization expense	(2,479)	(2,198)	(5,055)	(4,717)
Non-GAAP selling, general and administrative expenses	$ 90,285	$ 63,693	$ 173,586	$ 116,884

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

	As of June 30, 2023	As of December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 851,929	$ 966,777
Short-term investments	1,008,786	1,022,597
Accounts receivable	236,808	214,628
Inventory	226,876	203,968
Other current assets	148,215	149,891
Total current assets	2,472,614	2,557,861
Property and equipment, net	188,874	180,037
Right of use assets	134,728	64,954
Non-current inventory	166,635	162,545
Other non-current assets	163,039	162,969
Total assets	$ 3,125,890	$ 3,128,366

Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 109,796	$ 95,875
Accrued expenses	326,877	418,996
Deferred revenue, current portion	44,989	89,244
Other current liabilities	16,992	15,489
Total current liabilities	498,654	619,604
Long-term debt	1,235,517	1,544,292
Lease liabilities, net of current portion	129,170	57,578
Deferred revenue, net of current portion	485,000	485,000
Contingent consideration	36,100	36,900
Other non-current liabilities	38	42
Total liabilities	2,384,479	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; 93,273,541 and 87,950,117 issued and outstanding at June 30, 2023, and December 31, 2022, respectively	9	9
Additional paid-in capital	5,193,388	4,296,841
Accumulated other comprehensive loss, net of tax	(1,055)	(1,664)
Accumulated deficit	(4,450,931)	(3,910,236)
Total stockholders' equity	741,411	384,950
Total liabilities and stockholders' equity	$ 3,125,890	$ 3,128,366

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