Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2022 Financial Results and Recent Corporate Developments

2/28/23

- ***Total revenues, which consist of net product revenues and collaboration revenues, for the fourth quarter and full-year 2022 totaled $258.4 million and $933.0 million, respectively***
- ***Net product revenues for the fourth quarter and full-year 2022 totaled $235.9 million and $843.8 million, respectively***
- ***Fourth quarter 2022 net product revenues increased approximately 32% over the fourth quarter of 2021; full-year 2022 net product revenues increased approximately 38% over the prior year***
- ***Mid-cycle meeting complete; FDA does not plan to hold advisory committee for SRP-9001***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 28, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the fourth quarter and full-year 2022.

“Sarepta’s proven ability to launch Duchenne therapies and effectively serve the Duchenne patient community was once again on display in 2022 as we delivered nearly $236 million and $844 million in net product revenue for the quarter and year, respectively, and grew at 32% for the quarter and 38% for the year. Sarepta achieved these stellar results by prioritizing our patients and without taking a single price increase in over 6 years,” said Doug Ingram, Sarepta’s president and chief executive officer. “2023 will be a bellwether year for the patient community we serve and for the promise of gene therapy. Sarepta continues to work toward the May 29, 2023 action date for our gene therapy SRP-9001 Biologics License Application, answering questions, preparing for scheduled pre-approval inspections and preparing for launch. As of the mid-cycle review, which is now complete, the FDA posed CMC questions which have been answered by Sarepta, formally confirmed that there are no significant safety issues of concern and informed Sarepta that they are not planning to hold an Advisory Committee for this application.”

Fourth Quarter 2022 and Recent Developments:

- ***SRP-9001 Biologics License Application (BLA) accepted for filing and granted Priority Review by U.S. FDA:*** In November, the FDA accepted Sarepta’s BLA seeking accelerated approval of SRP-9001 (delandistrogene moxeparvovec) for the treatment of ambulant individuals with Duchenne muscular dystrophy. SRP-9001 has been granted Priority Review by the FDA, with a regulatory action date of May 29, 2023. SRP-9001 is an investigational gene therapy for Duchenne being developed in partnership with Roche. It is designed to treat the proximate cause of Duchenne by delivering to muscle a gene that codes for a shortened, functional form of dystrophin. In addition to a wealth of pre-clinical evidence, the BLA for SRP-9001 included efficacy and safety data from Study SRP-9001-103 (also known as ENDEAVOR), as well as from Studies SRP-9001-101 and SRP-9001-102, and an integrated analysis across these three clinical studies comparing functional results to a propensity-score-weighted external control (EC). In clinical results from more than 80 treated patients, SRP-9001 has demonstrated positive results at multiple time points, including one-, two- and up to four-years after treatment, in addition to demonstrating a consistent safety profile. In addition to Studies SRP-9001-101, SRP-9001-102 and SRP-9001-103, SRP-9001 is also being studied in EMBARK (Study SRP-9001-301), a global, randomized, double-blind, placebo-controlled clinical trial of SRP-9001 which has recruited 125 participants with Duchenne between the ages of 4 to 7. Results from EMBARK are expected by the end of 2023. Sarepta has proposed EMBARK as the post-marketing confirmatory trial for SRP-9001.

- ***Initiated VOYAGENE, a clinical study of SRP-9003 for the treatment of limb-girdle muscular dystrophy Type 2E/R4:*** In mid-February 2023, Sarepta announced that the first patient had been dosed in Study SRP-9003-102, also known as VOYAGENE. VOYAGENE is a phase 1 study of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E). VOYAGENE is a U.S.-only study enrolling ambulant patients aged 18 years or older and non-ambulant patients aged 4 to 50 years old, using clinical process SRP-9003 material.

- ***Expanded strategic manufacturing partnership with Catalent for commercial supply agreement for Duchenne gene therapy candidate:*** In early January, Sarepta and Catalent announced the signing of a commercial supply agreement for Catalent to manufacture SRP-9001 (delandistrogene moxeparvovec), the Company’s most advanced gene therapy candidate for the treatment of Duchenne. Under the terms of this expanded agreement, Catalent will be Sarepta’s primary commercial manufacturing partner for this therapy. The agreement also structures how Catalent may support multiple gene therapy candidates in Sarepta’s pipeline for limb-girdle muscular dystrophy.

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

On a GAAP basis, for the three months ended December 31, 2022, the Company reported a net loss of $109.2 million, or $1.24 per basic and diluted share, compared to a net loss of $122.0 million reported for the same period of 2021, or $1.42 per basic and diluted share. On a non-GAAP basis, the net loss for the three months ended December 31, 2022 was $46.5 million, or $0.53 per basic and diluted share, compared to a net loss of $66.0 million, or $0.77 per basic and diluted share for the same period of 2021.

On a GAAP basis, for the twelve months ended December 31, 2022, the Company reported a net loss of $703.5 million, or $8.03 per basic and diluted share, compared to a net loss of $418.8 million reported for the same period of 2021, or $5.15 per basic and diluted share. On a non-GAAP basis, the net loss for the twelve months ended December 31, 2022 was $268.2 million, or $3.06 per basic and diluted share, compared to a net loss of $308.7 million, or $3.80 per basic and diluted share for the same period of 2021.

Revenues

For the three months ended December 31, 2022, the Company recorded total revenues of $258.4 million, which consist primarily of net product revenues and collaboration revenues, compared to total revenues of $201.5 million for the same period of 2021, an increase of $56.9 million. For the twelve months ended December 31, 2022, the Company recorded total revenues of $933.0 million, compared to total revenues of $701.9 million for the same period of 2021, an increase of $231.1 million.

For the three months ended December 31, 2022, the Company recorded net product revenues of $235.9 million, compared to net product revenues of $178.7 million for the same period of 2021, an increase of $57.2 million. For the twelve months ended December 31, 2022, the Company recorded net product revenues of $843.8 million, compared to net product revenues of $612.4 million for the same period of 2021, an increase of $231.4 million. The increase primarily reflects the continuing increase in demand for the Company's products in the U.S. and a full period of AMONDYS 45 sales during the twelve months ended December 31, 2022 given its commercial launch in February 2021.

For the three and twelve months ended December 31, 2022, the Company recognized $22.5 million and $89.2 million of collaboration and other revenues, respectively. For the three and twelve months ended December 31, 2021, the Company recognized $22.7 million and $89.5 million of collaboration and other revenues, respectively. For all periods presented, collaboration revenue primarily relates to the F. Hoffman-La Roche Ltd. (Roche) collaboration arrangement.

Cost and Operating Expenses

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

For the three months ended December 31, 2022, cost of sales (excluding amortization of in-licensed rights) was $30.8 million, compared to $31.7 million for the same period of 2021, a decrease of $0.9 million. The decrease primarily reflects write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the three months ended December 31, 2021, with no similar activity in the same period of 2022 and a decrease in royalty payments during the three months ended December 31, 2022 due to changes in the BioMarin Pharmaceutical, Inc. (BioMarin) royalty terms. For the twelve months ended December 31, 2022, cost of sales (excluding amortization of in-licensed rights) was $140.0 million, compared to $97.0 million for the same period of 2021, an increase of $43.0 million. The increase primarily reflects increasing demand for the Company's products as well as an increase in write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the twelve months ended December 31, 2022, as compared to the same period of 2021.

Research and development

Research and development expenses were $213.8 million for the three months ended December 31, 2022, compared to $197.3 million for the same period of 2021, an increase of $16.5 million. The increase in research and development expenses primarily reflects the following:

- $18.2 million increase in up-front and milestone expenses primarily due to $18.3 million of up-front payments as a result of the execution of certain research and license agreements during the fourth quarter of 2022, offset by $0.1 million of similar activity during the fourth quarter of 2021;
- $13.8 million increase in clinical trial expenses primarily due to a continuing ramp-up of the Company's SRP-9001 gene therapy programs, including the Company's EMBARK program;
- $9.9 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $6.8 million increase in research and other expenses driven by increases in lab-related expenses, partially offset by a decrease in sponsored research with academic institutions during the three months ended December 31, 2022;
- $4.5 million increase in stock-based compensation expense primarily due to changes in headcount and the value of stock awards;
- $4.0 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $1.4 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors;
- $1.3 million decrease in collaboration cost-sharing expenses primarily due to the termination of the Lysogene S.A. (Lysogene) license and collaboration agreement and timing of expense incurred related to Genethon's micro-dystrophin drug candidate;
- $3.5 million decrease in pre-clinical expenses primarily due to a decrease in toxicology study activity in the Company's PPMO platform;
- $15.5 million decrease in manufacturing expenses primarily due to Company's accelerated amortization of nonrefundable advance payments due to capacity changes associated with its manufacturing and supply agreement with Thermo Fisher Scientific, Inc. (Thermo) during 2021 with no similar activity in 2022; and
- $21.9 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 $877.1 million for the twelve months ended December 31, 2022, compared to $771.2 million for the same period of 2021, an increase of $105.9 million. The increase in research and development expenses primarily reflects the following:

- $61.1 million increase in manufacturing expenses incurred primarily related to the gene therapy manufacturing and supply agreement with Thermo, including charges of $54.0 million related to recognition of minimum purchase requirements and a continuing ramp-up of SRP-9001 manufacturing;
- $33.0 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $31.1 million increase in clinical trial expenses primarily due to a continuing ramp-up of the Company's SRP-9001 gene therapy programs including the Company's EMBARK program;
- $14.5 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $10.8 million increase in stock-based compensation expense primarily due to changes in headcount and the value of stock awards;
- $5.4 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors;
- $3.2 million increase in research and other expenses primarily driven by increases in lab-related expenses, partially offset by a decrease in sponsored research with academic institutions during 2022;
- $5.2 million decrease in up-front, milestone and other expenses primarily due to a $28.7 million increase of an accrued sublicense fee to Nationwide Children's Hospital and $11.6 million of expense incurred as a result of up-front and milestone payments related to certain research and license agreements during 2021. This was offset primarily by $26.1 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 during 2022;
- $8.2 million decrease in collaboration cost sharing primarily due to the termination of the Lysogene license and collaboration agreement and timing of expense incurred related to Genethon's micro-dystrophin drug candidate;
- $12.7 million decrease in pre-clinical expenses primarily due to a decrease in toxicology study activity in the Company's PPMO platform; and
- $27.0 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 $186.8 million and $175.5 million for the three months ended December 31, 2022 and 2021, respectively, an increase of $11.3 million. Non-GAAP research and development expenses were $784.1 million and $693.4 million for the twelve months ended December 31, 2022 and 2021, respectively, an increase of $90.7 million.

Selling, general and administrative

Selling, general and administrative expenses were $120.5 million for the three months ended December 31, 2022, compared to $78.1 million for the same period in 2021, an increase of $42.4 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $16.3 million increase in stock-based compensation expense primarily due to the Chief Executive Officer grant modification executed during 2022;
- $14.9 million increase in other expenses primarily related to charitable contributions made during 2022;
- $7.6 million increase in compensation and other personnel expenses primarily due to changes in headcount; and
- $3.3 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors.

Selling, general and administrative expenses were $451.4 million for the twelve months ended December 31, 2022, compared to $282.7 million for the same period in 2021, an increase of $168.7 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $108.3 million increase in stock-based compensation expense primarily due to the Chief Executive Officer grant modification executed during 2022;
- $23.7 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors;
- $18.6 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $16.4 million increase in other expenses primarily related to charitable contributions made during 2022; and
- $2.0 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts.

Non-GAAP selling, general and administrative expenses were $86.6 million and $60.1 million for the three months ended December 31, 2022 and 2021, respectively, an increase of $26.5 million. Non-GAAP selling, general and administrative expenses were $270.3 million and $209.2 million for the twelve months ended December 31, 2022 and 2021, respectively, an increase of $61.1 million.

Settlement and license charges

In February 2021, the Company recognized a $10.0 million settlement charge related to contingent settlement payments to BioMarin as a result of the

approval of AMONDYS 45 in the U.S. This was a result of a settlement and license agreement with BioMarin in July 2017. There was no such expense recognized during the same period of 2022.

Amortization of in-licensed rights

For both the three months ended December 31, 2022 and 2021, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For both the twelve months ended December 31, 2022 and 2021, the Company recorded amortization of in-licensed rights of approximately $0.7 million. This is related to the amortization of the in-licensed right assets recognized as a result of agreements the Company entered into with BioMarin and the University of Western Australia in July 2017 and April 2013, respectively.

Other income (expense), net

For the three months ended December 31, 2022 and 2021, other income, net was $5.5 million and other expense, net was $16.1 million, respectively. For the twelve months ended December 31, 2022 and 2021, other expense, net was $35.0 million and $68.4 million, respectively. The changes are primarily due to an increase in interest income and accretion of investment discount 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, partially offset by losses on the disposal of assets.

Loss on debt extinguishment

On September 14, 2022, the Company entered into separate, privately negotiated transactions to repurchase a portion of the outstanding senior convertible notes due on November 15, 2024 (the "2024 Notes"). The holders exchanged $150.6 million in aggregate principal value of 2024 Notes held by them plus accrued interest of $0.8 million for an aggregate payment of $248.6 million. The Company accounted for the repurchase of the 2024 Notes as a debt extinguishment by recognizing the difference between the repurchase price of the debt and the net carrying amount of the extinguished debt as loss on debt extinguishment. The loss incurred on the extinguishment was $98.5 million.

On September 16, 2022, the Company repaid in full all of its amounts outstanding with respect to the December 2019 Term Loan and repaid in full all obligations to the lenders. The aggregate payoff amount was approximately $585.5 million, which includes $550.0 million of principal amounts, additional loan consideration and premiums of $25.4 million, and accrued interest of $10.1 million through the repayment date. The loss incurred on the extinguishment was $26.9 million and represents the difference between the aggregate payoff amount and the net carrying amount of the December 2019 Term Loan.

Gain on contingent consideration, net

The gain on contingent consideration, net, relates to the fair value adjustment of the Company's contingent consideration derivative liability related to regulatory-related contingent payments to Myonexus Therapeutics, Inc. selling shareholders as well as to two academic institutions under separate license agreements that meet the definition of a derivative. During the twelve months ended December 31, 2022 and 2021, the Company recognized a $6.7 million and $7.2 million net gain, respectively, to adjust the fair value of the contingent consideration.

Gain from sale of Priority Review Voucher

In February 2021, the Company entered into an agreement to sell the rare pediatric disease Priority Review Voucher (PRV) it received from the FDA in connection with the approval of AMONDYS 45. Following the termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in April 2021, the Company completed its sale of the PRV and received proceeds of $102.0 million, with no commission costs, which was recorded as a gain from sale of the 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 of 2022.

Income tax expense (benefit)

Income tax expense for the three and twelve months ended December 31, 2022 was approximately $7.9 million and $13.5 million, respectively. Income tax expense for the three months ended December 31, 2021 was approximately $0.1 million, while income tax benefit for the twelve months ended December 31, 2021 was $0.2 million. Income tax expense (benefit) for all periods presented relates to state and foreign taxes.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had approximately $2.0 billion and $2.1 billion in cash, cash equivalents, investments and long-term restricted cash as of December 31, 2022 and 2021, respectively. This is driven by cash used to fund the Company's ongoing operations during 2022 and repayment of the term loan and a portion of the convertible debt, partially offset by net proceeds from the Company's convertible note offering and sales of the Company's products.

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 (benefit), 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, gain from sale of PRV, impairment of equity investment and net 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 sale of the PRV obtained as a result of the FDA approval of AMONDYS 45 in February 2021 as it is a non-recurring event.
- 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 net gain on contingent consideration related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to two 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.

Beginning in the fourth quarter of 2021, up-front and milestone payments associated with the Company's license and collaboration agreements, settlement and license charges and collaboration revenue, along with the related transaction costs incurred, are no longer excluded from the non-GAAP results.

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 (benefit), 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 (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval 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 (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval 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. 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 (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 45 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval 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 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, collaborations and partnerships, and technologies; the potential benefits of our technologies and scientific approaches; the potential benefits of SRP-9001; the potential for 2023 to be a bellwether year for the patient community we serve and for the promise of gene therapy; and our plans and milestones, including the anticipated May 29, 2023 action date for our gene therapy SRP-9001 Biologics License Application, preparing for scheduled pre-approval inspections and launch of SRP-9001, if approved, the potential for EMBARK to be our post-marketing confirmatory trial for SRP-9001, anticipating results from EMBARK by the end of 2023, and the FDA's current plan not to hold an Advisory Committee for SRP-9001.

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 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2022	**2021**	**2022**	**2021**
Revenues:				
Products, net	$ 235,933	$ 178,725	$ 843,769	$ 612,401
Collaboration and other	22,494	22,736	89,244	89,486
Total revenues	258,427	201,461	933,013	701,887
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	30,799	31,744	139,989	97,049
Research and development	213,804	197,296	877,090	771,182
Selling, general and administrative	120,478	78,055	451,421	282,660
Settlement and license charges	—	—	—	10,000
Amortization of in-licensed rights	179	179	714	706
Total cost and expenses	365,260	307,274	1,469,214	1,161,597
Operating loss	(106,833)	(105,813)	(536,201)	(459,710)
Other income (loss), net:				
Other income (expense), net	5,527	(16,076)	(35,021)	(68,438)
Loss on debt extinguishment	—	—	(125,441)	—
Gain on contingent consideration, net	—	—	6,700	7,200
Gain from sale of Priority Review Voucher	—	—	—	102,000
Total other income (loss), net	5,527	(16,076)	(153,762)	40,762
Loss before income tax expense (benefit)	(101,306)	(121,889)	(689,963)	(418,948)
Income tax expense (benefit)	7,938	92	13,525	(168)

Net loss	$ (109,244)	$ (121,981)	$ (703,488)	$ (418,780)
Net loss per share — basic and diluted	$ (1.24)	$ (1.42)	$ (8.03)	$ (5.15)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	87,838	85,951	87,559	81,262

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2022	**2021**	**2022**	**2021**
GAAP net loss	$ (109,244)	$ (121,981)	$ (703,488)	$ (418,780)
Interest (income) expense, net	(8,865)	15,953	25,525	63,014
Income tax expense (benefit)	7,938	92	13,525	(168)
Depreciation and amortization expense	10,553	10,145	41,864	38,017
Stock-based compensation expense	50,510	29,782	233,018	113,943
Impairment of equity investment	2,575	—	2,575	4,488
Loss on debt extinguishment	—	—	125,441	—
Gain on contingent consideration, net	—	—	(6,700)	(7,200)
Gain from sale of Priority Review Voucher	—	—	—	(102,000)
Non-GAAP net loss*	$ (46,533)	$ (66,009)	$ (268,240)	$ (308,686)
Non-GAAP net loss per share:				
Basic and diluted	$ (0.53)	$ (0.77)	$ (3.06)	$ (3.80)
Weighted average number of shares of common stock used in computing earnings per share:				
Basic and diluted	87,838	85,951	87,559	81,262

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2022	**2021**	**2022**	**2021**
GAAP research and development expenses	$ 213,804	$ 197,296	$ 877,090	$ 771,182
Stock-based compensation expense	(18,963)	(14,509)	(61,293)	(50,526)
Depreciation and amortization expense	(8,013)	(7,250)	(31,713)	(27,293)
Non-GAAP research and development expenses	$ 186,828	$ 175,537	$ 784,084	$ 693,363

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2022	**2021**	**2022**	**2021**
GAAP selling, general and administrative expenses	$ 120,478	$ 78,055	$ 451,421	$ 282,660
Stock-based compensation expense	(31,547)	(15,273)	(171,725)	(63,417)
Depreciation and amortization expense	(2,361)	(2,716)	(9,437)	(10,018)
Non-GAAP selling, general and administrative expenses	$ 86,570	$ 60,066	$ 270,259	$ 209,225

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

	As of December 31,	
	2022	**2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 966,777	$ 2,115,869
Short-term investments	1,022,597	—

Accounts receivable	214,628	152,990
Inventory	203,968	186,212
Other current assets	149,891	149,028
Total current assets	2,557,861	2,604,099
Property and equipment, net	180,037	191,156
Intangible assets, net	7,578	14,239
Right of use assets	64,954	45,531
Other non-current assets	317,936	292,949
Total assets	$ 3,128,366	$ 3,147,974
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 95,875	$ 76,741
Accrued expenses	418,996	271,697
Deferred revenue, current portion	89,244	89,244
Other current liabilities	15,489	15,051
Total current liabilities	619,604	452,733
Long-term debt	1,544,292	1,096,876
Lease liabilities, net of current portion	57,578	41,512
Deferred revenue, net of current portion	485,000	574,244
Contingent consideration	36,900	43,600
Other non-current liabilities	42	11,000
Total liabilities	2,743,416	2,219,965
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; 87,950,117 and 87,126,974 issued and outstanding at December 31, 2022 and 2021, respectively	9	9
Additional paid-in capital	4,296,841	4,134,768
Accumulated other comprehensive loss, net of tax	(1,664)	(20)
Accumulated deficit	(3,910,236)	(3,206,748)
Total stockholders' equity	384,950	928,009
Total liabilities and stockholders' equity	$ 3,128,366	$ 3,147,974

View source version on businesswire.com: https://www.businesswire.com/news/home/20230228006240/en/

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