Sarepta Therapeutics Announces Second Quarter 2022 Financial Results and Recent Corporate Developments

8/2/22

- Total revenues, which consist of net product revenues and collaboration revenues, for the second quarter 2022 totaled $233.5 million

- Net product revenues for the second quarter 2022 totaled $211.2 million, a 49% increase over the same quarter of prior year

- Increased full-year total revenue and net product revenue guidance to a range of $905-$920 million and $825-$840 million, respectively

CAMBRIDGE, Mass., Aug. 02, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the second quarter 2022.

“Our performance so far this year represents the culmination of years of dedicated patient-centered execution. Over the course of the second quarter, we engaged in an in-depth and cross-departmental engagement with the FDA about the possibility of submitting a biologics license application (“BLA”) for the accelerated approval of our gene therapy, SRP-9001, to treat ambulatory Duchenne patients. Supported by a wealth of safety, biomarker, and functional data, including new analyses presented at the 2022 International Congress on Neuromuscular Diseases in early July, we received the necessary written feedback from the FDA that gives us conviction to submit our BLA in the fall of this year,” said Doug Ingram, president and chief executive officer of Sarepta. “If successful, our BLA would be approved by the middle of 2023 and represents a seminal moment in the treatment of Duchenne muscular dystrophy and an important milestone in the field of gene therapy. At the same time, we have remained dedicated to serving the Duchenne community with our three currently approved RNA-based PMO therapies, reaching total revenues of $233.5 million and net product revenues of $211.2 million in the second quarter, a nearly 50% growth over the second quarter of last year. As a result of our overperformance, we are updating our full-year guidance for net product revenue to a range of between $825 million and $840 million.”

Second Quarter 2022 and Recent Developments:

- ***Sarepta announces intent to submit an accelerated approval Biologics License Application (BLA) for SRP-9001:*** At the end of July 2022, following FDA feedback from a thorough and in-depth review, the Company announced that this fall it intends to submit a BLA for SRP-9001, their gene therapy (delandistrogene moxeparvovec) to treat Duchenne muscular dystrophy. SRP-9001 was granted Fast Track designation in July 2020. In addition to Fast Track, SRP-9001 has also been granted Rare Pediatric Disease (RPD) designation in the United States, and Orphan Drug status in the United States, the European Union, Switzerland and Japan.

- ***Sarepta and partner Roche presented new data for investigational gene therapy SRP-9001 at the 2022 International Congress on Neuromuscular Diseases (ICNMD 2022):*** In early July 2022, the company shared new functional data across multiple studies from the clinical development program for SRP-9001 (delandistrogene moxeparvovec) for the treatment of Duchenne muscular dystrophy. Key findings included new, one-year functional results from Study SRP-9001-103 (ENDEAVOR), which employs commercially representative SRP-9001 material at the target commercial dose. This is the same material currently being used in the ongoing trial, Study SRP-9001-301 (EMBARK), Sarepta’s global, randomized, double-blind, placebo-controlled clinical trial evaluating SRP-9001 in boys with Duchenne between the ages of 4 to 7 (n=120). Additionally, the Company presented long-term functional results from Study SRP-9001-101 four years after treatment with SRP-9001 along with results from an integrated analysis of Studies 101, 102 and 103 versus propensity weighted external control. These new analyses showed that SRP-9001 treated patients demonstrated statistically significant and clinically meaningful benefit versus propensity-matched external controls and results positively diverge from the natural history of this degenerative disease over time. The safety and tolerability profile of SRP-9001 remains consistent across treated patients.

Results from Study SRP-9001-103, ENDEAVOR (commercially representative material):

- In Cohort 1 (n=20, ages 4 to 7), SRP-9001-treated patients demonstrated a 3.8-point improvement (unadjusted means) and a 3.2-point improvement (least squared means) on the NSAA one-year after treatment when compared to a propensity-score weighted external control (p=0.0001).
    - At one year, using unadjusted means, NSAA total scores in the SRP-9001 treated patients improved 4 points from 22.1 to 26.1 and participants in the external control improved 0.2 points from 21.9 to 22.1.
    - At one year, using least squared means, NSAA total scores in the SRP-9001 treated patients improved 3.9 points and participants in the external control improved 0.8 points (p<0.0001).

- Participants in the study also recorded statistically significant improvements in timed function tests one year after treatment compared to external control
    - At one year, using unadjusted means, time to rise in the SRP-9001 treated patients improved 0.9 seconds

compared to external control
  - At one year, using least squared means, time to rise in the SRP-9001 treated patients improved 1.2 seconds compared to external control (p <0.0001)
  - At one year, using unadjusted means, SRP-9001 treated patients improved 1.0 seconds on the ten-meter walk test compared to external control
  - At one year, using least squared means, SRP-9001 treated patients improved 1.0 seconds on the ten-meter walk test compared to external control (p = 0.0018)

Results from Study SRP-9001-101:

- In long-term results from Study SRP-9001-101, after four years, SRP-9001-treated participants (n=4, ages 4-7 at time of treatment) had a positive mean 7.0-point difference on total NSAA scores compared to baseline. These patients are now on average over 9 years old, an age where one would expect to see rapid declines in function.
- When compared to a propensity-weighted external control, total NSAA scores for the SRP-9001 treated patients were 9.9 points (unadjusted means) and 9.4 points (least square means) (p=0.0125).

Results from Integrated Analysis of Studies 101, 102 and 103 versus propensity weighted external control:

- In an integrated analysis of one-year functional data from patients who received the target dose of SRP-9001 in Studies 101, 102 and 103 (n=52), SRP-9001-treated patients improved 3.1 points (unadjusted means) and 2.3 points (least squared means) in NSAA total scores from baseline.
- When compared to the propensity-weighted external control group, NSAA change from baseline one-year after treatment for SRP-9001 treated patients was 2.4 points higher (least square means; p<0.0001).

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 June 30, 2022, the Company reported a net loss of $231.5 million, or $2.65 per basic and diluted share, compared to a net loss of $81.4 million reported for the same period of 2021, or $1.02 per basic and diluted share. On a non-GAAP basis, the net loss for the three months ended June 30, 2022 was $103.0 million, or $1.18 per basic and diluted share, compared to a net loss of $130.6 million[1], or $1.64 per basic and diluted share for the same period of 2021.

On a GAAP basis, for the six months ended June 30, 2022, the Company reported a net loss of $336.5 million, or $3.85 per basic and diluted share, compared to a net loss of $248.7 million reported for the same period of 2021, or $3.12 per basic and diluted share. On a non-GAAP basis, the net loss for the six months ended June 30, 2022 was $151.7 million, or $1.74 per basic and diluted share, compared to a net loss of $245.1 million[1], or $3.08 per basic and diluted share for the same period of 2021.

Revenues

For the three months ended June 30, 2022, the Company recorded total revenues of $233.5 million, which consist of net product revenues and collaboration revenues, compared to total revenues of $164.1 million for the same period of 2021, an increase of $69.4 million. For the six months ended June 30, 2022, the Company recorded total revenues of $444.3 million, compared to total revenues of $311.0 million for the same period of 2021, an increase of $133.3 million.

For the three months ended June 30, 2022, the Company recorded net product revenues of $211.2 million, compared to net product revenues of $141.8 million for the same period of 2021, an increase of $69.4 million. For the six months ended June 30, 2022, the Company recorded net product revenues of $400.1 million, compared to net product revenues of $266.8 million for the same period of 2021, an increase of $133.3 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 six months ended June 30, 2022 given its commercial launch in February 2021.

For both the three and six months ended June 30, 2022 and 2021, 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 Operating Expenses

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

For the three months ended June 30, 2022, cost of sales (excluding amortization of in-licensed rights) was $37.8 million, compared to $19.5 million for the same period of 2021, an increase of $18.3 million. For the six months ended June 30, 2022, cost of sales (excluding amortization of in-licensed rights) was $69.2 million, compared to $41.9 million for the same period of 2021, an increase of $27.3 million. The changes for both periods primarily reflect increasing demand for the Company's products as well as the write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the three and six months ended June 30, 2022, as well as the six months ended June 30, 2021, with no similar activity for the three months ended June 30, 2021.

Research and development

Research and development expenses were $252.3 million for the three months ended June 30, 2022, compared to $239.6 million for the same period of 2021, an increase of $12.7 million. The increase in research and development expenses primarily reflects the following:

- $30.5 million increase in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the

gene therapy manufacturing and supply agreement with Thermo, partially offset by a decrease in gene therapy manufacturing costs incurred pursuant to the terms of the third amendment to the Thermo manufacturing and supply agreement, which removed capacity access fees and reduced the total number of manufacturing batches in 2022;

- $5.8 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $3.3 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $2.8 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;
- $2.2 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors;
- $1.6 million increase in stock-based compensation expense primarily due to changes in headcount and stock price;
- $4.8 million decrease in pre-clinical expenses primarily due to a decrease in toxicology study activity in the Company's PPMO platform;
- $20.4 million decrease in up-front, milestone and other expenses primarily due to a $28.7 million increase of an accrued sublicense fee to Nationwide and a $3.0 million expense incurred as a result of a milestone achievement in a research and license agreement during the three months ended June 30, 2021, offset by $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 during the same period of 2022; and
- $8.3 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 $446.6 million for the six months ended June 30, 2022, compared to $434.8 million for the same period of 2021, an increase of $11.8 million. The increase in research and development expenses primarily reflects the following:

- $37.5 million increase in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo, partially offset by a decrease in gene therapy manufacturing costs incurred pursuant to the third amendment to the Thermo manufacturing and supply agreement, which removed capacity access fees and reduced the total number of manufacturing batches in 2022;
- $7.8 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $6.7 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $4.7 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;
- $3.5 million increase in stock-based compensation expense primarily due to changes in headcount and stock price;
- $3.4 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors;
- $4.2 million decrease in collaboration cost sharing primarily due to the termination of the Lysogene S.A. license and collaboration agreement and timing of expense incurred related to Genethon's micro-dystrophin drug candidate;
- $5.1 million decrease in pre-clinical expenses primarily due to due to a decrease in toxicology study activity in the Company's PPMO platform;
- $5.2 million decrease in research and other expenses primarily driven by decreases in sponsored research with academic institutions during the six months ended June 30, 2022;
- $24.4 million decrease in up-front and milestone expenses primarily due to a $28.7 million increase of an accrued sublicense fee to Nationwide and $7.0 million of expense incurred as a result of milestone achievements in certain research and license agreements during the six months ended June 30, 2021, offset by $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 during the same period of 2022; and
- $12.8 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 $230.4 million and $220.7 million for the three months ended June 30, 2022 and 2021, respectively, an increase of $9.7 million. Non-GAAP research and development expenses were $403.5 million and $398.2 million for the six months ended June 30, 2022 and 2021, respectively, an increase of $5.3 million.

Selling, general and administrative

Selling, general and administrative expenses were $154.3 million for the three months ended June 30, 2022, compared to $72.3 million for the same period in 2021, an increase of $82.0 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $72.3 million increase in stock-based compensation expense primarily due to the CEO grant modification agreement executed during the three months ended June 30, 2022;
- $5.7 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors; and
- $2.4 million increase in compensation and other personnel expenses primarily due to a net increase in headcount.

Selling, general and administrative expenses were $226.2 million for the six months ended June 30, 2022, compared to $143.5 million for the same period in 2021, an increase of $82.7 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $71.1 million increase in stock-based compensation expense primarily due to the CEO grant modification executed during the three months ended June 30, 2022;
- $7.5 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors;
- $1.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $1.5 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 $63.7 million and $54.0 million for the three months ended June 30, 2022 and 2021, respectively, an increase of $9.7 million. Non-GAAP selling, general and administrative expenses were $116.9 million and $105.6 million for the six months ended June 30, 2022 and 2021, respectively, an increase of $11.3 million.

Settlement and license charges

In February 2021, the Company recognized a $10.0 million settlement charge related to contingent settlement payments to BioMarin Pharmaceutical, Inc. (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 June 30, 2022 and 2021, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For the six months ended June 30, 2022 and 2021, the Company recorded amortization of in-licensed rights of approximately $0.4 million and $0.3 million, respectively. This is related to the amortization of the in-licensed right assets recognized as a result of agreements the Company entered into with BioMarin and the University of Western Australia in July 2017 and April 2013, respectively.

Other expense, net

For the three months ended June 30, 2022 and 2021, other expense, net was $17.0 million and $16.2 million, respectively. For the six months ended June 30, 2022 and 2021, other expense, net was $34.2 million and $31.7 million, respectively. The increases are primarily due to losses on disposal of assets, an increase in the mark-to-market adjustment of the Company's Level 1 strategic investment, offset by an increase in interest income due to the investment mix of the Company's investment portfolio.

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 six months ended June 30, 2022 was $3.4 million and $4.3 million, respectively. Income tax benefit for the three and six months ended June 30, 2021 was approximately $0.4 million and $0.5 million, respectively. 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 $1.9 billion in cash, cash equivalents, restricted cash and investments as of June 30, 2022, compared to $2.1 billion as of December 31, 2021. The decrease is primarily driven by cash used to fund the Company's ongoing operations during 2022.

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 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 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.

The sale of the PRVs obtained as a result of the FDA approval of AMONDYS 45 in February 2021 is a non-recurring event and excluded from the Company's non-GAAP results.

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 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. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse reactions observed in at least 20% of treated patients and greater than placebo were (VYONDYS 53, placebo): headache (41%, 10%), pyrexia (41%, 14%), fall (29%, 19%), abdominal pain (27%, 10%), nasopharyngitis (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

For further information, please see the full Prescribing Information.

About AMONDYS 45

AMONDYS 45 (casimersen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

AMONDYS 45 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

AMONDYS 45 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for AMONDYS 45

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 and technologies; the potential benefits of our technologies and scientific approaches; and expected plans and milestones, including the Company's intent to submit an accelerated approval BLA for SRP-9001 in the fall and if successful, the potential for an approval by the middle of 2023.

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: the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations; 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; different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; 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, 2021 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,	
	2022	**2021**	**2022**	**2021**
Revenues:				
Products, net	$ 211,237	$ 141,839	$ 400,062	$ 266,765
Collaboration	22,250	22,250	44,255	44,255
Total revenues	233,487	164,089	444,317	311,020
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	37,795	19,515	69,238	41,861
Research and development	252,329	239,622	446,579	434,771
Selling, general and administrative	154,316	72,347	226,156	143,478
Settlement and license charges	—	—	—	10,000
Amortization of in-licensed rights	179	179	357	349
Total cost and expenses	444,619	331,663	742,330	630,459
Operating loss	(211,132)	(167,574)	(298,013)	(319,439)
Other (loss) income, net:				
Other expense, net	(16,961)	(16,185)	(34,226)	(31,713)
Gain from sale of Priority Review Voucher	—	102,000	—	102,000
Total other (loss) income, net	(16,961)	85,815	(34,226)	70,287

Loss before income tax expense (benefit)	(228,093)	(81,759)	(332,239)	(249,152)
Income tax expense (benefit)	3,388	(354)	4,267	(497)
Net loss	$ (231,481)	$ (81,405)	$ (336,506)	$ (248,655)
Net loss per share - basic and diluted	$ (2.65)	$ (1.02)	$ (3.85)	$ (3.12)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	87,511	79,746	87,383	79,601

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,	
	2022	2021	2022	2021
GAAP net loss	$ (231,481)	$ (81,405)	$ (336,506)	(248,655)
Interest expense, net	12,288	15,758	27,869	31,214
Income tax expense (benefit)	3,388	(354)	4,267	(497)
Gain from sale of Priority Review Voucher	—	(102,000)	—	(102,000)
Depreciation and amortization expense	9,889	8,447	20,608	17,377
Stock-based compensation expense	102,892	28,969	132,090	57,477
Non-GAAP net loss*	$ (103,024)	$ (130,585)	$ (151,672)	$ (245,084)
Non-GAAP net loss per share:				
Basic and diluted	$ (1.18)	$ (1.64)	$ (1.74)	$ (3.08)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	87,511	79,746	87,383	79,601

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2022	2021	2022	2021
GAAP research and development expenses	$ 252,329	$ 239,622	$ 446,579	$ 434,771
Stock-based compensation expense	(14,467)	(12,860)	(27,535)	(23,986)
Depreciation and amortization expense	(7,512)	(6,060)	(15,534)	(12,598)
Non-GAAP research and development expenses	$ 230,350	$ 220,702	$ 403,510	$ 398,187

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2022	2021	2022	2021
GAAP selling, general and administrative expenses	$ 154,316	$ 72,347	$ 226,156	$ 143,478
Stock-based compensation expense	(88,425)	(16,109)	(104,555)	(33,491)
Depreciation and amortization expense	(2,198)	(2,208)	(4,717)	(4,430)
Non-GAAP selling, general and administrative expenses	$ 63,693	$ 54,030	$ 116,884	$ 105,557

* 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. Non-GAAP financial results for the second quarter 2021 have been updated to reflect this change for comparable purposes.

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

	As of June 30, 2022	As of December 31, 2021

Assets		
Current assets:		
Cash and cash equivalents	$ 868,565	$ 2,115,869
Short-term investments	1,059,454	—
Accounts receivable	203,854	152,990
Inventory	208,095	186,212
Other current assets	129,332	149,028
Total current assets	2,469,300	2,604,099
Property and equipment, net	183,292	191,156
Intangible assets, net	13,062	14,239
Right of use assets	46,999	45,531
Other non-current assets	284,200	292,949
Total assets	$ 2,996,853	$ 3,147,974
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 56,207	$ 76,741
Accrued expenses	383,699	271,697
Deferred revenue, current portion	89,244	89,244
Other current liabilities	16,416	15,051
Total current liabilities	545,566	452,733
Long-term debt	1,100,873	1,096,876
Lease liabilities, net of current portion	39,368	41,512
Deferred revenue, net of current portion	529,989	574,244
Contingent consideration	43,600	43,600
Other non-current liabilities	11,000	11,000
Total liabilities	2,270,396	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; 87,535,299 and 87,126,974 issued and outstanding at June 30, 2022, and December 31, 2021, respectively	9	9
Additional paid-in capital	4,272,187	4,134,768
Accumulated other comprehensive loss, net of tax	(2,485)	(20)
Accumulated deficit	(3,543,254)	(3,206,748)
Total stockholders' equity	726,457	928,009
Total liabilities and stockholders' equity	$ 2,996,853	$ 3,147,974

Source: Sarepta Therapeutics, Inc.

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[1] 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. Non-GAAP financial results for the second quarter 2021 have been updated to reflect this change for comparable purposes.