Sarepta Therapeutics Announces Second Quarter 2021 Financial Results and Recent Corporate Developments

8/4/21

- ***Sarepta announces successful completion of end-of-phase-2 meeting for SRP-9001 micro-dystrophin with FDA's Office of Tissues and Advanced Therapies (OTAT) and plans to initiate pivotal trial, SRP-9001-301 (Study 301 or EMBARK), in September of 2021***

- ***Net product sales for the second quarter of 2021 reached $141.8 million, a 27% increase over the same quarter of prior year***

- ***In light of its over-performance, Sarepta raises its full-year product revenue guidance by nearly $30 million to between $565 million to $575 million***

CAMBRIDGE, Mass., Aug. 04, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the second quarter of 2021.

"I am pleased to report that the Sarepta team continues to execute, serving our patient community and advancing our portfolio. In the second quarter, our net product revenue stood at $141.8 million, a 27% increase over the same quarter last year," stated Doug Ingram, Sarepta's president and chief executive officer. "We also advanced our broad genetic medicine portfolio. Significantly and representing years of work and enormous investment into process and analytical development for SRP-9001, our gene therapy for Duchenne muscular dystrophy, we announced very positive results from our first cohort of Study SRP-9001-103 (ENDEAVOR) to test the performance of our commercially representative material. From there, we scheduled and have now completed a very productive and informative end-of-phase-2 meeting with the FDA and on that basis we anticipate initiating Study SRP-9001-301 (EMBARK), our pivotal trial for SRP-9001 in September of this year in the United States and globally. This is an extraordinarily important moment for our program and for the many families living with Duchenne. We intend to move as fast as science will permit to confirm the benefits of SRP-9001 and, if successful, to rapidly advance this therapy to waiting Duchenne patients around the world."

Second Quarter 2021 and Recent Corporate Developments:

- ***Sarepta announced that it concluded its end-of-phase-2 meeting with OTAT:*** In July of 2021, Sarepta completed a successful end-of-phase-2 meeting with the United States Food and Drug Administration's Office of Tissues and Advanced Therapies (OTAT) regarding the Company's proposed pivotal trial, Study SRP-9001-301 (EMBARK), and the commercially representative material to be used in that trial. Based on the meeting with OTAT, Sarepta anticipates initiating Study 301, as submitted to the Division, in September of this year.

- ***Sarepta executes licensing agreement for gene therapy program from Nationwide Children's Hospital to treat limb-girdle muscular dystrophy type 2A:*** Today Sarepta announced that upon completion of a number of preclinical and safety studies, the Company executed its licensing agreement to an investigational gene therapy candidate, calpain 3 (CAPN-3), to treat limb-girdle muscular dystrophy type 2A (LGMD2A). The candidate was developed by the Abigail Wexner Research Institute at Nationwide Children's Hospital. Also known as calpainopathy, LGMD2A is caused by mutations in the CAPN-3 gene and is the most common type of LGMD, accounting for almost a third of cases. Like SRP-9001, Sarepta's lead investigational gene transfer therapy for Duchenne, and the Company's five other LGMD programs, the LGMD2A program uses the AAVrh74 vector, designed to systematically and robustly deliver treatment to skeletal muscle making it an ideal candidate to treat muscle disease.

- ***Sarepta announced positive 12-week expression and safety results for SRP-9001-103, the first results from a clinical trial using SRP-9001 commercially representative material:*** In May 2021, the Company announced results from the first 11 participants enrolled in Study SRP-9001-103, an open-label study known as ENDEAVOR being conducted in partnership with Roche. SRP-9001 is an investigational gene transfer therapy being developed for the treatment of Duchenne muscular dystrophy. In the open-label study, 20 participants between the ages of four and seven were treated with a single infusion of SRP-9001 at a dose of $1.33x10^{14}$ vg/kg.

Results include:

- All patients demonstrated robust transduction, with mean micro-dystrophin expression of 55.4% of normal, as measured by western blot.
- Muscle dystrophin levels demonstrated a mean of 70.5% (baseline 12.8%) muscle fibers expressing micro-dystrophin at 12

weeks with a mean intensity at the sarcolemma of 116.9% (baseline 41.0%) compared to normal biopsies, as measured by immunofluorescence. Comparisons between baseline and post-treatment measures were statistically significant (p=0.001 for positive fibers, and p=0.002 for intensity).
- Mean vector genome copies per nucleus reached 3.87.
- The safety profile of SRP-9001 observed in the first 11 participants is consistent with the safety seen in earlier studies using clinical manufacturing process material. In line with previously reported clinical data, no clinically relevant complement activation was observed in these 11 patients. Two patients experienced serious adverse events (transaminase elevation in one patient and nausea and vomiting in a second patient) that fully resolved.

Conference Call

The Company will be hosting a conference call at 4:30 p.m. Eastern Time to discuss Sarepta's financial results and provide a corporate update. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 8374717. Please specify to the operator that you would like to join the "Sarepta Second Quarter 2021 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

Financial Results

On a GAAP basis, for the three months ended June 30, 2021, the Company reported a net loss of $81.4 million, or $1.02 per basic and diluted share, compared to a net loss of $150.8 million reported for the same period of 2020, or $1.93 per basic and diluted share. On a non-GAAP basis, the net loss for the three months ended June 30, 2021 was $121.2 million, or $1.52 per basic and diluted share, compared to a net loss of $117.9 million, or $1.51 per basic and diluted share for the same period of 2020.

On a GAAP basis, for the six months ended June 30, 2021, the Company reported a net loss of $248.7 million, or $3.12 per basic and diluted share, compared to a net loss of $168.3 million reported for the same period of 2020, or $2.18 per basic and diluted share. On a non-GAAP basis, the net loss for the six months ended June 30, 2021 was $243.7 million, or $3.06 per basic and diluted share, compared to a net loss of $197.7 million, or $2.56 per basic and diluted share for the same period of 2020.

Revenues

For the three months ended June 30, 2021, the Company recorded net product revenues of $141.8 million, compared to net product revenues of $111.3 million for the same period of 2020, an increase of $30.5 million. For the six months ended June 30, 2021, the Company recorded net product revenues of $266.8 million, compared to net product revenues of $211.8 million for the same period of 2020, an increase of $55.0 million. The increase primarily reflects the launch of AMONDYS 45 in the first quarter of 2021 and the continuing increase in demand for the Company's products in the U.S.

For the three months ended June 30, 2021 and 2020, the Company recognized $22.3 million and $26.0 million of collaboration revenue, respectively. For the six months ended June 30, 2021 and 2020, the Company recognized $44.3 million and $39.2 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, 2021, cost of sales (excluding amortization of in-licensed rights) was $19.5 million, compared to $13.3 million for the same period of 2020, an increase of $6.2 million. The increase in cost of sales is primarily due to increased demand for the Company's products. For the six months ended June 30, 2021, cost of sales (excluding amortization of in-licensed rights) was $41.9 million, compared to $26.0 million for the same period of 2020, an increase of $15.9 million. The increase is primarily due to increasing demand for the Company's products and the write-offs of certain batches of EXONDYS 51 not meeting the Company's quality specifications for the six months ended June 30, 2021, with no similar activity during the same period of 2020.

Research and development

Research and development expenses were $239.6 million for the three months ended June 30, 2021, compared to $188.5 million for the same period of 2020, an increase of $51.1 million. The increase in research and development expenses primarily reflects the following:

- $18.9 million increase in up-front and milestone expenses primarily due to a $28.7 million increase of an accrued sublicense fee to Nationwide Children's Hospital (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 $12.0 million of up-front payments as a result of the execution of certain research and license agreements during the same period of 2020;
- $16.6 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;
- $9.8 million increase in clinical trial expenses primarily due to increased patient enrollment for the Company's ESSENCE and MOMENTUM programs as well as certain start-up activities for the Company's SRP-9001 micro-dystrophin program;
- $6.0 million increase in research and other primarily driven by an increase in sponsored research with academic institutions during the three months ended June 30, 2021;
- $4.0 million increase in facility- and technology-related expenses due to the Company's continuing expansion efforts;
- $3.2 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $2.1 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $1.7 million increase in stock-based compensation expense primarily driven by increases in headcount and changes in stock price;

- $1.0 million decrease in collaboration cost sharing with Genethon on its micro-dystrophin drug candidate; and
- $9.3 million increase in the offset to expense associated with a collaboration reimbursement from Roche primarily due to continuing development of the Company's micro-dystrophin gene therapy.

Research and development expenses were $434.8 million for the six months ended June 30, 2021, compared to $324.7 million for the same period of 2020, an increase of $110.1 million. The increase in research and development expenses primarily reflects the following:

- $43.7 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;
- $19.8 million increase in clinical trial expenses primarily due to increased patient enrollment for the Company's ESSENCE and MOMENTUM programs as well as certain start-up activities for the Company's SRP-9001 micro-dystrophin program;
- $14.4 million increase in up-front, milestone and other 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 $9.3 million of expense related to sublicense payments accrued to Nationwide and $12.0 million of up-front payments as a result of the execution of certain research and license agreements during the same period of 2020;
- $12.9 million increase in research and other primarily driven by an increase in sponsored research with academic institutions during the six months ended June 30, 2021;
- $8.0 million increase in facility- and technology-related expenses due to the Company's continuing expansion efforts;
- $7.8 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $6.2 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $3.6 million increase in stock-based compensation expense primarily driven by increases in headcount and changes in stock price;
- $2.5 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidate;
- $2.7 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors; and
- $6.0 million increase in the offset to expense associated with a collaboration reimbursement from Roche primarily due to continuing development of the Company's SRP-9001 micro-dystrophin gene therapy.

Non-GAAP research and development expenses were $189.0 million and $160.4 million for the three months ended June 30, 2021 and 2020, respectively, an increase of $28.6 million. Non-GAAP research and development expenses were $362.5 million and $274.6 million for the six months ended June 30, 2021 and 2020, respectively, an increase of $87.9 million.

Selling, general and administration

Selling general and administrative expenses were approximately $72.3 million for the three months ended June 30, 2021, compared to $73.7 million for the same period in 2020, a decrease of $1.4 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $0.7 million decrease in compensation and other personnel expenses primarily due to a change in headcount period over period; and
- $0.6 million decrease in professional service expenses primarily due to a decrease in reliance on third-party selling, general and administrative contractors.

Selling general and administrative expenses were approximately $143.5 million for the six months ended June 30, 2021, compared to $156.5 million for the same period in 2020, a decrease of $13.0 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $16.2 million decrease in professional service expenses primarily due to a decrease in reliance on third-party selling, general and administrative contractors, as well as a transaction fee for the Roche transaction incurred during the six months ended June 30, 2020, with no similar activity incurred during the six months ended June 30, 2021; and
- $2.2 million increase in stock-based compensation primarily due to changes in stock price.

Non-GAAP selling, general and administrative expenses were $54.0 million and $55.1 million for the three months ended June 30, 2021 and 2020, respectively, a decrease of $1.1 million. Non-GAAP selling, general and administrative expenses were $105.6 million and $109.6 million for the six months ended June 30, 2021 and 2020, respectively, a decrease of $4.0 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 United States. 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 2020.

Amortization of in-licensed rights

For each of the three months ended June 30, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For each of the six months ended June 30, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.3 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.

Other expense, net

For the three months ended June 30, 2021 and 2020, other expense, net was approximately $16.2 million and $12.4 million, respectively. For the six months ended June 30, 2021 and 2020, other expense, net was approximately $31.7 million and $19.9 million, respectively. The increase primarily reflects an increase in interest expense incurred on the Company's term loan debt facilities due to an increase in the outstanding balance partially offset by a reduction of interest expense incurred on the Company's convertible debt related to the adoption of ASU 2020-06, as well as a decrease in interest income and the amortization of investment discounts due to changes in 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.

In February 2020, the Company entered into an agreement to sell the PRV it received from the FDA in connection with the approval of VYONDYS 53. In March 2020, the Company completed its sale of the PRV and received proceeds of $108.1 million, net of commission, 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.

Cash, Cash Equivalents, Investments and Restricted Cash and Investments

The Company had approximately $1.7 billion in cash, cash equivalents and investments as of June 30, 2021, compared to $1.9 billion as of December 31, 2020. The decrease is primarily driven by cash used to fund the Company's ongoing operations during 2021.

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 (benefit) expense, depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined by the Company as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by the Company as GAAP selling, general and administrative expenses excluding depreciation and amortization expense, stock-based compensation expense and other items.

1. Interest, tax, depreciation and amortization

Interest 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 collaboration revenue and transaction cost related to the Roche transaction, up-front and milestone payments, acquired in-process research and development expense, gain from sale of PRV and loss on contingent consideration.

The Company excludes collaboration revenue and transaction cost associated with the Roche transaction from its non-GAAP results. While collaboration revenue is recurring, as the Company's ordinary activities do not include contracting with third parties to provide them with research and development services, collaboration revenue is treated as a non-GAAP adjustment item. Additionally, the transaction fee related to the Roche transaction is non-recurring and is excluded from its non-GAAP results. However, the Company does not exclude reimbursement of costs by Roche from its non-GAAP results.

The Company excludes up-front and milestone payments associated with its license and collaboration agreements from its non-GAAP results and research and development expenses because the Company does not consider them to be normal operating expenses due to their nature, variability of amounts, and lack of predictability as to occurrence and/or timing. Up-front payments are made at the commencement of a collaborative relationship or a license agreement anticipated to continue for a multi-year period and provide the Company with intellectual property rights, option rights and other rights with respect to particular programs. Milestone payments are made when certain development, regulatory and sales milestone events are achieved. The variability of amounts and lack of predictability of collaboration- and license-related up-front and milestone payment makes the identification of trends in the Company's ongoing research and development activities more difficult.

The sale of the PRVs obtained as a result of the FDA approval of VYONDYS 53 and AMONDYS 45 in December 2019 and February 2021, respectively, are non-recurring events and excluded from the Company's non-GAAP results.

The Company excludes from its non-GAAP results loss on contingent consideration related to the Company's acquisition of Myonexus in 2019 as it is a non-cash item and is not considered to be normal operating expenses due to its variability of amounts and lack of predictability as to occurrence and/or timing.

The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. The Company also believes these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating the Company's performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of the Company's financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP other income and loss

adjustments, non-GAAP income tax (benefit) expense, non-GAAP net loss, and non-GAAP basic and diluted net loss per share may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table "Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures."

About EXONDYS 51

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 51 of dystrophin pre-mRNA, resulting in "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 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, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

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

In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

For further information, please see the full Prescribing Information.

About VYONDYS 53

VYONDYS 53 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 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 DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

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

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

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

For further information, please see the full Prescribing Information.

About AMONDYS 45
AMONDYS 45 (casimersen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping. AMONDYS 45 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 approved under accelerated review based on an increase in dystrophin production in skeletal muscle of patients amenable to exon 45 skipping. 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 DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

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

Adverse reactions 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 (DMD) 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, including the design of the AAVrh74 vector to systematically and robustly deliver treatment to skeletal muscle making it an ideal candidate to treat muscle disease; and expected plans and milestones, including our plan to initiate pivotal trial, SRP-9001-301 (Study 301 or EMBARK), in September of 2021 in the United States and globally, and our plan to move as fast as science will permit to confirm the benefits of SRP-9001 and, if successful, to rapidly advance this therapy to waiting Duchenne patients around the world.

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; 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, 2020 and 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,	
	2021	2020	2021	2020
Revenues:				
Products, net	$ 141,839	$ 111,344	$ 266,765	$ 211,792
Collaboration	22,250	26,019	44,255	39,245
Total revenues	164,089	137,363	311,020	251,037
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	19,515	13,341	41,861	25,963
Research and development	239,622	188,522	434,771	324,666
Selling, general and administrative	72,347	73,688	143,478	156,456
Settlement and license charges	—	—	10,000	—
Amortization of in-licensed rights	179	165	349	331
Total cost and expenses	331,663	275,716	630,459	507,416
Operating loss	(167,574)	(138,353)	(319,439)	(256,379)
Other income (loss):				
Gain from sale of Priority Review Voucher	102,000	—	102,000	108,069
Other expense, net	(16,185)	(12,447)	(31,713)	(19,867)
Total other income (loss)	85,815	(12,447)	70,287	88,202
Loss before income tax (benefit) expense	(81,759)	(150,800)	(249,152)	(168,177)
Income tax (benefit) expense	(354)	20	(497)	135
Net loss	$ (81,405)	$ (150,820)	$ (248,655)	$ (168,312)
Net loss per share - basic and diluted	$ (1.02)	$ (1.93)	$ (3.12)	$ (2.18)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	79,746	77,968	79,601	77,200

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,	
	2021	2020	2021	2020
GAAP net loss	$ (81,405)	$ (150,820)	$ (248,655)	$ (168,312)
Interest expense, net	15,758	12,076	31,214	20,588
Income tax (benefit) expense	(354)	20	(497)	135
Gain from sale of Priority Review Voucher	(102,000)	—	(102,000)	(108,069)
Collaboration revenue	(22,250)	(26,019)	(44,255)	(39,245)
Depreciation and amortization expense	8,447	6,475	17,377	13,004
Stock-based compensation expense	28,969	27,616	57,477	51,640
Roche transaction costs	—	—	—	11,292
Up-front, milestone, and other expenses	31,677	12,750	35,677	21,283
Settlement and license charges	—	—	10,000	—
Non-GAAP net loss	$ (121,158)	$ (117,902)	$ (243,662)	$ (197,684)
Non-GAAP net loss per share:				
Basic and diluted	$ (1.52)	$ (1.51)	$ (3.06)	$ (2.56)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	79,746	77,968	79,601	77,200

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2021	2020	2021	2020

GAAP research and development expenses	$ 239,622	$ 188,522	$ 434,771	$ 324,666
Up-front, milestone, and other expenses	(31,677)	(12,750)	(35,677)	(21,283)
Stock-based compensation expense	(12,860)	(11,140)	(23,986)	(20,389)
Depreciation and amortization expense	(6,060)	(4,199)	(12,598)	(8,376)
Non-GAAP research and development expenses	$ 189,025	$ 160,433	$ 362,510	$ 274,618

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2021	2020	2021	2020
GAAP selling, general and administrative expenses	$ 72,347	$ 73,688	$ 143,478	$ 156,456
Stock-based compensation expense	(16,109)	(16,476)	(33,491)	(31,251)
Depreciation and amortization expense	(2,208)	(2,111)	(4,430)	(4,297)
Roche transaction costs	—	—	—	(11,292)
Non-GAAP selling, general and administrative expenses	$ 54,030	$ 55,101	$ 105,557	$ 109,616

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

	As of June 30, 2021	As of December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 1,697,275	$ 1,502,648
Short-term investments	30,000	435,923
Accounts receivable	127,520	101,340
Inventory	268,756	231,961
Other current assets	141,666	213,324
Total current assets	2,265,217	2,485,196
Property and equipment, net	203,330	190,430
Intangible assets, net	14,105	13,628
Right of use assets	72,868	91,761
Other non-current assets	203,553	203,703
Total assets	$ 2,759,073	$ 2,984,718
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 76,681	$ 111,090
Accrued expenses	240,141	193,553
Deferred revenue, current portion	89,244	89,244
Other current liabilities	18,956	22,139
Total current liabilities	425,022	416,026
Long-term debt	1,092,985	992,493
Lease liabilities, net of current portion	65,285	80,367
Deferred revenue, net of current portion	619,233	663,488
Contingent consideration	50,800	50,800
Other non-current liabilities	20,640	19,785
Total liabilities	2,273,965	2,222,959
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; 79,830,411 and 79,374,247 issued and outstanding at June 30, 2021 and December 31, 2020, respectively	8	8
Additional paid-in capital	3,521,721	3,609,877
Accumulated other comprehensive income, net of tax	2	3
Accumulated deficit	(3,036,623)	(2,848,129)
Total stockholders' equity	485,108	761,759
Total liabilities and stockholders' equity	$ 2,759,073	$ 2,984,718

Source: Sarepta Therapeutics, Inc.

Investor Contact:
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Media Contact:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com