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

5/5/21

- ***Net product sales for the first quarter 2021 of $124.9 million, a 24% increase over the same quarter of prior year***

CAMBRIDGE, Mass., May 05, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the first quarter of 2021.

"In the first quarter, we obtained FDA approval for and launched our third RNA therapy for Duchenne, AMONDYS 45™ (casimersen). The first AMONDYS 45 patient was treated within the first week of approval. Serving the community with EXONDYS 51, VYONDYS 53, and AMONDYS 45, we achieved net product revenue of $124.9 million, a 24% increase over the same quarter in 2020," stated Doug Ingram, Sarepta's president and CEO. "We also made significant strides in the advancement of our pipeline. Two days ago we announced positive results for the 30 mg/kg cohort of SRP-5051, our lead PPMO candidate. As we reported, after a median of only 12-weeks and three doses, the PPMO showed a significant dose-dependent increase in expression, exhibiting an 18 times greater level of exon skipping and nearly an order of magnitude greater level of dystrophin production as compared to EXONDYS 51, the currently approved standard of care for those with Duchenne amenable to exon 51 skipping. SRP-5051 achieved this in about half the time as EXONDYS 51 and with only about 12% of the dose exposure."

Mr. Ingram continued, "Also in the first quarter we reported positive data from our ongoing study of SRP-9003, our investigational gene therapy for limb-girdle muscular dystrophy Type 2E at the 2021 Muscular Dystrophy Association Annual Clinical and Scientific Conference, reporting robust expression, a good safety profile, good durability to two years, and significantly better functional results than age-matched natural history. For SRP-9001, our investigational gene therapy for Duchenne, we have gained invaluable and proprietary insight from the read out of Part 1 of Study 102 which we have used to refine the protocol of our next trial, Study 301. We remain on track to report data in the second quarter from Study 103 (ENDEAVOR), our open-label study evaluating the performance of our commercially representative SRP-9001 material."

First Quarter 2021 and Recent Corporate Developments:

- ***Reported positive clinical results from Phase 2 MOMENTUM study of SRP-5051 in patients with Duchenne muscular dystrophy amenable to skipping exon 51:*** These results were from the 30 mg/kg arm of Part A of the MOMENTUM study (Study 5051-201), a global, Phase 2, multi-ascending dose clinical trial of SRP-5051, Sarepta's next-generation peptide phosphorodiamidate morpholino oligomer (PPMO) treatment for patients with Duchenne muscular dystrophy amenable to skipping exon 51. Strong, dose-dependent exon-skipping and dystrophin expression results with monthly dosing of SRP-5051 were observed in ambulant and non-ambulant Duchenne patients when compared to earlier dosing cohorts in Part A and a control group who received weekly dosing of eteplirsen. Hypomagnesemia was identified in patients taking SRP-5051. Cases have resolved with oral magnesium supplementation and an analysis of all available data indicate that the hypomagnesemia is monitorable and manageable. Part A of MOMENTUM is now complete, and the Company is engaging regulatory agencies to outline next steps for the program. Part B of MOMENTUM is intended to be a pivotal trial supporting an accelerated approval in the United States. The full results will be presented at a future medical meeting.

Results from the 30 mg/kg dose cohort:

- In biopsies taken at week 12, 30 mg/kg of SRP-5051 dosed monthly resulted in mean exon skipping of 10.79% (n=4). Exon skipping was measured by digital drop polymerase chain reaction (ddPCR).
  - This correlates to a >4x increase in exon skipping compared to the 20 mg/kg cohort of SRP-5051 at 12 weeks (mean exon skipping of 2.57%, n=2) and an 18x increase in exon skipping compared a weekly 30 mg/kg dose of eteplirsen at 24 weeks (mean exon skipping of 0.59%, n=16).
- At week 12, 30 mg/kg of SRP-5051 resulted in mean dystrophin production of 6.55% of normal. Dystrophin expression was measured by western blot.
  - This is twice the dystrophin expression compared to the 20 mg/kg cohort at week 12 (mean expression of 3.06%) and eight times that of the eteplirsen comparison group (mean expression of 0.82%).

- ***Sarepta's investigational gene therapy, SRP-9003 being developed for the treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E/R4), showed sustained expression and functional improvements at two years after administration:*** At the 2021 Muscular Dystrophy Association (MDA) Annual Clinical and Scientific Conference held in March, the Company presented the first expression data from biopsies of participants in Cohort 1 (low-dose cohort) taken two years after a single administration of SRP-9003. The results showed sustained protein expression in muscle tissue. In functional outcomes assessments taken two years following treatment in Cohort 1 and one year after treatment in Cohort 2 (high-dose cohort), patients continued to demonstrate stability in their NSAD (North Star Assessment for Dysferlinopathies)

total score and improvements on timed function tests. The results from both cohorts reinforce the safety and tolerability profile of SRP-9003.

Cohort 1 (Dosed at 1.85×10^{13} vg/kg), 24 months following treatment:

- As measured by western blot, mean beta-SG of 54% at 24 months of normal control, compared to 36% measured at Day 60.
- Percent immunofluorescent (IF) positive fibers was 48% compared to normal control, compared to 51% at Day 60.
- Participants showed a mean intensity of 35% of transduced beta-SG, properly localized to the muscle sarcolemma, as measured by IF, compared to 47% at Day 60.
- The mean NSAD improvement from baseline of 5.7 points at 18 months was sustained through 24 months.
- All three participants demonstrated continued improvements from baseline across all functional measures, including time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test.

Cohort 2 (Dosed at 7.41×10^{13} vg/kg), 12 months following treatment:

- At Day 60, the expression of beta-SG (72% mean positive fibers and 73% mean intensity) resulted in increased expression of delta-sarcoglycan, with 65% mean positive fibers and 103% intensity, and gamma-sarcoglycan, 60% mean positive fibers and 97% intensity. These results suggest treatment with SRP-9003 demonstrates reconstitution of the dystrophin-associated protein complex, which could lead to improved membrane integrity and thus improved clinical motor outcomes measures.
- All three participants demonstrated improvements from baseline across all functional measures, including the NSAD, time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test.
- The mean NSAD improvement from baseline was 4.0 points at one year, compared to 3.7 at 6 months.

- ***Presented results from gene therapy and RNA platforms at the 2021 MDA Annual Clinical and Scientific Conference:*** Sarepta held two symposia and presented four podium presentations and six posters at MDA. In addition to the new two- and one-year data for SRP-9003, the three other podium presentations covered data from Part 1 of Study 9001-102, an ongoing clinical trial of SRP-9001, Sarepta's investigational gene therapy for Duchenne muscular dystrophy and pre-clinical approaches to the challenge of pre-existing antibodies. An analysis of time to loss of ambulation in patients taking eteplirsen compared to standard of care was also presented.

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 6429649. Please specify to the operator that you would like to join the "Sarepta First 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, the Company reported a net loss of $167.3 million and $17.5 million, or $2.10 and $0.23 per basic and diluted share for the first quarter of 2021 and 2020, respectively. On a non-GAAP basis, the net loss for the first quarter of 2021 was $122.5 million, or $1.54 per basic and diluted share, compared to a net loss of $79.8 million, or $1.04 per basic and diluted share for the same period of 2020.

Revenues

For the three months ended March 31, 2021, the Company recorded net product revenues of $124.9 million, compared to net product revenues of $100.4 million for the same period of 2020, an increase of $24.5 million. The increase primarily reflects the continuing increase in demand for the Company's products in the U.S.

For the three months ended March 31, 2021 and 2020, the Company recognized $22.0 million and $13.2 million of collaboration revenue, respectively, which relates to the Company's collaboration arrangement with Roche Holding A.G. (Roche).

Cost and Operating Expenses

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

For the three months ended March 31, 2021, cost of sales (excluding amortization of in-licensed rights) was $22.3 million, compared to $12.6 million for the same period of 2020, an increase of $9.7 million. The increase in cost of sales 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 three months ended March 31, 2021, with no similar activity for the three months ended March 31, 2020.

Research and development

Research and development expenses were $195.1 million for the three months ended March 31, 2021, compared to $136.1 million for the same period of 2020, an increase of $59.0 million. The increase in research and development expenses primarily reflects the following:

- $27.1 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;
- $10.0 million increase in clinical trial expenses primarily due to increased patient enrollment for the Company's ESSENCE program as well as certain start-up activities for the Company's micro-dystrophin program;

- $6.9 million increase in research activities primarily driven by an increase in sponsored research with academic institutions during the three months ended March 31, 2021;
- $4.6 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $4.1 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $3.9 million increase in facility- and technology-related expenses due to the Company's continuing expansion efforts;
- $3.6 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene on its MPS IIIA drug candidate;
- $1.9 million increase in stock-based compensation expense primarily driven by increases in headcount and changes in stock price;
- $1.9 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors as a result of an increase in hiring and headcount;
- $4.5 million decrease in up-front, milestone and other expenses primarily due to $4.0 million of milestone expense incurred in the three months ended March 31, 2021, offset by $8.8 million of milestone expense accrued to an academic institution during the same period of 2020; and
- $3.3 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.

Non-GAAP research and development expenses were $173.5 million and $114.2 million for the three months ended March 31, 2021 and 2020, respectively, an increase of $59.3 million.

Selling, general and administration

Selling general and administrative expenses were approximately $71.1 million for the three months ended March 31, 2021, compared to $82.8 million for the same period in 2020, a decrease of $11.7 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $2.6 million increase in stock-based compensation primarily due to increases in headcount and changes in stock price;
- $1.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $15.6 million decrease in professional services primarily due to a decrease in reliance on third-party selling, general and administrative contractors as a result of an increase in hiring and headcount, as well as a transaction fee for the Roche transaction incurred during the three months ended March 31, 2020, with no similar activity incurred during the three months ended March 31, 2021.

Non-GAAP selling, general and administrative expenses were $51.5 million and $54.5 million for the three months ended March 31, 2021 and 2020, respectively, a decrease of $3.0 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. 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 March 31, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.2 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 UWA.

Other expense, net

For the three months ended March 31, 2021 and 2020, other expense, net was approximately $15.5 million and $7.4 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 for consideration of $102.0 million. The closing of the transaction is subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary conditions. The transaction closed on April 13, 2021 and the net proceeds will be recorded as a gain from sale of the PRV as it does not have a carrying value at the time of the sale during the quarter ended June 30, 2021.

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 March 31, 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 PRV obtained as a result of the FDA approval of VYONDYS 53 in December 2019 is a non-recurring event 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 and expected plans and milestones, including reporting data in Q2 2021 from SRP-9001 Study 103, the plan that Part B of MOMENTUM will be a pivotal trial supporting an accelerated approval in the U.S., and reporting the full results of our MOMENTUM study at a future medical meeting.

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 March 31,	
	2021	**2020**
Revenues:		
Products, net	$ 124,926	$ 100,448
Collaboration	22,005	13,226
Total revenues	146,931	113,674
Cost and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	22,346	12,622
Research and development	195,149	136,144
Selling, general and administrative	71,131	82,768
Settlement and license charges	10,000	—
Amortization of in-licensed rights	170	166
Total cost and expenses	298,796	231,700
Operating loss	(151,865)	(118,026)
Other (loss) income:		
Other expense, net	(15,528)	(7,420)
Gain from sale of Priority Review Voucher	—	108,069
Total other (loss) income	(15,528)	100,649

Loss before income tax (benefit) expense	(167,393)	(17,377)
Income tax (benefit) expense	(143)	115
Net loss	$ (167,250)	$ (17,492)
Net loss per share - basic and diluted	$ (2.10)	$ (0.23)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	79,454	76,432

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 March 31,	
	2021	**2020**
GAAP net loss	$ (167,250)	$ (17,492)
Interest expense, net	15,456	8,512
Income tax (benefit) expense	(143)	115
Gain from sale of Priority Review Voucher	—	(108,069)
Collaboration revenue	(22,005)	(13,226)
Depreciation and amortization expense	8,930	6,529
Stock-based compensation expense	28,508	24,024
Roche transaction costs	—	11,292
Up-front, milestone, and other expenses	4,000	8,533
Settlement and license charges	10,000	—
Non-GAAP net loss	$ (122,504)	$ (79,782)
Non-GAAP net loss per share:		
Basic and diluted	$ (1.54)	$ (1.04)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	79,454	76,432

	For the Three Months Ended March 31,	
	2021	**2020**
GAAP research and development expenses	$ 195,149	$ 136,144
Up-front, milestone, and other expenses	(4,000)	(8,533)
Stock-based compensation expense	(11,126)	(9,249)
Depreciation and amortization expense	(6,538)	(4,177)
Non-GAAP research and development expenses	$ 173,485	$ 114,185

	For the Three Months Ended March 31,	
	2021	**2020**
GAAP selling, general and administrative expenses	$ 71,131	$ 82,768
Stock-based compensation expense	(17,382)	(14,775)
Depreciation and amortization expense	(2,222)	(2,186)
Roche transaction costs	—	(11,292)
Non-GAAP selling, general and administrative expenses	$ 51,527	$ 54,515

Condensed Consolidated Balance Sheets
(unaudited, in thousands, except share and per share data)

	As of March 31, 2021	As of December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 1,481,836	$ 1,502,648
Short-term investments	255,997	435,923
Accounts receivable	118,203	101,340
Inventory	240,333	231,961
Other current assets	174,981	213,324
Total current assets	2,271,350	2,485,196
Property and equipment, net	203,107	190,430
Intangible assets, net	14,124	13,628
Right of use assets	65,068	91,761
Other non-current assets	211,584	203,703
Total assets	$ 2,765,233	$ 2,984,718
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 76,651	$ 111,090
Accrued expenses	180,636	193,553
Deferred revenue, current portion	89,244	89,244
Other current liabilities	18,205	22,139
Total current liabilities	364,736	416,026
Long-term debt	1,091,110	992,493
Lease liabilities, net of current portion	60,675	80,367
Deferred revenue, net of current portion	641,483	663,488
Contingent consideration	50,800	50,800
Other non-current liabilities	20,984	19,785
Total liabilities	2,229,788	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; 79,748,109 and 79,374,247 issued and outstanding at March 31, 2021, and December 31, 2020, respectively	8	8
Additional paid-in capital	3,490,658	3,609,877
Accumulated other comprehensive (loss) income, net of tax	(3)	3
Accumulated deficit	(2,955,218)	(2,848,129)
Total stockholders' equity	535,445	761,759
Total liabilities and stockholders' equity	$ 2,765,233	$ 2,984,718

Source: Sarepta Therapeutics, Inc.

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