Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2020 Financial Results and Recent Corporate Developments

3/1/21

– Net product sales for the fourth quarter and full-year 2020 of $122.6 million and $455.9 million, respectively, were pre-announced in January 2021 at the J.P. Morgan Healthcare Conference –

– Fourth quarter 2020 net product sales increased approximately 23% over the fourth quarter of 2019; full-year 2020 product revenue increased almost 20% over the prior year –

CAMBRIDGE, Mass., March 01, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the fourth quarter and full-year 2020.

"In the midst of a challenging pandemic, in 2020 the Sarepta team executed and stayed focused on the patients we serve. We advanced our multi-platform portfolio and achieved a number of important milestones, both in our gene therapy and in our RNA platform, including the submission of our FDA application for the approval of AMONDYS 45™ (casimersen), which resulted in the approval we reported last week. This is our third internally developed RNA therapy approved in the U.S. to treat Duchenne muscular dystrophy and we are now able to offer treatment to the 8% of Duchenne patients with a confirmed exon 45 amenable mutation. By providing a treatment option for patients with an exon 45 amenable mutation, AMONDYS 45 will contribute to the steady growth of our RNA product revenue, which in the fourth quarter of 2020 achieved net product sales of $122.6 million, a 23% increase over the same quarter last year and $455.9 million for full year 2020, a nearly 20% increase over the prior year," stated Doug Ingram, Sarepta's president and CEO. "This approval moves us closer to our goal of treating the greatest percentage of the Duchenne community as possible. Our three therapies together can treat nearly 30% of Duchenne patients in the U.S. and with our RNA-PMO technology, or its next generation version, the PPMO, we could build constructs to ultimately treat over 80% of Duchenne mutations."

Mr. Ingram continued, "In January and at the end of the fourth quarter we reported results from two clinical trials for two of our key value-driving pipeline assets in development for Duchenne: Part 1 of Study 102 for our gene therapy SRP-9001, and Part A of the MOMENTUM study for SRP-5051, our first candidate developed using our next-generation PPMO technology. Although Study 102 did not achieve statistical significance on the primary functional endpoint, the top-line results reinforce our confidence in the potentially transformative benefits of SRP-9001 and generated key insights that will inform the protocol for our upcoming trial, Study 301. Additionally, we plan to report data in the second quarter from Study 103 called ENDEAVOR, our open-label study that is testing commercially representative SRP-9001 material. For SRP-5051 we are pleased with the higher tissue concentration, exon skipping and dystrophin production in the 20 mg/kg dosing group observed at an early 12-week timepoint at an order of magnitude lower cumulative drug exposure compared to our current PMO technology. We are on track to report data from the 30 mg/kg arm of the MOMENTUM trial in the second quarter. Additionally, later this month at the 2021 MDA Clinical and Scientific Conference, we will present the data for SRP-9001-102 Part 1 and new long-term functional data from study SRP-9003-101, our gene therapy in development for limb-girdle muscular dystrophy type 2E."

Fourth Quarter 2020 and Recent Corporate Developments:

- **Received FDA Approval of AMONDYS 45 (casimersen) Injection for the Treatment of Duchenne Muscular Dystrophy (DMD) in Patients Amenable to Skipping Exon 45, Sarepta's third RNA exon-skipping treatment for DMD approved in the U.S.:** AMONDYS 45 is an antisense oligonucleotide from Sarepta's phosphorodiamidate morpholino oligomer (PMO) platform, indicated for the treatment of DMD in patients with a confirmed mutation amenable to exon 45 skipping. This indication is based on a statistically significant increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45, which is reasonably likely to predict clinical benefit for those patients who are exon 45 amenable. Consistent with the accelerated approval pathway, the continued approval of AMONDYS 45 may be contingent on confirmation of a clinical benefit in confirmatory trials. The ESSENCE trial, a placebo-controlled, confirmatory trial to support both the AMONDYS 45 and VYONDYS 53 approvals is ongoing and expected to conclude in 2024.

- **Reported top-line results for Part 1 of Study SRP-9001-102 (Study 102) evaluating SRP-9001, Sarepta's investigational gene transfer therapy for the treatment of Duchenne muscular dystrophy:** Study 102 is a double-blind, 1:1 randomized, placebo-controlled clinical trial of SRP-9001 in 41 participants with DMD, ages of 4-7, using clinical process SRP-9001. Primary endpoints are micro-dystrophin expression at 12 weeks and change in NSAA total score at 48 weeks compared to placebo. In Part 1 results from the treatment and placebo groups are compared through 48 weeks following treatment. Data from the Part 1 of the study showed the following:

    - Met the primary biological endpoint of micro-dystrophin protein expression at 12 weeks post-treatment (P<0.0001), as measured by western blot, in SRP-9001-treated participants versus placebo with mean micro-dystrophin expression of 28.1% for treated participants.

    - SRP-9001-treated participants showed an increase in North Star Ambulatory Assessment (NSAA) total score compared to placebo at 48 weeks.

- Study did not achieve statistical significance (P=0.37) on the primary functional endpoint of improvement in NSAA total score compared to placebo at 48 weeks post-treatment.

- No new safety signals for SRP-9001 were identified.

  In the pre-specified analysis of participants aged 4-5 (n=16) at the time of treatment, the treatment group demonstrated a statistically significant improvement on NSAA compared to the age-matched placebo group (P=0.0172). The functional status at baseline for the 4-5 age group was balanced across the placebo and treatment cohorts. A statistically significant imbalance (P=0.0046) in baseline NSAA total score was present in the cohort of 6-7-year-old participants (n=25), resulting in milder participants in the placebo arm (n=13) than in the treated arm (n=12). The significantly different baseline characteristics between treatment and control groups in the 6-7 age group may have contributed to the inability to observe a treatment effect in the 6-7 age group at the week 48 timepoint in Part 1.

  Study 102 is ongoing and remains blinded with all participants having entered the Part 2 crossover phase. Participants continue to be monitored for safety and will undergo another biopsy at week 12 in Part 2 to assess expression and biological markers, in addition to longer-term assessments of functional outcomes.

- **Announced positive clinical results from MOMENTUM, a global Phase 2 clinical trial of SRP-5051 in patients with Duchenne muscular dystrophy amenable to skipping exon 51:** The multiple-ascending dose trial of Sarepta's investigational peptide phosphorodiamidate morpholino oligomer (PPMO), its next-generation technology for the treatment of Duchenne, demonstrated proof-of-concept for SRP-5051 and supports continued dose escalation. At a total dose exposure approximately 10x lower than eteplirsen, SRP-5051 at 20 mg/kg showed enhanced tissue exposure, greater exon skipping, and greater dystrophin production with no negative renal or other laboratory findings. When compared to a control group of Duchenne patients from the PROMOVI study who received biopsies at 24 weeks after taking a weekly 30 mg/kg dose of eteplirsen, once-monthly dosing of SRP-5051 resulted in higher muscle concentration, increased exon-skipping and dystrophin at 12 weeks. A dose-dependent increase in exon-skipping and dystrophin was observed, with patients in the 20 mg/kg dose group of SRP-5051 seeing a 1.6-fold increase in exon skipping (n=4) and a 5-fold increase in the % of normal dystrophin (n=2) when compared to the group taking eteplirsen at 24 weeks.

- **Entered into research collaboration with Genevant Sciences for lipid nanoparticle (LNP) -based gene editing therapeutics:** LNPs offer the potential for a non-viral approach to gene editing and can provide both optimal uptake into desired cells and efficient release, resulting in functional delivery of gene editing cargo, such as CRISPR-Cas, to target tissues. Genevant will design and collaborate with Sarepta in the development of muscle targeted LNPs to be applied to gene editing targets in early-stage development. Sarepta has rights to an exclusive license to Genevant's LNP technology for up to four neuromuscular indications, including Duchenne muscular dystrophy.

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 9672289. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 2020 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 December 31, 2020 and 2019, the Company reported a net loss of $189.3 million and $235.7 million, or $2.40 and $3.16 per basic and diluted share, respectively. On a non-GAAP basis, the net loss for the three months ended December 31, 2020 and 2019 was $145.1 million and $116.9 million, or $1.84 and $1.57 per basic and diluted share, respectively.

On a GAAP basis, for the twelve months ended December 31, 2020 and 2019, the Company reported a net loss of $554.1 million and $715.1 million, or $7.11 and $9.71 per basic and diluted share, respectively. On a non-GAAP basis, the net loss for the twelve months ended December 31, 2020 and 2019 was $454.3 million and $316.3 million, or $5.83 and $4.30 per basic and diluted share, respectively.

Revenues

For the three months ended December 31, 2020, the Company recorded net product revenues of $122.6 million, compared to net product revenues of $100.1 million for the same period of 2019, an increase of $22.5 million. For the twelve months ended December 31, 2020, the Company recorded net product revenues of $455.9 million, compared to net product revenues of $380.8 million for the twelve months ended December 31, 2019, an increase of $75.1 million. The increase primarily reflects the continuing increase in demand for EXONDYS 51 and the launch of VYONDYS 53 in the U.S.

For the three and twelve months ended December 31, 2020, the Company recognized $22.5 million and $84.2 million of collaboration revenue, respectively, which primarily relates to the Company's collaboration arrangement with F. Hoffman-La Roche Ltd. (Roche). In February 2020, the Company received an aggregate of approximately $1.2 billion in cash consideration from Roche, consisting of an up-front payment for the rights

granted to Roche and pre-paid development costs, and an equity investment in the Company. Of that amount, $348.7 million is recorded as deferred revenue and is being recognized as collaboration revenue on a straight-line basis over the performance period, estimated to be through the fourth quarter of 2023. There was no such transaction in 2019.

Cost and Operating Expenses

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

For the three months ended December 31, 2020, cost of sales (excluding amortization of in-licensed rights) was $22.4 million, compared to $15.6 million for the same period of 2019, an increase of $6.8 million. For the twelve months ended December 31, 2020, cost of sales (excluding amortization of in-licensed rights) was approximately $63.4 million, compared to $56.6 million for the same period of 2019, an increase of $6.8 million. The increase in cost of sales is primarily due to an increase in royalty payments to BioMarin Pharmaceutical (BioMarin) and University of Western Australia (UWA) that reflects increasing demand for the Company's products.

Research and development

Research and development expenses were $207.2 million for the three months ended December 31, 2020, compared to $223.1 million for the same period of 2019, a decrease of $15.9 million. The decrease in research and development expenses primarily reflects the following:

- $58.1 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's micro-dystrophin gene therapy program as well as other gene therapy programs in the Company's pipeline;
- $9.8 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;
- $2.6 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts;
- $2.2 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.2 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's gene therapy platforms during the fourth quarter of 2020, offset by the completion of certain toxicology studies in the Company's PPMO platform;
- $1.9 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $1.4 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;
- $5.8 million decrease in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene on its MPS IIIA drug candidate;
- $64.2 million decrease in up-front, milestone and other expenses primarily due to a $46.9 million up-front payment to StrideBio, Inc. (StrideBio) and a $28.0 million up-front payment to Genethon as a result of the execution of a license and collaboration agreement with each company, respectively, in the fourth quarter of 2019. This was offset primarily by $10.6 million of up-front payments as a result of the execution of certain research, option and license agreements during the fourth quarter of 2020; and
- $24.0 million offset to expense incurred in the fourth quarter of 2020 associated with a collaboration reimbursement from Roche related to the micro-dystrophin project.

Research and development expenses were $722.3 million for the twelve months ended December 31, 2020, compared to $560.9 million for the same period of 2019, an increase of $161.4 million. The increase in research and development expenses primarily reflects the following:

- $223.9 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's micro-dystrophin gene therapy program as well as other gene therapy programs in the Company's pipeline;
- $17.5 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $16.1 million increase in clinical expenses primarily due to increased patient enrollment in the Company's ESSENCE program as well as certain start-up activities for the Company's micro-dystrophin program;
- $14.0 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $8.8 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts;

- $5.4 million increase in research and other primarily driven by an increase in sponsored research with academic institutions during 2020;

- $3.7 million increase in collaboration cost-sharing with Genethon on its micro-dystrophin drug candidates and Lysogene on its MPS IIIA drug candidate;

- $1.6 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company's PPMO platform, offset by an increase of toxicology studies in the Company's gene therapy programs;

- $4.6 million decrease in professional services primarily due to a decrease in reliance on third-party research and development contractors as a result of an increase in hiring and headcount;

- $55.9 million decrease in up-front, milestone, and other expenses, primarily due to $9.3 million of milestone expense related to payments accrued to an academic institution and $38.0 million of up-front payments as a result of the execution of certain research, option and license agreements during 2020. This was offset primarily by $46.9 million of up-front payments to StrideBio and a $28.0 million up-front payment to Genethon as a result of the execution of a license and collaboration agreement with each company, respectively, and $25.6 million of up-front and milestone payments made to various academic institutions throughout 2019; and

- $65.9 million offset to expense incurred during 2020 associated with a collaboration reimbursement from Roche related to the micro-dystrophin project.

Non-GAAP research and development expenses were $180.8 million and $135.4 million for the three months ended December 31, 2020 and 2019, respectively, an increase of $45.4 million. Non-GAAP research and development expenses were $615.3 million and $414.8 million for the twelve months ended December 31, 2020 and 2019, respectively, an increase of $200.5 million.

Selling, general and administration

Selling general and administrative expenses were approximately $86.0 million for the three months ended December 31, 2020, compared to $81.4 million for the same period in 2019, an increase of $4.6 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $5.5 million increase in stock-based compensation partially due to increases in headcount and stock price;
- $3.9 million increase in professional services primarily due to continuing global expansion; and
- $3.6 million decrease in compensation and other personnel expenses primarily due to a decrease in bonus expense.

Selling general and administrative expenses were $317.9 million for the twelve months ended December 31, 2020, compared to $284.8 million for the same period of 2019, an increase of $33.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $15.5 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- $15.2 million and $1.4 million increases in professional services and facility- and technology-related expense, respectively, both of which primarily due to continuing global expansion.

Non-GAAP selling, general and administrative expenses were $65.2 million and $65.8 million for the three months ended December 31, 2020 and 2019, respectively, a decrease of $0.6 million. Non-GAAP selling, general and administrative expenses were $232.0 million and $225.5 million for the twelve months ended December 31, 2020 and 2019, respectively, an increase of $6.5 million.

Acquired in-process research and development

As a result of the Myonexus acquisition, the Company recorded acquired in-process research and development expense of approximately $173.2 million during the second quarter of 2019. There was no such transaction during 2020.

Settlement and license charges

In December 2019, the Company recognized a $10.0 million settlement charge related to contingent settlement payments to BioMarin as a result of the approval of VYONDYS 53. This was a result of a settlement and license agreement with BioMarin in July 2017. There was no such expense recognized during 2020.

Amortization of in-licensed rights

For each of the three months ended December 31, 2020 and 2019, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For the twelve months ended December 31, 2020 and 2019, the Company recorded amortization of in-licensed rights of approximately $0.7 million and $0.8 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 UWA.

Gain from sale of Priority Review Voucher

In February 2020, the Company entered into an agreement with Vifor (International) Ltd. to sell the rare pediatric disease Priority Review Voucher ("PRV") it received from the FDA in connection with the approval of VYONDYS 53. Following the early termination of the applicable waiting period

under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, 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. There was no similar activity during 2019.

Loss on contingent consideration

The loss on contingent consideration relates to the fair value adjustment of the Company's contingent consideration liability related to regulatory-related contingent payments to Myonexus selling shareholders as well as to an academic institution under separate license agreements that meet the definition of a derivative. For the year ended December 31, 2020, the Company recognized $45.0 million of expense to adjust the fair value of the contingent consideration liabilities based on the most current assumptions relating to the achievement of the milestones. There was no similar activity during 2019.

Other expense, net

For the three months and twelve months ended December 31, 2020, other expense, net was approximately $17.8 million and $52.0 million, respectively. For the three and twelve months ended December 31, 2019, other expense, net was approximately $4.8 million and $8.3 million, respectively. The increases for both periods primarily reflect the interest expense on the Company's debt facilities as well as a decrease in interest income and the amortization of investment discounts due to the investment mix of the Company's investment portfolio.

Cash, Cash Equivalents, Investments and Restricted Cash and Investments

The Company had approximately $1.9 billion in cash, cash equivalents and investments as of December 31, 2020, compared to $1.1 billion as of December 31, 2019. The increase is primarily driven by the $1.2 billion of up-front payments received from the Roche as a result of the execution of the collaboration and equity investment agreements offset by cash used to fund the Company's ongoing operations during 2020.

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, 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, milestone, and acquired in-process research and development expenses 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.

As a result of the Myonexus acquisition, the Company recorded acquired in-process research and development expense, which represents a non-recurring expense and, therefore, was treated as a non-GAAP adjustment item. The Company believes the presentation of adjusted research and development, which does not include license- and collaboration-related up-front and milestone expenses, provides useful and meaningful information

about its ongoing research and development activities by enhancing investors' understanding of the Company's normal, recurring operating research and development expenses and facilitates comparisons between periods and with respect to projected performance.

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 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

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy 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 potential of AMONDYS 45 to treat 8% of Duchenne patients with a confirmed exon 45 amendable mutation; our goal of treating the greatest percentage of the Duchenne community as possible; the potential of our three therapies together to treat nearly 30% of Duchenne patients in the U.S.; the potential of our RNA technology to build constructs to treat over 80% of Duchenne mutations; the potentially transformative benefits of SRP-9001; the potential benefits of our collaboration with Genevant and the obligations, rights, responsibilities, potential payments and fees under the collaboration agreement; and expected plans and milestones, including reporting data from Study SRP-9001-103 in Q2 2021, reporting data from the 30 mg/kg arm of the MOMENTUM trial in Q2 2021, our plan to present data at the 2021 MDA Clinical and Scientific Conference from SRP-9001-102 Part 1 and new long-term functional data from study SRP-9003-101, and concluding our ESSENCE trial in 2024.

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 commercial launch for AMONDYS 45 in the U.S. may not be successful for various reasons, including the actual market size and drug supply needed may not be consistent with the company's expectations, the degree to which AMONDYS 45 is accepted by patients and prescribed by physicians, manufacturing limitations, and competitive, reimbursement and regulatory conditions that could negatively impact the commercial launch of AMONDYS 45; 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; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; success in preclinical testing and early 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; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; 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 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Internet Posting of Information

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

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2020	2019	2020	2019
Revenues:				
Products, net	$ 122,644	$ 100,113	$ 455,865	$ 380,833
Collaboration	22,494	—	84,234	—
Total revenues	145,138	100,113	540,099	380,833
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	22,404	15,567	63,382	56,586
Research and development	207,239	223,141	722,343	560,909
Selling, general and administrative	86,046	81,424	317,875	284,812
Acquired in-process research and development	—	—	—	173,240
Settlement and license charges	—	10,000	—	10,000
Amortization of in-licensed rights	165	200	662	849
Total cost and expenses	315,854	330,332	1,104,262	1,086,396
Operating loss	(170,716)	(230,219)	(564,163)	(705,563)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	108,069	—
Loss on contingent consideration	—	—	(45,000)	—
Other expense, net	(17,769)	(4,773)	(51,971)	(8,317)
Total other (loss) income	(17,769)	(4,773)	11,098	(8,317)
Loss before income tax expense	(188,485)	(234,992)	(553,065)	(713,880)
Income tax expense	832	711	1,063	1,195
Net loss	$ (189,317)	$ (235,703)	$ (554,128)	$ (715,075)
Net loss per share - basic and diluted	$ (2.40)	$ (3.16)	$ (7.11)	$ (9.71)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	78,905	74,557	77,956	73,615

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

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2020	2019	2020	2019
GAAP net loss	$ (189,317)	$ (235,703)	$ (554,128)	$ (715,075)
Interest expense, net	18,446	4,562	52,488	8,081
Income tax expense	832	711	1,063	1,195
Gain from sale of Priority Review Voucher	—	—	(108,069)	—
Loss on contingent consideration	—	—	45,000	—
Collaboration revenue	(22,494)	—	(84,234)	—
Depreciation and amortization expense	7,288	6,646	26,911	24,500
Stock-based compensation expense	29,527	22,064	108,070	78,602
Roche transaction costs	—	—	11,292	—
Up-front, milestone, and other expenses	10,622	74,816	47,280	103,162
Settlement and license charges	—	10,000	—	10,000
Acquired in-process research and development	—	—	—	173,240
Non-GAAP net loss	$ (145,096)	$ (116,904)	$ (454,327)	$ (316,295)
Non-GAAP net loss per share:				
Basic and diluted	$ (1.84)	$ (1.57)	$ (5.83)	$ (4.30)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	78,905	74,557	77,956	73,615

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2020	2019	2020	2019
GAAP research and development expenses	$ 207,239	$ 223,141	$ 722,343	$ 560,909
Up-front, milestone, and other expenses	(10,622)	(74,816)	(47,280)	(103,162)
Stock-based compensation expense	(10,637)	(8,699)	(41,671)	(27,681)
Depreciation and amortization expense	(5,162)	(4,188)	(18,054)	(15,240)
Non-GAAP research and development expenses	$ 180,818	$ 135,438	$ 615,338	$ 414,826

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2020	2019	2020	2019
GAAP selling, general and administrative expenses	$ 86,046	$ 81,424	$ 317,875	$ 284,812
Stock-based compensation expense	(18,890)	(13,365)	(66,399)	(50,921)
Depreciation and amortization expense	(1,961)	(2,258)	(8,195)	(8,411)
Roche transaction costs	—	—	(11,292)	—
Non-GAAP selling, general and administrative expenses	$ 65,195	$ 65,801	$ 231,989	$ 225,480

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

	As of December 31,	
	2020	2019
Assets		
Current assets:		

Cash and cash equivalents	$ 1,502,648	$ 835,080
Short-term investments	435,923	289,668
Accounts receivable	101,340	90,879
Inventory	231,961	171,379
Other current assets	213,324	81,907
Total current assets	2,485,196	1,468,913
Property and equipment, net	190,430	129,620
Intangible assets, net	13,628	12,497
Right of use assets	91,761	37,933
Other non-current assets	203,703	173,859
Total assets	$ 2,984,718	$ 1,822,822
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 111,090	$ 68,094
Accrued expenses	193,553	185,527
Deferred revenue, current portion	89,244	3,303
Other current liabilities	22,139	7,843
Total current liabilities	416,026	264,767
Long-term debt	992,493	681,900
Lease liabilities, net of current portion	80,367	47,720
Deferred revenue, net of current portion	663,488	—
Contingent consideration	50,800	5,200
Other non-current liabilities	19,785	5,048
Total liabilities	2,222,959	1,004,635
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; 79,374,247 and 75,184,863 issued and outstanding at December 31, 2020 and 2019, respectively	8	8
Additional paid-in capital	3,609,877	3,112,130
Accumulated other comprehensive income, net of tax	3	50
Accumulated deficit	(2,848,129)	(2,294,001)
Total stockholders' equity	761,759	818,187
Total liabilities and stockholders' equity	$ 2,984,718	$ 1,822,822

Source: Sarepta Therapeutics, Inc.

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