# Sarepta Therapeutics Announces Third Quarter 2021 Financial Results and Recent Corporate Developments

11/3/21

- ***Net product sales for the third quarter of 2021 reached $166.9 million, a 37% increase over the same quarter of prior year***

- ***In light of its continued over-performance, Sarepta raises its full-year product revenue guidance by $40 million to between $605 million to $615 million***

CAMBRIDGE, Mass., Nov. 03, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the third quarter of 2021.

"We are pleased to report another quarter of strong performance serving the Duchenne community with our three currently approved therapies and on that basis have once again raised our full-year product revenue guidance. In total, we have raised guidance by some $70 million this year and are now guiding to $605 million to $615 million. This represents our 20th straight quarter of strong revenue growth and we anticipate this growth continuing in 2022," said Doug Ingram, president and chief executive officer, Sarepta. "We have now initiated Part B of MOMENTUM, our pivotal trial for SRP-5051, our next-generation PPMO candidate for exon 51 skip amenable Duchenne patients as well as EMBARK, our pivotal trial for SRP-9001, our micro-dystrophin gene therapy for Duchenne. Also, this quarter we shared additional compelling data across three studies for SRP-9001, providing additional conviction as we execute on EMBARK and prepare to unblind and release Study 102 Part 2 results in the first quarter of next year. As we track out of 2021 and into a milestone-rich 2022, we are delivering on our approved therapies, seeing successes across our programs, and as of today, with greater than $2 billion of cash and cash equivalents on our balance sheet and a first-in-class team of genetic and rare disease professionals, have the resources and talent to deliver on the promise of our multi-platform pipeline."

***Third Quarter 2021 and Recent Corporate Developments:***

- ***At SRP-9001 Micro-dystrophin R&D Day Sarepta showed sustained functional improvements in multiple studies of individuals with Duchenne:*** In October of 2021, the Company presented new analyses and functional data from its SRP-9001 development program and details of EMBARK (Study SRP-9001-301); its global pivotal Phase 3 trial of SRP-9001 for the treatment of Duchenne. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. The results presented highlight the breadth, depth and strength of the clinical evidence to date for SRP-9001 in treating Duchenne.
    - ***SRP-9001-101 (Study 101):*** Results from participants treated with SRP-9001 in Study 101 (n=4, ages 4 to 7) found that participants improved 8.6 points on the North Star Ambulatory Assessment (NSAA) compared to a matched natural history cohort three years following a single administration of SRP-9001 ($p<0.0001$).
    - ***SRP-9001-102 (Study 102):*** The results from Study 102 Part 1 found that SRP-9001-treated participants (n=12, ages 6 to 7) had a positive 2.9-point difference on NSAA compared to a matched natural history cohort one year after treatment ($p=0.0129$).
    - ***ENDEAVOR, SRP-9001-103 (Study 103):*** The first functional results presented from Study 103, which uses commercially representative SRP-9001 material, the first 11 participants in Cohort 1, ages 4 to 7, demonstrated a 3.0-point improvement from baseline on NSAA six months after treatment.
    - Across three clinical studies, the tolerability profile remains consistent in all treated patients.

- ***Initiated EMBARK (Study SRP-9001-301), the first global pivotal study of SRP-9001 for the treatment of Duchenne:*** The EMBARK study is a multi-center clinical trial initiating in the US, Europe and Asia. It is the first global, randomized, double-blind, placebo-controlled clinical trial of commercially representative SRP-9001 material and will enroll 120 participants with Duchenne between the ages of 4 to 7. The primary endpoint will assess the change in NSAA total score from baseline to week 52 compared to placebo. Key features include stratification of participants by age and baseline NSAA, with a minimum of 50 percent of patients ages 4 to 5 to be enrolled. Inclusion criteria include a Time to Rise from Floor of less than 5 seconds, a stable daily dose of oral corticosteroids for at least 12 weeks before screening and rAAVrh74 antibody titers of less than 1:400. Participants with mutations between or including exons 1-17 or mutations fully contained within exon 45 (inclusive) are not eligible. The Company expects the trial to fully enroll in the first half of 2022.

- ***Initiated Part B of MOMENTUM (Study SRP-5051-201) in patients with Duchenne amenable to exon 51 skipping:*** In the fourth quarter of 2021, the Company initiated Part B of MOMENTUM, a global trial investigating the use of SRP-5051, the Company's next-generation peptide-conjugated phosphorodiamidate morpholino oligomer (PPMO) to treat patients with Duchenne who are amenable to exon 51 skipping. The study plans to enroll between 20-40 patients between ages 7 to 21

amenable to exon 51 skipping who are naïve to SRP-5051. Additionally, those previously dosed in Study 5051-201, Part A or Study 5051-102 who meet the entrance criteria will be eligible to participate. Both ambulatory and non-ambulatory patients are eligible for participation. Sarepta anticipates Part B of MOMENTUM to serve as a pivotal study for SRP-5051 and plans to seek Accelerated Approval if the trial is successful.

- ***Showcased data from gene therapy and RNA platforms at World Muscle Society (WMS) 2021 Virtual Congress:*** Sarepta's presentations at WMS 2021 highlighted scientific leadership and innovation from across the Company's deep, multi-platform portfolio and reflect a continued commitment to advancing life-changing therapies for those with rare genetic diseases. Notably, new research was presented on the prevalence of pre-existing antibodies to the AAVrh74 vector, which is used in several of Sarepta's gene transfer therapy programs. The posters are available on the Investor Relations section of www.sarepta.com.

***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 7131019. Please specify to the operator that you would like to join the "Sarepta Third 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 September 30, 2021, the Company reported a net loss of $48.1 million, or $0.60 per basic and diluted share, compared to a net loss of $196.5 million reported for the same period of 2020, or $2.50 per basic and diluted share. On a non-GAAP basis, the net loss for the three months ended September 30, 2021 was $15.6 million, or $0.19 per basic and diluted share, compared to a net loss of $111.5 million, or $1.42 per basic and diluted share for the same period of 2020.

On a GAAP basis, for the nine months ended September 30, 2021, the Company reported a net loss of $296.8 million, or $3.72 per basic and diluted share, compared to a net loss of $364.8 million reported for the same period of 2020, or $4.70 per basic and diluted share. On a non-GAAP basis, the net loss for the nine months ended September 30, 2021 was $259.2 million, or $3.25 per basic and diluted share, compared to a net loss of $309.2 million, or $3.98 per basic and diluted share for the same period of 2020.

***Revenues***

For the three months ended September 30, 2021, the Company recorded total revenues of $189.4 million, compared to total revenues of $143.9 million for the same period of 2020, an increase of $45.5 million. For the nine months ended September 30, 2021, the Company recorded total revenues of $500.4 million, compared to total revenues of $395.0 million for the same period of 2020, an increase of $105.4 million.

For the three months ended September 30, 2021, the Company recorded net product revenues of $166.9 million, compared to net product revenues of $121.4 million for the same period of 2020, an increase of $45.5 million. For the nine months ended September 30, 2021, the Company recorded net product revenues of $433.7 million, compared to net product revenues of $333.2 million for the same period of 2020, an increase of $100.5 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 other two products in the U.S.

For both the three months ended September 30, 2021 and 2020, the Company recognized $22.5 million of collaboration revenue. For the nine months ended September 30, 2021 and 2020, the Company recognized $66.8 million and $61.7 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 September 30, 2021, cost of sales (excluding amortization of in-licensed rights) was $23.4 million, compared to $15.0 million for the same period of 2020, an increase of $8.4 million. The increase in cost of sales is primarily due to increasing demand for the Company's products. For the nine months ended September 30, 2021, cost of sales (excluding amortization of in-licensed rights) was $65.3 million, compared to $41.0 million for the same period of 2020, an increase of $24.3 million. The increase is primarily due to increasing demand for the Company's products and the write-offs of certain batches of the Company's products not meeting the Company's quality specifications for the nine months ended September 30, 2021, with no similar activity during the same period of 2020.

Research and development

Research and development expenses were $139.1 million for the three months ended September 30, 2021, compared to $190.4 million for the same period of 2020, a decrease of $51.3 million. The decrease in research and development expenses primarily reflects the following:

- $27.5 million decrease in manufacturing expenses primarily due to timing of production activity related to the Company's gene therapy programs;
- $10.9 million decrease in up-front and milestone expenses primarily due to $15.1 million of up-front payments as a result of the execution of certain research, option and license agreements during the third quarter of 2020, offset primarily by $4.5 million of similar activity during the third quarter of 2021;
- $6.2 million decrease in clinical trial expenses primarily due to a ramp-down of enrollment for certain clinical trials as well as the timing of contract research organization activities;
- $2.1 million decrease in collaboration cost sharing expenses with Genethon on its micro-dystrophin drug candidate and Lysogene S.A. on its MPS IIIA drug candidate;

- $1.6 million decrease in compensation and other personnel expenses primarily due to changes in headcount;
- $1.4 million increase in stock-based compensation expense primarily driven by changes in headcount and stock price;
- $2.5 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the three months ended September 30, 2021;
- $2.8 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $3.8 million increase in facility- and technology-related expenses due to the Company's continuing expansion efforts; and
- $12.7 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.

Research and development expenses were $573.9 million for the nine months ended September 30, 2021, compared to $515.1 million for the same period of 2020, an increase of $58.8 million. The increase in research and development expenses primarily reflects the following:

- $16.2 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;
- $15.4 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the nine months ended September 30, 2021;
- $13.6 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 including for the Company's EMBARK program;
- $11.8 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $10.5 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $5.0 million increase in stock-based compensation expense primarily due to changes in headcount and stock price;
- $4.6 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $3.5 million increase in up-front, milestone and other expenses primarily due to a $28.7 million increase of an accrued sublicense fee to Nationwide Children's Hospital (Nationwide) and $11.5 million of expense incurred as a result of up-front payments and milestone achievements in certain research and license agreements during the nine months ended September 30, 2021, offset by $9.3 million of expense related to sublicense payments accrued to Nationwide and $27.1 million of up-front payments as a result of the execution of certain research and license agreements during the same period of 2020;
- $3.4 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors; and
- $18.7 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 $115.1 million and $159.9 million for the three months ended September 30, 2021 and 2020, respectively, a decrease of $44.8 million. Non-GAAP research and development expenses were $477.6 million and $434.5 million for the nine months ended September 30, 2021 and 2020, respectively, an increase of $43.1 million.

Selling, general and administration

Selling, general and administrative expenses were approximately $61.1 million for the three months ended September 30, 2021, compared to $75.4 million for the same period in 2020, a decrease of $14.3 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $9.3 million decrease in professional service expenses primarily due to a decrease in reliance on third-party selling, general and administrative contractors;
- $4.8 million decrease in compensation and other personnel expenses primarily due to a net decrease in headcount period over period;
- $1.6 million decrease in stock-based compensation expense primarily due to changes in headcount and stock prices; and
- $1.1 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts.

Selling, general and administrative expenses were approximately $204.6 million for the nine months ended September 30, 2021, compared to $231.8 million for the same period in 2020, a decrease of $27.2 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $25.5 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 nine months ended September 30, 2020, with no similar activity incurred during the nine months ended September 30, 2021;
- $4.2 million decrease in compensation and other personnel expenses primarily due to a net decrease in headcount period over period; and
- $1.8 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts.

Non-GAAP selling, general and administrative expenses were $43.6 million and $57.2 million for the three months ended September 30, 2021 and 2020, respectively, a decrease of $13.6 million. Non-GAAP selling, general and administrative expenses were $149.2 million and $166.8 million for the nine months ended September 30, 2021 and 2020, respectively, a decrease of $17.6 million.

Settlement and license charges
In February 2021, the Company recognized a $10.0 million settlement charge related to contingent settlement payments to BioMarin Pharmaceutical, Inc. (BioMarin) as a result of the approval of AMONDYS 45 in the U.S. This was a result of a settlement and license agreement with BioMarin in July 2017. There was no such expense recognized during the same period of 2020.

Amortization of in-licensed rights
For each of the three months ended September 30, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For each of the nine months ended September 30, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.5 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.

Gain (loss) on contingent consideration, net
The gain (loss) on contingent consideration, net, relates to the fair value adjustment of the Company's contingent consideration derivative liability related to regulatory-related contingent payments to Myonexus Therapeutics, Inc. (Myonexus) selling shareholders as well as to two academic institutions under separate license agreements that meet the definition of a derivative. During the three and nine months ended September 30, 2021 and 2020, the Company recognized a $7.2 million net gain and $45.0 million net loss, respectively, to adjust the fair value of the contingent consideration.

Other expense, net
For the three months ended September 30, 2021 and 2020, other expense, net was $20.6 million and $14.3 million, respectively. For the nine months ended September 30, 2021 and 2020, other expense, net was $52.4 million and $34.2 million, respectively. The increases primarily reflect an increase in interest expense incurred on the Company's term loan debt facilities due to an increase in the outstanding balance as well as an impairment loss related to a strategic investment, partially offset by a reduction of interest expense incurred on the Company's convertible debt related to the adoption of ASU 2020-06.

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 $1.6 billion in cash, cash equivalents and investments as of September 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, gain from sale of PRV, settlement and license charges, impairment of equity investment and net gain (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 settlement and license charges from its non-GAAP results 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.

- The Company excludes from its non-GAAP results the impairment of any equity investments as it is a non-cash item and is not considered to be a normal operating expense due to the variability of amount and lack of predictability as to the occurrence and/or timing of such impairments.

- The Company excludes from its non-GAAP results the net gain (loss) on contingent consideration related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to two academic institutions under separate license agreements as it is a non-cash item and is not considered to be normal operating expenses due to its variability of amounts and lack of predictability as to occurrence and/or timing.

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 (eteplirsen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

EXONDYS 51 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

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

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 (golodirsen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

VYONDYS 53 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

***Important Safety Information for VYONDYS 53***
Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy.

Kidney toxicity was observed in animals who received golodirsen. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in Duchenne patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

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

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

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

For further information, please see the full Prescribing Information.

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

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

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

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

***Important Safety Information for AMONDYS 45***
Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in Duchenne patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio

should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

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

Adverse reactions observed in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were (AMONDYS 45, placebo): upper respiratory tract infections (65%, 55%), cough (33%, 26%), pyrexia (33%, 23%), headache (32%, 19%), arthralgia (21%, 10%), and oropharyngeal pain (21%, 7%).

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently in the placebo group, were: ear pain, nausea, ear infection, post-traumatic pain, and dizziness and light-headedness.

For further information, please see the full Prescribing Information.

***About Sarepta Therapeutics***

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (Duchenne) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

***Forward-Looking Statements***

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs; the potential for SRP-9001 to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein; the potential benefits of SRP-5051; and expected plans and milestones, including releasing Study SRP-9001-102 Part 2 results in the first quarter of 2022, enrolling 120 participants with Duchenne between ages 4 to 7 in Study SRP-9001-301 in the first half of 2022, enrolling between 20-40 patients between ages 7 to 21 amenable to exon 51 skipping who are naïve to SRP-5051 in our MOMENTUM Study, and our plan for Part B of MOMENTUM to serve as a pivotal study for SRP-5051 and to seek Accelerated Approval if the trial is successful.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, some of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, the COVID-19 pandemic and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 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 September 30, | | For the Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Revenues: | | | | |
| Products, net | $ 166,911 | $ 121,429 | $ 433,676 | $ 333,221 |
| Collaboration | 22,495 | 22,495 | 66,750 | 61,740 |
| Total revenues | 189,406 | 143,924 | 500,426 | 394,961 |
| Cost and expenses: | | | | |
| Cost of sales (excluding amortization of in-licensed rights) | 23,444 | 15,015 | 65,305 | 40,978 |
| Research and development | 139,115 | 190,438 | 573,886 | 515,104 |

| | | | | |
|---|---|---|---|---|
| Selling, general and administrative | 61,127 | 75,373 | 204,605 | 231,829 |
| Settlement and license charges | — | — | 10,000 | — |
| Amortization of in-licensed rights | 178 | 166 | 527 | 497 |
| Total cost and expenses | 223,864 | 280,992 | 854,323 | 788,408 |
| Operating loss | (34,458) | (137,068) | (353,897) | (393,447) |
| Other (loss) income: | | | | |
| Gain (loss) on contingent consideration, net | 7,200 | (45,000) | 7,200 | (45,000) |
| Other expense, net | (20,649) | (14,335) | (52,362) | (34,202) |
| Gain from sale of Priority Review Voucher | — | — | 102,000 | 108,069 |
| Total other (loss) income | (13,449) | (59,335) | 56,838 | 28,867 |
| Loss before income tax expense (benefit) | (47,907) | (196,403) | (297,059) | (364,580) |
| Income tax expense (benefit) | 237 | 96 | (260) | 231 |
| Net loss | $ (48,144) | $ (196,499) | $ (296,799) | $ (364,811) |
| Net loss per share - basic and diluted | $ (0.60) | $ (2.50) | $ (3.72) | $ (4.70) |
| Weighted average number of shares of common stock used in computing basic and diluted net loss per share | 79,880 | 78,501 | 79,695 | 77,637 |

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 September 30, | | For the Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| GAAP net loss | $ (48,144) | $ (196,499) | $ (296,799) | $ (364,811) |
| Interest expense, net | 15,847 | 13,454 | 47,061 | 34,042 |
| Income tax expense (benefit) | 237 | 96 | (260) | 231 |
| Gain from sale of Priority Review Voucher | — | — | (102,000) | (108,069) |
| (Gain) loss on contingent consideration, net | (7,200) | 45,000 | (7,200) | 45,000 |
| Collaboration revenue | (22,495) | (22,495) | (66,750) | (61,740) |
| Depreciation and amortization expense | 10,495 | 6,619 | 27,872 | 19,623 |
| Stock-based compensation expense | 26,684 | 26,903 | 84,161 | 78,543 |
| Roche transaction costs | — | — | — | 11,292 |
| Up-front, milestone, and other expenses | 4,515 | 15,375 | 40,192 | 36,658 |
| Settlement and license charges | — | — | 10,000 | — |
| Impairment of equity investment | 4,488 | — | 4,488 | — |
| Non-GAAP net loss | $ (15,573) | $ (111,547) | $ (259,235) | $ (309,231) |
| Non-GAAP net loss per share: | | | | |
| Basic and diluted | $ (0.19) | $ (1.42) | $ (3.25) | $ (3.98) |
| Weighted average number of shares of common stock used in computing basic and diluted net loss per share | 79,880 | 78,501 | 79,695 | 77,637 |

| | For the Three Months Ended September 30, | | For the Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| GAAP research and development expenses | $ 139,115 | $ 190,438 | $ 573,886 | $ 515,104 |
| Up-front, milestone, and other expenses | (4,515) | (15,375) | (40,192) | (36,658) |
| Stock-based compensation expense | (12,031) | (10,645) | (36,017) | (31,034) |
| Depreciation and amortization expense | (7,445) | (4,516) | (20,043) | (12,892) |

| | | | | |
|---|---|---|---|---|
| Non-GAAP research and development expenses | $ 115,124 | $ 159,902 | $ 477,634 | $ 434,520 |

| | For the Three Months Ended September 30, | | For the Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| GAAP selling, general and administrative expenses | $ 61,127 | $ 75,373 | $ 204,605 | $ 231,829 |
| Stock-based compensation expense | (14,653) | (16,258) | (48,144) | (47,509) |
| Depreciation and amortization expense | (2,872) | (1,937) | (7,302) | (6,234) |
| Roche transaction costs | — | — | — | (11,292) |
| Non-GAAP selling, general and administrative expenses | $ 43,602 | $ 57,178 | $ 149,159 | $ 166,794 |

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

| | As of September 30, 2021 | As of December 31, 2020 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 1,599,113 | $ 1,502,648 |
| Short-term investments | — | 435,923 |
| Accounts receivable | 149,787 | 101,340 |
| Inventory | 288,469 | 231,961 |
| Other current assets | 147,941 | 213,324 |
| Total current assets | 2,185,310 | 2,485,196 |
| Property and equipment, net | 199,249 | 190,430 |
| Intangible assets, net | 14,204 | 13,628 |
| Right of use assets | 72,663 | 91,761 |
| Other non-current assets | 190,792 | 203,703 |
| Total assets | $ 2,662,218 | $ 2,984,718 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 44,163 | $ 111,090 |
| Accrued expenses | 221,009 | 193,553 |
| Deferred revenue, current portion | 89,244 | 89,244 |
| Other current liabilities | 19,027 | 22,139 |
| Total current liabilities | 373,443 | 416,026 |
| Long-term debt | 1,094,912 | 992,493 |
| Lease liabilities, net of current portion | 63,428 | 80,367 |
| Deferred revenue, net of current portion | 596,738 | 663,488 |
| Contingent consideration | 43,600 | 50,800 |
| Other non-current liabilities | 20,569 | 19,785 |
| Total liabilities | 2,192,690 | 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; 79,958,527 and 79,374,247 issued and outstanding at September 30, 2021, and December 31, 2020, respectively | 8 | 8 |
| Additional paid-in capital | 3,554,307 | 3,609,877 |
| Accumulated other comprehensive (loss) income, net of tax | (20) | 3 |
| Accumulated deficit | (3,084,767) | (2,848,129) |
| Total stockholders' equity | 469,528 | 761,759 |
| Total liabilities and stockholders' equity | $ 2,662,218 | $ 2,984,718 |

Source: Sarepta Therapeutics, Inc.

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