Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2021 Financial Results and Recent Corporate Developments

3/1/22

- ***Total revenues, which consist primarily of net product revenues and collaboration revenues, for the fourth quarter and full-year 2021 totaled $201.5 million and $701.9 million, respectively***

- ***Net product revenues for the fourth quarter and full-year 2021 totaled $178.7 million and $612.4 million, respectively***

- ***Fourth quarter 2021 net product revenues increased approximately 46% over the fourth quarter of 2020; full-year 2021 net product revenues increased approximately 34% over the prior year***

CAMBRIDGE, Mass., March 01, 2022 (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 2021.

"In 2021, Sarepta distinguished itself as a well-funded, fully integrated, commercial-stage biotech that executes against its ambitious goals. Launching our third RNA-based therapy, AMONDYS 45®(casimersen) in 2021, we enjoyed our 21st straight quarter of strong quarter-over-quarter growth. In the fourth quarter of 2021, total revenues reached $201.5 million and net product revenue for our now three RNA-based therapies reached $178.7 million, a 46% increase over the same quarter of the prior year. For full-year 2021, total revenue reached $701.9 million and net product revenue was $612.4 million, a 34% increase over the prior year. In 2021, we also commenced pivotal trials for our lead candidates in both our RNA and our gene therapy platforms. We initiated Part B of the MOMENTUM study, our global pivotal trial of SRP-5051, our next-generation RNA-based therapy intended to treat Duchenne patients with exon 51 amenable mutations; and we initiated EMBARK, our global pivotal trial of SRP-9001, the only global trial currently enrolling using a gene therapy micro-dystrophin to treat Duchenne," stated Doug Ingram, Sarepta's president and CEO.

Mr. Ingram continued, "We entered 2022 in a position of strength, with over $2.1 billion of cash and cash equivalents, total revenue guidance of over $880 million and net product revenue guidance of over $800 million. Further, in the first quarter of 2022, we announced statistically significant functional results and demonstrated a differentiated safety profile for SRP-9001 from Part 2 of Study 102. We are continuing to enroll and dose patients in the EMBARK and MOMENTUM studies, and are actively advancing our deep, multi-platform pipeline."

Fourth Quarter 2021 and Recent Corporate Developments:

- ***In Part 2 of Study SRP-9001-102 Sarepta's SRP-9001 micro-dystrophin showed statistically significant functional improvements compared to pre-specified matched external control:*** In January 2022, at the 40th Annual J.P. Morgan Healthcare Conference, Sarepta announced topline results from Part 2 of Study SRP-9001-102 (Study 102), an ongoing, randomized, double-blind, placebo-controlled clinical trial evaluating the safety, efficacy and tolerability of a single dose of SRP-9001 (delandistrogene moxeparvovec) in 41 patients with Duchenne muscular dystrophy, 21 of whom were in the placebo crossover cohort. The treated participants from the placebo crossover group (n=20, aged 5-8 at time of dosing SRP-9001) scored a statistically significant 2.0 points higher on the mean North Star Ambulatory Assessment (NSAA) at 48 weeks compared to propensity-score weighted external controls (p value=0.0009). Mean NSAA scores from these Part 2 participants improved 1.3 points from baseline for the SRP-9001 treated group and the NSAA scores in the external control group (n=103) declined 0.7 points from baseline. The safety profile of patients treated in Part 2 of Study 102 is consistent with that seen in Part 1. For patients treated in Part 1, no new safety signals emerged after two years of follow up. Study 102 remains ongoing and all participants continue to be monitored for safety in addition to longer-term assessments of functional outcomes. Additional results will be shared at a future medical congress.

- ***Sarepta and GenEdit shared progress on research collaboration and announced agreement to develop gene editing therapeutics for neuromuscular diseases:*** GenEdit, Inc. develops genetic medicines that leverage its NanoGalaxy™ platform of non-viral, non-lipid polymer nanoparticles for tissue-selective delivery. Through this research collaboration and exclusive option agreement, the companies are employing GenEdit's NanoGalaxy platform and Sarepta's gene editing technology to develop up to four neuromuscular indications selected by Sarepta. Initial *in vivo* results from the research collaboration have demonstrated the potential of GenEdit's polymer nanoparticles to deliver therapeutic cargo to specific muscle tissue after systemic administration to allow for targeted, non-viral systemic delivery of genetic medicines.

- ***Appointed Stephen L. Mayo, Ph.D. to Sarepta's Board of Directors:*** Dr. Mayo is a world-renowned expert in protein engineering. He is currently the Bren Professor of Biology and Chemistry at California Institute of Technology (Caltech), and serves on the board of directors for Merck and on the scientific advisory board of Rubryc Therapeutics, Inc. Dr. Mayo

co-founded several companies: Molecular Simulations Inc. (now BIOVIA), Xencor, Inc. and, Protabit LLC, where he serves on the scientific advisory board. Dr. Mayo received his undergraduate degree in chemistry from the Pennsylvania State University, his Ph.D. in chemistry from Caltech, and did postdoctoral work at both UC Berkeley and Stanford University School of Medicine.

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 8075225. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 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 December 31, 2021 and 2020, the Company reported a net loss of $122.0 million or $1.42 per basic and diluted share, compared to a net loss of $189.3 million reported for the same period of 2020, or $2.40 per basic and diluted share. On a non-GAAP basis, the net loss for the three months ended December 31, 2021 was $66.0 million, or $0.77 per basic and diluted share, compared to a net loss of $133.2 million[1], or $1.69 per basic and diluted share for the same period of 2020.

On a GAAP basis, for the twelve months ended December 31, 2021, the Company reported a net loss of $418.8 million, or $5.15 per basic and diluted share, compared to a net loss of $554.1 million reported for the same period of 2020, or $7.11 per basic and diluted share. On a non-GAAP basis, the net loss for the twelve months ended December 31, 2021 and 2020 was $308.7 million and $428.7 million, or $3.80 and $5.50 per basic and diluted share, respectively.

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[1] Beginning in the fourth quarter of 2021, up-front and milestone payments associated with the Company's license and collaboration agreements, settlement and license charges and collaboration revenue, along with the related transaction costs incurred, are no longer excluded from the Non-GAAP expenses and income. Non-GAAP financial results for the fourth quarter and full-year 2020 have been updated to reflect this change for comparable purposes.

Revenues

For the three months ended December 31, 2021, the Company recorded total revenues of $201.5 million, which consist primarily of net product revenues and collaboration revenues, compared to total revenues of $145.1 million for the same period of 2020, an increase of $56.4 million. For the twelve months ended December 31, 2021, the Company recorded total revenues of $701.9 million, compared to total revenues of $540.1 million for the same period of 2020, an increase of $161.8 million.

For the three months ended December 31, 2021, the Company recorded net product revenues of $178.7 million, compared to net product revenues of $122.6 million for the same period of 2020, an increase of $56.1 million. For the twelve months ended December 31, 2021, the Company recorded net product revenues of $612.4 million, compared to net product revenues of $455.9 million for the same period of 2020, an increase of $156.5 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 the three months ended December 31, 2021 and 2020, the Company recognized $22.7 million and $22.5 million of collaboration and other revenues, respectively. For the twelve months ended December 31, 2021 and 2020, the Company recognized $89.5 million and $84.2 million of collaboration and other revenues, 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 December 31, 2021, cost of sales (excluding amortization of in-licensed rights) was $31.7 million, compared to $22.4 million for the same period of 2020, an increase of $9.3 million. For the twelve months ended December 31, 2021, cost of sales (excluding amortization of in-licensed rights) was $97.0 million, compared to $63.4 million for the same period of 2020, an increase of $33.6 million. The increases are primarily due to increasing demand for the Company's products.

Research and development

Research and development expenses were $197.3 million for the three months ended December 31, 2021, compared to $207.2 million for the same period of 2020, a decrease of $9.9 million. The decrease in research and development expenses primarily reflects the following:

- $10.5 million decrease in up-front and milestone expenses primarily due to $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, offset by $0.1 million of similar activity during the fourth quarter of 2021;
- $4.2 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;
- $1.0 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors;
- $1.0 million decrease in collaboration cost sharing expenses with Lysogene S.A. (Lysogene) on its MPS IIIA drug candidate and Genethon on its micro-dystrophin drug candidate;

- $1.7 million increase in manufacturing expenses primarily due to the Company's accelerated amortization of nonrefundable advance payments due to capacity changes associated with the execution of the Third Amendment to its manufacturing and supply agreement with Thermo Fisher Scientific, Inc. (Thermo), offset partially by timing of production activity related to the Company's gene therapy programs;
- $3.4 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $3.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $3.9 million increase in stock-based compensation expense primarily driven by changes in headcount and stock price; and
- $5.6 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 $771.2 million for the twelve months ended December 31, 2021, compared to $722.3 million for the same period of 2020, an increase of $48.9 million. The increase in research and development expenses primarily reflects the following:

- $17.8 million increase in manufacturing expenses primarily due to the Company's accelerated amortization of nonrefundable advance payments amortization due to capacity changes associated with the execution of the Third Amendment to its manufacturing and supply agreement with Thermo;
- $15.2 million increase in facility- and technology-related expenses primarily due to the Company's continuing expansion efforts;
- $14.5 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during 2021;
- $11.3 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's PPMO platforms;
- $9.4 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 and patient enrollment for the Company's SRP-9001 micro-dystrophin program including for the Company's EMBARK program;
- $8.9 million increase in stock-based compensation expense primarily due to changes in headcount and stock price;
- $8.2 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $0.7 million decrease in collaboration cost sharing expenses with Lysogene on its MPS IIIA drug candidate offset by an increase in cost sharing expenses with Genethon on its micro-dystrophin drug candidate;
- $4.4 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors;
- $7.0 million decrease 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 and $11.6 million of expense incurred as a result of up-front and milestone payments related to certain research and license agreements during 2021. This was offset primarily by $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; and
- $24.3 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 $175.5 million and $191.4 million for the three months ended December 31, 2021 and 2020, respectively, a decrease of $15.9 million. Non-GAAP research and development expenses were $693.4 million and $662.6 million for the twelve months ended December 31, 2021 and 2020, respectively, an increase of $30.8 million.

Selling, general and administration

Selling, general and administrative expenses were $78.1 million for the three months ended December 31, 2021, compared to $86.0 million for the same period in 2020, a decrease of $7.9 million. The decrease in selling, general and administrative expenses primarily reflects the following:

- $7.5 million decrease in professional service expenses primarily due to a decrease in reliance on third-party selling, general and administrative contractors;
- $3.6 million decrease in stock-based compensation expense primarily due to changes in headcount and stock prices; and
- $2.5 million increase in compensation and other personnel expenses primarily due to changes in headcount.

Selling, general and administrative expenses were approximately $282.7 million for the twelve months ended December 31, 2021, compared to $317.9 million for the same period in 2020, a decrease of $35.2 million. The decrease in selling, general and administrative expenses primarily reflects

the following:

- $33.0 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 2020, with no similar activity incurred during 2021;
- $3.0 million decrease in stock-based compensation expense primarily due to changes in headcount and stock price;
- $1.7 million decrease in compensation and other personnel expenses primarily due to changes in headcount; and
- $2.5 million increase in facility- and technology-related expense primarily due to the Company's continuing expansion efforts.

Non-GAAP selling, general and administrative expenses were $60.1 million and $65.2 million for the three months ended December 31, 2021 and 2020, respectively, a decrease of $5.1 million. Non-GAAP selling, general and administrative expenses were $209.2 million and $243.3 million for the twelve months ended December 31, 2021 and 2020, respectively, a decrease of $34.1 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 December 31, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For each of the twelve months ended December 31, 2021 and 2020, the Company recorded amortization of in-licensed rights of approximately $0.7 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 in July 2017 and April 2013, respectively.

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 twelve months ended December 31, 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 December 31, 2021 and 2020, other expense, net was $16.1 million and $17.8 million, respectively. For the twelve months ended December 31, 2021 and 2020, other expense, net was $68.4 million and $52.0 million, respectively. The quarter-over-quarter decrease primarily reflects a reduction of interest expense incurred on the Company's convertible debt related to the adoption of ASU 2020-06. The year-over-year increase primarily reflects an increase in non-cash 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 $2.1 billion in cash, cash equivalents and investments as of December 31, 2021, compared to $1.9 billion as of December 31, 2020. The increase is primarily driven by proceeds received from the October 2021 equity offering, offset 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 expense (benefit), depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined by the Company as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by the Company as GAAP selling, general and administrative expenses excluding depreciation and amortization expense, stock-based compensation expense and other items.

1. Interest, tax, depreciation and amortization
Interest expense, net amounts can vary substantially from period to period due to changes in cash and debt balances and interest rates driven by market conditions outside of the Company's operations. Tax amounts can vary substantially from period to period due to tax adjustments that are not directly related to underlying operating performance. Depreciation expense can vary substantially from period to period as the purchases of property and equipment may vary significantly from period to period and without any direct correlation to the Company's operating performance. Amortization expense primarily associated with in-licensed rights as well as patent costs are amortized over a period of several years after acquisition or patent application or renewal and generally cannot be changed or influenced by management.

2. Stock-based compensation expenses

Stock-based compensation expenses represent non-cash charges related to equity awards granted by the Company. Although these are recurring charges to operations, the Company believes the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within the Company's control. Therefore, the Company believes that excluding these charges facilitates comparisons of the Company's operational performance in different periods.

3. Other items
The Company evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to the Company's ongoing business operations, and (c) whether the Company expects the items to continue on a regular basis. These other items include gain from sale of PRV, impairment of equity investment and net gain (loss) on contingent consideration.

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

- The Company excludes from its non-GAAP results 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.

Beginning in the fourth quarter of 2021, up-front and milestone payments associated with the Company's license and collaboration agreements, settlement and license charges and collaboration revenue, along with the related transaction costs incurred, are no longer excluded from the non-GAAP expenses and income.

The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. The Company also believes these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating the Company's performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of the Company's financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP other income and loss adjustments, non-GAAP income tax expense (benefit), non-GAAP net loss, and non-GAAP basic and diluted net loss per share may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table "Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures."

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

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

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

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

Important Safety Information About EXONDYS 51
Hypersensitivity reactions, bronchospasm, chest pain, cough, tachycardia, and urticaria have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

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

The following adverse reactions have been identified during observational studies that were conducted as part of the clinical development program and continued post approval.

In open-label observational studies, 163 patients received at least one intravenous dose of EXONDYS 51, with doses ranging between 0.5 mg/kg (0.017 times the recommended dosage) and 50 mg/kg (1.7 times the recommended dosage). All patients were male and had genetically confirmed Duchenne muscular dystrophy. Age at study entry was 6 months to 19 years. Most (85%) patients were Caucasian.

The most common adverse reactions from observational clinical studies (N=163) seen in greater than 10% of the study population were headache, cough, rash, and vomiting.

For further information, please see the full Prescribing Information.

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

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

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

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

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

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

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

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

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

For further information, please see the full Prescribing Information.

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

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

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

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

Important Safety Information for AMONDYS 45
Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in Duchenne patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

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

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

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

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

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

Forward-Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs and technologies; our plan to share additional results for SRP-9001-102 at a future medical congress; the rights and obligations of the parties to, and potential benefits of, the collaboration and option agreement with GenEdit, including the potential for GenEdit's NanoGalaxy platform and Sarepta's gene editing technology to develop up to four neuromuscular indications selected by Sarepta and the potential of GenEdit's polymer nanoparticles to deliver therapeutic cargo to specific muscle tissue after systemic administration to allow for targeted, non-viral systemic delivery of genetic medicines.

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, 2021 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,	
	2021	**2020**	**2021**	**2020**
Revenues:				
Products, net	$ 178,725	$ 122,644	$ 612,401	$ 455,865
Collaboration and other	22,736	22,494	89,486	84,234
Total revenues	201,461	145,138	701,887	540,099
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	31,744	22,404	97,049	63,382
Research and development	197,296	207,239	771,182	722,343
Selling, general and administrative	78,055	86,046	282,660	317,875
Settlement and license charges	—	—	10,000	—
Amortization of in-licensed rights	179	165	706	662
Total cost and expenses	307,274	315,854	1,161,597	1,104,262
Operating loss	(105,813)	(170,716)	(459,710)	(564,163)
Other (loss) income:				
Other expense, net	(16,076)	(17,769)	(68,438)	(51,971)
Gain from sale of Priority Review Voucher	—	—	102,000	108,069
Gain (loss) on contingent consideration, net	—	—	7,200	(45,000)
Total other (loss) income	(16,076)	(17,769)	40,762	11,098
Loss before income tax expense (benefit)	(121,889)	(188,485)	(418,948)	(553,065)
Income tax expense (benefit)	92	832	(168)	1,063
Net loss	$ (121,981)	$ (189,317)	$ (418,780)	$ (554,128)

Net loss per share - basic and diluted	$ (1.42)	$ (2.40)	$ (5.15)	$ (7.11)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	85,951	78,905	81,262	77,956

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,	
	2021	**2020**	**2021**	**2020**
GAAP net loss	$ (121,981)	$ (189,317)	$ (418,780)	$ (554,128)
Interest expense, net	15,953	18,446	63,014	52,488
Income tax expense (benefit)	92	832	(168)	1,063
Depreciation and amortization expense	10,145	7,288	38,017	26,911
Stock-based compensation expense	29,782	29,527	113,943	108,070
Gain from sale of Priority Review Voucher	—	—	(102,000)	(108,069)
(Gain) loss on contingent consideration, net	—	—	(7,200)	45,000
Impairment of equity investment	—	—	4,488	—
Non-GAAP net loss*	$ (66,009)	$ (133,224)	$ (308,686)	$ (428,665)
Non-GAAP net loss per share:				
Basic and diluted	$ (0.77)	$ (1.69)	$ (3.80)	$ (5.50)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	85,951	78,905	81,262	77,956

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2021	**2020**	**2021**	**2020**
GAAP research and development expenses	$ 197,296	$ 207,239	$ 771,182	$ 722,343
Stock-based compensation expense	(14,509)	(10,637)	(50,526)	(41,671)
Depreciation and amortization expense	(7,250)	(5,162)	(27,293)	(18,054)
Non-GAAP research and development expenses	$ 175,537	$ 191,440	$ 693,363	$ 662,618

	For the Three Months Ended December 31,		For the Twelve Months Ended December 31,	
	2021	**2020**	**2021**	**2020**
GAAP selling, general and administrative expenses	$ 78,055	$ 86,046	$ 282,660	$ 317,875
Stock-based compensation expense	(15,273)	(18,890)	(63,417)	(66,399)
Depreciation and amortization expense	(2,716)	(1,961)	(10,018)	(8,195)
Non-GAAP selling, general and administrative expenses	$ 60,066	$ 65,195	$ 209,225	$ 243,281

* Beginning in the fourth quarter of 2021, up-front and milestone payments associated with the Company's license and collaboration agreements, settlement and license charges and collaboration revenue, along with the related transaction costs incurred, are no longer excluded from the non-GAAP expenses and income. Non-GAAP financial results for the fourth quarter and full-year 2020 have been updated to reflect this change for comparable purposes.

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

	As of December 31, 2021	As of December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 2,115,869	$ 1,502,648
Short-term investments	—	435,923
Accounts receivable	152,990	101,340
Inventory	186,212	231,961
Other current assets	149,028	213,324
Total current assets	2,604,099	2,485,196
Property and equipment, net	191,156	190,430
Intangible assets, net	14,239	13,628
Right of use assets	45,531	91,761
Other non-current assets	292,949	203,703
Total assets	$ 3,147,974	$ 2,984,718
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 76,741	$ 111,090
Accrued expenses	271,697	193,553
Deferred revenue, current portion	89,244	89,244
Other current liabilities	15,051	22,139
Total current liabilities	452,733	416,026
Long-term debt	1,096,876	992,493
Lease liabilities, net of current portion	41,512	80,367
Deferred revenue, net of current portion	574,244	663,488
Contingent consideration	43,600	50,800
Other non-current liabilities	11,000	19,785
Total liabilities	2,219,965	2,222,959
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 87,126,974 and 79,374,247 issued and outstanding at December 31, 2021 and 2020, respectively	9	8
Additional paid-in capital	4,134,768	3,609,877
Accumulated other comprehensive (loss) income, net of tax	(20)	3
Accumulated deficit	(3,206,748)	(2,848,129)
Total stockholders' equity	928,009	761,759
Total liabilities and stockholders' equity	$ 3,147,974	$ 2,984,718

Source: Sarepta Therapeutics, Inc.

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