Sarepta Therapeutics Announces Third Quarter 2020 Financial Results and Recent Corporate Developments

11/5/20

– Net product sales of $121.4 million, a 23% increase over same quarter of prior year –

CAMBRIDGE, Mass., Nov. 05, 2020 (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 2020.

"Even as the pandemic created challenges throughout the third quarter, I am pleased to report that the Sarepta team was able to continue to serve the Duchenne muscular dystrophy community with little interruption, achieving net product revenue of $121.4 million for EXONDYS 51 (eteplirsen) and VYONDYS 53 (golodirsen), representing a 23% increase over the same quarter last year," said Doug Ingram, Sarepta's president and CEO. "At the same time, we advanced our RNA pipeline with a filing of and the FDA's acceptance of the NDA for our next PMO candidate, casimersen, and prepared our next-generation candidate, the peptide-conjugated PMO, SRP-5051 for a data readout by the end of 2020. We also announced, at the World Muscle Society congress, positive data for SRP-9001 and SRP-9003, two critical drivers of our first-in-class gene therapy engine, and in support of these programs continued to build our strength in manufacturing. As we reported in a separate release today, the FDA has agreed on an approach for a potency assay which should permit us to dose patients with commercial process material in Study SRP-9001-103, an important milestone in our goal of bringing a potentially profound therapy to a waiting Duchenne community."

Third Quarter 2020 and Recent Corporate Developments:

- **Announced positive 2-year functional data of SRP-9001 micro-dystrophin gene therapy in Duchenne muscular dystrophy (DMD) patients:** The clinical results, presented at the 25th International Annual Congress of the World Muscle Society (WMS), demonstrated that two years after a one-time infusion of SRP-9001, trial participants exhibited a mean 7.0 point improvement on the North Star Ambulatory Assessment (NSAA) compared to baseline (at one year post treatment the mean increase was 5.5 points from baseline). NSAA is a validated scale developed to measure functional motor abilities in ambulant children with DMD. The data were generated from four ambulatory participants, ages 4 to 7, in Sarepta's open-label trial, Study 101, and showed continued safety and tolerability of a one-time infusion of SRP-9001 at a dose of $2x10^{14}$ vg/kg. All adverse events were considered mild or moderate, and occurred within 90 days of treatment. There were no serious adverse events or evidence of complement activation.

- **Reported positive results for SRP-9003, an investigational gene therapy for limb-girdle muscular dystrophy type 2E (LGMD2E):** Results, presented at WMS, included 18-month functional data from three clinical trial participants in the low-dose cohort and 6-month functional data from three participants in the high-dose cohort.

  Cohort 1 (low dose) at 18 months:

  - All three participants continued to show improvements from baseline across all functional measures, including the North Star Assessment for Dysferlinopathies (NSAD), time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test.
  - The mean NSAD improvement from baseline was 3.0 points at 6 months and 5.7 points at 18 months.
  - There have been no new drug-related safety signals observed since the one-year update in June 2020, and no decreases in platelet counts outside of the normal range or signs of complement activation were observed.

  Cohort 2 (high dose) at 6 months:

  - All three participants demonstrated improvements from baseline across all functional measures, including the NSAD, time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test.
  - The mean NSAD improvement from baseline was 3.7 points.
  - There have been no new drug-related safety signals observed since expression results were shared in June 2020, and no decreases in platelet counts outside of the normal range or signs of complement activation were observed.

- **U.S. Food and Drug Administration (FDA) accepted casimersen (SRP-4045) new drug application (NDA) for patients with DMD amenable to skipping exon 45; grants Priority Review Status:** The FDA accepted Sarepta's NDA seeking accelerated approval for casimersen (SRP-4045); regulatory action date set for February 25, 2021. FDA also granted priority review status for casimersen. The FDA has indicated it does not currently plan to hold an advisory committee meeting to discuss the application. Sarepta also received FDA's conditional approval of AMONDYS 45™ as the

brand name for casimersen, a phosphorodiamidate morpholino oligomer (PMO) engineered to treat patients with DMD who have genetic mutations that are amenable to skipping exon 45 of the dystrophin gene.

- **Invested $15.0 million in AavantiBio, Inc.:** Sarepta, along with Perceptive Advisors, Bain Capital Life Sciences, and RA Capital Management participated in a $107.0 million Series A financing to fund AavantiBio, a gene therapy company focused on Friedrich's Ataxia. Bo Combo, most recently Sarepta's Chief Commercial Officer and Executive Vice President, was appointed Chief Executive Officer and President of AavantiBio, which was co-founded by renowned gene therapy researchers Barry Byrne, M.D., Ph.D., and Manuela Corti, P.T., Ph.D., both from the University of Florida's Powell Gene Therapy Center.

- **Announced collaboration with University of Florida (UF) to accelerate the discovery and development of therapies for rare genetic diseases:** Sarepta will fund multiple research programs at UF and will have an exclusive option to further develop any new therapeutic compounds that result from the funded research programs. Funding has been allocated for four innovative projects, including: exploratory research in novel gene therapy vectors, next-generation capsids and gene editing technologies, as well as work in new therapeutic areas in degenerative genetic diseases.

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 9452027. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2020 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

Financial Results

On a GAAP basis, for the three months ended September 30, 2020 and 2019, the Company reported a net loss of $196.5 million and $126.3 million, or $2.50 and $1.70 per basic and diluted share, respectively. On a non-GAAP basis, the net loss for the three months ended September 30, 2020 and 2019 was $111.5 million and $84.4 million, or $1.42 and $1.14 per basic and diluted share, respectively.

On a GAAP basis, for the nine months ended September 30, 2020 and 2019, the Company reported a net loss of $364.8 million and $479.4 million, or $4.70 and $6.54 per basic and diluted share, respectively. On a non-GAAP basis, the net loss for the nine months ended September 30, 2020 and 2019 was $309.2 million and $199.4 million, or $3.98 and $2.72 per basic and diluted share, respectively.

Revenues

For the three months ended September 30, 2020, the Company recorded net product revenues of $121.4 million, compared to net product revenues of $99.0 million for the same period of 2019, an increase of $22.4 million. For the nine months ended September 30, 2020, the Company recorded net product revenues of $333.2 million, compared to net product revenues of $280.7 million for nine months ended September 30, 2019, an increase of $52.5 million. The increase primarily reflects the continuing increase in demand for the Company's products in the U.S.

For the three and nine months ended September 30, 2020, the Company recognized $22.5 million and $61.7 million of collaboration revenue, respectively, which primarily relates to the Company's collaboration arrangement with F. Hoffman-La Roche Ltd. ("Roche"). In February 2020, the Company received an aggregate of approximately $1.2 billion in cash consideration from Roche, consisting of an up-front payment and an equity investment in the Company. Of that amount, $348.7 million is being recognized as revenue on a straight-line basis over the performance period, estimated to be through the fourth quarter of 2023.

Cost and Operating Expenses

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

For the three months ended September 30, 2020, cost of sales (excluding amortization of in-licensed rights) was $15.0 million, compared to $13.0 million for the same period of 2019, an increase of $2.0 million. For the nine months ended September 30, 2020 and 2019, cost of sales (excluding amortization of in-licensed rights) was approximately $41.0 million. The increase in cost of sales for the three months ended September 30, 2020 is primarily due to increasing demand for the Company's products, partially offset by write-offs of certain batches of EXONDYS 51 not meeting the Company's quality specifications for the three months ended September 30, 2019, with no similar activity for the three months ended September 30, 2020 and an increase in royalty payments to BioMarin Pharmaceutical ("BioMarin") and University of Western Australia ("UWA") as a result of increasing demand for the Company's products.

Research and development

Research and development expenses were $190.4 million for the three months ended September 30, 2020, compared to $133.9 million for the same period of 2019, an increase of $56.5 million. The increase in research and development expenses primarily reflects the following:

- $42.5 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;

- $11.2 million increase in clinical trial expenses primarily due to increased patient enrollment for the Company's ESSENCE program as well as certain start-up activities for the Company's micro-dystrophin program;

- $5.6 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene on

its MPS IIIA drug candidate;

- $4.2 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $3.7 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $3.2 million increase in up-front, milestone and other 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 $10.0 million of similar activity during the third quarter of 2019;
- $2.2 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts;
- $1.0 million increase in pre-clinical expenses primarily due to an increase of toxicology studies in the Company's gene therapy platforms during the third quarter of 2020, offset by the completion of certain toxicology studies in the Company's PPMO platform;
- $2.1 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors as a result of an increase in hiring and headcount; and
- $16.7 million offset to expense incurred in the third quarter of 2020 associated with a collaboration reimbursement from Roche.

Research and development expenses were $515.1 million for the nine months ended September 30, 2020, compared to $337.8 million for the same period of 2019, an increase of $177.3 million. The increase in research and development expenses primarily reflects the following:

- $165.8 million increase in manufacturing expenses primarily due to a continuing ramp-up of the Company's gene therapy programs;
- $15.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $12.1 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $9.5 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene on its MPS IIIA drug candidate;
- $8.3 million increase in up-front, milestone and other expenses primarily due to $9.3 million of milestone expense related to payments accrued to an academic institution and $27.1 million of up-front payments as a result of the execution of certain research, option and license agreements during the nine months ended September 30, 2020, offset primarily by $25.5 million of up-front payments as a result of license agreements executed during the same period of 2019;
- $6.3 million increase in clinical trial expenses primarily due to increased patient enrollment for the Company's ESSENCE program as well as certain start-up activities for the Company's micro-dystrophin program;
- $6.2 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts;
- $2.7 million increase in research and other primarily driven by an increase in sponsored research with academic institutions during the nine months ended September 30, 2020;
- $3.2 million decrease in professional service expenses primarily due to a decrease in reliance on third-party research and development contractors as a result of an increase in hiring and headcount;
- $3.8 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company's PPMO platform, offset by an increase of toxicology studies in the Company's gene therapy platforms; and
- $41.9 million offset to expense associated with a collaboration reimbursement from Roche during the nine months ended September 30, 2020.

Non-GAAP research and development expenses were $159.9 million and $110.5 million for the three months ended September 30, 2020 and 2019, respectively, an increase of $49.4 million. Non-GAAP research and development expenses were $434.5 million and $279.4 million for the nine months ended September 30, 2020 and 2019, respectively, an increase of $155.1 million.

Selling, general and administration

Selling general and administrative expenses were approximately $75.4 million for both the three months ended September 30, 2020 and 2019. The changes in selling, general and administrative expenses primarily reflect the following:

- $3.0 million decrease in professional services primarily due to a decrease in reliance on third-party contractors as a result of an increase in hiring and headcount; and
- $2.6 million increase in stock-based compensation primarily due to increases in headcount and stock price.

Selling general and administrative expenses were $231.8 million for the nine months ended September 30, 2020, compared to $203.4 million for the same period of 2019, an increase of $28.4 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $11.3 million increase in professional services primarily due to a transaction fee for the Roche transaction;
- $10.0 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- $2.8 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $1.3 million increase in facility- and technology-related expense primarily due to continuing global expansion.

Non-GAAP selling, general and administrative expenses were $57.2 million and $59.6 million for the three months ended September 30, 2020 and 2019, respectively, a decrease of $2.4 million. Non-GAAP selling, general and administrative expenses were $166.8 million and $159.7 million for the nine months ended September 30, 2020 and 2019, respectively, an increase of $7.1 million.

Acquired in-process research and development

As a result of the Myonexus acquisition, the Company recorded acquired in-process research and development expense of approximately $173.2 million during the nine months ended September 30, 2019. There was no such transaction during the nine months ended September 30, 2020.

Amortization of in-licensed rights

For both the three months ended September 30, 2020 and 2019, the Company recorded amortization of in-licensed rights of approximately $0.2 million. For the nine months ended September 30, 2020 and 2019, the Company recorded amortization of in-licensed rights of approximately $0.5 million and $0.6 million, respectively. This is related to the amortization of the in-licensed right assets recognized as a result of agreements the Company entered into with BioMarin and UWA upon the first commercial sale of EXONDYS 51 and VYONDYS 53.

Gain from Sale of Priority Review Voucher

In February 2020, the Company entered into an agreement with Vifor (International) Ltd. to sell the rare pediatric disease Priority Review Voucher (“PRV”) it received from the FDA in connection with the approval of VYONDYS 53. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2020, the Company completed its sale of the PRV and received proceeds of $108.1 million, net of commission, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale. There was no similar activity during the nine months ended September 30, 2019.

Loss on contingent consideration

The loss on contingent consideration relates to the fair value adjustment of the Company’s contingent consideration liability related to regulatory-related contingent payments to Myonexus selling shareholders as well as to an academic institution under separate license agreements that meet the definition of a derivative. During the three months ended September 30, 2020, the Company recognized $45.0 million of expense to adjust the fair value of the contingent consideration based on the most current assumptions relating to the achievement of the milestone. There was no similar activity during the three or nine months ended September 30, 2019.

Other expense, net

For the three months and nine months ended September 30, 2020, other expense, net was approximately $14.3 million and $34.2 million, respectively. For the three and nine months ended September 30, 2019, other expense, net was approximately $2.5 million and $3.5 million, respectively. The increase primarily reflects the interest expense on the Company’s debt facility entered into in December 2019 as well as a decrease in interest income and the amortization of investment discounts due to the investment mix of the Company’s investment portfolio.

Cash, Cash Equivalents, Investments and Restricted Cash and Investments

The Company had approximately $1.8 billion in cash, cash equivalents and investments as of September 30, 2020 compared to $1.1 billion as of December 31, 2019. The increase is primarily driven by the $1.2 billion up-front payments received from the Roche as a result of the execution of the collaboration and equity investment agreements offset by cash used to fund the Company’s ongoing operations during 2020.

Use of Non-GAAP Measures

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements. The non-GAAP loss is defined by the Company as GAAP net loss excluding interest expense/(income), 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 income and expense 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 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 Sarepta. Although these are recurring charges to operations, the Company believes the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within the Company's control. Therefore, the Company believes that excluding these charges facilitates comparisons of the Company's operational performance in different periods.

3. Other items

The Company evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to the Company's ongoing business operations, and (c) whether the Company expects the items to continue on a regular basis. These other items include collaboration revenue and transaction cost related to the Roche transaction, up-front and milestone payments, acquired in-process research and development expense, gain from sale of PRV and loss on contingent consideration.

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

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

As a result of the Myonexus acquisition, the Company recorded acquired in-process research and development expense, which represents a non-recurring expense and, therefore, was treated as a non-GAAP adjustment item. The Company believes the presentation of adjusted research and development, which does not include license- and collaboration-related up-front and milestone expenses, provides useful and meaningful information about its ongoing research and development activities by enhancing investors' understanding of the Company's normal, recurring operating research and development expenses and facilitates comparisons between periods and with respect to projected performance.

The sale of the PRV obtained as a result of the FDA approval of VYONDYS 53 in December 2019 is a non-recurring event and excluded from the Company's non-GAAP results.

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

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

About EXONDYS 51

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

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

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

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

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

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

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

For further information, please see the full Prescribing Information.

About VYONDYS 53

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

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

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

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

Important Safety Information for VYONDYS 53

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

Renal toxicity was observed in animals who received golodirsen. Although renal toxicity was not observed in the clinical studies with VYONDYS 53, renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Renal 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 renal function in DMD patients. Measurement of glomerular filtration rate (GFR) by 24-hour urine collection prior to initiation of therapy is recommended. Monthly monitoring for proteinuria by dipstick urinalysis and monitoring of serum cystatin C every three months is recommended. In the case of a confirmed dipstick proteinuria of 2+ or greater or elevated serum cystatin C, a 24-hour urine collection to quantify proteinuria and assess GFR should be performed.

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 Sarepta Therapeutics

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Forward-Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs including the potential of casimersen to treat patients with DMD who have genetic mutations that are amenable to skipping exon 45 of the Duchenne gene; the expected regulatory action date of February 25, 2021 for casimersen; the expectation that the FDA will not hold an advisory committee to discuss the casimersen application; the potential for the collaboration with UF to accelerate the discovery and development of therapies for rare genetic diseases; our goal of bringing a potentially profound therapy to the DMD community; and expected plans and milestones, including the expectation of a data readout for SRP-5051 by the end of 2020 and our plan to commence dosing patients with commercial process material in Study SRP-9001-103.

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; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; 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, 2019 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.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

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,	
	2020	2019	2020	2019
Revenues:				
Products, net	$ 121,429	$ 99,041	$ 333,221	$ 280,720
Collaboration	22,495	—	61,740	—
Total revenues	143,924	99,041	394,961	280,720
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	15,015	13,037	40,978	41,019
Research and development	190,438	133,949	515,104	337,768
Selling, general and administrative	75,373	75,429	231,829	203,388
Acquired in-process research and development	—	—	—	173,240
Amortization of in-licensed rights	166	216	497	649
Total cost and expenses	280,992	222,631	788,408	756,064
Operating loss	(137,068)	(123,590)	(393,447)	(475,344)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	108,069	—
Loss on contingent consideration	(45,000)	—	(45,000)	—
Other expense, net	(14,335)	(2,510)	(34,202)	(3,544)
Total other (loss) income	(59,335)	(2,510)	28,867	(3,544)
Loss before income tax expense	(196,403)	(126,100)	(364,580)	(478,888)
Income tax expense	96	226	231	484
Net loss	$ (196,499)	$ (126,326)	$ (364,811)	$ (479,372)
Net loss per share - basic and diluted	$ (2.50)	$ (1.70)	$ (4.70)	$ (6.54)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	78,501	74,177	77,637	73,298

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,	
	2020	2019	2020	2019
GAAP net loss	$ (196,499)	$ (126,326)	$ (364,811)	$ (479,372)
Interest expense, net	13,454	2,136	34,042	3,519
Income tax expense	96	226	231	484
Gain from sale of Priority Review Voucher	—	—	(108,069)	—
Loss on contingent consideration	45,000	—	45,000	—
Collaboration revenue	(22,495)	—	(61,740)	—
Depreciation and amortization expense	6,619	6,740	19,623	17,853
Stock-based compensation expense	26,903	20,637	78,543	56,538
Roche transaction costs	—	—	11,292	—
Up-front, milestone, and other expenses	15,375	12,146	36,658	28,346
Acquired in-process research and development	—	—	—	173,240
Non-GAAP net loss	$ (111,547)	$ (84,441)	$ (309,231)	$ (199,392)
Non-GAAP net loss per share:				
Basic and diluted	$ (1.42)	$ (1.14)	$ (3.98)	$ (2.72)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	78,501	74,177	77,637	73,298

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2020	2019	2020	2019
GAAP research and development expenses	$ 190,438	$ 133,949	$ 515,104	$ 337,768
Up-front, milestone, and other expenses	(15,375)	(12,146)	(36,658)	(28,346)
Stock-based compensation expense	(10,645)	(6,972)	(31,034)	(18,982)
Depreciation and amortization expense	(4,516)	(4,364)	(12,892)	(11,051)
Non-GAAP research and development expenses	$ 159,902	$ 110,467	$ 434,520	$ 279,389

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2020	2019	2020	2019
GAAP selling, general and administrative expenses	$ 75,373	$ 75,429	$ 231,829	$ 203,388
Stock-based compensation expense	(16,258)	(13,665)	(47,509)	(37,556)
Depreciation and amortization expense	(1,937)	(2,160)	(6,234)	(6,153)
Roche transaction costs	—	—	(11,292)	—
Non-GAAP selling, general and administrative expenses	$ 57,178	$ 59,604	$ 166,794	$ 159,679

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

	As of September 30, 2020	As of December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 1,474,637	$ 835,080
Short-term investments	341,467	289,668
Accounts receivable	121,827	90,879
Inventory	220,118	171,379
Other current assets	164,624	81,907
Total current assets	2,322,673	1,468,913
Property and equipment, net	171,715	129,620
Intangible assets, net	13,344	12,497
Right of use assets	71,085	37,933

Other non-current assets	201,847	173,859
Total assets	$ 2,780,664	$ 1,822,822
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 74,528	$ 68,094
Accrued expenses	199,063	185,527
Deferred revenue, current portion	89,244	3,303
Other current liabilities	15,553	7,843
Total current liabilities	378,388	264,767
Long-term debt	700,470	681,900
Lease liabilities	68,229	47,720
Deferred revenue, net of current portion	685,982	—
Contingent consideration	50,500	5,200
Other non-current liabilities	5,048	5,048
Total liabilities	1,888,617	1,004,635
Commitments and contingencies		
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; 78,789,760 and 75,184,863 issued and outstanding at September 30, 2020 and December 31, 2019, respectively	8	8
Additional paid-in capital	3,550,857	3,112,130
Accumulated other comprehensive (loss) income, net of tax	(6)	50
Accumulated deficit	(2,658,812)	(2,294,001)
Total stockholders' equity	892,047	818,187
Total liabilities and stockholders' equity	$ 2,780,664	$ 1,822,822

Source: Sarepta Therapeutics, Inc.

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