Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2019 Financial Results and Recent Corporate Developments

2/26/20

CAMBRIDGE, Mass., Feb. 26, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the three and twelve months ended December 31, 2019.

"The Sarepta team made great progress in service of our vision to be one of the world's most meaningful leaders in precision genetic medicine in 2019, and profoundly so in the fourth quarter," stated Doug Ingram, Sarepta's President and Chief Executive Officer. "We continued to serve the Duchenne community with EXONDYS 51, achieving revenue of $100 million in the fourth quarter and $381 million for full year 2019, a 26% increase over prior year. With our RNA platform, we also received approval and launched our second RNA therapy, VYONDYS 53, advanced our third RNA therapy, casimersen, to a rolling submission, and continued our multi-ascending dose study for our next-generation RNA technology, PPMO."

Mr. Ingram continued, "We progressed our gene therapy programs such as LGMD2E and MPS IIIA, and significantly advanced our lead gene therapy program, SRP-9001, intended to use micro-dystrophin to treat Duchenne muscular dystrophy. We have now dosed all patients in our blinded, placebo-controlled trial, Study 102, and are preparing to commence Study 301, our next placebo-controlled trial using commercial process material. And importantly, in the fourth quarter we entered into our alliance with Roche, which not only gives us a very strong balance sheet to execute our plans but also, assuming the success of SRP-9001, accelerates our mission to bring a potentially life-enhancing therapy to Duchenne patients around the world."

Fourth Quarter 2019 and Recent Corporate Developments:

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

- **Closed Licensing Transaction with Roche in Territories Outside the United States for Investigational Micro-dystrophin Gene Therapy for Duchenne Muscular Dystrophy (DMD), SRP-9001:** On February 14, 2020, Sarepta and Roche closed the License, Collaboration, and Option Agreement (the "Collaboration Agreement") and the Stock Purchase Agreement announced on December 23, 2019. The Collaboration Agreement provides Roche exclusive commercial rights to SRP-9001 (AAVrh74.MHCK7.micro-dystrophin), Sarepta's investigational gene therapy for DMD, outside the U.S. At closing, Sarepta received $1.2 billion in an up-front payment comprised of $750.0 million in cash and approximately $400.0 million in exchange for Sarepta stock priced at $158.29 per share of common stock. The Company is also eligible to receive up to $1.7 billion in regulatory and sales milestones plus royalties on net sales, anticipated to be in the mid-teens. In addition, Roche and Sarepta will equally share joint global development expenses. Sarepta retains all rights to SRP-9001 in the United States. The collaboration combines Sarepta's leading gene therapy candidate for DMD with Roche's global reach, commercial presence and regulatory expertise to accelerate access to SRP-9001 for patients outside the U.S. As part of the agreement, the Company will continue to be responsible for performing the joint global development plan and manufacturing build out for SRP-9001. Through its leading hybrid manufacturing platform, Sarepta will remain responsible for manufacturing of clinical and commercial supplies. The Company has also granted Roche an option to acquire ex-U.S. rights to certain future programs in DMD, in exchange for separate, up-front milestone and royalty considerations, and cost sharing.

- **Appointed Biotech Executive John C. Martin, Ph.D. to Board of Directors:** Dr. Martin brings decades of executive leadership to Sarepta's board, having played an instrumental role in building Gilead Sciences into one of the world's foremost biotechnology companies. During his 20-year tenure as chief executive officer at Gilead, he oversaw the growth of the company and development of its scientific portfolio into 24 marketed products.

- **Announced Multi-target Strategic Collaboration with StrideBio to Advance Novel Gene Therapies:** Sarepta and StrideBio Inc. ("StrideBio") signed a collaboration and license agreement to develop *in vivo* AAV-based therapies for up to

eight central nervous system (CNS) and neuromuscular targets. Pursuant to the agreement, Sarepta was granted an exclusive license on selected targets to leverage StrideBio's novel, structure-driven capsid technology, intended to enhance specific tropism to tissues of interest and evade neutralizing antibodies. The parties also plan to focus on strategies intended to address re-dosing challenges in patients who have received AAV-delivered gene therapy. StrideBio will conduct all investigational new drug enabling research, development and manufacturing for the first four CNS targets, which are MECP2 (Rett syndrome), SCN1A (Dravet syndrome), UBE3A (Angelman syndrome), and NPC1 (Niemann-Pick). Additionally, Sarepta has an exclusive option to four additional targets based on StrideBio's capsid technology.

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 4749068. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 2019 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

Financial Results

On a GAAP basis, the Company reported a net loss of $235.7 million and $140.9 million, or $3.16 and $2.05 per basic and diluted share for the fourth quarter of 2019 and 2018, respectively. On a non-GAAP basis, the net loss for the fourth quarter of 2019 was $116.9 million, or $1.57 per basic and diluted share, compared to a net loss of $58.7 million, or $0.85 per basic and diluted share for the same period of 2018.

On a GAAP basis, for the twelve months ended December 31, 2019, the Company reported a net loss of $715.1 million, or $9.71 per basic and diluted share, compared to a net loss of $361.9 million, or $5.46 per basic and diluted share for the same period of 2018. On a non-GAAP basis, the net loss for the twelve months ended December 31, 2019 was $316.3 million, or $4.30 per basic and diluted share, compared to a net loss of $141.7 million, or $2.14 per basic and diluted share for the same period of 2018.

Net Revenues

For the three months ended December 31, 2019, the Company recorded net revenues of $100.1 million, compared to net revenues of $84.4 million for the same period of 2018, an increase of $15.7 million. For the twelve months ended December 31, 2019, the Company recorded net revenues of $380.8 million, compared to net revenues of $301.0 million for the same period of 2018, an increase of $79.8 million. The increases primarily reflect the continuing increase in demand for EXONDYS 51 in the U.S.

Cost and Operating Expenses

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

For the fourth quarter of 2019, cost of sales (excluding amortization of in-licensed rights) was $15.6 million, compared to $13.1 million for the same period of 2018, an increase of $2.5 million. For the twelve months ended December 31, 2019, cost of sales (excluding amortization of in-licensed rights) was $56.6 million, compared to $34.2 million for the same period of 2018, an increase of $22.4 million. The increases primarily reflect royalty payments to BioMarin Pharmaceuticals ("BioMarin") and University of Western Australia ("UWA"), and higher product costs as a result of increasing demand for EXONDYS 51.

Research and development

Research and development expenses were $223.1 million for the fourth quarter of 2019, compared to $146.2 million for the same period of 2018, an increase of $76.9 million. The increase in research and development expenses primarily reflects the following:

- $40.0 million increase in clinical and manufacturing expenses primarily due to a ramp-up of manufacturing activities for the Company's gene therapy programs (including its micro-dystrophin program), increased patient enrollment in the Company's ongoing ESSENCE trial, initiation of certain post-market studies for EXONDYS 51 and its PPMO platform. These increases were partially offset by a ramp-down of clinical trials in EXONDYS 51, including the PROMOVI trial, and the Phase 1/2 trial in VYONDYS 53;

- $10.8 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;

- $10.4 million increase in up-front, milestone, and other expenses, primarily due to $46.9 million up-front payment to StrideBio as a result of the execution of a collaboration and license agreement in November 2019, $28.0 million up-front payment to Genethon as a result of the execution of a collaboration agreement with Genethon in November 2019, as compared to $44.8 million up-front and milestone payments to Lysogene S.A. ("Lysogene") as a result of the execution of the collaboration and license agreement with Lysogene in October 2018 as well as certain development milestones becoming probable of being achieved and $15.0 million milestone payments to Myonexus Therapeutics, Inc. ("Myonexus") as a result of certain development milestones being achieved or becoming probable of being achieved;

- $8.0 million increase in collaboration cost-sharing expense primarily driven by collaboration cost sharing with Genethon on its microdystrophin platform;

- $7.1 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts as well as a change in methodology in allocation of technology expense;

- $4.9 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;

- $0.8 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of the Company's research and development pipeline;

- $0.7 million increase in research and other primarily driven by an increase in lab supplies as a result of an increase in headcount as well as sponsored research with academic institutions; and

- $5.9 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company's PPMO platform.

Research and development expenses were $560.9 million for the twelve months ended December 31, 2019, compared to $401.8 million for the same period of 2018, an increase of $159.1 million. The increase in research and development expenses primarily reflects the following:

- $111.1 million increase in clinical and manufacturing expenses primarily due to a ramp-up of manufacturing activities for the Company's gene therapy programs (including its micro-dystrophin program), increased patient enrollment in the Company's ESSENCE trial, and initiation of certain post-market studies for EXONDYS 51. These increases were partially offset by a ramp-down of clinical trials in EXONDYS 51, including the PROMOVI trial and the Phase 1/2 trial in VYONDYS 53;

- $39.9 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;

- $30.0 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts as well as a change in methodology in allocation of technology expense;

- $13.5 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;

- $9.2 million increase in research and other primarily driven by a $7.1 million increase in lab supplies as a result of an increase in headcount as well as a $3.0 million increase in sponsored research with academic institutions, partially offset by a reduction of $3.8 million in loss due to impairment of certain patents from 2018;

- $5.0 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of the Company's research and development pipeline;

- $0.8 million increase in collaboration cost-sharing driven by collaboration cost-sharing with Genethon on its microdystrophin platform, offset by a decrease in collaboration cost-sharing with Summit (Oxford) Ltd. as it wound down activities on its Utrophin platform;

- $39.3 million decrease in up-front, milestone, and other expenses, primarily due to $46.9 million of up-front cash and equity payments to StrideBio as a result of the execution of a license and collaboration agreement in November 2019, a $28.0 million up-front payment to Genethon as a result of the execution of a license and collaboration agreement in November 2019, and $25.6 million of up-front and milestone payments made to various academic institutions throughout 2019, as compared with $85.0 million up-front and milestone payments to Myonexus as a result of the execution of a warrant agreement in May 2018 as well as certain development milestones being achieved, $44.8 million up-front and milestone payments to Lysogene as a result of the execution of a collaboration and license agreement in October 2018 as well as certain development milestones becoming probable of being achieved, and $8.0 million related to the purchase of a license to develop, manufacture and commercialize a pre-clinical Pompe product candidate under a license agreement with Lacerta in August 2018; and

- $11.3 million decrease in pre-clinical expenses primarily due to the completion of certain toxicology studies in the Company's PPMO platform.

Non-GAAP research and development expenses were $135.4 million and $77.0 million for the fourth quarter of 2019 and 2018, respectively, an increase of $58.4 million. Non-GAAP research and development expenses were $414.8 million and $241.5 million for the twelve months ended December 31, 2019 and 2018, respectively, an increase of $173.3 million.

Selling, general and administration

Selling general and administrative expenses were $81.4 million for the fourth quarter of 2019, compared to $64.2 million for the same period of 2018,

an increase of $17.2 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $8.6 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology;
- $6.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $4.4 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- $2.8 million increase in professional services primarily due to continuing global expansion.

Selling, general and administrative expenses were $284.8 million for the twelve months ended December 31, 2019, compared to $207.8 million for the same period of 2018, an increase of $77.0 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $34.0 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $16.5 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology;
- $15.0 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- $12.5 million increase in professional services primarily due to continuing global expansion; and
- $2.2 million decrease in restructuring credits due to the relief of cease-use liabilities as a result of the termination of the rental agreement for the Company's Corvallis facility recorded during the second quarter of 2018.

Non-GAAP selling, general and administrative expenses were $65.8 million and $52.9 million for the fourth quarter of 2019 and 2018, respectively, an increase of $12.9 million. Non-GAAP selling, general and administrative expenses were $225.5 million and $166.4 million for the twelve months ended December 31, 2019 and 2018, respectively, an increase of $59.1 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 second quarter of 2019. There was no such transaction during the same period of 2018.

Settlement and License Charges

In December 2019, the Company recognized a $10.0 million settlement charge related to contingent settlement payments to BioMarin as a result of the approval of VYONDYS 53. This was a result of a settlement and license agreement with BioMarin in July 2017. There was no such expense recognized during the same period of 2018.

Amortization of in-licensed rights

For the three and twelve months ended December 31, 2019, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $0.8 million, respectively. For the three and twelve months ended December 31, 2018, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $0.9 million, respectively. This is related to the amortization of the in-licensed right assets recognized as a result of agreements we entered into with BioMarin and UWA upon the first commercial sale of EXYONDYS 51 and VYONDYS 53.

Other expense, net

For the three and twelve months ended December 31, 2019, other expense, net was approximately $4.8 million and $8.3 million, respectively. For the three and twelve months ended December 31, 2018, other expense, net was approximately $2.3 million and $19.0 million, respectively. The quarter-over- quarter increase primarily reflects an increase in interest expense recognized from a new term loan that was received by the Company in December 2019. The year- over- year decrease primarily reflected decreases in a previous term loan termination expense and an increase in amortization of investment discount as a result of an increase in interest rates.

Cash, Cash Equivalents, Investments and Restricted Cash and Investments

The Company had approximately $1.1 billion in cash, cash equivalents and investments as of December 31, 2019 compared to $1.2 billion as of December 31, 2018. The decrease is primarily driven by cash used to fund the Company's ongoing operations during 2019 offset by the proceeds of the public offering of common stock in March 2019, the debt offering in December 2019, and proceeds from the sale of the Company's products.

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, management 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 management's control. Therefore, management 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 up-front and milestone payments and acquired in-process research and development expense. The Company excludes up-front, milestone, and acquired in-process research and development expenses associated with its license and collaboration agreements from its financial 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 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 skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion 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.

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 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. 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 approved under accelerated review based on an increase in dystrophin production in skeletal muscle of patients amenable to exon 53 skipping. 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 Sarepta's vision to be one of the world's most meaningful leaders in precision genetic medicine; Sarepta's plan to commence its next placebo-controlled trial using commercial process for SRPT-9001 (Study 301); the likelihood of increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53 to predict clinical benefit for those patients who are exon 53 amenable; the expectation to conclude Sarepta's placebo-controlled, post-marketing confirmatory trial to support the VYONDYS 53 accelerated approval (ESSENCE) by 2024; the expected benefits of the collaboration agreement with Roche, including the acceleration of Sarepta's mission to bring a potentially life-enhancing therapy to Duchenne patients around the world, and expected regulatory and sales milestones and royalties; and the expected benefits of Sarepta's collaboration with StrideBio and the parties' intention to focus on strategies intended to address re-dosing challenges in patients who have received AAV-delivered gene therapy.

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 meet expectations with respect to sales of our products or attain the net revenues we anticipate for 2020, profitability or positive cash-flow from operations; 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; Sarepta's dependence on certain manufacturers to produce its products and product candidates, including any inability on Sarepta's 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 early results from a clinical trial do not necessarily predict final results; our data for casimersen, SRP-5051, SRP-9001, SRP-9003 and/or other programs may not be sufficient for obtaining regulatory approval; 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; 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; Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing its product candidates to market, for various reasons, some of which may be outside of Sarepta's control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's 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 December 31,		For the Twelve Months Ended December 31,	
	2019	**2018**	**2019**	**2018**
Revenues:				
Product, net	$ 100,113	$ 84,415	$ 380,833	$ 301,034
Total revenues	100,113	84,415	380,833	301,034
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	15,567	13,135	56,586	34,193
Research and development	223,141	146,207	560,909	401,843
Selling, general and administrative	81,424	64,220	284,812	207,761
Acquired in-process research and development	—	—	173,240	—
Settlement and license charges	10,000	—	10,000	—
Amortization of in-licensed rights	200	216	849	865
Total cost and expenses	330,332	223,778	1,086,396	644,662
Operating loss	(230,219)	(139,363)	(705,563)	(343,628)
Other (loss) income:				
Other expense, net	(4,773)	(2,311)	(8,317)	(18,982)
Total other (loss) income	(4,773)	(2,311)	(8,317)	(18,982)
Loss before income tax expense (benefit)	(234,992)	(141,674)	(713,880)	(362,610)
Income tax expense (benefit)	711	(779)	1,195	(692)
Net loss	$ (235,703)	$ (140,895)	$ (715,075)	$ (361,918)
Net loss per share - basic and diluted	$ (3.16)	$ (2.05)	$ (9.71)	$ (5.46)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	74,557	68,653	73,615	66,250

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018

GAAP net loss	$ (235,703)	$ (140,895)	$ (715,075)	$ (361,918)
Interest expense, net	4,562	2,225	8,081	18,326
Income tax expense (benefit)	711	(779)	1,195	(692)
Depreciation and amortization expense	6,646	3,527	24,500	12,245
Stock-based compensation expense	22,064	12,838	78,602	50,127
Restructuring benefit	—	—	—	(2,222)
Up-front, milestone, and other expenses	74,816	64,413	103,162	142,413
Settlement and license charges	10,000	—	10,000	—
Acquired in-process research and development	—	—	173,240	—
Non-GAAP net loss	$ (116,904)	$ (58,671)	$ (316,295)	$ (141,721)
Non-GAAP net loss per share:				
Basic and diluted	$ (1.57)	$ (0.85)	$ (4.30)	$ (2.14)
Weighted average number of shares of common stock outstanding for computing:				
Basic and diluted	74,557	68,653	73,615	66,250

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP research and development expenses	$ 223,141	$ 146,207	$ 560,909	$ 401,843
Up-front, milestone, and other expenses	(74,816)	(64,413)	(103,162)	(142,413)
Stock-based compensation expense	(8,699)	(3,865)	(27,681)	(14,214)
Depreciation and amortization expense	(4,188)	(924)	(15,240)	(3,717)
Non-GAAP research and development expenses	$ 135,438	$ 77,005	$ 414,826	$ 241,499

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP selling, general and administrative expenses	$ 81,424	$ 64,220	$ 284,812	$ 207,761
Stock-based compensation expense	(13,365)	(8,973)	(50,921)	(35,913)
Depreciation and amortization expense	(2,258)	(2,387)	(8,411)	(7,663)
Restructuring benefit	—	—	—	2,222
Non-GAAP selling, general and administrative expenses	$ 65,801	$ 52,860	$ 225,480	$ 166,407

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

	As of December 31, 2019	As of December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 835,080	$ 370,829
Short-term investments	289,668	803,083
Accounts receivable	90,879	49,044
Inventory	171,379	125,445
Other current assets	81,907	77,782
Total current assets	1,468,913	1,426,183
Property and equipment, net	129,620	97,024
Intangible assets, net	12,497	11,574

Right of use assets, net [1]	37,933	—
Other non-current assets	173,859	107,294
Total assets	$ 1,822,822	$ 1,642,075
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 68,094	$ 33,829
Accrued expenses	185,527	134,095
Other current liabilities	11,146	5,766
Total current liabilities	264,767	173,690
Long-term debt	681,900	420,554
Lease liabilities [1]	47,720	—
Other non-current liabilities	10,248	15,555
Total liabilities	1,004,635	609,799
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 99,000,000 shares authorized; 75,184,863 and 71,071,887 issued and outstanding at December 31, 2019 and 2018, respectively	8	7
Additional paid-in capital	3,112,130	2,611,294
Accumulated other comprehensive income (loss), net of tax	50	(99)
Accumulated deficit	(2,294,001)	(1,578,926)
Total stockholders' equity	818,187	1,032,276
Total liabilities and stockholders' equity	$ 1,822,822	$ 1,642,075

(1) As of January 1, 2019, the Company adopted the requirements of Accounting Standards Codification 842, Leases, using the modified retrospective method as of the effective date, and as a result, these captions are not comparable to the prior periods presented.

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

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Source: Sarepta Therapeutics, Inc.