Sarepta Therapeutics Announces Fourth Quarter 2018 and Full-Year 2018 Financial Results and Recent Corporate Developments

RNA Franchise Advances

-Reported EXONDYS 51® (eteplirsen) net sales of $84.4M for the quarter and full-year net sales of $301.0M, in line with guidance-

-Filed NDA for golodirsen with priority review, PDUFA August 19th-

Gene Therapy Engine Advances

-Presented positive preliminary clinical data from the Limb-girdle muscular dystrophy (LGMD) Type 2E program, MYO-101, 51% beta-sarcoglycan gene expression, exceeding the threshold of ≥20% of beta-SG positive fiber expression above baseline in patients dosed at 5E13vg/kg-

-Exercised Option to Acquire Myonexus Therapeutics, including rights to Limb-girdle muscular dystrophy (LGMD) portfolio of five programs-

-Enhanced hybrid gene therapy manufacturing strategy with three distinct partnerships - Aldevron, Brammer Biosciences and Paragon-

CAMBRIDGE, Mass., February 27, 2019 (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, 2018, and announced that it has exercised its option to acquire Myonexus Therapeutics following positive preliminary clinical data from the Limb-girdle muscular dystrophy (LGMD) Type 2E program.

“2018 was a year of transformation for Sarepta. We continued to execute commercially, announced unprecedented early results in our micro-dystrophin gene therapy program, advanced our RNA strategy with a filing for golodirsen, and, by building out a 25-program/10 therapeutic area genetic medicine portfolio second to no other in biotech, cemented our reputation as a science-focused leader in rare disease,” said Doug Ingram, Sarepta’s president and chief executive officer. “And yet, with all of that progress, we have just begun to execute against our vision. We at Sarepta are dedicated to the proposition that a genetic medicine era is upon us, and we intend to play a central role in translating this promise to a better, longer, richer life for those living with rare disease.”

Fourth Quarter 2018 and Recent Corporate Developments

- **Positive, Preliminary Gene Therapy Clinical Results in LGMD2E Patients:** In Cohort 1 of the MYO-101 study, three patients ages 4 – 13, were treated with an infusion of MYO-101 at a dose of 5E13vg/kg, with post-treatment biopsies taken at approximately two months. The first three patients in the MYO-101 trial demonstrated robust and properly localized expression of the protein beta-SG, the lack of which causes LGMD2E, in skeletal muscle. Expression was also correlated with a dramatic 90% mean drop in creatine kinase levels, the enzyme released by muscle as it is being damaged by LGMD2E. Two patients had elevated liver enzymes, one of which was designated a serious adverse event (SAE), as the patient had associated transient increase in bilirubin. Both events occurred when the patients were tapered off oral steroids and, in both instances, symptoms quickly resolved and elevated liver enzymes returned to baseline following supplemental steroid treatment. The first two patients have completely tapered off steroids, and liver enzymes have remained at baseline. There were no other clinically significant laboratory findings and no decreases in platelet counts were observed.

- **Myonexus Acquisition:** Exercised option to acquire Myonexus Therapeutics for $165 million. Upon completion of the transaction and satisfaction of closing conditions, Sarepta will own its 5-program Limb-girdle muscular dystrophies (LGMD) portfolio. The acquisition will enable the rapid development of the LGMD portfolio.

- **Dosing of the First Patient in AAVance, a Phase 2/3 Clinical Trial Investigating LYS-SAF302, a Gene Therapy for MPS IIIA:** AAVance is a single-arm trial aimed at evaluating the effectiveness of a one-time delivery of a recombinant adeno-associated virus vector rh.10 carrying the N-sulfoglucosamine sulfohydrolase (SGSH) gene. MPS IIIA is caused by mutations in the SGSH gene, which is involved in producing an enzyme necessary for the breakdown and disposal of long chain sugar molecules. LAF-SAF302 is intended to deliver a functional copy of the SGSH gene and allow the brain to secrete the missing enzyme. The goal of the trial is to show improved or stabilized neurodevelopmental status of MPS IIIA patients. The trial will enroll 20 patients at eight sites in the U.S. and Europe. Sarepta is collaborating on the program with Lysogene, a pioneering biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases.

- **FDA Accepted Sarepta's New Drug Application Seeking Accelerated Approval for Golodirsen (SRP-4053) for Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 53:** If approved, golodirsen would serve up to another 8 percent of the Duchenne community. PDUFA date is August 19th.

- **Mary Ann Gray, Ph.D. Added to Sarepta's Board of Directors:** Dr. Gray has more than three decades of biotechnology and healthcare experience, with a track record of successfully guiding high-potential companies evolve to their next stage of growth. Dr. Gray serves as a member of both Sarepta's Compensation and Nominating and Corporate Governance Committees.

- **Agreement with Aldevron for GMP-grade Plasmid in Support of Gene Therapy Development and Commercial Manufacturing Strategy:** Entered into a long-term strategic relationship for the supply of plasmid DNA to fulfill Sarepta's needs for its gene therapy clinical trials and commercial supply. Under the terms of the agreement, Aldevron will provide GMP-grade plasmid for Sarepta's micro-dystrophin Duchenne muscular dystrophy gene therapy program and Limb-girdle muscular dystrophy programs, as well as plasmid source material for future gene therapy programs, such as Charcot-Marie-Tooth, MPS IIIA, Pompe and other CNS 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 3768408. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 2018 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, Sarepta reported a net loss of $140.9 million and $24.0 million, or $2.05 and $0.37 per basic and diluted share for the fourth quarter of 2018 and 2017, respectively. On a non-GAAP basis, the net loss for the fourth quarter of 2018 was $58.7 million, or $0.85 per basic and diluted share, compared to a net loss of $13.3 million for the same period of 2017, or $0.21 per basic and diluted share.

On a GAAP basis, for the twelve months ended December 31, 2018, Sarepta reported a net loss of $361.9 million, or $5.46 per basic and diluted share, compared to a net loss of $50.7 million reported for the same period of 2017, or $0.86 per basic and diluted share. On a non-GAAP basis, the net loss for the twelve months ended December 31, 2018 was $141.7 million, or $2.14 per basic and diluted share, compared to a net loss of $79.0 million for the same period of 2017, or $1.34 per basic and diluted share.

Net Revenues

For the three months ended December 31, 2018, the Company recorded net revenues of $84.4 million, compared to net revenues of $57.3 million for the same period of 2017, an increase of $27.1 million. For the twelve months ended December 31, 2018, the Company recorded net revenues of $301.0 million, compared to net revenues of $154.6 million for the same period of 2017, an increase of $146.4 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 three months ended December 31, 2018, cost of sales (excluding amortization of in-licensed rights) was $13.1 million, compared to $3.5 million for the same period of 2017. For the twelve months ended December 31, 2018, cost of sales (excluding amortization of in-licensed rights) was $34.2 million, compared to $7.4 million for the same period of 2017. The increase primarily reflects royalty payments to BioMarin Pharmaceuticals (BioMarin) and higher inventory costs as a result of in increasing demand for EXONDYS 51, as well as an inventory write-off related to certain batches of product not meeting our quality specifications. In addition, prior to the approval of EXONDYS 51, the Company expensed related manufacturing and material costs as research and development expenses.

Research and development

Research and development expenses were $146.2 million for the fourth quarter of 2018, compared to $44.4 million for the same period of 2017, an increase of $101.8 million. The increase in research and development expenses primarily reflects the following:

- $64.4 million increase in up-front and milestone payments primarily consisting of (1) $44.8 million up-front and milestone payments to 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 (2)$15.0 million milestone payments to Myonexus as a result of certain development milestones being achieved or becoming probable of being achieved;
- $10.4 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for Golodirsen, our gene therapy programs, and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in Eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $7.4 million and $2.9 million increases in compensation and other personnel expenses and facility-related expenses and lab supplies, respectively, primarily due to an net increase in headcount;

- $5.7 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform and other follow-on exons;
- $3.8 million increase in loss due to impairment of certain capitalized patent costs;
- $3.0 million increase in professional services as a result of the expansion of our R&D pipeline; and
- $1.0 million increase in collaboration cost sharing with Summit on its utrophin platform.

Research and development expenses were $401.8 million for the twelve months ended December 31, 2018, compared to $166.7 million for the same period of 2017, an increase of $235.1 million. The increase in research and development expenses primarily reflects the following:

- $120.4 million increase in up-front and milestone payments, primarily consisting of (1) $85.0 million up-front and milestone payments to Myonexus as a result of the execution of the Myonexus Warrant Agreement in May 2018 as well as certain development milestones being achieved or becoming probable of being achieved, (2) $44.8 million up-front and milestone payments to 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 (3) $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, partially offset by a $22.0 million payment to Summit in 2017 as a result of achieving the milestone of the last patient being dosed in the safety arm cohort to the PhaseOut DMD study;
- $35.4 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, our gene therapy programs, and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $24.1 million and $7.6 million increases in compensation and other personnel expenses and facility-related expenses, respectively, primarily due to a net increase in headcount;
- $13.6 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform;
- $7.8 million increase in professional services primarily due to continuing accelerated company growth as a result of the expansion of our research and development pipeline;
- $5.7 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;

- $8.6 million increase in collaboration expense driven by collaboration cost sharing with Summit on its Utrophin platform;
- $4.0 million increase in sponsored research with institutions such as Duke University and Nationwide Children's Hospital;
- $3.8 million increase in loss due to impairment of certain capitalized patent costs; and
- $2.9 million increase in lab supplies.

Non-GAAP research and development expenses were $77.0 million and $41.0 million for the fourth quarter of 2018 and 2017, respectively. Non-GAAP research and development expenses were $241.5 million and $133.2 million for the twelve months ended December 31, 2018 and 2017, respectively.

Selling, general and administration

Selling general and administrative expenses were $64.2 million for the fourth quarter of 2018, compared to $32.2 million for the same period of 2017, an increase of $32.0 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $12.4 million increase in professional services, primarily due to continued global expansion;
- $12.0 million and $2.0 million increases in compensation and other personnel expenses and facility-related expenses, respectively, primarily due to an increase in headcount; and
- $4.2 million increase in stock-based compensation primarily due to increases in headcount and stock price.

Selling general and administrative expenses were $207.8 million for the twelve months ended December 31, 2018, compared to $122.7 million for the same period of 2017, an increase of $85.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $34.2 million increase in professional services primarily due to continuing global expansion;
- $35.1 million and $4.9 million increases in compensation and other personnel expenses and facility-related expenses, respectively, primarily reflect an increase in headcount;
- $16.1 million increase in stock-based compensation primarily due to increases in headcount and stock price, the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions as well as the impact of a revised forfeiture rate assumption for officers and members of our Board of Directors;
- $3.5 million decrease in severance expense as a result of the termination of our former CEO in June 2017; and

- $5.1 million decrease in restructuring expenses primarily due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility.

Non-GAAP selling, general and administrative expenses were $52.9 million and $26.2 million for the fourth quarter of 2018 and 2017, respectively. Non-GAAP selling, general and administrative expenses were $166.4 million and $93.6 million for the twelve months ended December 30, 2018 and 2017, respectively.

EXONDYS 51 litigation and license charges

As a result of the execution of the settlement and license agreements with BioMarin in July 2017, the Company recognized EXONDYS 51 litigation and license charges of $28.4 million in 2017. There was no such a transaction in 2018.

Amortization of in-licensed rights

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. For the three and twelve months ended December 31, 2017, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $1.1 million, respectively.

Other (loss) income

Gain from sale of Priority Review Voucher

In connection with the completion of the sale of the Priority Review Voucher (PRV) in March 2017, the Company recorded a gain of $125.0 million from sale of the PRV in the first quarter of 2017.

Interest expense and other, net

For the three months ended December 31, 2018 and 2017, the Company recorded $2.3 million and $2.7 million, respectively, of interest expense and other, net. The decrease was primarily driven by the pay-off of certain of the Company's debt facilities. For the twelve months ended December 31, 2018 and 2017, the Company recorded $19.0 million and $2.0 million, respectively, of interest expense and other, net. The year over year increase is primarily due to the $570.0 million convertible debt offering partially offset by interest income from higher balances of cash, cash equivalents and investments.

Cash, Cash Equivalents, Investments and Restricted Investment

The Company had approximately $1.174 billion in cash, cash equivalents and investments as of December 31, 2018 compared to $1.1 billion as of December 31, 2017. The increase is primarily driven by the proceeds of the public offering of common stock in November 2018 offset by cash used to fund operations.

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, restructuring 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, restructuring 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, restructuring 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. Restructuring expenses

The Company believes that adjusting for these items more closely represents the Company's ongoing operating performance and financial results.

4. 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 relates 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 the aforementioned gain from the sale of the Company's PRV and up-front and milestone payments. In particular, the Company excludes up-front and milestone expenses associated with the Company's license and collaboration agreements from its financial results and research and development expenses because the Company does not consider them to be normal, recurring 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-related up-front and milestone payment makes the identification of trends in the Company's ongoing research and development activities more difficult. 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 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 Sarepta Therapeutics

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 5 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit www.sarepta.com.

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 intention to play a central role in translating the promise of the genetic medicine to a better, longer, richer life for those living with rare disease; the satisfaction of closing conditions related to the acquisition of Myonexus; the expectation that the acquisition of Myonexus will enable the rapid development of the LGMD portfolio; LAF-SAF302's potential to deliver a functional copy of the SGSH gene and allow the brain to secrete the missing enzyme; golodirsen's potential to serve up to another 8 percent of the Duchenne community; the expected PDUFA date of August 19th; the potential benefits of our agreement with Aldevron; and Sarepta's mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.

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 EXONDYS 51 sales or attain the net revenues we anticipate for 2019, 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 EXONDYS 51 in a timely manner or at all; there can be no assurance that Sarepta will be able to complete the acquisition of Myonexus on the anticipated terms, or at all; Sarepta may not realize the anticipated benefits of the acquisition, which involves various risks, including disruption of Sarepta's ongoing business and distraction of its management and employees from other opportunities and challenges, potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of Myonexus or the product candidates, liability for activities of Myonexus before the acquisition, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities; the acquisition of Myonexus may not result in any viable treatments suitable for clinical research or commercialization; the expected benefits and opportunities related to the agreement with Aldevron may not be realized or may take longer to realize than expected; Sarepta's dependence on certain manufacturers to produce its 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 initial results from a clinical trial do not necessarily predict final results; our data for golodirsen, casimersen, SRP-9001, the LGMD programs and/or other programs may not be sufficient for obtaining regulatory approval; if the actual number of patients suffering from DMD, LGMD, pompe disease, CMT and/or MPS IIIA is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; various factors may decrease the market size of our product and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our product candidates; 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, 2017 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.

Consolidated Statements of Operations

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended December 31, 2018		For the Twelve Months Ended December 31, 2018	
	2018	2017	2018	2017
Revenues:				
Product, net	$ 84,415	$ 57,277	$ 301,034	$ 154,584
Total revenues	84,415	57,277	301,034	154,584
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	13,135	3,546	34,193	7,353
Research and development	146,207	44,441	401,843	166,707
Selling, general and administrative	64,220	32,221	207,761	122,682
EXONDYS 51 litigation and license charges	—	—	—	28,427
Amortization of in-licensed rights	216	216	865	1,053
Total costs and expenses	223,778	80,424	644,662	326,222
Operating loss	(139,363)	(23,147)	(343,628)	(171,638)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	—	125,000
Interest expense and other, net	(2,311)	(2,693)	(18,982)	(1,990)
Other (loss) income	(2,311)	(2,693)	(18,982)	123,010
Loss before income tax (benefit) expense	(141,674)	(25,840)	(362,610)	(48,628)
Income tax (benefit) expense	(779)	(1,842)	(692)	2,060
Net loss	$ (140,895)	$ (23,998)	$ (361,918)	$ (50,688)
Net loss per share - basic and diluted	$ (2.05)	$ (0.37)	$ (5.46)	$ (0.86)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	68,653	64,277	66,250	58,818

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,	
	2018	2017	2018	2017
GAAP net loss	$ (140,895)	$ (23,998)	$ (361,918)	$ (50,688)
Interest expense, net	2,225	2,772	18,326	2,591
Income tax (benefit) expense	(779)	(1,842)	(692)	2,060
Depreciation and amortization expense	3,527	2,124	12,245	8,092
Stock-based compensation expense	12,838	7,366	50,127	30,465
Restructuring expense	—	247	(2,222)	3,020
Up-front and milestone payments	64,413	—	142,413	22,000
EXONDYS 51 litigation and license charges	—	—	—	28,427
Gain from sale of Priority Review Voucher	—	—	—	(125,000)
Non-GAAP net loss [1]	$ (58,671)	$ (13,331)	$ (141,721)	$ (79,033)
Non GAAP net loss per share:				
Basic and diluted	$ (0.85)	$ (0.21)	$ (2.14)	$ (1.34)
Weighted average number of shares of common stock outstanding for computing:				
Basic and diluted	68,653	64,277	66,250	58,818

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	2017	2018	2017
GAAP research and development expenses	$ 146,207	$ 44,441	$ 401,843	$ 166,707
Up-front and milestone payments	(64,413)	—	(142,413)	(22,000)
Stock-based compensation expense	(3,865)	(2,661)	(14,214)	(8,542)
Depreciation and amortization expense	(924)	(795)	(3,717)	(2,761)
Restructuring expense	—	(4)	—	(188)
Non-GAAP research and development expenses [1]	$ 77,005	$ 40,981	$ 241,499	$ 133,216

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	2017	2018	2017
GAAP selling, general and administrative expenses	$ 64,220	$ 32,221	$ 207,761	$ 122,682
Stock-based compensation expense	(8,973)	(4,705)	(35,913)	(21,923)
Depreciation and amortization expense	(2,387)	(1,113)	(7,663)	(4,278)
Restructuring (expense) credit	—	(243)	2,222	(2,832)
Non-GAAP selling, general and administrative expenses [1]	$ 52,860	$ 26,160	$ 166,407	$ 93,649

(1) Commencing in the first quarter of 2018, the Company has excluded interest expense (income), net, and depreciation and amortization expense from the computation of its non-GAAP financial measures. The Company has revised prior year presentation in the tables above in order to conform to the current year presentation.

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(unaudited, in thousands, except share and per share data)

	As of December 31, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 370,829	$ 599,691
Short-term investments	803,083	479,369
Accounts receivable	49,044	29,468
Inventory	125,445	83,605
Other current assets	77,782	36,511
Total Current Assets	1,426,183	1,228,644
Property and equipment, net of accumulated depreciation of $28,149 and $18,022 as of December 31, 2018, and 2017, respectively	97,024	43,156
Intangible assets, net of accumulated amortization of $3,852 and $4,145 as of December 31, 2018, and 2017, respectively	11,574	14,355
Other assets	107,294	21,809
Total Assets	$ 1,642,075	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 33,829	$ 8,467
Accrued expenses	134,095	68,982
Current portion of long-term debt	—	6,175
Deferred revenue	3,303	3,316
Other current liabilities	2,463	1,392
Total Current Liabilities	173,690	88,332
Long-term debt	420,554	424,876
Deferred rent and other	15,555	5,539
Total Liabilities	609,799	518,747
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, 99,000,000 shares authorized; 71,017,887 and 64,791,670 issued and outstanding at December 31, 2018, and December 31, 2017, respectively	7	6
Additional paid-in capital	2,611,294	2,006,598
Accumulated other comprehensive loss	(99)	(379)
Accumulated deficit	(1,578,926)	(1,217,008)
Total Stockholders' Equity	1,032,276	789,217
Total Liabilities and Stockholders' Equity	$ 1,642,075	$ 1,307,964

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

Investors:
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Media:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com