Sarepta Therapeutics Announces Third Quarter 2018 Financial Results and Recent Corporate Developments

-- Third quarter 2018 EXONDYS 51® (eteplirsen) total net revenues of $78.5 million --

CAMBRIDGE, Mass., October 24, 2018 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a leader in precision genetic medicine for rare diseases, today reported financial results for the third quarter of 2018.

“We are pleased to report another positive quarter, delivering strong EXONDYS 51 sales and tracking to achieve our full-year sales objectives while both advancing our RNA pipeline and making substantial progress in the creation of an enduring gene therapy engine,” stated Doug Ingram, Sarepta’s president and chief executive officer. “We continued this quarter to advance our RNA pipeline, PMOs and next-generation PPMO platform, with urgency. Further, the strides we have taken in service of our gene therapy engine, including unprecedented results in our micro-dystrophin program, rights to what are now 14 gene therapy programs, the addition of manufacturing partners, and the continued hiring of gene therapy talent, speak to our vision. Others may be content with steady progress. We see a revolution and it is our intention to lead that revolution to the benefit of countless genetic disease patients awaiting life-enhancing therapies.”

Third Quarter 2018 and Recent Corporate Developments

Lysogene Agreement - Signed a license agreement with Lysogene, a biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, for the development of a gene therapy, LYS-SAF302, to treat Mucopolysaccharidosis type IIIA (MPS IIIA), also called Sanfilippo syndrome type A, a rare, severe and fatal inherited neurodegenerative lysosomal storage disorder. The pivotal gene therapy study is scheduled to start by year-end 2018; and the trial will assess the efficacy of LYS-SAF302 in improving or stabilizing the neurodevelopmental status of MPS IIIA patients. Sarepta receives full commercial rights to LYS-SAF302 in the U.S. and other markets outside of Europe, while Lysogene retains full commercial rights in Europe.

Paragon Bioservices Agreement - Entered into long-term manufacturing partnership with Paragon Bioservices, significantly expanding Sarepta's commercial capacity for its micro-dystrophin gene therapy program, as well as bolstering the Company's clinical and commercial capacity for its other pipeline programs.

Nationwide Children's Hospital Gene Therapy Partnership, Charcot-Marie-Tooth (CMT) Neuropathy - Forged another agreement with Nationwide Children's Hospital giving Sarepta the certain exclusive rights to the Nationwide Children's gene therapy candidate, neurotrophin 3 (NT-3), to treat CMT neuropathies, including CMT type 1A. CMT is a group of hereditary, degenerative nerve diseases that can affect motor skills, resulting in muscle weakness, and limiting patients' ability to walk or use their hands. CMT is the most common inherited neuromuscular disorder, affecting over 2.8 million people worldwide. A clinical trial is scheduled to begin in 2019 in the most prevalent subtype of CMT, CMT type 1A.

Positive Micro-Dystrophin Gene Therapy Clinical Results in DMD Patients Presented at the 23rd International Congress of the World Muscle Society (Mendoza, Argentina) - Jerry Mendell, M.D., of Nationwide Children's, presented positive updated results from the gene therapy clinical trial assessing AAVrh74.MHCK7.micro-Dystrophin in individuals with Duchenne muscular dystrophy (DMD). Dr. Mendell's presentation included positive results from the biopsy of the fourth patient showing robust micro-dystrophin expression as measured by Western blot and immunohistochemistry. In all patients, expression of micro-dystrophin was associated with significant expression and up regulation of the dystrophin-associated protein complex, an additional indication of functionality of dystrophin. All patients showed significant decreases of serum creatine kinase (CK) levels at last measure versus baseline, and positive functional improvements were shown across all measures. No serious adverse events (SAEs) were observed.

Clinical Hold Lifted for DMD Micro-dystrophin Gene Therapy Program - The Food and Drug Administration (FDA) lifted the clinical hold for Sarepta's DMD micro-dystrophin gene therapy program. Sarepta previously announced on July 25, 2018 that the FDA placed the program on clinical hold due to the presence of trace amounts of DNA fragment in research-grade third-party supplied plasmid in a manufacturing lot. In response, and in collaboration with Nationwide Children's, an action plan was developed and submitted to the FDA, including an audit of the plasmid supplier and a commitment to use GMP-s plasmid for all future production lots.

Negative CHMP Re-examination Opinion Received for Eteplirsen - The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) confirmed its May 31, 2018 negative

opinion for a Conditional Marketing Application for eteplirsen. Relying upon CHMP advice and input, Sarepta will seek further scientific advice from the EMA on a possible path to bring eteplirsen to patients in Europe.

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 +1-574-990-1451 for international callers. The passcode for the call is 6099548. Please specify to the operator that you would like to join the "Sarepta Third Quarter 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 $76.4 million and $47.7 million, or $1.15 and $0.78 per basic and diluted share for the third quarter of 2018 and 2017, respectively. On a non-GAAP basis, the net loss for the third quarter of 2018 was $37.1 million, or $0.56 per basic and diluted share, compared to a net loss of $10.7 million for the same period of 2017, or $0.17 per basic and diluted share.

On a GAAP basis, for the nine months ended September 30, 2018, Sarepta reported a net loss of $221.0 million, or $3.38 per basic and diluted share, compared to a net loss of $26.7 million reported for the same period of 2017, or $0.47 per basic and diluted share. On a non-GAAP basis, the net loss for the nine months ended September 30, 2018 was $83.1 million, or $1.27 per basic and diluted share, compared to a net loss of $65.7 million for the same period of 2017, or $1.15 per basic and diluted share.

Net Revenues

For the three months ended September 30, 2018, the Company recorded net revenues of $78.5 million, compared to net revenues of $46.0 million for the same period of 2017, an increase of $32.5 million. For the nine months ended September 30, 2018, the Company recorded net revenues of $216.6 million, compared to net revenues of $97.3 million for the same period of 2017, an increase of $119.3 million. The increases primarily reflect increasing 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 September 30, 2018, cost of sales (excluding amortization of in-licensed rights) was $8.7 million, compared to $3.1 million for the same period of 2017. For the nine months ended September 30, 2018, cost of sales (excluding amortization of in-licensed rights) was $21.1 million, compared to $3.8 million for the same period of 2017. The increase primarily reflects royalty payments to BioMarin Pharmaceuticals (BioMarin) as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as higher inventory costs related to increasing demand for EXONDYS 51 during 2018. 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 $86.6 million for the third quarter of 2018, compared to $34.2 million for the same period of 2017, an increase of $52.4 million. The increase in research and development expenses primarily reflects the following:

- $18.0 million increase in up-front and milestone payments. The Company made a milestone payment of $10.0 million to Myonexus Therapeutics (Myonexus) for the achievement of one of the development milestones. In addition, the Company expensed $8.0 million related to the purchase of license to develop, manufacture and commercialize a pre-clinical Pompe product candidate under a license agreement with Lacerta Therapeutics (Lacerta);
- $12.8 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in the on-going ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, our micro-dystrophin program 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;
- $8.1 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- $3.8 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform;
- $2.6 million increase in facility-related expenses due to our continuing expansion efforts;
- $2.6 million increase in sponsored research with institutions such as Duke University and Nationwide Children’s Hospital;

- $1.6 million increase in collaboration cost sharing with Summit on its utrophin platform; and
- $1.4 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price.

Research and development expenses were $255.6 million for the nine months ended September 30, 2018, compared to $122.3 million for the same period of 2017, an increase of $133.3 million. The increase in research and development expenses primarily reflects the following:

- $56.0 million increase in up-front and milestone payments. The Company made an up-front payment of $60.0 million to Myonexus upon execution of the warrant to purchase common stock agreement in May 2018 and a milestone payment of $10.0 million to Myonexus upon achievement of one of the development milestones in September 2018. In addition, the Company expensed $8.0 million related to the purchase of license to develop, manufacture and commercialize a pre-clinical Pompe product candidate under a license agreement with Lacerta. In May 2017, the Company made a milestone payment of $22.0 million to Summit as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved;
- $25.0 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in the on-going ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, casimersen, our micro-dystrophin program 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;
- $16.7 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- $7.9 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $7.6 million increase in collaboration cost sharing with Summit on its utrophin platform;
- $4.8 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of our R&D pipeline;
- $4.7 million increase in facility-related expenses due to our continuing expansion efforts;
- $4.5 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price as well as achievement of a milestone related to the September 2016 restricted stock awards with a performance condition; and

- $4.0 million increase in sponsored research with institutions such as Duke University, Genethon and Nationwide Children's Hospital.

Non-GAAP research and development expenses were $64.2 million and $31.5 million for the third quarter of 2018 and 2017, respectively. Non-GAAP research and development expenses were $164.5 million and $92.2 million for the nine months ended September 30, 2018 and 2017, respectively.

Selling, general and administration

Selling general and administrative expenses were $53.0 million for the third quarter of 2018, compared to $28.2 million for the same period of 2017, an increase of $24.8 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $9.8 million and $1.7 million increase in professional services and facility-related expenses, respectively, primarily due to continuing global expansion;
- $9.4 million increase in compensation and other personnel expenses primarily due to an increase in headcount; and
- $3.0 million increase in stock-based compensation primarily due to increases in headcount and stock price.

Selling general and administrative expenses were $143.5 million for the nine months ended September 30, 2018, compared to $90.5 million for the same period of 2017, an increase of $53.0 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $23.1 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- $21.8 million and $2.9 million increase in professional services and facility-related expenses, respectively, primarily due to continuing global expansion;
- $11.9 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 granted with a performance condition, as well as the impact of a revised forfeiture rate assumption for equity awards granted to officers and directors;
- $4.8 million decrease in restructuring expenses due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility; and
- $3.5 million decrease in severance expense as a result of termination of our former CEO in June 2017.

Non-GAAP selling, general and administrative expenses were $42.5 million and $22.2 million for the third quarter of 2018 and 2017, respectively. Non-GAAP selling, general and administrative expenses were $113.5 million and $67.5 million for the nine months ended September 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 litigation and license charges of $25.6 million and $28.4 million during the three and nine months ended September 30, 2017, respectively. There was no such a transaction in 2018.

Amortization of in-licensed rights

For the three and nine months ended September 30, 2018, the Company recorded amortization of in-licensed rights of approximately $0.2 million and $0.6 million, respectively. For both the three and nine months ended September 30, 2017, the Company recorded amortization of in-licensed rights of approximately $0.8 million.

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 from sale of the PRV in the first quarter of 2017.

Interest (expense) income and other, net

For the three and nine months ended September 30, 2018, the Company recorded $7.0 million and $16.7 million, respectively, of interest expense and other, net. For the same periods of 2017, the Company recorded $0.2 million and $0.7 million, respectively, of interest income and other, net. The period over period unfavorable change primarily reflects the interest expense accrued on the convertible notes issued in November 2017 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 $793.9 million in cash, cash equivalents and investments as of September 30, 2018 compared to $1.1 billion as of December 31, 2017. The decrease is primarily driven by the use of cash to fund the Company's ongoing operations during the first three quarters of 2018.

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.

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 Limb-girdle muscular dystrophy (LGMD), Charcot-Marie-Tooth (CMT) and CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs span across several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare 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 future operations, financial performance and projections, business plans, priorities and development of product candidates including: Sarepta being on track to achieve its full-year sales objectives while both advancing its RNA pipeline and making substantial progress in the creation of an enduring gene therapy engine; Sarepta's intention to lead the gene therapy revolution to the benefit of countless genetic disease patients awaiting life-enhancing therapies; the goal of the LYS-SAF302 pivotal study and the expectation to start the study by

year-end 2018; Sarepta's partnership with Paragon significantly expanding Sarepta's commercial capacity for its micro-dystrophin gene therapy program, as well as bolstering Sarepta's clinical and commercial capacity for its other pipeline programs; the expectation to begin a clinical trial for NT-3 to treat CMT type 1A in 2019; Sarepta's plan to seek further scientific advice from the EMA on a possible path to bring eteplirsen to patients in Europe; and Sarepta being poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare 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 2018, 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; the expected benefits and opportunities related to the transactions with Lysogene and Nationwide Children's Hospital may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership with Lysogene and Nationwide Children's Hospital may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including 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 or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreements, including any inability by us to fulfill our financial commitments to Lysogene and/or Nationwide Children's Hospital; the expected benefits and opportunities related to the agreement with Paragon may not be realized or may take longer to realize than expected; Sarepta's dependence on Paragon 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; if Paragon were to cease providing quality manufacturing and related services to Sarepta, and Sarepta is not able to engage appropriate replacements in a timely manner, Sarepta's ability to manufacture its gene therapy product candidates in sufficient quality and quantity would adversely affect Sarepta's various product research, development and commercialization efforts; 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; Sarepta may not be able to eventually obtain regulatory approval for eteplirsen, or any other product candidates, from EMA; 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 September 30		For the Nine Months Ended September 30	
	2018	2017	2018	2017
Revenues:				
Product, net	$ 78,486	$ 45,954	$ 216,619	$ 97,307
Total revenues	78,486	45,954	216,619	97,307
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	$ 8,741	3,078	$ 21,058	3,807
Research and development	86,584	34,239	255,636	122,266
Selling, general and administrative	53,044	28,176	143,541	90,461
EXONDYS 51 litigation and license charges	—	25,588	—	28,427
Amortization of in-licensed rights	216	780	649	837
Total costs and expenses	148,585	91,861	420,884	245,798
Operating loss	(70,099)	(45,907)	(204,265)	(148,491)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	—	125,000
Interest (expense) income and other, net	(6,968)	184	(16,671)	703
Other (loss) income	(6,968)	184	(16,671)	125,703
Loss before income tax (benefit) expense	(77,067)	(45,723)	(220,936)	(22,788)
Income tax (benefit) expense	(674)	2,011	87	3,902
Net loss	(76,393)	(47,734)	(221,023)	(26,690)
Net loss per share - basic and diluted	$ (1.15)	$ (0.78)	$ (3.38)	$ (0.47)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	66,209	61,528	65,454	57,166

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
GAAP net loss	$ (76,393)	$ (47,734)	$ (221,023)	$ (26,690)
Interest expense (income), net	6,909	(69)	16,101	(181)
Income tax (benefit) expense	(674)	2,011	87	3,902
Depreciation and amortization expense	3,593	2,559	8,718	5,968
Stock-based compensation expense	11,484	6,922	37,289	23,099
Restructuring expense	—	13	(2,222)	2,773
Up-front and milestone payments	18,000	—	78,000	22,000
EXONDYS 51 litigation and license charges	—	25,588	—	28,427
Gain from sale of Priority Review Voucher	—	—	—	(125,000)
Non-GAAP net loss [1]	$ (37,081)	$ (10,709)	$ (83,050)	$ (65,702)
Non GAAP net loss per share:				
Basic and diluted	$ (0.56)	$ (0.17)	$ (1.27)	$ (1.15)
Weighted average number of shares of common stock outstanding for computing:				
Basic and diluted	66,209	61,528	65,454	57,166

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
GAAP research and development expenses	86,584	$ 34,239	255,636	122,266
Up-front and milestone payments	(18,000)	$ —	(78,000)	(22,000)
Stock-based compensation expense	(3,260)	$ (1,812)	(10,349)	(5,881)
Depreciation and amortization expense	(1,092)	$ (937)	(2,793)	(1,966)
Restructuring expense	—	$ (10)	—	(184)
Non-GAAP research and development expenses [1]	64,232	31,480	164,494	92,235

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
GAAP selling, general and administrative expenses	53,044	$ 28,176	143,541	90,461
Stock-based compensation expense	(8,224)	$ (5,110)	(26,940)	(17,218)
Depreciation and amortization expense	(2,285)	$ (842)	(5,276)	(3,165)
Restructuring (expense) credit	—	$ (3)	2,222	(2,589)
Non-GAAP selling, general and administrative expenses [1]	42,535	22,221	113,547	67,489

(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 September 30, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 209,702	$ 599,691
Short-term investments	583,158	479,369
Accounts receivable	48,601	29,468
Inventory	115,816	83,605
Other current assets	54,800	36,511
Total current assets	1,012,077	1,228,644
Property and equipment, net of accumulated depreciation of $25,224 and $18,022 as of September 30, 2018, and December 31, 2017, respectively	76,841	43,156
Intangible assets, net of accumulated amortization of $5,532 and $4,145 as of September 30, 2018, and December 31, 2017, respectively	15,324	14,355
Other assets	78,664	21,809
Total assets	$ 1,182,906	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 20,408	$ 8,467
Accrued expenses	88,687	68,982
Current portion of long-term debt	—	6,175
Deferred revenue	3,303	3,316
Other current liabilities	1,995	1,392
Total current liabilities	114,393	88,332
Long-term debt	415,446	424,876
Deferred rent and other	13,219	5,539
Total liabilities	543,058	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; 66,693,348 and 64,791,670 issued and outstanding at September 30, 2018, and December 31, 2017, respectively	7	6
Additional paid-in capital	2,077,864	2,006,598
Accumulated other comprehensive loss	8	(379)
Accumulated deficit	(1,438,031)	(1,217,008)
Total stockholders' equity	639,848	789,217
Total liabilities and stockholders' equity	$ 1,182,906	$ 1,307,964

Source: Sarepta Therapeutics, Inc.

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