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

-- Second quarter 2018 EXONDYS 51® (eteplirsen) total net revenues of $73.5 million --

CAMBRIDGE, Mass., August 8, 2018 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, today reported financial results for the second quarter of 2018.

“We are very pleased to report another strong quarter, marrying our vision to become one of the most meaningful precision genetic medicine companies globally with a strong focus on execution against both our near- and long-term range goals,” stated Doug Ingram, Sarepta’s president and chief executive officer. “Our robust quarterly sales for EXONDYS 51, increasing patients on therapy and strong adherence and compliance speaks to the benefits of EXONDYS 51 and to our ability as a fully integrated, commercial-stage genetic medicine company to support our therapies in the community and deliver on our commitments. At the same time we took enormous strides in the quarter in the direction of our lofty strategic vision by announcing very positive preliminary results from our first gene therapy program focused on the use of micro-dystrophin to treat Duchenne muscular dystrophy, accelerating the development of our gene therapy center of excellence and enhancing our senior management team with the addition of two scientific leaders, Drs. Gilmore O’Neill and Louise Rodino-Klapac, and advancing our hybrid gene therapy manufacturing strategy with our long-term relationship with gold standard manufacturer, Brammer Bio.”

Mr. Ingram continued, “We have entered into our next gene therapy collaboration, with Lacerta Therapeutics, gaining access to world-leading talent, next-generation tools, and three additional gene therapy programs, one focused on Pompe Disease and two additional CNS-targeted programs, with the purpose of supporting our strategic expansion. Over the remainder of 2018, we have much to do and also numerous catalysts that have the potential to solidify us as a leader in precision genetic medicine and a

company focused on rapidly developing therapies to extend and enhance the lives of those living with rare, genetic-based disease."

Second Quarter 2018 and Recent Corporate Developments

- **Lacerta Therapeutics Partnership:** After the close of the quarter, on August 8, 2018, Sarepta signed a long-term strategic partnership with Lacerta Therapeutics, a spin out of the University of Florida, one of the top gene therapy research centers of excellence. Under the terms of the agreement, Sarepta will receive exclusive rights to Lacerta's program focused on CNS-targeted gene therapy to treat Pompe Disease, along with an option to two additional rare CNS-targeted gene therapy programs. Lacerta will manage the majority of the pre-clinical development while Sarepta will lead clinical development and commercialization. Sarepta will owe development and sales-based milestones to Lacerta and pay single-digit royalties on net sales. Sarepta will make an equity investment of $30 million.

- **Positive Preliminary Phase 1/2a Gene Therapy Micro-dystrophin Trial Results in DMD Patients:** At Sarepta's first R&D Day held on June 19, 2018, Jerry Mendell, M.D. of Nationwide Children's Hospital presented positive preliminary results from its Phase 1/2a gene therapy clinical trial assessing AAVrh74.MHCK7.micro-Dystrophin in individuals with DMD. In all three patients, the three-month biopsy results showed robust gene expression as measured by Western blot and immunohistochemistry and unprecedented drops in levels of creatine kinase, an enzyme associated with muscle damage which is a hallmark of Duchenne muscular dystrophy.

- **Executive Leadership Appointments:** Gilmore O'Neill, M.B., M.M.Sc. was appointed as Sarepta's chief medical officer on June 7, 2018. Dr. O'Neill leads all clinical development, medical affairs, pharmacovigilance, and regulatory affairs. At Biogen, he held leadership roles of increasing responsibility over a 15-year period in research and development, most recently as senior vice president responsible for all late-stage clinical development, and oversaw development programs for Alzheimer's disease, movement disorders, acute neurology, multiple sclerosis, pain, neuromuscular disease, gene and cell therapy, and rare diseases. He played a leadership role in seeking, receiving and maintaining global marketing approvals for Tecfidera®, Zinbryta®, Plegridy® and Spinraza®.

Louise Rodino-Klapac, Ph.D. will lead Sarepta's newly created gene therapy business unit as vice president, gene therapy, overseeing the development of the Company's gene therapy and gene editing programs and serving as a member of the Company's executive team. Until her appointment with Sarepta, Dr. Rodino-Klapac was head of the laboratory for gene therapy research for Muscular Dystrophies at Nationwide Children's and is renowned for her work in molecular genetics and gene therapy. Her ground-breaking work has involved 11 investigational new drug applications for gene therapy research, resulting in numerous field-advancing peer-reviewed publications, and the advancement of six gene therapy programs from bench to human clinical development.

- **CHMP Opinion:** On June 1, 2018, The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), adopted a negative opinion for the eteplirsen Marketing Authorization Application (MAA), as anticipated. Sarepta has commenced a re-examination of the CHMP opinion and CHMP is convening a Scientific Advisory Group (SAG), made up of neuromuscular experts, in the fall of 2018 to review the MAA. A final decision is expected by year-end 2018.

- **Brammer Bio Partnership:** Sarepta signed a long-term strategic manufacturing partnership with Brammer Bio, granting Sarepta access to clinical and commercial manufacturing capacity for its micro-dystrophin DMD gene therapy program and a manufacturing platform for future gene therapy programs.

  Sarepta has adopted a hybrid internal and external development and manufacturing model. Under this model, Sarepta will continue to build internal expertise in all aspects of AAV-based manufacturing while partnering with Brammer Bio to provide scalable best-in-class manufacturing capabilities. The collaboration model will integrate process development, clinical production and testing, and commercial manufacturing with the goal of bringing micro-dystrophin gene therapies to the patient community urgently and in sufficient supply.

  Brammer Bio will partner with Sarepta to design and build dedicated commercial manufacturing capacity within their facility with cutting-edge capabilities. Once complete, the facility is expected to provide robust manufacturing capacity to support the unusually high demands for systemic administration of the micro-dystrophin therapy for Duchenne muscular dystrophy.

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 5899719. Please specify to the operator that you would like to join the "Sarepta Second 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 $109.3 million and $63.0 million, or $1.67 and $1.15 per basic and diluted share for the second quarter of 2018 and 2017, respectively. On a non-GAAP basis, the net loss for the second quarter of 2018 was $28.0 million, or $0.43 per share, compared to a net loss of $26.5 million for the same period of 2017, or $0.48 per share.

On a GAAP basis, for the six months ended June 30, 2018, Sarepta reported a net loss of $144.6 million, or $2.22 per basic and diluted share, compared to a net income of $21.0 million reported for the same period of 2017, or $0.38 per basic share and $0.37 per diluted share. On a non-GAAP basis, the net loss for the six months ended June 30, 2018 was $46.0 million, or $0.71 per share, compared to a net loss of $57.8 million for the same period of 2017, or $1.05 per share.

Net Revenues

For the three months ended June 30, 2018, the Company recorded net revenues of $73.5 million, compared to net revenues of $35.0 million for three months ended June 30, 2017, an increase of $38.5 million. For the six months ended June 30, 2018, the Company recorded net revenues of $138.1 million, compared to net revenues of $51.4 million for six months ended June 30, 2017, an increase of $86.7 million. The increases primarily reflects 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 June 30, 2018, cost of sales (excluding amortization of in-licensed rights) was $6.7 million, compared to $0.5 million for the same period of 2017. For the six months ended June 30,

2018, cost of sales (excluding amortization of in-licensed rights) was $12.3 million, compared to $0.7 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 $122.8 million for the second quarter of 2018, compared to $58.9 million for the same period of 2017, an increase of $63.9 million. The increase in research and development expenses primarily reflects the following:

- $38.0 million increase in up-front and milestone payments. The Company made an up-front payment of $60.0 million to Myonexus Therapeutics (Myonexus) upon execution of the partnership agreement in May 2018. In May 2017, the Company made a milestone payment of $22.0 million to Summit (Oxford) Ltd. (Summit) as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved;
- $7.8 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, casimersen 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;
- $6.0 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.9 million increase in collaboration cost sharing with Summit on its utrophin platform;
- $2.8 million in stock-based compensation expense primarily driven by change in headcount as well as achievement of a milestone related to the September 2016 restricted stock awards with performance condition;
- $2.5 million increase in preclinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $1.6 million increase in professional services primarily due to accelerated company growth as a result of expansion of our R&D pipeline; and

- $1.4 million increase in facility-related expenses due to our continuing expansion.

Research and development expenses were $169.1 million for the six months ended June 30, 2018, compared to $88.0 million for the same period of 2017, an increase of $81.1 million. The increase in research and development expenses primarily reflects the following:

- $38.0 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. 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;
- $12.2 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 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;
- $8.6 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $6.1 million increase in collaboration cost sharing with Summit on its utrophin platform;
- $4.1 million increase in preclinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $4.0 million increase in professional services primarily due to accelerated company growth as a result of expansion of our R&D pipeline;
- $3.0 million increase in stock-based compensation expense primarily driven by change in headcount as well as achievement of a milestone related to the September 2016 restricted stock awards with performance condition;
- $2.1 million increase in facility-related expenses due to our continuing expansion efforts; and
- $1.4 million increase in sponsored research with institutions such as Duke University and Genethon.

Non-GAAP research and development expenses were $57.0 million and $34.1 million for the second quarter of 2018 and 2017, respectively. Non-GAAP research and development expenses were $100.3 million and $60.8 million for the six months ended June 30, 2018 and 2017, respectively.

Selling, general and administration

Selling general and administrative expenses were $47.2 million for the second quarter of 2018, compared to $36.1 million for the same period of 2017, an increase of $11.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $8.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $5.6 million increase in professional services primarily due to continuing global expansion;
- $4.2 million increase in stock-based compensation primarily due to an increase in headcount and the achievement of a milestone related to the September 2016 restricted stock awards with performance condition;
- $4.6 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
- $3.4 million decrease in severance expense as a result of termination of our former CEO in June 2017.

Selling general and administrative expenses were $90.5 million for the six months ended June 30, 2018, compared to $62.3 million for the same period of 2017, an increase of $28.2 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $13.6 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $12.0 million and $1.2 million increase in professional services and facility related expenses, respectively, primarily due to continuing global expansion;
- $8.8 million increase in stock-based compensation primarily due to an increase in headcount, 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 associated with our Corvallis facility; and
- $3.4 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 $37.3 million and $24.2 million for the second quarter of 2018 and 2017, respectively. Non-GAAP selling, general and administrative expenses were $71.0 million and $45.3 million for the six months ended June 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 Pharmaceuticals (BioMarin) in July 2017, the Company recognized litigation and license charges of $2.8 million during the second quarter of 2017 related to estimated royalties incurred between September 2016 and June 2017.

Amortization of in-licensed rights

Amortization of in-licensed rights was $0.2 million and $0.4 million for the three and six months ended June 30, 2018. Amortization of in-licensed rights was less than $0.1 million for the same periods of 2017. The increase was primarily due to the BioMarin settlement and license agreements that were executed in July 2017.

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 six months ended June 30, 2018, the Company recorded $5.2 million and $9.7 million, respectively, of interest expense and other, net. For the same periods of 2017, the Company recorded $0.2 million and $0.5 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 Investments

The Company had approximately $950 million in cash, cash equivalents, investments and restricted investments as of June 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 and second 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 Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. 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's vision to become one of the most meaningful precision genetic medicine companies globally with a strong focus on execution against both its near- and long-term range goals; the benefits of EXONDYS 51 and Sarepta's ability as a fully integrated, commercial-stage genetic medicine company to support its therapies in the community and deliver on its commitments; the transactions with Lacerta providing Sarepta with access to world-leading talent, next-generation tools, and three additional gene therapy programs, one focused on Pompe Disease and two additional CNS-targeted programs, with the purpose of supporting Sarepta's strategic expansion; the potential of Sarepta's numerous catalysts to solidify Sarepta as a leader in precision genetic medicine and a company focused on rapidly developing therapies to extend and enhance the lives of those living with rare, genetic-based disease; the potential benefits of the transactions with Lacerta and payments that Sarepta is expected to make in connection with these transactions; Sarepta's plan to make an equity investment of $30 in Lacerta; Sarepta's expectation that a SAG will be convened in the fall of 2018 and that a final CHMP decision will be made by year-end 2018; Sarepta's plan to continue to build internal expertise in all aspects of AAV-based manufacturing while partnering with Brammer Bio to provide scalable best-in-class manufacturing capabilities; Sarepta's goal of bringing micro-dystrophin gene therapies to the patient community urgently and in sufficient supply; Sarepta's plan to partner with Brammer Bio to design and build dedicated commercial manufacturing capacity within Brammer Bio's facility with cutting-edge capabilities; and the expectation that the facility will provide robust manufacturing capacity to support the unusually high demands for systemic administration of the micro-dystrophin therapy for DMD.

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, 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 CHMP may render a negative final decision and we may not be able to obtain regulatory approval for eteplirsen from the EMA; the expected benefits and opportunities related to the transactions with Lacerta 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 Lacerta 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 Lacerta; the expected benefits and opportunities related to the agreement with Brammer Bio may not be realized or may take longer to realize than expected; Sarepta's dependence on Brammer Bio 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 Brammer Bio 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's ongoing research and development efforts may not result in any viable treatments suitable for commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including the results of future research may fail to meet regulatory approval requirements for the safety and efficacy of product candidates, 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 even if Sarepta's programs result in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; 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 June 30,		For the Six Months Ended June 30,	
	2018	2017	2018	2017
Revenues:				
Product, net	$ 73,529	$ 35,011	$ 138,133	$ 51,353
Total revenues	73,529	35,011	138,133	51,353
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	$ 6,735	506	$ 12,317	729
Research and development	122,848	58,908	169,052	88,027
Selling, general and administrative	47,156	36,069	90,497	62,285
EXONDYS 51 litigation and license charges	—	2,839	—	2,839
Amortization of in-licensed rights	217	28	433	57
Total costs and expenses	176,956	98,350	272,299	153,937
Operating loss	(103,427)	(63,339)	(134,166)	(102,584)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	—	125,000
Interest (expense) income and other, net	(5,218)	184	(9,703)	519
Other (loss) income	(5,218)	184	(9,703)	125,519
(Loss) income before income tax expense (benefit)	(108,645)	(63,155)	(143,869)	22,935
Income tax expense (benefit)	622	(109)	761	1,891
Net (loss) income	(109,267)	(63,046)	(144,630)	21,044
Net (loss) income per share				
Basic (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.38
Diluted (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.37
Weighted average number of shares of common stock used in computing:				
Basic (loss) earnings per share	65,484	54,976	65,060	54,913
Diluted (loss) earnings per share	65,484	54,976	65,060	56,176

Sarepta Therapeutics, Inc.

Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures

(unaudited, in thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
GAAP net (loss) income	$ (109,267)	$ (63,046)	$ (144,630)	$ 21,044
Interest expense (income), net	4,689	(83)	9,192	(112)
Income tax expense (benefit)	622	(109)	761	1,891
Depreciation and amortization expense	2,873	1,772	5,125	3,409
Stock-based compensation expense	15,279	10,465	25,805	16,177
Restructuring expense	(2,222)	2,524	(2,222)	2,760
Up-front and milestone payments	60,000	22,000	60,000	22,000
Gain from sale of Priority Review Voucher	—	—	—	(125,000)
Non-GAAP net loss [1]	$ (28,026)	$ (26,477)	$ (45,969)	$ (57,831)
Non GAAP net loss per share:				
Basic and diluted	$ (0.43)	$ (0.48)	$ (0.71)	$ (1.05)
Weighted average number of shares of common stock outstanding for computing:				
Basic and diluted	65,484	54,976	65,060	54,913

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
GAAP research and development expenses	122,848	58,908	169,052	88,027
Up-front and milestone payments	(60,000)	(22,000)	(60,000)	(22,000)
Stock-based compensation expense	(5,029)	(2,195)	(7,089)	(4,069)
Depreciation and amortization expense	(853)	(517)	(1,701)	(1,029)
Restructuring expense	—	(104)	—	(174)
Non-GAAP research and development expenses [1]	56,966	34,092	100,262	60,755

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
GAAP selling, general and administrative expenses	47,156	36,069	90,497	62,285
Stock-based compensation expense	(10,250)	(8,270)	(18,716)	(12,108)
Depreciation and amortization expense	(1,803)	(1,198)	(2,991)	(2,323)
Restructuring credit (expense)	2,222	(2,420)	2,222	(2,586)
Non-GAAP selling, general and administrative expenses [1]	37,325	24,181	71,012	45,268

(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 June 30, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 410,375	$ 599,691
Short-term investments	538,769	479,369
Accounts receivable	42,985	29,468
Inventory	104,126	83,605
Other current assets	42,989	36,511
Total current assets	1,139,244	1,228,644
Property and equipment, net of accumulated depreciation of $22,124 and $18,022 as of June 30, 2018 and December 31, 2017, respectively	57,624	43,156
Intangible assets, net of accumulated amortization of $5,100 and $4,145 as of June 30, 2018 and December 31, 2017, respectively	14,857	14,355
Other assets	35,435	21,809
Total assets	$ 1,247,160	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,382	$ 8,467
Accrued expenses	72,477	68,982
Current portion of long-term debt	9,514	6,175
Deferred revenue	3,303	3,316
Other current liabilities	2,011	1,392
Total current liabilities	104,687	88,332
Long-term debt	429,925	424,876
Deferred rent and other	13,501	5,539
Total liabilities	548,113	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; 66,346,248 and 64,791,670 issued and outstanding at June 30, 2018 and December 31, 2017, respectively	7	6
Additional paid-in capital	2,061,039	2,006,598
Accumulated other comprehensive loss	(361)	(379)
Accumulated deficit	(1,361,638)	(1,217,008)
Total stockholders' equity	699,047	789,217
Total liabilities and stockholders' equity	$ 1,247,160	$ 1,307,964

Source: Sarepta Therapeutics, Inc.

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