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

-- First quarter 2017 EXONDYS 51® (eteplirsen) total net revenues of $64.6 million --

-- Sarepta signs exclusive partnership and buy-out option with Myonexus Therapeutics; pipeline expands from 16 to 21 programs --

-- Company announces date of first R&D day, at which clinical data from gene therapy micro-dystrophin program will be announced --

-- Company receives negative trend vote following its CHMP oral explanation; will request re-examination and Scientific Advisory Group to be convened --

CAMBRIDGE, Mass., May 3, 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 three months ended March 31, 2018.

“In the first quarter, we continued our successful launch of EXONDYS 51 and advanced our pipeline to bring life-enhancing therapies to those suffering from rare disease around the world,” said Doug Ingram, Sarepta’s president and chief executive officer. “We accelerated our gene therapy and RNA platform, and in that regard are excited to announce that our first R&D day will take place on June 19 to showcase the breadth, depth and progress of our pipeline. Significantly, at this event we will report preliminary safety and gene expression data from at least two patients from our micro-dystrophin gene therapy trial underway with Nationwide Children’s Hospital.”

Mr. Ingram continued, “Aligned with our stated goal of leveraging our expertise beyond DMD, we announced today a collaboration with Myonexus Therapeutics for the development of five potentially transformative gene therapies to treat a debilitating set of diseases, all under the umbrella of Limb-girdle muscular dystrophy. Through this collaboration, we have expanded our pipeline to 21 therapies in development. Our confidence in the Myonexus collaboration comes from the similarities between the Myonexus

and Sarepta approaches to gene therapy. Both are seeking to treat rare neuromuscular disease through the AAVrh.74 vector; and both rely upon the unparalleled expertise of Dr. Louise Rodino-Klapac in developing and executing gene therapy constructs. This partnership with Myonexus enables us to expand our efforts beyond DMD while maintaining our unwavering commitment to those suffering from DMD."

Mr. Ingram concluded, "Unfortunately, in addition to our successes in the first quarter, we also have had a delay in our effort to bring eteplirsen to patients in Europe who could potentially benefit from it. I could not be prouder of our Sarepta team and the team of experts who spoke on behalf of eteplirsen at the CHMP oral explanation last week. The rigorous work that was done to prepare for the hearing only strengthened our resolve that eteplirsen should urgently be made available to those waiting in Europe. Unfortunately, the CHMP's trend vote was negative. Based on discussions with CHMP representatives, it is our understanding that the CHMP did not conclude that eteplirsen is ineffective for exon 51 amenable patients, but rather that Sarepta has not yet met the regulatory threshold for conditional approval, in part due to the use of external controls as comparators in the studies. Sarepta plans to file for re-examination and will request that a Scientific Advisory Group (SAG), which is made up of DMD and neuromuscular specialists, be convened to provide expert guidance and insight into, among other things, the validity of the external controls used and the importance of slowing pulmonary decline in patients with DMD."

Financial Results

For the first quarter of 2018, on a GAAP basis, Sarepta reported a net loss of $35.4 million, or $0.55 per basic and diluted share, compared to net income of $84.1 million reported for the same period of 2017, or $1.53 per basic share and $1.50 per diluted share. On a non-GAAP basis, the net loss for the first quarter of 2018 was $17.9 million, or $0.28 per share, compared to a net loss of $31.4 million for the same period of 2017, or $0.57 per share.

Net Revenues

For the three months ended March 31, 2018, the Company recorded net product revenues of $64.6 million, compared to net revenues of $16.3 million for first quarter of 2017. The increase 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 March 31, 2018, cost of sales (excluding amortization of in-licensed rights) was $5.6 million, compared to $0.2 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. 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 $46.2 million for the first quarter of 2018, compared to $29.1 million for the same period of 2017, an increase of $17.1 million. The increase in research and development expenses primarily reflects the following:

- $4.4 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in the Company's ongoing clinical trials in golodirsen and casimersen, as well as a ramp-up of manufacturing activities for the Company's 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;
- $3.2 million increase in collaboration cost sharing with Summit on its utrophin platform;
- $2.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.4 million increase in professional services primarily due to an expansion of the Company's research and development pipeline; and
- $1.6 million increase in preclinical expenses primarily due to the continuing ramp-up of toxicology studies in the Company's PPMO platform as well as golodirsen and casimersen.

Non-GAAP research and development expenses were $43.3 million for the first quarter of 2018, compared to $26.7 million for the same period of 2017, an increase of $16.6 million.

Selling, general and administration

Selling general and administrative expenses were $43.3 million for the first quarter of 2018, compared to $26.2 million for the same period of 2017, an increase of $17.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $6.4 million increase in professional services primarily due to continuing global expansion as well as preparation for a potential product launch in the EU should the Company's Marketing Authorization Application be approved by the European Medicines Agency;
- $5.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $4.6 million increase in stock-based compensation primarily due to the impact of revising the forfeiture rate assumption for officers and Board of Directors as well as an increase in stock price.

Non-GAAP selling, general and administrative expenses were $33.7 million for the first quarter of 2018, compared to $21.1 million for the same period of 2017, an increase of $12.6 million.

Amortization of in-licensed rights

Amortization of in-licensed rights was $0.2 million during the first quarter of 2018, compared to less than $0.1 million for the same period of 2017. The increase was primarily due to the BioMarin transactions that occurred 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. There was no similar activity in the first quarter of 2018.

Interest (expense) income and other, net

For the three months ended March 31, 2018 and 2017, the Company recorded $4.5 million interest expense and other, net and $0.3 interest income and other, net, respectively. The period over period unfavorable change primarily reflects the interest expense accrued on the Company's debt facilities partially offset by interest income from higher balances of cash, cash equivalents and investments.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had $1.0 billion in cash, cash equivalents, restricted cash and investments as of March 31, 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 quarter 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.

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 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 to Non-GAAP Net Loss."

First Quarter and Recent Corporate Developments

- **Golodirsen (SRP-4053):** Based on Sarepta's Type C meeting with the FDA's Division of Neurology Products to solicit the Division's guidance on the development pathway for golodirsen, the Company remains on track to complete a rolling NDA submission by year-end 2018, seeking accelerated approval based on an increase in dystrophin protein as a surrogate endpoint.

- **Myonexus Therapeutics Partnership:** Sarepta and Myonexus Therapeutics entered into a partnership to advance multiple gene therapies for various forms of Limb-girdle muscular dystrophies

(LGMDs). The lead program, MYO-101, has generated encouraging pre-clinical safety and efficacy data utilizing the AAVrh.74 vector system, the same vector used in the micro-dystrophin gene therapy program Sarepta is developing with Nationwide Children's Hospital. A Phase 1/2a study of MYO-101 is scheduled to begin in mid-2018. The companies plan to report on 60-day biopsy data in late-2018 or early 2019. Additionally, Myonexus is advancing MYO-102 for LGMD2D, MYO-103 for LGMD2C, MYO-201 for LGMD2B, and MYO-301 for LGMD2L. Under the terms of the agreement, Sarepta will make an upfront payment of $60 million and additional development-related milestone payments to purchase an exclusive option to acquire Myonexus at a pre-negotiated, fixed price with sales-related contingent payments. If all development-related milestone payments are met, Sarepta will make payments of up to $45 million over an approximately two-year evaluation period. Sarepta has the option to purchase Myonexus at any time, including upon review of proof-of-concept data.

- **Sarepta R&D Day (Tuesday, June 19, 2018):** Sarepta management, along with several key-opinion leaders, will provide an in-depth look into the Company's pipeline programs across several modalities, included RNA-targeted therapies, gene therapy and gene editing. Of particular note, we look forward to presenting our micro-dystrophin expression data from at least two patients enrolled in the Phase 1/2a gene therapy clinical trial underway with Drs. Jerry Mendell and Louise Rodino-Klapac of Nationwide Children's Hospital. To date, the Company has enrolled four patients in this study and no significant adverse events have been reported. In addition, Dr. Rodino-Klapac, who is also chief scientific officer and co-founder of Myonexus, will present data from Myonexus' entire LGMD program. For all to access, Sarepta's R&D day will be webcast live under the investor relations section of the Company's website at: www.sarepta.com and will be archived there following the event for 90 days.

Conference Call

The Company will be hosting a conference call at 4:30 p.m. Eastern Time, to discuss these 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 2798939. Please specify to the operator that you would like to join the "Sarepta First 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.

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 goal of leveraging its expertise beyond DMD; expected milestones, including reporting preliminary safety and gene expression data from at least two patients from our micro-dystrophin gene therapy trial on June 19, 2018, completing a rolling NDA submission for golodirsen by year-end 2018, which seeks accelerated approval based on an increase in dystrophin protein as a surrogate endpoint, initiating a Phase 1/2a study of MYO-101 in mid-2018, and reporting on 60-day biopsy data in late-2018 or early 2019; Sarepta's collaboration with Myonexus involving five potentially transformative gene therapies to treat LGMD; the partnership with Myonexus enabling Sarepta to expand its efforts beyond DMD while maintaining its unwavering commitment to those suffering from DMD; payments that Sarepta is expected to make under the agreement with Myonexus; and Sarepta's plan to file for re-examination of its marketing authorization application (MAA) for eteplirsen and to request that a SAG be convened.

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; we may not be granted a re-examination of our MAA for eteplirsen, a SAG may not be convened, and even if a re-examination and a related SAG are granted, the CHMP may render a negative opinion and we may not be able to obtain regulatory approval for eteplirsen from the European Medicines Agency; our data for golodirsen may not be sufficient for a filing for or obtaining regulatory approval; the expected benefits and opportunities related to the agreement with Myonexus 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 Myonexus 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 agreement, including any inability by us to fulfill our financial commitments to Myonexus; and even if the agreement results in new commercialized products, we may not achieve any significant revenues from the sale of such products; we may not be able to execute on our business plans, including meeting our expectations with respect to EXONDYS 51 sales, meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons 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 CHMP on eteplirsen or the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in 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 share and per share amounts)

	For the Three Months Ended March 31,	
	2018	**2017**
Revenues:		
Product, net	$ 64,604	$ 16,342
Total revenues	64,604	16,342
Costs and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	5,582	223
Research and development	46,204	29,119
Selling, general and administrative	43,341	26,216
Amortization of in-licensed rights	216	29
Total costs and expenses	95,343	55,587
Operating loss	(30,739)	(39,245)
Other (loss) income:		
Gain from sale of Priority Review Voucher	—	125,000
Interest (expense) income and other, net	(4,485)	335
Other (loss) income	(4,485)	125,335
(Loss) income before income tax expense	(35,224)	86,090
Income tax expense	139	2,000
Net (loss) income	(35,363)	84,090
Net (loss) income per share		
Basic (loss) earnings per share	$ (0.55)	$ 1.53
Diluted (loss) earnings per share	$ (0.55)	$ 1.50
Weighted average number of shares of common stock used in computing:		
Basic (loss) earnings per share	64,631	54,850
Diluted (loss) earnings per share	64,631	56,012

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

	Three Months Ended March 31,	
	2018	2017
GAAP net (loss) income	$ (35,363)	$ 84,090
Interest expense (income), net	4,503	(29)
Income tax expense	139	2,000
Depreciation and amortization expense	2,252	1,637
Stock-based compensation expense	10,526	5,712
Restructuring expense	—	236
Gain from sale of Priority Review Voucher	—	(125,000)
Non-GAAP net loss [(1)]	$ (17,943)	$ (31,354)
Non GAAP net loss per share:		
Basic and diluted	$ (0.28)	$ (0.57)
Weighted average number of shares of common stock outstanding for computing:		
Basic and diluted	64,631	54,850

	Three Months Ended March 31,	
	2018	2017
GAAP research and development expenses	46,204	29,119
Stock-based compensation expense	(2,060)	(1,874)
Depreciation and amortization expense	(848)	(512)
Restructuring expense	—	(70)
Non-GAAP research and development expenses [(1)]	43,296	26,663

	Three Months Ended March 31,	
	2018	2017
GAAP selling, general and administrative expenses	43,341	26,216
Stock-based compensation expense	(8,466)	(3,838)
Depreciation and amortization expense	(1,188)	(1,125)
Restructuring expense	—	(166)
Non-GAAP selling, general and administrative expenses [(1)]	33,687	21,087

(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 March 31, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 557,234	$ 599,691
Short-term investments	491,757	479,369
Accounts receivable	39,848	29,468
Inventory	99,375	83,605
Other current assets	31,203	36,511
Total current assets	1,219,417	1,228,644
Property and equipment, net of accumulated depreciation of $19,817 and $18,022 as of March 31, 2018 and December 31, 2017, respectively	53,927	43,156
Intangible assets, net of accumulated amortization of $4,659 and $4,145 as of March 31, 2018 and December 31, 2017, respectively	14,473	14,355
Other assets	12,466	21,809
Total assets	$ 1,300,283	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,379	$ 8,467
Accrued expenses	65,648	68,982
Current portion of long-term debt	3,446	6,175
Other current liabilities	4,723	4,708
Total current liabilities	91,196	88,332
Long-term debt	427,365	424,876
Deferred rent and other	4,962	5,539
Total liabilities	523,523	518,747
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; 65,493,293 and 64,791,670 issued and outstanding at March 31, 2018 and December 31, 2017, respectively	7	6
Additional paid-in capital	2,029,767	2,006,598
Accumulated other comprehensive loss	(643)	(379)
Accumulated deficit	(1,252,371)	(1,217,008)
Total stockholders' equity	776,760	789,217
Total liabilities and stockholders' equity	$ 1,300,283	$ 1,307,964

Source: Sarepta Therapeutics, Inc.

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