



## **Sarepta Therapeutics Announces First Quarter 2017 Financial Results and Recent Corporate Developments**

-- First quarter 2017 EXONDYS 51 total net revenues of \$16.3 million --

-- Company anticipates net revenues for the year will exceed \$95 million --

CAMBRIDGE, Mass., April 27, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, today reported financial results for the first quarter of 2017.

"The first quarter of 2017 showed significant progress across all areas of the business, including executing a strong launch for EXONDYS 51™ (eteplirsen), building our pipeline, working to expand globally, and forging strategic partnerships that support our goal to help as many DMD patients as possible," said Edward Kaye, Sarepta's chief executive officer. "Additionally, our pipeline is advancing on schedule, we have taken an important first step in building our European operations, and we look forward to entering the clinic later in the year with our gene therapy and PPMO programs. Moving through 2017, we remain focused on continuing the momentum of the EXONDYS 51 launch while building the foundation for long-term growth through re-investment in our R&D programs."

Management also noted that due to continued patient and physician interest in EXONDYS 51, coupled with progress in the reimbursement landscape, the Company anticipates that net revenues for the year will exceed \$95 million, an increase to its previously reported annual net revenue guidance of exceeding at least \$80 million.

### ***Financial Results***

For the first quarter of 2017, Sarepta reported GAAP net income of \$84.1 million, or \$1.50 per diluted share, compared to a net loss of \$59.8 million for the same period of 2016, or \$1.31 per diluted share. The increase in income was primarily driven by the gain on sale of the Company's Rare Pediatric Disease Priority Review Voucher (PRV) and sales of EXONDYS 51. Non-GAAP net loss for the first quarter of 2017

was \$33.0 million, or \$0.60 per share, compared to a non-GAAP net loss of \$52.5 million for the same period of 2016, or \$1.15 per share.

### ***Net Revenues***

For the first quarter of 2017, the Company recognized net revenues of \$16.3 million in product sales. No revenue was recognized for the same period of 2016.

### ***Operating Expenses***

Research and development expenses were \$29.1 million for the first quarter of 2017, compared to \$38.8 million for the same period of 2016, a decrease of \$9.7 million. The decrease was primarily driven by lower manufacturing expenses due to the capitalization of inventory following the approval of EXONDYS 51 by the U.S. Food and Drug Administration (FDA), partially offset by increased patient enrollment in our ongoing clinical trials. Non-GAAP research and development expenses were \$27.2 million for the first quarter of 2017, compared to \$35.9 million for the same period of 2016, a decrease of \$8.7 million.

Selling, general and administrative expenses were \$26.2 million for the first quarter of 2017, compared to \$20.9 million for the same period of 2016, an increase of \$5.3 million, which was primarily driven by increases in professional services due to increased legal fees and commercial initiatives, compensation and other personnel expenses. Non-GAAP selling, general and administrative expenses were \$22.2 million for the first quarter of 2017, compared to \$16.6 million for the same period of 2016, an increase of \$5.6 million.

### ***Cash, Cash Equivalents and Investments***

The Company had \$391.1 million in cash, cash equivalents and investments as of March 31, 2017 compared to \$329.3 million as of December 31, 2016, an increase of \$61.8 million. The increase was driven by the proceeds received from the sale of the Company's PRV, offset by the use of cash to fund the company's ongoing operations.

### ***Use of Non-GAAP Measures***

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements: non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP other income adjustments, non-GAAP net loss, and non-GAAP basic and diluted net loss per share, which present operating results on a basis adjusted for stock-based compensation, restructuring expenses, and other items.

### 1. 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.

### 2. Restructuring expenses

Restructuring expenses have been excluded as the Company believes that adjusting for these items more closely represents the Company's ongoing operating performance and financial results.

### 3. Other items

Management 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 associated taxes.

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 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."

## **Recent Corporate Developments**

- Sarepta Therapeutics Announces Appointment of Catherine Stehman-Breen, M.D., M.S. as Chief Medical Officer
- Sarepta Therapeutics Announces Addition of Kenneth Fischbeck, M.D. and Matthew Wood M.D., Ph.D. to the Company's Strategic and Scientific Advisory Board
- Sarepta Therapeutics Announces Presentations at the 2017 MDA Scientific Conference
- Sarepta Therapeutics Agrees to Sale of Priority Review Voucher for \$125M
- Sarepta Therapeutics Enters into License Agreement with Nationwide Children's Hospital for Galgt2 Gene Therapy Program
- Sarepta Therapeutics Enters into Research Agreement and Option Agreement with Nationwide Children's Hospital for Micro-Dystrophin Gene Therapy Program
- Sarepta Therapeutics Announces EMA Validation of Eteplirsen Authorization Application for Treatment of Duchenne Muscular Dystrophy Amenable to Exon Skipping 51

## **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 574-990-1451 for domestic callers and 1-844-534-7313 for international callers. The passcode for the call is 9321329. Please specify to the operator that you would like to join the "Sarepta First Quarter 2017 Earnings Call." The conference call, along with a slide presentation, will be webcast live under the investor relations section of Sarepta's website at [www.sarepta.com](http://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™ (eteplirsen) Injection:**

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. Data from clinical studies of EXONDYS 51 in a

small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

### **Important Safety Information**

- Adverse reactions in DMD patients (N=8) treated with 30 or 50 mg/kg/week of EXONDYS 51 with incidence of at least 25% more than placebo (N=4) (Study 1) were: balance disorder (38%), vomiting (38%) and contact dermatitis (25%). 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  $\geq 30$  mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in  $\geq 10\%$  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.
- There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

*Please see the U.S. Full Prescribing Information for EXONDYS 51 at [www.EXONDYS51.com](http://www.EXONDYS51.com).*

### **About Sarepta Therapeutics**

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit [www.sarepta.com](http://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 plans for the rest of 2017, including its pipeline advancing on schedule, building its European operations, Sarepta's plans for its gene therapy and PPMO programs entering the clinic later in the year, and remaining focused on continuing the momentum for EXONDYS 51 while building the foundation for long-term growth through re-investment in Sarepta's R&D programs, and Sarepta's anticipation that EXONDYS 51 net revenues for 2017 will exceed \$95 million.*

*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 2017, 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 able to obtain regulatory approval for EXONDYS 51 in jurisdictions outside of the U.S. including from the European Medicines Agency; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of any of our product candidates; the results of our ongoing research and development efforts and clinical trials for our product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, 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 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, 2016 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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

Sarepta Therapeutics, Inc.  
Condensed Consolidated Statements of Operations  
(in thousands, except per share amounts)  
(unaudited)

	<b>Three Months Ended March 31,</b>	
	<b>2017</b>	<b>2016</b>
Revenues:		
Product, net	\$ 16,342	\$ -
Total revenues	16,342	-
Cost and expenses:		
Cost of sales	252	-
Research and development	29,119	38,826
Selling, general and administrative	26,216	20,876
Total cost and expenses	55,587	59,702
Operating loss	(39,245)	(59,702)
Other income (loss):		
Gain from sale of intangible asset	125,000	-
Interest income (expense) and other, net	335	(68)
Income (loss) before income tax expense	86,090	(59,770)
Income tax expenses	2,000	-
Net income (loss)	\$ 84,090	\$ (59,770)
Net income (loss) per share:		
Basic earnings (loss) per share	\$ 1.53	\$ (1.31)
Diluted earnings (loss) per share	\$ 1.50	\$ (1.31)
Weighted average number of shares of common stock used in calculating:		
Basic earnings (loss) per share	54,850	45,697
Diluted earnings (loss) per share	56,012	45,697

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

	Three Months Ended March 31,	
	2017	2016
Net income (loss) - GAAP	\$ 84,090	\$ (59,770)
Research and development:		
Stock-based compensation expense	1,874	2,448
Restructuring expense	70	502
Total research and development non-GAAP adjustments	1,944	2,950
Selling, general and administrative:		
Stock-based compensation expense	3,838	4,241
Restructuring expense	166	31
Total selling, general and administrative non-GAAP adjustments	4,004	4,272
Other income (loss):		
(Gain) from sale of intangible asset	(125,000)	-
Total other income non-GAAP adjustments	(125,000)	-
Income tax expense non-GAAP adjustments	2,000	-
Net income (loss) - non-GAAP	\$ (32,962)	\$ (52,548)
Non-GAAP net loss per share - basic and diluted	\$ (0.60)	\$ (1.15)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	54,850	45,697

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	<b>As of March 31, 2017</b>	<b>As of December 31, 2016</b>
Cash, cash equivalents and investments	\$ 391,097	\$ 329,324
Total assets	511,533	424,104
Total liabilities	82,391	87,413
Total stockholders' equity	\$ 429,142	\$ 336,691

Source: Sarepta Therapeutics, Inc.

Media and Investors:

Sarepta Therapeutics, Inc.

Ian Estepan, 617-274-4052

[iestepan@sarepta.com](mailto:iestepan@sarepta.com)

or

W2O Group

Brian Reid, 212-257-6725

[breid@w2ogroup.com](mailto:breid@w2ogroup.com)