



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

- Achieved net revenue of \$35 million for the second quarter 2017 --
- Increased revenue guidance range to \$125 – \$130 million for the year –
- Entered into global settlement and license agreements resolving exon skipping patent disputes with BioMarin --
- Entered into a micro-dystrophin gene therapy research collaboration with Genethon --

CAMBRIDGE, Mass., July 19, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a U.S. 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 second quarter of 2017.

“We are pleased with the progress made in the second quarter and anticipate continued momentum for the remainder of the year. In an effort to become a global pharmaceutical company, we settled our patent dispute with BioMarin, which gave us the ability to launch our Managed Access Program and reach patients around the world,” said Douglas Ingram, Sarepta’s president and chief executive officer. “We are particularly excited with the continued success of the EXONDYS 51® (eteplirsen) launch, the progress with our internal pipeline and strategic partnerships, all toward the goal of maintaining our leadership position in DMD. We look forward to numerous catalysts between now and the end of the year. Specifically, announcing the results of our 4053-101 dystrophin data and potentially having up to seven experimental DMD therapies in the clinic.”

### **Financial Results**

For the second quarter of 2017, Sarepta reported a GAAP net loss of \$63.0 million, or \$1.15 per diluted share, compared to a net loss of \$62.3 million for the same period of 2016, or \$1.35 per diluted share. The

increase in loss for the quarter was primarily driven by increased operating expenses offset by net revenue. Non-GAAP net loss for the second quarter of 2017 was \$25.3 million, or \$0.46 per share, compared to a non-GAAP net loss of \$54.8 million for the same period of 2016, or \$1.19 per share.

### ***Net Revenues***

For the second quarter of 2017, the Company recognized net revenues of \$35.0 million from product sales. No revenue was recognized for the same period of 2016.

### ***Operating Expenses***

Research and development expenses were \$58.9 million for the second quarter of 2017, compared to \$44.3 million for the same period of 2016, an increase of \$14.6 million. The increase was primarily due to a one-time milestone payment to Summit Therapeutics for \$22.0 million, increased preclinical expenses due to a ramp up of preclinical studies for the Company's PPMO platform and other follow-on exons, and increased patient enrollment in our clinical trials, offset in part 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). Non-GAAP research and development expenses were \$34.6 million for the second quarter of 2017, compared to \$41.4 million for the same period of 2016, a decrease of \$6.8 million.

Selling, general and administrative expenses were \$36.1 million for the second quarter of 2017, compared to \$17.8 million for the same period of 2016, an increase of \$18.3 million, which was primarily driven by increases in professional services due to increased legal fees and commercial initiatives, restructuring charges, compensation and other personnel expenses. Non-GAAP selling, general and administrative expenses were \$25.4 million for the second quarter of 2017, compared to \$13.2 million for the same period of 2016, an increase of \$12.2 million.

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

The Company had \$301.7 million in cash, cash equivalents, restricted cash and investments as of June 30, 2017 compared to \$329.3 million as of December 31, 2016, a decrease of \$27.6 million. The decrease is primarily due to a one-time milestone payment to Summit Therapeutics for \$22 million and the use of cash to fund the Company's ongoing operations, offset by the proceeds received from the sale of the Company's PRV.

### ***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 income tax expense, 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 income taxes, upfront license and milestone payments to Summit, and EXONDYS 51 litigation and license charges.

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."

### **Recent Corporate Developments**

- Sarepta Therapeutics and Clinigen Launch a Managed Access Program to Treat Patients with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping
- Sarepta Therapeutics Secures \$100 Million in Debt Financing
- Sarepta Therapeutics and BioMarin Pharmaceutical Inc. Announce Execution of a Global Settlement and a License Agreement Resolving Exon Skipping Patent Litigation
- Sarepta Therapeutics Appoints Douglas S. Ingram as President and Chief Executive Officer
- Sarepta Therapeutics Announces Grand Opening of its Research and Manufacturing Center at Andover
- Sarepta Therapeutics and Genethon Announce a Gene Therapy Research Collaboration for the Treatment of Duchenne Muscular Dystrophy

### **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 46830427. Please specify to the operator that you would like to join the "Sarepta Second Quarter 2017 Earnings Call". The conference call 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**

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 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  $\geq 30$  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.

### **About Sarepta Therapeutics**

Sarepta Therapeutics is a U.S. 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 anticipated continued momentum for the remainder of 2017; the impact of the agreements with BioMarin and Sarepta’s efforts to become a global pharmaceutical company, including Sarepta’s ability to reach patients around the world; Sarepta’s goal of maintaining its leadership position in DMD by, among other things, continuing the success of the EXONDYS 51 launch and the progress of Sarepta’s internal pipeline and strategic partnerships; the expectation for numerous catalysts between now and the end of the year, including plans to announce the dystrophin data results of Sarepta’s 4053-101 study and potentially having up to seven experimental DMD therapies in the clinic; and Sarepta’s anticipation that EXONDYS 51 net revenues for 2017 will be in the range of \$125 to \$130 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 eteplirsén 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, including those with strategic partners, and clinical trials for our product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact our business; our rights to commercialize EXONDYS 51 and our follow-on exons across the world may not be fully protected by our patents and/or third party agreements; we may not be able to establish and successfully conduct a Managed Access Program in one or more countries, and even if such program(s) are successfully conducted in each country targeted, we may not achieve any significant revenues from sales of eteplirsén under these programs; 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)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues:				
Product, net	35,011	-	51,353	-
Total revenues	<u>35,011</u>	<u>-</u>	<u>51,353</u>	<u>-</u>
Cost and expenses:				
Cost of sales	534	-	786	-
Research and development	58,908	44,348	88,027	83,174
Selling, general and administrative	36,069	17,752	62,285	38,628
EXONDYS 51 litigation and license charges	2,839	-	2,839	-
Total cost and expenses	<u>98,350</u>	<u>62,100</u>	<u>153,937</u>	<u>121,802</u>
Operating loss	<u>(63,339)</u>	<u>(62,100)</u>	<u>(102,584)</u>	<u>(121,802)</u>
Other income (loss):				
Gain from sale of intangible asset	-	-	125,000	-
Interest income (expense) and other, net	184	(201)	519	(269)
Income (loss) before income tax expense	<u>(63,155)</u>	<u>(62,301)</u>	<u>22,935</u>	<u>(122,071)</u>
Income tax expense (benefit)	(109)	-	1,891	-
Net income (loss)	<u>\$ (63,046)</u>	<u>\$ (62,301)</u>	<u>\$ 21,044</u>	<u>\$ (122,071)</u>
Net income (loss) per share:				
Basic earnings (loss) per share	\$ (1.15)	\$ (1.35)	\$ 0.38	\$ (2.66)
Diluted earnings (loss) per share	\$ (1.15)	\$ (1.35)	\$ 0.37	\$ (2.66)
Weighted average number of shares of common stock used in calculating:				
Basic earnings (loss) per share	54,976	46,157	54,913	45,927
Diluted earnings (loss) per share	54,976	46,157	56,176	45,927

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net income (loss) - GAAP	\$ (63,046)	\$ (62,301)	\$ 21,044	\$ (122,071)
Research and development:				
Milestone payments	22,000	-	22,000	-
Stock-based compensation expense	2,195	2,404	4,069	4,853
Restructuring expense	104	511	174	1,013
Total research and development non-GAAP adjustments	24,299	2,915	26,243	5,866
Selling, general and administrative:				
Stock-based compensation expense	8,270	4,426	12,108	8,667
Restructuring expense	2,420	115	2,586	146
Total selling, general and administrative non-GAAP adjustments	10,690	4,541	14,694	8,813
EXONDYS 51 litigation and license charges - non-GAAP adjustment	2,839	-	2,839	-
Other income (loss):				
(Gain) from sale of intangible asset	-	-	(125,000)	-
Total other income (loss) non-GAAP adjustments	-	-	(125,000)	-
Income tax expense (benefit) - non-GAAP adjustments	(109)	-	1,891	-
Net income (loss) - non-GAAP	<u>\$ (25,327)</u>	<u>\$ (54,845)</u>	<u>\$ (58,289)</u>	<u>\$ (107,392)</u>
Non-GAAP net loss per share - basic and diluted	\$ (0.46)	\$ (1.19)	\$ (1.06)	\$ (2.34)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	54,976	46,157	54,913	45,927

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	<b>As of June 30, 2017</b>	<b>As of December 31, 2016</b>
Cash, cash equivalents, restricted cash and investments	\$ 301,730	\$ 329,324
Total assets	450,139	424,104
Total liabilities	72,367	87,413
Total stockholders' equity	\$ 377,772	\$ 336,691

Source: Sarepta Therapeutics, Inc.

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