Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2016 Financial Results and Recent Corporate Developments

CAMBRIDGE, Mass.--(BUSINESS WIRE)—February 28, 2017-- 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 three and twelve months ended December 31, 2016.

"2016 was a transformative year, with the FDA accelerated approval of EXONDYS 51. In 2017, we are focused on our strategy to build shareholder value by executing a successful launch of EXONDYS 51 in the US, reaching more patients through global expansion, and rapidly advancing our pipeline through internal and external development efforts," said Edward Kaye, Sarepta's chief executive officer. "We are pleased with the interest from the patient and physician community for EXONDYS 51, and with the progress we have made in discussions with payers. We believe this positions us well for potential growth and towards our goal at Sarepta Therapeutics to help all boys with Duchenne muscular dystrophy."

Financial Results

For the fourth quarter of 2016, Sarepta reported a net loss of $88.5 million, or $1.62 per share, compared to a net loss of $64.7 million for the same period of 2015, or $1.44 per share. The incremental loss of $23.8 million was primarily driven by expense recorded in connection with an up-front payment of $40.0 million related to an exclusive license agreement with Summit Therapeutics plc. ("Summit") offset by lower manufacturing expenses that were previously captured as research and development expenses, which are now capitalized as inventory because of the approval of EXONDYS 51 by the Food and Drug Administration ("FDA"). Non-GAAP net loss for the fourth quarter of 2016 was $38.7 million, or $0.71 per share, compared to a non-GAAP net loss of $58.3 million for the fourth quarter of 2015, or $1.30 per share. The reduction of $19.7

million in Non-GAAP net loss was primarily driven by the capitalization of inventory upon the approval of EXONDYS 51 by the FDA.

For the year ended December 31, 2016, Sarepta reported a net loss of $267.3 million, or $5.49 per share, compared to a net loss of $220.0 million for the prior year, or $5.20 per share. The incremental loss of $47.2 million was primarily driven by $47.9 million of research and development expenses recorded in connection with up-front license and milestone payments related to certain license and collaboration agreements and increased costs for our on-going clinical trials primarily due to increased patient enrollment, partially offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA. Non-GAAP net loss for the year ended December 31, 2016 was $192.0 million, or $3.94 per share, compared to a non-GAAP net loss of $187.9 million for the prior year, or $4.44 per share. The incremental loss of $4.0 million was primarily driven by increased costs for our on-going clinical trials primarily due to increased patient enrollment partially offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA.

Net revenues

The Company commenced shipments of EXONDYS 51 to customers at the end of the third quarter of 2016 following the accelerated approval by the FDA on September 19, 2016. For both the fourth quarter and full-year of 2016, the Company recognized net revenues of $5.4 million. For the same periods of 2015, the Company recognized $1.3 million of revenue from the contract finalization of the Ebola portion of the July 2010 Department of Defense contract.

Operating expenses

Research and development expenses were $70.7 million for the fourth quarter of 2016, compared to $41.4 million for the same period of 2015, an increase of $29.4 million, which was primarily driven by expense recorded in connection with an up-front payment of $40.0 million related to the exclusive license agreement with Summit offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA. Non-GAAP research and development expenses were $27.8 million for the fourth quarter of 2016, compared to $38.6 million for the same period of 2015, a decrease of $10.8 million, which was primarily driven by lower manufacturing expense because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA.

Research and development expenses were $188.3 million for the year ended December 31, 2016, compared to $146.4 million for the prior year, an increase of $41.9 million, which was primarily driven by $47.9 million of expenses recorded in connection with up-front license and milestone payments related to certain license and collaboration agreements and increased clinical trial costs, partially offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA. Non-GAAP research and development expenses were $136.0 million for both the year ended December 31, 2016, and the prior year.

Selling, general and administrative expenses were $22.9 million for the fourth quarter of 2016, compared to $24.3 million for the same period of 2015, a decrease of $1.4 million, which was primarily driven by decreased external professional services due to lower litigation activities partially offset by increases in restructuring expense and stock-based compensation expense. Non-GAAP selling, general and administrative expenses were $16.1 million for the fourth quarter of 2016, compared to $20.7 million for the same period of 2015, a decrease of $4.6 million, which was primarily driven by decreased external professional fees due to lower litigation activities.

Selling, general and administrative expenses for the year ended December 31, 2016 were $83.7 million, compared to $75.0 million for the prior year, an increase of $8.7 million, which was primarily driven by increases in compensation expenses due to increases in commercial headcount and restructuring expenses offset by decreases in severance expense related to the resignation of our former CEO in March 2015 and professional services primarily due to lower litigation activities. Non-GAAP selling, general and administrative expenses were $60.7 million for the year ended December 31, 2016, compared to $53.3 million for the same period of 2015, an increase of $7.4 million, which was primarily driven by increase in compensation expenses due to increased commercial headcount offset by decreased external professional fees due to lower litigation activities.

Cash, cash equivalents and restricted cash and investments

The Company had $329.3 million in cash, cash equivalents and restricted cash and investments as of December 31, 2016 compared to $204.0 million as of December 31, 2015, an increase of $125.4 million. The increase was driven by the net proceeds received from the Company's public offerings in June and September 2016, 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 operating expense 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 and 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 from non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP net loss and non-GAAP net loss per share facilitates comparisons of the Company’s operational performance in different periods.

2. Restructuring expenses

   Restructuring expenses have been excluded from non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP net loss and non-GAAP net loss per share as the Company believes that the adjustments for these items represent more closely the sustainability of the Company’s 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 up-front and options payments related to existing collaboration and option agreements.

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 operating expense 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 Agrees to Sale of Priority Review Voucher for $125M

-Sarepta Therapeutics Enters into Research Agreement and Option Agreement with Nationwide Children's Hospital for Microdystrophin Gene Therapy Program

-Sarepta Therapeutics Enters into License Agreement with Nationwide Children's Hospital for Galgt2 Gene Therapy Program

-Sarepta Therapeutics Announces EMA Validation of Eteplirsen Authorization Application for Treatment of Duchenne Muscular Dystrophy Amenable to Exon Skipping 51

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 drug candidates. For more information, please visit us at 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 con-

tained 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 2017, including executing a successful launch of EXONDYS 51 in the US, reaching more patients through global expansion and rapidly advancing Sarepta's pipeline through internal and external development efforts, and Sarepta's belief that it is well positioned for potential growth and towards its goal to help all boys with Duchenne muscular dystrophy.

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 profitability and 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 complete clinical trials required by the FDA for approval 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, bringing EXONDYS 51 to markets outside the United States 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 or Quarterly Reports 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 web site at 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 December 31,		Twelve Months Ended December 31,	
	2016	**2015**	**2016**	**2015**
Revenues:				
Product, net	$ 5,421	$ —	$ 5,421	$ —
Revenue from research contracts and other grants	—	1,253	—	1,253
Total revenues	5,421	1,253	5,421	1,253
Cost and expenses:				
Cost of sales	130	—	130	—
Research and development	70,749	41,376	188,272	146,394
Selling, general and administrative	22,937	24,329	83,749	75,043
Total cost and expenses	93,816	65,705	272,151	221,437
Operating loss	(88,395)	(64,452)	(266,730)	(220,184)
Interest (expense) income and other, net	(57)	(229)	(535)	154
Net loss	$ (88,452)	$ (64,681)	$ (267,265)	$ (220,030)
Net loss per share — basic and diluted	$ (1.62)	$ (1.44)	$ (5.49)	$ (5.20)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	54,619	44,882	48,697	42,290

Sarepta Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Net Loss

(in thousands, except per share amounts)

(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2016	**2015**	**2016**	**2015**
Net loss - GAAP	$ (88,452)	$ (64,681)	$ (267,265)	$ (220,030)
Research and development:				
Up-front license and milestone payments	40,785	—	40,785	—
Stock-based compensation expense	1,972	2,764	9,499	10,403
Restructuring expense	230	—	2,013	—
Total research and development non-GAAP adjustments	42,987	2,764	52,297	10,403
Selling, general and administrative:				
Stock-based compensation expense	4,897	3,584	20,463	21,714

Restructuring expense	1,909	—	2,549	—
Total selling, general and administrative non-GAAP adjustments	6,806	3,584	23,012	21,714
Net loss - non-GAAP	$ (38,659)	$ (58,333)	$ (191,956)	$ (187,913)
Non-GAAP net loss per share - basic and diluted	$ (0.71)	$ (1.30)	$ (3.94)	$ (4.44)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	54,619	44,882	48,697	42,290

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	As of December 31, 2016	**As of December 31, 2015**

Cash, cash equivalents and short-term investments	$ 317,845	$ 192,491
Restricted cash and investments	11,479	11,478
Total assets	424,104	273,782
Total liabilities	87,413	83,435
Total stockholders' equity	$ 336,691	$ 190,347

Source: Sarepta Therapeutics, Inc.

Media and Investors:

Sarepta Therapeutics, Inc.

Ian Estepan, 617-274-4052

iestepan@sarepta.com

or

W2O Group

Brian Reid, 212-257-6725

breid@w2ogroup.com