



Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2015 Financial Results and Recent Corporate Developments

- Announced May 26, 2016 PDUFA goal date for eteplirsen

- Ended year with \$204 million cash, cash equivalents, and marketable securities

CAMBRIDGE, Mass.--(BUSINESS WIRE)--February 25, 2016--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today reported financial results for the three months and year ended December 31, 2015, and provided an update of recent corporate developments.

“2015 marked a year of significant achievements for Sarepta, the most important being the filing of our New Drug Application for eteplirsen,” said Edward Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “2016 will be a pivotal year for the company as we prepare for an FDA advisory committee meeting and May 26th PDUFA date for eteplirsen.”

“Our 2015 year ending cash balance of \$204 million well positions the company to focus on our 2016 business objectives,” said Sandy Mahatme, Sarepta’s chief financial officer. “The majority of the cash used in the quarter was related to pre-commercialization activities for eteplirsen, manufacturing, and R&D. The rate of future cash expenditures will be largely determined by the upcoming FDA decision regarding the approval of eteplirsen.”

Financial Results

For the fourth quarter of 2015, Sarepta reported a non-GAAP net loss of \$58.3 million, or \$1.30 per share, compared to a non-GAAP net loss of \$38.6 million for the fourth quarter of 2014, or \$0.94 per share. The incremental loss of \$19.7 million was primarily the result of increased operating expenses.

On a GAAP basis, the net loss for the fourth quarter of 2015 was \$64.7 million, or \$1.44 per share (including \$6.3 million of stock-based compensation), compared to a



net loss of \$44.4 million, or \$1.08 per share (including \$5.8 million of stock-based compensation and restructuring expenses) for the fourth quarter of 2014. The increase in net loss is primarily the result of an increase of \$21.0 million in operating expenses offset by an increase of \$1.2 million in revenue from the Company's government contracts. The increase in operating expenses was primarily caused by research and development personnel growth, increased clinical activity in connection with our DMD programs and increased consulting and professional fees in the normal course of business.

Revenue for the fourth quarter of 2015 increased by \$1.2 million primarily due to the contract finalization of the Ebola portion of the Company's Ebola-Marburg U.S. government contract.

Non-GAAP research and development expenses were \$38.6 million for the fourth quarter of 2015, compared to \$28.4 million for the fourth quarter of 2014, an increase of \$10.2 million. GAAP research and development expenses were \$41.4 million for the fourth quarter of 2015 (including \$2.8 million of stock-based compensation), compared to \$30.8 million for the fourth quarter of 2014 (including \$2.4 million of stock-based compensation and restructuring expenses), an increase of \$10.6 million.

Non-GAAP general and administrative expenses were \$20.7 million for the fourth quarter of 2015, compared to \$10.5 million for the fourth quarter of 2014, an increase of \$10.2 million. GAAP general and administrative expenses were \$24.3 million for the fourth quarter of 2015 (including \$3.6 million of stock-based compensation expense), compared to \$13.9 million for the fourth quarter of 2014 (including \$3.4 million of stock-based compensation), an increase of \$10.4 million.

For the year ended December 31, 2015, the operating loss was \$220.2 million, compared to an operating loss of \$133.8 million for the prior year. The \$86.4 million increase was the result of a \$52.2 million increase in research and development expenses and a \$25.7 million increase in general and administrative expenses as well as an \$8.5 million decrease in revenue from the Company's government contracts.



Revenue for the year ended December 31, 2015 decreased by \$8.5 million from \$9.8 million for the year ended December 31, 2014 primarily due to the July 2010 expiration of the Marburg portion of the Company's Ebola-Marburg U.S. government contract.

Research and development expenses were \$146.4 million for the year ended December 31, 2015, compared to \$94.2 million for the prior year, an increase of \$52.2 million. The increase was primarily due to increases in clinical and manufacturing expenses, driven by increased enrollment in our ongoing clinical trials and timing of manufacturing activities (including raw material purchases) as well as expense incurred in connection with an amendment to a supply agreement, compensation expenses primarily driven by increases in headcount, stock-based compensation and facility-related expenses primarily driven by corporate growth.

General and administrative expenses for the year ended December 31, 2015 were \$75.0 million, compared to \$49.3 million for the prior year, an increase of \$25.7 million. The increase was primarily due to professional services driven by preparation for the potential product launch for eteplirsen if marketing approval is granted and increased legal fees, severance expense, including stock-based compensation as a result of the resignation of our former CEO, and compensation expenses primarily driven by increases in headcount.

The Company had \$204.0 million in cash, cash equivalents, short-term investments and restricted cash as of December 31, 2015 compared to \$211.1 million as of December 31, 2014, a decrease of \$7.1 million. The decrease was primarily driven by the use of cash to fund the Company's ongoing operations, offset by the net proceeds received from the exercises of warrants and stock options and the Company's public offering in October 2015.

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 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 certain items. The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating performance internally. The Company also believes these non-GAAP measures provide the Company's investors with useful information regarding the Company's historical operating results. These non-GAAP measures are not intended 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 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. 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

Duchenne Muscular Dystrophy Program

--Sarepta Therapeutics Has Not Yet Been Informed of a Rescheduled Date for the Peripheral and Central Nervous System Advisory Committee Meeting to Review Eteplirsen

--FDA Postpones Advisory Committee Meeting to Review Eteplirsen Due to Severe Weather Storm in the Washington D.C. Area

--Sarepta Therapeutics Receives Notification of PDUFA Extension for Eteplirsen

--Sarepta Therapeutics Announces Publication of Positive Long-Term Safety and Efficacy Data for Eteplirsen in the Annals of Neurology

About Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious, and other life-threatening diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne Muscular Dystrophy (DMD) drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the



treatment of infectious diseases, such as drug-resistant and other rare human diseases. 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 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, business plans, priorities and development of product candidates including: the potential Advisory Committee meeting to review the NDA for eteplirsen and the applicable PDUFA date, Sarepta's plans for 2016 including preparations for an Advisory Committee meeting and the PDUFA date, Sarepta's current cash balance well positioning the Company to focus on its 2016 business objectives, and the potential factors that may impact Sarepta's rate of future cash expenditures including any FDA decision on approval of the NDA for eteplirsen. .

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: the FDA may further delay scheduling of or cancel the advisory committee meeting or may further delay the new PDUFA date of May 26, 2016; we may not be able to comply with all FDA requests, including with respect to our eteplirsen NDA submission and the addendums we have submitted to the FDA or with respect to our ongoing or planned clinical trials, in a timely manner or at all; we may not be able to complete clinical trials required by the FDA for approval of our products or any submissions made in connection with our pipeline of product candidates; the results of our ongoing research and development efforts and clinical trials for eteplirsen and our other product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit,



support a positive advisory committee recommendation or approval of our NDA for eteplirsen or positive decisions on regulatory submissions for our other product candidates and/or Sarepta's antisense based technology platform; 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, including the planned commercialization of eteplirsen, 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, 2015 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,		Year ended December 31,	
	2015	2014	2015	2014
Revenues from grants and research contracts	\$ 1,253	\$ 27	\$ 1,253	\$ 9,757
Operating expenses:				
Research and development	41,376	30,832	146,394	94,231
General and administrative	24,329	13,917	75,043	49,315
Operating loss	(64,452)	(44,722)	(220,184)	(133,789)
Other income (loss):				
Interest (expense) income and other, net	(229)	306	154	779
Loss on change in warrant valuation	-	-	-	(2,779)
Net loss	\$ (64,681)	\$ (44,416)	\$ (220,030)	\$ (135,789)
Net loss per share - basic and diluted	\$ (1.44)	\$ (1.08)	\$ (5.20)	\$ (3.39)
Shares used in per share calculation basic and diluted	44,882	41,304	42,290	40,026



Sarepta Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Net Loss

(in thousands, except per share amounts)

(unaudited)

	Three months ended December 31,		Year ended December 31,	
	2015	2014	2015	2014
Net loss - GAAP	\$ (64,681)	\$ (44,416)	\$ (220,030)	\$ (135,789)
Research and development:				
Stock-based compensation expense	2,764	2,383	10,403	8,269
Restructuring Expense	-	3	-	14
Total research and development non-GAAP adjustments ¹	2,764	2,386	10,403	8,283
General and administrative:				
Stock-based compensation expense	3,584	3,384	21,714	12,076
Total general and administrative non-GAAP adjustments ¹	3,584	3,384	21,714	12,076
Other non-operating loss:				
(Gain) loss on change in warrant valuation non-GAAP adjustment	-	-	-	2,779
Net loss - non-GAAP	<u>\$ (58,333)</u>	<u>\$ (38,646)</u>	<u>\$ (187,913)</u>	<u>\$ (112,651)</u>
Non-GAAP net loss per share - basic and diluted	<u>\$ (1.30)</u>	<u>\$ (0.94)</u>	<u>\$ (4.44)</u>	<u>\$ (2.81)</u>
Shares used in per share calculations - basic and diluted	44,882	41,304	42,290	40,026

¹ Non-GAAP operating expense adjustments are comprised of total general and administrative non-GAAP adjustments and total research and development non-GAAP adjustments. Total non-GAAP operating expense adjustments were \$6,348 and \$5,770 for the three months ended December 31, 2015 and 2014, respectively. Total non-GAAP operating expense adjustments were \$32,117 and \$20,359 for the year ended December 31, 2015 and 2014, respectively.



Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	December 31, 2015	December 31, 2014
Cash, cash equivalents and short-term investments	\$ 192,491	\$ 210,344
Restricted investments	11,478	782
Total assets	273,782	295,033
Total liabilities	83,435	47,380
Total stockholders' equity	\$ 190,347	\$ 247,653

Source: Sarepta Therapeutics, Inc.

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