



Sarepta Therapeutics Announces Third Quarter 2015 Financial Results and Recent Corporate Developments

- *Well capitalized with \$111 million in cash and other investments at quarter end, with an additional \$120 million raised post-quarter end*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--November 5, 2015-- Sarepta Therapeutics, Inc.(NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today reported financial results for the three and nine months ended September 30, 2015, and provided an update of recent corporate developments.

“We are encouraged by the established body of clinical, biochemical and safety data for eteplirsen, which we plan to present at the tentatively scheduled Advisory Committee meeting in January,” said Edward Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “As our understanding of exon-skipping continues to evolve based on the data from our eteplirsen program, we remain focused on advancing additional programs and making new therapies available to Duchenne patients.”

Financial Results

For the third quarter of 2015, Sarepta reported a non-GAAP net loss of \$46.3 million, or \$1.11 per share, compared to a non-GAAP net loss of \$28.8 million for the third quarter of 2014, or \$0.70 per share. The incremental loss of \$17.5 million was primarily the result of increased operating expenses as well as a decrease in revenue from the Company’s government contracts.

On a GAAP basis, the net loss for the third quarter of 2015 was \$51.9 million, or \$1.25 per share (including \$5.7 million of stock-based compensation), compared to a net loss of \$29.2 million, or \$0.71 per share (including \$4.6 million of stock-based compensation) for the third quarter of 2014. The increase in net loss was primarily due to a decrease of \$1.1 million from government contract revenue and increases of \$14.8



million in research and development expenses and \$2.2 million in general and administrative expenses. The increase in operating expenses was primarily due to the timing of manufacturing activities, including the purchase of raw materials, increased clinical activity in connection with our DMD programs, research and development personnel growth and increased stock compensation expense. In addition, there was a gain of \$4.3 million from a gain on change in warrant valuation as all warrants were exercised or expired during 2014.

Revenue for the third quarter of 2015 decreased by \$1.1 million primarily due to the July 2014 expiration of the Marburg portion of the Company's Ebola-Marburg U.S. government contract.

Non-GAAP research and development expenses were \$34.0 million for the third quarter of 2015, compared to \$20.2 million for the third quarter of 2014, an increase of \$13.8 million. GAAP research and development expenses were \$36.7 million for the third quarter of 2015 (including \$2.6 million of stock-based compensation), compared to \$21.9 million for the third quarter of 2014 (including \$1.7 million of stock-based compensation), an increase of \$14.8 million. Non-GAAP general and administrative expenses were \$12.0 million for the third quarter of 2015, compared to \$9.9 million for the third quarter of 2014, an increase of \$2.1 million. GAAP general and administrative expenses were \$15.1 million for the third quarter of 2015 (including \$3.1 million of stock-based compensation expense), compared to \$12.9 million for the third quarter of 2014 (including \$3.0 million of stock-based compensation), an increase of \$2.2 million.

The Company had cash, cash equivalents, short-term investments and restricted investments of \$111.4 million as of September 30, 2015 compared to \$211.1 million as of December 31, 2014, a decrease of \$99.7 million. The decrease was primarily driven by the use of cash to fund the Company's ongoing operations.

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 expenses, 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 expenses, 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 Announces Tentative FDA Advisory Committee Meeting to Review Eteplirsen as a Treatment for Duchenne Muscular Dystrophy
- Sarepta Therapeutics Announces Additional Long-Term Efficacy and Safety Data from Phase IIb Program of Eteplirsen for the Treatment of Duchenne Muscular Dystrophy
- Sarepta Therapeutics Announces FDA Filing of Eteplirsen NDA for the Potential Treatment of Duchenne Muscular Dystrophy for Patients Amenable to Exon 51 Skipping
- Sarepta Therapeutics Receives Rare Pediatric Disease Designation from FDA for Eteplirsen for the Potential Treatment of Duchenne Muscular Dystrophy

Corporate Updates

- Sarepta Therapeutics Announces Public Offering of Common Stock



--Sarepta Therapeutics Announces Collaborative Research Agreement with Murdoch University Researchers Steve Wilton and Sue Fletcher

--Sarepta Therapeutics Appoints Jean-Paul Kress, M.D., to the Company's Board of Directors

--Sarepta Therapeutics Announces USPTO Decision in Patent Interference Case with BioMarin Pharmaceutical

-- Sarepta Therapeutics Announces Formation of Strategic and Scientific Advisory Board

Conference Call

The Company will be hosting a conference call at 8:00 a.m. EST, to discuss these financial results and other corporate updates. The conference call may be accessed by dialing 866-436-9172 for domestic callers and 630-691-2760 for international callers. The passcode for the call is 41044861. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2015 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. An audio replay will be accessible through November 19, 2015 by calling 888-843-7419 or 630-652-3042 and entering access code 4104 4861#.

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: Sarepta being well capitalized; Sarepta's plans to present the established body of clinical, biochemical and safety data for eteplirsen in the advisory committee meeting tentatively scheduled for January; Sarepta's evolving understanding of exon-skipping based on the data from the eteplirsen program; and focus on advancing additional programs and making new therapies available to Duchenne patients.

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 or cancel the tentative advisory committee meeting; we may not be able to comply with all FDA requests, including with respect to our eteplirsen NDA submission and 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 anti-sense 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 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 or 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 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 September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Revenues from grants and research contracts	\$ -	\$ 1,059	\$ -	\$ 9,730
Operating expenses:				
Research and development	36,673	21,852	105,018	63,399
General and administrative	15,090	12,882	50,714	35,398
Operating loss	<u>(51,763)</u>	<u>(33,675)</u>	<u>(155,732)</u>	<u>(89,067)</u>
Other income (loss):				
Interest (expense) income and other, net	(176)	193	383	473
Gain (loss) on change in warrant valuation	-	4,256	-	(2,779)
Net loss	<u>\$ (51,939)</u>	<u>\$ (29,226)</u>	<u>\$ (155,349)</u>	<u>\$ (91,373)</u>
Net loss per share - basic and diluted	<u>\$ (1.25)</u>	<u>\$ (0.71)</u>	<u>\$ (3.75)</u>	<u>\$ (2.31)</u>
Shares used in per share calculation basic and diluted	<u>41,565</u>	<u>41,066</u>	<u>41,416</u>	<u>39,595</u>



Sarepta Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Net Loss

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Net loss - GAAP	\$ (51,939)	\$ (29,226)	\$ (155,349)	\$ (91,373)
Research and development:				
Stock-based compensation expense	2,631	1,668	7,639	5,886
Restructuring Expense	-	-	-	11
Total research and development non-GAAP adjustments ¹	2,631	1,668	7,639	5,897
General and administrative:				
Stock-based compensation expense	3,052	2,981	18,130	8,692
Total general and administrative non-GAAP adjustments ¹	3,052	2,981	18,130	8,692
Other non-operating loss:				
(Gain) loss on change in warrant valuation non-GAAP adjustment	-	(4,256)	-	2,779
Net loss - non-GAAP	<u>\$ (46,256)</u>	<u>\$ (28,833)</u>	<u>\$ (129,580)</u>	<u>\$ (74,005)</u>
Non-GAAP net loss per share - basic and diluted	<u>\$ (1.11)</u>	<u>\$ (0.70)</u>	<u>\$ (3.13)</u>	<u>\$ (1.87)</u>
Shares used in per share calculations - basic and diluted	41,565	41,066	41,416	39,595

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 \$5,683 and \$4,649



for the three months ended September 30, 2015 and 2014, respectively. Total non-GAAP operating expense adjustments were \$25,769 and \$14,589 for the nine months ended September 30, 2015 and 2014, respectively.

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	September 30, 2015	December 31, 2014
Cash, cash equivalents and short-term investments	\$ 99,909	\$ 210,344
Restricted investments	11,478	782
Total assets	184,751	295,033
Total liabilities	61,515	47,380
Total stockholders' equity	\$ 123,236	\$ 247,653

Source: Sarepta Therapeutics, Inc.

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