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

- Pre-NDA meeting with FDA calendared for second quarter 2015 -

- NDA submission for eteplirsen planned for mid-year 2015 -

- Cash and Other Investments of $167 Million -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 7, 2015-- Sarepta Therapeutics, Inc.(NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today reported financial results for the three months ended March 31, 2015, and provided an update of recent corporate developments.

“Our highest priority remains the compilation of the data requested in the FDA’s most recent guidance. We have scheduled a meeting with the agency to discuss our data package and to ensure our expectations regarding an NDA submission for eteplirsen mid-year are aligned,” said Edward Kaye, Sarepta’s interim chief executive officer and chief medical officer. “The team at Sarepta is highly focused on developing our technology and committed to the advancement of our pipeline and treatments for follow-on exons. We have a number of active trials in the clinic and are continuing to work toward reaching more families affected by Duchenne muscular dystrophy.”

Financial Results

For the first quarter of 2015, Sarepta reported a non-GAAP net loss of $47.4 million, or $1.15 per share, compared to a non-GAAP net loss of $20.7 million for the first quarter of 2014, or $0.55 per share. The incremental loss of $26.7 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 first quarter of 2015 was $61.6 million, or $1.49 per share (including $14.2 million of stock-based compensation expense), compared with a net loss of $28.3 million for the first quarter of 2014, or $0.75 per

share (including $4.4 million of stock-based compensation and restructuring expenses). The increase in net loss was primarily due to a decrease of $6.1 million from government contract revenue and increases of $18.3 million from research and development expenses and $12.4 million from 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. These increases were offset by a decrease of $3.3 million from a loss on change in warrant valuation as all warrants were exercised or expired during 2014.

Revenue for the first quarter of 2015 decreased by $6.1 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 $36.7 million for the first quarter of 2015, compared to $19.0 million for the first quarter of 2014, an increase of $17.7 million. GAAP research and development expenses were $39.2 million for the first quarter of 2015 (including $2.4 million of stock-based compensation expense), compared to $20.9 million for the first quarter of 2014 (including $1.9 million of stock-based compensation and restructuring expenses), an increase of $18.3 million. Non-GAAP general and administrative expenses were $11.0 million for the first quarter of 2015, compared to $7.8 million for the first quarter of 2014, an increase of $3.2 million. GAAP general and administrative expenses were $22.7 million for the first quarter of 2015 (including $11.7 million of stock-based compensation expense), compared to $10.3 million for the first quarter of 2014 (including $2.5 million of stock-based compensation expense), an increase of $12.4 million.

The Company had cash, cash equivalents, short-term investments and restricted investments related to its letters of credit of $167.0 million as of March 31, 2015 compared to $211.1 million as of December 31, 2014, a decrease of $44.1 million.

The decrease was primarily driven by the use of cash to fund the Company's ongoing operations in the first quarter of 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 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

--Pre-NDA meeting with the FDA, to discuss the current guidance for eteplirsen, scheduled for second quarter, 2015.

--Sarepta remains on track in compiling the data requested by the FDA for a planned NDA submission for our lead drug candidate, eteplirsen, mid-year 2015.

Corporate Updates

--Sarepta Therapeutics appoints chief medical officer, Edward Kaye M.D., as interim chief executive officer.

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 39538738. Please specify to the operator that you would like to join the “Sarepta First 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 May 21, 2015 by calling 888-843-7419 or 630-652-3042 and entering access code 39538738#.

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 diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of drug-resistant bacteria and infectious, rare and other 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: prioritizing the compilation of data requested by the U.S. Food and Drug Administration (FDA); our planned meeting with the FDA in the second quarter to discuss our data package and to ensure our expectations regarding an NDA submission for eteplirsen mid-year are aligned; our focus to develop our technology and commitment to the advancement of our pipeline and treatments for follow-on exons; our active trials in the clinic and our continued work towards reaching more families affected by 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 capitalize on our executive team’s relationship and expertise to meet our expected timelines for our planned NDA submission, clinical development plans and bringing our product candidates to market; we may not be able to comply with all FDA requests, including with respect to our planned NDA submission, in a timely manner or at all; the FDA may determine that our NDA submission for eteplirsen is incomplete or does not qualify for filing, even if we provide additional supporting information and datasets requested; the additional information and data we collect for the eteplirsen NDA submission may not be consistent with prior data or results or may not support an eteplirsen NDA submission, filing or approval; we may not be able to complete clinical trials required by the FDA for approval of eteplirsen or our pipeline of product candidates and the results of our ongoing and new clinical trials may not be positive or consistent with prior results and may not support the safety and efficacy of or an NDA submission, filing or approval of eteplirsen, our other product candidates and/or Sarepta’s anti-sense based technology platform; there may be delays in our projected timelines relating to eteplirsen clinical studies, our planned NDA submission for eteplirsen, our planned meetings and discussions with the FDA, initiating new clinical trials for our product candidates, or making a product commercially available for various reasons including possible limitations of Company resources and regulatory or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; scale-up of manufacturing may not be successful and any or all of the Company’s drug candidates may fail in development or may not receive required regulatory approvals for commercialization (including potentially under an accelerated pathway); we may need and may not be able to obtain additional funds to conduct our planned research and development efforts and execute our business plans; 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 March 31,	
	2015	**2014**
Revenues from grants and research contracts	$ -	$ 6,088
Operating expenses:		
Research and development	39,165	20,906
General and administrative	22,697	10,303
Operating loss	(61,862)	(25,121)
Other non-operating income (loss):		
Interest income and other, net	303	99
Loss on change in warrant valuation	-	(3,251)
Net loss	$ (61,559)	$ (28,273)
Net loss per share – basic and diluted	$ (1.49)	$ (0.75)
Shares used in per share calculations – basic and diluted	41,324	37,821

Sarepta Therapeutics, Inc.

Reconciliation of GAAP to non-GAAP Net Loss
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2015	**2014**
Net loss – GAAP	$ (61,559)	$ (28,273)
Research and development:		
Stock-based compensation expense	2,446	1,873
Restructuring expense	-	9
Total research and development non-GAAP adjustments[1]	2,446	1,882
General and administrative:		
Stock-based compensation expense	11,710	2,469
Total general and administrative non-GAAP adjustments[1]	11,710	2,469
Other non-operating loss:		
Loss on change in warrant valuation non-GAAP adjustment	-	3,251
Net loss – non-GAAP	$ (47,403)	$ (20,671)
Non-GAAP net loss per share – basic and diluted	$ (1.15)	$ (0.55)
Shares used in per share calculations – basic and diluted	41,324	37,821

[1] Non-GAAP operating expense adjustments are comprised of total general and administrative non-GAAP adjustments plus total research and development non-GAAP adjustments. Total non-GAAP operating expense adjustments were $14,156 and $4,351 for the three months ended March 31, 2015 and 2014, respectively (in thousands).

Sarepta Therapeutics, Inc.

Balance Sheet Highlights
(in thousands)
(unaudited)

	March 31, 2015	*December 31, 2014*
Cash, cash equivalents and short-term investments	$ 166,223	$ 210,344
Restricted investments	783	782
Total assets	242,708	295,033
Total liabilities	41,949	47,380
Total stockholders' equity	$ 200,759	$ 247,653

Source: Sarepta Therapeutics, Inc.

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