



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

- NDA submission to FDA for eteplirsen completed on June 26, 2015 -

- Cash and Other Investments of \$158 Million -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--August 6, 2015-- Sarepta Therapeutics, Inc.(NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today reported financial results for the three and six months ended June 30, 2015, and provided an update of recent corporate developments.

“We made significant achievements from both a regulatory and clinical perspective this quarter and look to build on this momentum as we continue through the regulatory process for eteplirsen, our lead product candidate,” said Edward Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “We believe the recently published FDA draft guidance for Duchenne muscular dystrophy illustrates that the FDA is being responsive to the needs of patients and is open to providing appropriate flexibility in cases of unmet medical need and serious and life threatening diseases, such as Duchenne muscular dystrophy.”

Financial Results

For the second quarter of 2015, Sarepta reported a non-GAAP net loss of \$35.9 million, or \$0.87 per share, compared to a non-GAAP net loss of \$24.5 million for the second quarter of 2014, or \$0.61 per share. The incremental loss of \$11.4 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 second quarter of 2015 was \$41.9 million, or \$1.01 per share (including \$5.9 million of stock-based compensation), compared to a net loss of \$33.9 million, or \$0.85 per share (including \$5.6 million of stock-based



compensation and restructuring expenses) for the second quarter of 2014. The increase in net loss was primarily due to a decrease of \$2.6 million from government contract revenue and increases of \$8.5 million from research and development expenses and \$0.7 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 \$3.8 million from a loss on change in warrant valuation as all warrants were exercised or expired during 2014.

Revenue for the second quarter of 2015 decreased by \$2.6 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 \$26.6 million for the second quarter of 2015, compared to \$18.3 million for the second quarter of 2014, an increase of \$8.3 million. GAAP research and development expenses were \$29.2 million for the second quarter of 2015 (including \$2.6 million of stock-based compensation), compared to \$20.6 million for the second quarter of 2014 (including \$2.3 million of stock-based compensation and restructuring expenses), an increase of \$8.5 million. Non-GAAP general and administrative expenses were \$9.6 million for the second quarter of 2015, compared to \$9.0 million for the second quarter of 2014, an increase of \$0.6 million. GAAP general and administrative expenses were \$12.9 million for the second quarter of 2015 (including \$3.4 million of stock-based compensation expense), compared to \$12.2 million for the second quarter of 2014 (including \$3.2 million of stock-based compensation), an increase of \$0.7 million.

The Company had cash, cash equivalents, short-term investments and restricted investments related to a letter of credit of \$157.7 million as of June 30, 2015 compared to \$211.1 million as of December 31, 2014, a decrease of \$53.4 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 Completes NDA Submission to FDA for eteplirsen for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping.
- Data and Safety Monitoring Board (DSMB) recommends European Study 4053-101 to proceed to the Part 2, maintenance phase, of the study.

Infectious Diseases Program

- Sarepta Therapeutics Announces New England Journal of Medicine Publication of Phase I Clinical Data of Marburg Drug Candidate, AVI-7288, Supporting Safety of the PMOplus® platform.



Corporate Updates

- Sarepta Therapeutics appoints Henri Termeer as Advisor to the Company.
- Sarepta Therapeutics secures a \$40 million senior secured term loan, \$20 million of which has been drawn down and \$20 million of which would be available upon acceptance of the NDA for eteplirsen by the FDA.

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 40347977. Please specify to the operator that you would like to join the "Sarepta Second 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 August 20, 2015 by calling 888-843-7419 or 630-652-3042 and entering access code 40347977#.

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's plans to continue building on momentum as Sarepta continues through the regulatory process for eteplirsen and the belief that the FDA is being responsive to the needs of patients and is open to providing appropriate flexibility in cases of unmet medical need and serious life threatening diseases such as DMD.

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 relationships and expertise to meet our expected or planned regulatory milestones and timelines, 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 eteplirsen NDA submission and ongoing or planned clinical trials, 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, the additional information and data we collect for the eteplirsen may not be consistent with prior data or results or may not support an eteplirsen NDA filing, advisory committee positive recommendation or FDA 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, positive advisory committee recommendation or approval of eteplirsen, our other product candidates and/or Sarepta's anti-sense based technology platform; there may be delays in our projected regulatory timelines relating to our eteplirsen NDA submission, clinical studies, 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, court 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, development or commercialization 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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Revenues from grants and research contracts	\$ -	\$ 2,583	\$ -	\$ 8,671
Operating expenses:				
Research and development	29,180	20,641	68,345	41,547
General and administrative	12,927	12,213	35,624	22,516
Operating loss	(42,107)	(30,271)	(103,969)	(55,392)
Other income (loss):				
Interest income (expense) and other, net	256	181	559	280
Loss on change in warrant valuation	-	(3,784)	-	(7,035)
Net loss	\$ (41,851)	\$ (33,874)	\$ (103,410)	\$ (62,147)
Net loss per share – basic and diluted	\$ (1.01)	\$ (0.85)	\$ (2.50)	\$ (1.60)
Shares used in per share calculations – basic and diluted	41,357	39,862	41,341	38,847



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,	
	2015	2014	2015	2014
Net loss – GAAP	\$ (41,851)	\$ (33,874)	\$ (103,410)	\$ (62,147)
Research and development:				
Stock-based compensation expense	2,562	2,345	5,008	4,218
Restructuring expense	-	2	-	11
Total research and development non-GAAP adjustments ¹	2,562	2,347	5,008	4,229
General and administrative:				
Stock-based compensation expense	3,368	3,242	15,078	5,711
Total general and administrative non-GAAP adjustments ¹	3,368	3,242	15,078	5,711
Other non-operating loss:				
Loss on change in warrant valuation non-GAAP adjustment	-	3,784	-	7,035
Net loss – non-GAAP	\$ (35,921)	\$ (24,501)	\$ (83,324)	\$ (45,172)
Non-GAAP net loss per share – basic and diluted	\$ (0.87)	\$ (0.61)	\$ (2.02)	\$ (1.16)
Shares used in per share calculations – basic and diluted	41,357	39,862	41,341	38,847

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 \$5,930 and \$5,589 for the three months ended June 30, 2015 and 2014, respectively. Total non-GAAP operating expense adjustments were \$20,086 and \$9,940 for the six months ended June 30, 2015 and 2014, respectively.



Sarepta Therapeutics, Inc.

Balance Sheet Highlights
(in thousands)
(unaudited)

	<i>June 30,</i>	<i>December 31,</i>
	2015	2014
Cash, cash equivalents and short-term investments	\$ 156,887	\$ 210,344
Restricted investments	783	782
Total assets	231,710	295,033
Total liabilities	66,334	47,380
Total stockholders' equity	\$ 165,376	\$ 247,653

Source: Sarepta Therapeutics, Inc.

Media and Investors:

Ian Estepan, 617-274-4052

iestepan@sarepta.com

W2O Group

Ryan Flinn, 415-946-1059

Mobile: 510-207-7616

rflinn@w2ogroup.com