

## Sarepta Therapeutics Announces Second Quarter 2013 Financial Results and Recent Corporate Developments

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### **Submission of New Drug Application for Eteplirsen Planned for First Half of 2014; Preparations for Confirmatory Eteplirsen Study and Manufacturing Scale Up Remain on Track; Well Capitalized With \$164.0 Million in Cash and Other Investments With an Additional \$37.9 Million Raised Post-Quarter End**

CAMBRIDGE, MA -- (Marketwired) -- 08/08/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today reported financial results for the three months and six months ended June 30, 2013, and provided an update of recent corporate developments.

"We are excited that the FDA is open to an NDA filing for our drug eteplirsen for the treatment of Duchenne muscular dystrophy and the organization is focused on all of the activities necessary for a successful NDA submission in the first half of next year," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "The Sarepta team is planning for success as we continue activities related to our eteplirsen confirmatory study and our manufacturing scale up, while we begin pre-commercial activities to prepare for the potential approval of eteplirsen."

#### ***Financial Results***

For the second quarter of 2013, Sarepta reported a Non-GAAP net loss of \$14.6 million, or \$0.46 per share, compared to a Non-GAAP net loss of \$5.0 million for the second quarter of 2012, or \$0.22 per share. The incremental loss is primarily the result of an \$8.3 million decrease in government contract revenues as well as a \$1.2 million increase in Non-GAAP operating expenses.

On a GAAP basis, the net loss for the second quarter of 2013 was \$19.1 million, or \$0.60 per share (including \$2.5 million of stock-based employee compensation expense and restructuring expense), compared with a net income of \$8.0 million for the second quarter of 2012, or \$0.35 per diluted share (including \$0.4 million of stock-based employee compensation expense). The incremental loss is the result of a \$15.4 million change in warrant valuation, an \$8.3 million decrease in government contract revenues and a \$3.3 million increase in operating expenses.

Revenue for the second quarter of 2013 was \$3.0 million, down from \$11.2 million for the second quarter of 2012. The \$8.3 million decrease was primarily due to the August 2012 stop-work-order and subsequent termination for convenience of the Ebola portion of the Ebola-Marburg U.S. government contract due to a lack of available U.S. government funding. The termination of the Ebola portion did not impact the Marburg portion of the contract. Revenues from the Marburg portion of the contract also decreased during the second quarter of 2013 due to the timing of activities throughout the normal progression of the contract. These decreases were partially offset by revenue from the intramuscular administration (IM) contract with the U.S. government for the Marburg virus that started in August 2012.

Non-GAAP research and development expenses were \$12.2 million for the second quarter of 2013, compared to \$13.6 million for the second quarter of 2012, a decrease of \$1.4 million. GAAP research and development expenses were \$13.0 million for the second quarter of 2013 (including \$0.8 million of stock-based employee compensation expense and restructuring expense), compared to \$13.8 million for the second quarter of 2012 (including \$0.3 million of stock-based employee compensation expense), a decrease of \$0.8 million.

Non-GAAP general and administrative expenses were \$5.3 million for the second quarter of 2013, compared to \$2.7 million for the second quarter of 2012, an increase of \$2.6 million. GAAP general and administrative expenses were \$7.1 million for the second quarter of 2013 (including \$1.7 million of stock-based employee compensation expense and restructuring expense), compared to \$2.9 million for the second quarter of 2012 (including \$0.2 million of stock-based employee compensation expense), an increase of \$4.2 million.

The increased operating expenses were primarily caused by corporate growth as the Company continues the development of its programs in Duchenne muscular dystrophy and infectious diseases.

Sarepta had cash, cash equivalents and restricted investments related to our letters of credit of \$164.0 million as of June 30, 2013 compared to \$187.7 million as of December 31, 2012, a decrease of \$23.7 million. The cash was used to fund our ongoing

operations in 2013. Subsequent to second quarter end and up to August 7, the Company raised \$37.9 million in proceeds and issued approximately 1.0 million shares of common stock under the At-The-Market (ATM) equity financing that was put in place in July 2013.

In connection with prior equity financings, Sarepta issued warrants that are classified as current liabilities and are adjusted to fair value on a quarterly basis with the change in fair value being included in net loss. The amount included in net loss is a non-cash item as Sarepta is not required to expend any cash to settle the warrant liability. The warrant liability is primarily affected by changes in Sarepta's stock price. In the second quarter of 2013, the appreciation in Sarepta's stock price caused the warrant valuation to increase, which resulted in other expense of \$1.9 million. In the second quarter of 2012, a decrease in Sarepta's stock price resulted in other income of \$13.5 million. In the first six months of 2013, the change in the warrant liability resulted in a \$28.9 million non-cash charge to other non-operating loss and resulted in a \$2.6 million non-cash increase in other non-operating income in the comparable period in 2012. The warrant valuation gain or losses as well as stock-based employee compensation expense and restructuring costs related to our corporate move to Cambridge, are excluded from our Non-GAAP results.

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 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. We also believe these non-GAAP measures provide our investors with useful information regarding our historical operating results. These non-GAAP measures are not intended to replace the presentation of our financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP general and administrative 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***

-- Announced plans to submit a New Drug Application (NDA) for the approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD) patients with genotypes amenable to skipping of exon 51. The decision to submit an NDA for eteplirsen was based on productive interactions with the FDA including a meeting with the agency in July. In pre-meeting comments, the FDA stated it was open to considering an NDA based on results from the Phase IIb clinical study of eteplirsen. Sarepta expects to submit the NDA to the FDA in the first half of 2014.

-- Announced updated data from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with DMD. Results at 84 weeks showed a continued stabilization of walking ability in eteplirsen-treated patients evaluable on the 6-minute walk test (6MWT). As previously reported, Study 202 met its primary endpoint of increased novel dystrophin as assessed by muscle biopsy at week 48 and is now in the long-term extension phase in which patients continue to be followed for safety and clinical outcomes. Eteplirsen is Sarepta's lead exon-skipping compound in development for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

#### ***Corporate Update***

-- Entered into an At-the-Market (ATM) equity offering sales agreement with Further Lane Securities, L.P., on July 3, 2013, under which Sarepta may, from time to time, offer and sell shares of its common stock having an aggregate value of up to \$125 million through Further Lane. Sarepta intends to use any proceeds from this offering for general corporate purposes, including for manufacturing scale up for eteplirsen, the planned confirmatory Phase III clinical study of eteplirsen and early development activities related to follow-on Duchenne muscular dystrophy drugs and other programs.

#### ***Conference Call***

The conference call may be accessed by dialing 888.895.5271 for domestic callers and 847.619.6547 for international callers. The passcode for the call is 35366636. Please specify to the operator that you would like to join the "Sarepta Second Quarter Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at [www.sarepta.com](http://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 available through August 22, 2013 by calling 888.843.7419 or 630.652.3042 and entering access code 35366636.

### ***About Sarepta Therapeutics***

Sarepta Therapeutics is focused on developing first-in-class RNA-based therapeutics to improve and save the lives of people affected by serious and life-threatening rare and infectious diseases. Sarepta's diverse pipeline includes its lead program eteplirsen, for Duchenne muscular dystrophy, as well as potential treatments for some of the world's most lethal infectious diseases. Sarepta aims to build a leading, independent biotech company dedicated to translating its RNA-based science into transformational therapeutics for patients who face significant unmet medical needs. For more information, please visit us at [www.sarepta.com](http://www.sarepta.com).

### ***Forward-Looking Statements and Information***

*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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the potential for dystrophin as a surrogate marker, the amount and type of data as well as our ability to provide the data that will be necessary for the FDA's regulatory determinations regarding eteplirsen, the safety, efficacy, development and potential of Sarepta's product candidates, the potential and timing for regulatory submissions and meetings, the potential and timing for regulatory filings, review and approval of Sarepta's product candidates (including potentially under Subpart H Accelerated Approval), Sarepta's ability to establish and protect intellectual property rights, Sarepta's timing and ability to manufacture product candidates and Sarepta's estimates regarding its future revenue, operating loss, cash reserves and expenses and expectations regarding future success and funding from government and other sources.*

*These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from these forward-looking statements as a result of such risks and uncertainties. Known risk factors include, among others: clinical trials may not demonstrate safety and efficacy of any of Sarepta's drug candidates and/or Sarepta's antisense-based technology platform; development of any of Sarepta's drug candidates may not result in funding from the U.S. government in the anticipated amounts or on a timely basis, if at all; scale-up of manufacturing may not be successful and any of Sarepta's drug candidates may fail in development, may not receive required regulatory approvals (including potentially under Subpart H Accelerated Approval), or be delayed to a point where they do not become commercially viable; Sarepta may need additional funds to conduct research and development efforts; and those risks identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, and filed with the Securities and Exchange Commission, as well as the other information we file with the SEC.*

*Any of the foregoing risks could materially and adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the official corporate documents filed with the Securities and Exchange Commission. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.*

### **Sarepta Therapeutics, Inc.**

(A Development-Stage Company)

(in thousands, except per share amounts)

(unaudited)

	<b><i>Three Months Ended</i></b>		<b><i>Six Months Ended</i></b>	
	<b><i>June 30,</i></b>		<b><i>June 30,</i></b>	
	<b><i>2013</i></b>	<b><i>2012</i></b>	<b><i>2013</i></b>	<b><i>2012</i></b>
Revenues from grants and research contracts	\$ 2,951	\$ 11,207	\$ 7,425	\$ 22,419
Operating expenses:				
Research and development	12,984	13,849	26,746	28,654

General and administrative	7,054	2,915	13,181	6,196
Operating loss	(17,087)	(5,557)	(32,502)	(12,431)
Other non-operating income (loss):				
Interest income and other, net	(19)	107	218	203
Income (loss) on change in warrant liability	(1,945)	13,488	(28,851)	2,562
Net income (loss)	<u>\$ (19,051)</u>	<u>\$ 8,038</u>	<u>\$ (61,135)</u>	<u>\$ (9,666)</u>
Net income (loss) per share - basic	<u>\$ (0.60)</u>	<u>\$ 0.36</u>	<u>\$ (1.92)</u>	<u>\$ (0.43)</u>
Net income (loss) per share - diluted	<u>\$ (0.60)</u>	<u>\$ 0.35</u>	<u>\$ (1.92)</u>	<u>\$ (0.43)</u>
Shares used in per share calculations - basic	<u>31,984</u>	<u>22,624</u>	<u>31,899</u>	<u>22,624</u>
Shares used in per share calculations - diluted	<u>31,984</u>	<u>22,658</u>	<u>31,899</u>	<u>22,624</u>

***Sarepta Therapeutics, Inc.***

(A Development-Stage Company)

Reconciliation of GAAP to Non-GAAP Net Loss

(in thousands, except per share amounts)

(unaudited)

	<b><i>Three Months Ended</i></b>		<b><i>Six Months Ended</i></b>	
	<b><i>June 30,</i></b>		<b><i>June 30,</i></b>	
	<b><i>2013</i></b>	<b><i>2012</i></b>	<b><i>2013</i></b>	<b><i>2012</i></b>
Net income (loss) - GAAP	\$ (19,051)	\$ 8,038	\$ (61,135)	\$ (9,666)
Research and development:				
Stock-based compensation expense	724	259	1,254	512
Restructuring expense	<u>78</u>	<u>0</u>	<u>342</u>	<u>16</u>
Total Research and development Non-GAAP adjustments	802	259	1,596	528
General and administrative:				
Stock-based compensation expense	1,593	181	2,734	636
Restructuring expense	<u>131</u>	<u>0</u>	<u>329</u>	<u>37</u>
Total General and administrative Non-GAAP adjustments	1,724	181	3,063	673
Other non-operating income (loss):				
Adjust for change in warrant liability	<u>1,945</u>	<u>(13,488)</u>	<u>28,851</u>	<u>(2,562)</u>
Net loss - Non-GAAP <sup>I</sup>	<u>\$ (14,580)</u>	<u>\$ (5,010)</u>	<u>\$ (27,625)</u>	<u>\$ (11,027)</u>
Net loss per share - basic and diluted	<u>\$ (0.46)</u>	<u>\$ (0.22)</u>	<u>\$ (0.87)</u>	<u>\$ (0.49)</u>
Shares used in per share calculations - basic and diluted	<u>31,984</u>	<u>22,624</u>	<u>31,899</u>	<u>22,624</u>

<sup>I</sup> Non-GAAP operating income (loss) differs from Non-GAAP net income (loss) due to \$19 of other expense, net, and \$107 of net interest income for the three months ended June 30, 2013 and June 30, 2012, respectively, and due to \$218 and \$203 of net interest income for the six months ended June 30, 2013 and June 30, 2012, respectively.

***BALANCE SHEET HIGHLIGHTS***

(in thousands)

	<b><i>June 30,</i></b>	<b><i>December 31,</i></b>
	<b><i>2013</i></b>	<b><i>2012</i></b>
Cash and cash equivalents	\$ 156,185	\$ 187,661

Restricted Investments		7,807		---
Total assets		183,633		204,993
Total liabilities		94,812		81,314
Total shareholders' equity	\$	88,821	\$	123,679

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Source: Sarepta Therapeutics, Inc.