



Sarepta Therapeutics Announces Second Quarter 2014 Financial Results and Recent Corporate Developments

Progress achieved across eteplirsen clinical studies with patient screening expected to begin this month

On track to submit New Drug Application for eteplirsen by year-end

Follow-on exon-skipping drugs targeting exons 45 and 53 advancing toward clinical testing

Well capitalized with \$284.2 million in cash and other investments at quarter end

CAMBRIDGE, MA, August 7, 2014 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today reported financial results for the three and six months ended June 30, 2014, and provided an update of recent corporate developments.

"We've made tremendous progress in advancing our eteplirsen clinical trials and are ready to begin our confirmatory ambulatory study this month, the first of three new studies with eteplirsen," said President and Chief Executive Officer Chris Garabedian. "We continue to work on preparing our NDA submissions, while making progress on both our additional studies with eteplirsen and studies with follow-on exon-skipping drug candidates for Duchenne muscular dystrophy patients with other genotypes."

Financial Results

For the second quarter of 2014, Sarepta reported a non-GAAP net loss of \$24.5 million, or \$0.61 per share, compared to a non-GAAP net loss of \$14.6 million for the second quarter of 2013, or \$0.46 per share. The incremental loss of \$9.9 million was primarily the result of increased research and development and general and administrative expenses as a result of corporate growth.

On a GAAP basis, the net loss for the second quarter of 2014 was \$33.9 million, or \$0.85 per share (including \$5.6 million of stock-based compensation and restructuring expenses), compared to a net loss of \$19.1 million, or \$0.60 per share (including \$2.5 million of stock-based compensation and restructuring expenses) for the second quarter of 2013. The increase in net loss is primarily due to an increase of \$12.8 million in operating expenses, an increase of \$1.8 million in loss on change in warrant valuation and a decrease of \$0.4 million in contract revenue. Revenue for the second quarter of 2014 was \$2.6 million, down from \$3.0 million for the second quarter of 2013. The \$0.4 million decrease was primarily due to decreases in revenue from the Company's government contracts.

Non-GAAP research and development expenses were \$18.3 million for the second quarter of 2014, compared to \$12.2 million for the second quarter of 2013, an increase of \$6.1 million. GAAP research and development expenses were \$20.6 million for the second quarter of 2014 (including \$2.3 million of stock-based compensation and restructuring expenses), compared to \$13.0 million for the second quarter of 2013 (including \$0.8 million of stock-based compensation and restructuring expenses), an increase of \$7.6 million.

Non-GAAP general and administrative expenses were \$9.0 million for the second quarter of 2014, compared to \$5.3 million for the second quarter of 2013, an increase of \$3.7 million.



GAAP general and administrative expenses were \$12.2 million for the second quarter of 2014 (including \$3.2 million of stock-based compensation expense), compared to \$7.1 million for the second quarter of 2013 (including \$1.7 million of stock-based compensation and restructuring expenses), an increase of \$5.1 million.

The increase in operating expenses was primarily caused by corporate growth, including an expansion of manufacturing, pre-commercial and medical affairs activities as the Company prepares for the possibility of a product approval next year. The company also continues to expand clinical and regulatory activities in support of the development of its programs in Duchenne muscular dystrophy (DMD).

The Company had cash, cash equivalents, short-term investments and restricted investments related to a letter of credit of \$284.2 million as of June 30, 2014 compared to \$264.9 million as of December 31, 2013, an increase of \$19.3 million. The increase was primarily driven by the net proceeds received from the Company's public offering in April 2014, offset by the use of cash to fund the Company's ongoing operations in the first half of 2014.

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."

2014 Guidance

The Company now expects that Non-GAAP loss from operations will range from \$135 to \$145 million, as compared with its previous guidance of \$110 to \$120 million. In addition, The Company is anticipating capital investments of approximately \$25 million for the remainder of 2014 in connection with its manufacturing facility in Andover and 2015 inventory commitments. The Company is not able to provide a reconciliation of this Non-GAAP guidance to its relevant GAAP measure because full year loss from operations could include incremental stock compensation expense related to the achievement of certain criteria for performance awards.

Recent Corporate Developments

Duchenne Muscular Dystrophy Program

-- Announced updated data from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with DMD. Results on the 6-minute walk test (6MWT) at 144 weeks showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history data. In addition, a continued stabilization of respiratory muscle function was observed. 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.

Corporate Updates

- Acquisition of the multifunctional manufacturing facility on 26 acres of land in Andover, Massachusetts supports large-scale manufacturing needs.
- John Hodgman was named interim chairman of the board of directors.

Conference Call

The Company will be hosting a conference call to discuss these financial results and other corporate updates. The conference call may be accessed by dialing 800-708-4539 for domestic callers and 847-619-6396 for international callers. The passcode for the call is 37748110. Please specify to the operator that you would like to join the "Sarepta Second Quarter 2014 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 21, 2014 by calling 888-843-7419 or 630-652-3042 and entering access code 37748110.

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. The Company'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 disease. 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.

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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements may include statements relating to Sarepta's future operations, financial performance, business plans and development of product candidates including; being well capitalized; the expected timing of (i) patient screening for eteplirsen clinical studies, (ii) planned submission of a New Drug Application for eteplirsen, and (iii) advancement of drug candidates targeting exons 45 and 53 toward clinical testing; and Sarepta's Non-GAAP anticipated loss from operations and capital investments.

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: risks specific to obtaining FDA approval for eteplirsen including: we may not be able to comply with all FDA requests in a timely manner or at all; the FDA may determine that our NDA submission for eteplirsen does not qualify for filing, even if we provide additional supporting information and data; we may not be able to complete clinical trials the FDA requires for approval of eteplirsen and the results of our ongoing and new clinical trials may not be positive or consistent with prior results including possible failure to obtain results supporting safety and efficacy of eteplirsen, other product candidates and/or Sarepta's anti-sense based technology platform; there may be delays in our projected timelines relating to patient



screenings for eteplirsen clinical studies, the NDA submission, initiating new clinical trials for eteplirsen or other product candidates in our pipeline, or making a product commercially available for various reasons including possible limitations of Company resources and regulatory or agency decisions, including decisions by the United States Patent and Trademark Office with respect to the 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 (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 Annual Report on Form 10-Q for the quarter ended June 30, 2014 filed with the Securities and Exchange Commission 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. 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.
 Condensed Consolidated Statements of Operations
 (in thousands, except per share amounts)
 (unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Revenues from grants and research contracts	\$ 2,583	\$ 2,951	\$ 8,671	\$ 7,425
Operating expenses:				
Research and development	20,641	12,984	41,547	26,746
General and administrative	12,213	7,054	22,516	13,181
Operating loss	<u>(30,271)</u>	<u>(17,087)</u>	<u>(55,392)</u>	<u>(32,502)</u>
Other income (loss):				
Interest income (expense) and other, net	181	(19)	280	218
Loss on change in warrant valuation	<u>(3,784)</u>	<u>(1,945)</u>	<u>(7,035)</u>	<u>(28,851)</u>
Net loss	<u>\$ (33,874)</u>	<u>\$ (19,051)</u>	<u>\$ (62,147)</u>	<u>\$ (61,135)</u>
Net loss per share – basic and diluted	<u>\$ (0.85)</u>	<u>\$ (0.60)</u>	<u>\$ (1.60)</u>	<u>\$ (1.92)</u>
Shares used in per share calculations – basic and diluted	39,862	31,984	38,847	31,899



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,	
	2014	2013	2014	2013
Net loss – GAAP	\$ (33,874)	\$ (19,051)	\$ (62,147)	\$ (61,135)
Research and development:				
Stock-based compensation expense	2,345	724	4,218	1,254
Restructuring expense	2	78	11	342
Total research and development non-GAAP adjustments ¹	2,347	802	4,229	1,596
General and administrative:				
Stock-based compensation expense	3,242	1,594	5,711	2,735
Restructuring expense	-	131	-	329
Total general and administrative non-GAAP adjustments ¹	3,242	1,725	5,711	3,064
Other non-operating loss:				
Loss on change in warrant valuation non-GAAP adjustment	3,784	1,945	7,035	28,851
Net loss – non-GAAP	\$ (24,501)	\$ (14,579)	\$ (45,172)	\$ (27,624)
Non-GAAP net loss per share – basic and diluted	\$ (0.61)	\$ (0.46)	\$ (1.16)	\$ (0.87)
Shares used in per share calculations – basic and diluted	39,862	31,984	38,847	31,899

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 \$5,589 and \$2,527 for the three months ended June 30, 2014 and 2013, respectively. Total non-GAAP operating expense adjustments were \$9,940 and \$4,660 for the six months ended June 30, 2014 and 2013, respectively.



Sarepta Therapeutics, Inc.

Balance Sheet Highlights
(in thousands)
(unaudited)

	June 30, 2014	December 31, 2013
Cash, cash equivalents and short-term investments	\$ 279,539	\$ 256,965
Restricted investments	4,647	7,897
Total assets	337,309	291,569
Total liabilities	37,388	44,377
Total stockholders' equity	\$ 299,921	\$ 247,192

Sarepta Investor Contact:
Lilian Stern, 212 362 1200
lilian@sternir.com

or

Sarepta Media Contact:
Tony Plohoros, 908 591 2839
tplohoros@6degreespr.com