



## **Sarepta Therapeutics Announces Fourth Quarter and Full-Year 2014 Financial Results and Recent Corporate Developments**

- *NDA submission for eteplirsen planned for mid-year 2015 -*
- *Plan to discuss new data and NDA submission with FDA in 2nd Quarter –*
- *Cash and Other Investments of \$211 Million at Year End 2014 -*

**CAMBRIDGE, MA, February 26, 2015** -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-targeted therapeutics, today reported financial results for the three months and year ended December 31, 2014, and provided an update of recent corporate developments.

“Last month we released 168 week data from our Phase IIb study of eteplirsen. We believe the long-term safety of our drug with the continued ambulation and stability of pulmonary function in these boys are encouraging considering the length of follow up and the advancing age of the boys,” said Chris Garabedian, president and chief executive officer. “We are actively collecting and analyzing the additional datasets requested by the FDA and plan to meet with the FDA in the second quarter to ensure that we are aligned with their expectations for an acceptable NDA filing. We continue to work towards a complete NDA submission by mid-year 2015.”

### ***Financial Results***

For the fourth quarter of 2014, Sarepta reported a non-GAAP net loss of \$38.6 million, or \$0.94 per share, compared to a non-GAAP net loss of \$29.1 million for the fourth quarter of 2013, or \$0.77 per share. The incremental loss of \$9.5 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 fourth quarter of 2014 was \$44.4 million, or \$1.08 per share (including \$5.8 million of stock-based compensation and restructuring expenses), compared to a net loss of \$8.8 million, or \$0.23 per share (including \$3.7 million of stock-based compensation and restructuring expenses) for the fourth quarter of 2013. The increase in net loss is primarily the result of a \$24.0 million decrease in gain on change in warrant valuation, an increase of \$9.3 million in operating expenses as a result of corporate growth and a decrease of \$2.6 million in revenue from the Company’s government contracts.

Revenue for the fourth quarter of 2014 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 \$28.4 million for the fourth quarter of 2014, compared to \$23.6 million for the fourth quarter of 2013, an increase of \$4.8 million. GAAP research and

development expenses were \$30.8 million for the fourth quarter of 2014 (including \$2.4 million of stock-based compensation and restructuring expenses), compared to \$25.1 million for the fourth quarter of 2013 (including \$1.5 million of stock-based compensation and restructuring expenses), an increase of \$5.7 million.

Non-GAAP general and administrative expenses were \$10.5 million for the fourth quarter of 2014, compared to \$8.2 million for the fourth quarter of 2013, an increase of \$2.3 million. GAAP general and administrative expenses were \$13.9 million for the fourth quarter of 2014 (including \$3.4 million of stock-based compensation expense), compared to \$10.4 million for the fourth quarter of 2013 (including \$2.2 million of stock-based compensation and restructuring expenses), an increase of \$3.5 million.

The increase in operating expenses was primarily caused by research and development personnel growth, increased clinical activity in connection with our DMD programs and increased consulting and professional fees in the normal course of business.

For the year ended December 31, 2014, the operating loss was \$133.8 million, compared to an operating loss of \$90.3 million for the prior year. The \$43.5 million increase was the result of a \$21.3 million increase in research and development expenses and a \$17.7 million increase in general and administrative expenses as well as a \$4.5 million decrease in revenue from the Company's government contracts.

Revenue for the year ended December 31, 2014 decreased to \$9.8 million from \$14.2 million for the year ended December 31, 2013 primarily due to the July 2014 expiration of the Marburg portion of the Company's Ebola-Marburg U.S. government contract.

Research and development expenses were \$94.2 million for the year ended December 31, 2014, compared to \$72.9 million for the prior year, an increase of \$21.3 million. The increase was primarily driven by preparation for the potential launch of eteplirsen, if market approval is obtained, as well as the advancement of our early- and late-stage research and development pipeline.

General and administrative expenses for the year ended December 31, 2014 were \$49.3 million, compared to \$31.6 million for the prior year, an increase of \$17.7 million. The increase was primarily due to increased personnel costs, professional services and facility-related expenses.

The Company had \$211.1 million in cash, cash equivalents, short-term investments and restricted cash as of December 31, 2014 compared to \$264.9 million as of December 31, 2013, a decrease of \$53.8 million. The decrease was primarily driven by the use of cash to fund the Company's ongoing operations, offset by the net proceeds received from the exercises of warrants and stock options and the Company's public offering in April 2014. Guidance on non-GAAP operating loss for 2015 will be provided during the first quarter earnings call.

*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 expense adjustments, 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 expense adjustments, 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*

--Presented 168 week data from Study 202, a long-term Phase IIb extension study of eteplirsen in patients with Duchenne muscular dystrophy (DMD). Results through more than three years of treatment showed continued stability of respiratory muscle function, as assessed by pulmonary function tests. Results of the 6 minute walk test showed continued ambulation across all patients evaluable on the test, however all patients showed a decline in distance walked on this measure since the week 144 timepoint.

--Announced dosing in an open-label study, 4658-204 (Study 204), of eteplirsen in non-ambulant patients with DMD. The study, which is being conducted at several sites across the United States and is designed to evaluate the safety of eteplirsen over 96 weeks of dosing, will include approximately 20 patients who have genotypes amenable to exon 51 skipping.

--Announced dosing in an open-label confirmatory study, 4658-301 (PROMOVI), of eteplirsen in ambulant patients with DMD. The study, which is being conducted at 39 sites across the United States and is designed to evaluate the efficacy and safety of eteplirsen in patients over 48 weeks of dosing, will include up to 80 boys, aged 7-16 years, with genotypes amenable to exon 51 skipping.

--Announced dosing in first European Phase I/II Study of SRP-4053 to assess the safety, tolerability, efficacy, and pharmacokinetics in DMD patients with genotypes amenable to exon-53 skipping. The study is being conducted at four sites in Europe under a consortium agreement between Sarepta and various European hospitals, institutions, and scientists established to conduct the study and which is funded in part by the European Union's Seventh Programme for research, technological development, and demonstration under grant agreement No. 305370.

### *Infectious Diseases Program-*

--Published results from a study done in collaboration with the U.S. Army Medical Research Institute of Infectious Diseases (USAMRIID) on the efficacy of Sarepta's PMO, AVI-7537, against the Ebola virus in Rhesus monkeys. The study demonstrated 75% effectiveness across the infected population treated with AVI-7537 compared to 0% survival in the control group.

## 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 800-708-4539 for domestic callers and 847-619-6396 for international callers. The passcode for the call is 38999966. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 2014 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 accessible through March 12, 2015 by calling 888-843-7419 or 630-652-3042 and entering access code 38999966#.

## 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](http://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 include statements relating to Sarepta's future operations, financial performance, business plans, priorities and development of product candidates including: our belief that the long-term safety of eteplirsen with the continued ambulation and stability of pulmonary function in boys participating in our eteplirsen extension study are encouraging; our continued efforts to collect and analyze the additional datasets requested by the U.S. Food and Drug Administration (FDA) and our plans to meet with the FDA in the second quarter to ensure that we are aligned with their expectations for an acceptable New Drug Application (NDA) filing, and our plans for and continued work towards a complete NDA submission by mid-year 2015 .*

*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 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 Annual Report on Form 10-K for the year ended December 31, 2014 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.*

#### **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](http://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 December 31,		Year Ended December 31,	
	2014	2013	2014	2013
Revenue from research contracts and other grants	\$ 27	\$ 2,626	\$ 9,757	\$ 14,219
Operating expenses:				
Research and development	30,832	25,076	94,231	72,909
General and administrative	13,917	10,399	49,315	31,594
Operating loss	(44,722)	(32,849)	(133,789)	(90,284)
Other income (loss):				
Interest income and other, net	306	45	779	326
Gain (loss) on change in warrant valuation	-	23,984	(2,779)	(22,027)
Net loss	\$ (44,416)	\$ (8,820)	\$ (135,789)	\$ (111,985)
Net loss per share – basic and diluted	\$ (1.08)	\$ (0.23)	\$ (3.39)	\$ (3.31)
Shares used in per share calculations – basic and diluted	41,304	37,596	40,026	33,850

**Sarepta Therapeutics, Inc.**  
Reconciliation of GAAP to Non-GAAP Net Loss  
(in thousands, except per share amounts)  
(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2014	2013	2014	2013
Net loss – GAAP	\$ (44,416)	\$ (8,820)	\$ (135,789)	\$ (111,985)
Research and development:				
Stock-based compensation expense	2,383	1,479	8,269	3,888
Restructuring expense	3	17	14	414
Total research and development non-GAAP adjustments <sup>1</sup>	2,386	1,496	8,283	4,302
General and administrative:				
Stock-based compensation expense	3,384	2,173	12,076	7,239
Restructuring expense	-	21	-	350
Total general and administrative non-GAAP adjustments <sup>1</sup>	3,384	2,194	12,076	7,589
Other non-operating loss:				
Gain (loss) on change in warrant valuation non-GAAP adjustment	-	23,984	(2,779)	(22,027)
Net loss – non-GAAP	\$ (38,646)	\$ (29,114)	\$ (112,651)	\$ (78,067)
Non-GAAP net loss per share – basic and diluted	\$ (0.94)	\$ (0.77)	\$ (2.81)	\$ (2.31)
Shares used in per share calculations – basic and diluted	41,304	37,596	40,026	33,850

<sup>1</sup> Non-GAAP operating expense adjustments are comprised of general and administrative non-GAAP adjustments and research and development non-GAAP adjustments. Total non-GAAP operating expense adjustments were \$5,770 and \$3,690 for the three months ended December 31, 2014 and 2013, respectively. Total non-GAAP operating expense adjustments were \$20,359 and \$11,891 for the year ended December 31, 2014 and 2013, respectively.

**Sarepta Therapeutics, Inc.**

**Balance Sheet Highlights**

(in thousands)

**(unaudited)**

	<b>December 31,</b> <b>2014</b>	<b>December 31,</b> <b>2013</b>
Cash, cash equivalents and short-term investments	\$ 210,344	\$ 256,965
Restricted cash and investments	782	7,897
Total assets	295,033	291,569
Total liabilities	47,380	44,377
Total stockholders' equity	\$ 247,653	\$ 247,192

Source: Sarepta Therapeutics, Inc.

Media and Investors:  
Sarepta Therapeutics, Inc.  
Ian Estepan, 617-274-4052  
iestepan@sarepta.com