Sarepta Therapeutics Announces Fourth Quarter and Full Year 2013 Financial Results and Recent Corporate Developments

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Clarity on Eteplirsen Confirmatory Trial Design Expected in Coming Weeks

2014 Financial Guidance of $110-120 Million in Non-GAAP Operating Loss

Cash and Other Investments of $265 Million at Year End 2013

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 27, 2014--

Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today reported financial results for the three months and year ended December 31, 2013, and provided an update of recent corporate developments.

“We continue to be encouraged by the eteplirsen clinical data through 120 weeks and the general stability we’ve observed on the 6-minute walk test and pulmonary function measures,” said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. “We look forward to our continued discussions with the FDA to gain clarity in the coming weeks on the clinical path forward for eteplirsen.”

Financial Results

For the fourth quarter of 2013, Sarepta reported a non-GAAP net loss of $29.1 million, or $0.77 per share, compared to a non-GAAP net loss of $8.9 million for the fourth quarter of 2012, or $0.34 per share. The incremental loss is primarily the result of a $4.7 million decrease in contract revenues as well as a $15.5 million increase in non-GAAP operating expenses, due to corporate growth.

On a GAAP basis, the net loss for the fourth quarter of 2013 was $8.8 million, or $0.23 per share (including $3.7 million of stock-based compensation and restructuring expenses), compared with a net loss of $62.1 million for the fourth quarter of 2012, or $2.36 per share (including $1.4 million of stock-based compensation and restructuring expenses). The decrease in net loss is the result of a $75.8 million decrease in expense incurred due to the change in valuation of the Company’s outstanding warrants, offset by a $4.7 million decrease in contract revenues and a $17.8 million increase in operating expenses.

Revenue for the fourth quarter of 2013 was $2.6 million, down from $7.3 million for the fourth quarter of 2012. The $4.7 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 fourth 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 Company’s European Union SKIP-NMD agreement supporting development of an exon 53 skipping therapeutic.

Non-GAAP research and development expenses were $23.6 million for the fourth quarter of 2013, compared to $12.4 million for the fourth quarter of 2012, an increase of $11.2 million. GAAP research and development expenses were $25.1 million for the fourth quarter of 2013 (including $1.5 million of stock-based compensation and restructuring expenses), compared to $12.8 million for the fourth quarter of 2012 (including $0.4 million of stock-based compensation and restructuring expenses), an increase of $12.3 million.

Non-GAAP general and administrative expenses were $8.2 million for the fourth quarter of 2013, compared to $3.9 million for the fourth quarter of 2012, an increase of $4.3 million. GAAP general and administrative expenses were $10.4 million for the fourth quarter of 2013 (including $2.2 million of stock-based compensation and restructuring expenses), compared to $4.9 million for the fourth quarter of 2012 (including $0.9 million of stock-based compensation and restructuring expenses), an increase of $5.5 million.

The increased operating expenses were primarily caused by corporate growth as the Company continues the development of its

programs in Duchenne muscular dystrophy (DMD).

For the year ended December 31, 2013 the operating loss was $90.3 million, compared to an operating loss of $29.7 million for the prior year. The $60.6 million increase was the result of a $20.5 million increase in research and development expenses and a $17.0 million increase in general and administrative expenses as well as a $23.1 million decrease in revenue from research contracts.

Revenue for the year ended December 31, 2013 decreased to $14.2 million from $37.3 million in 2012 primarily due to the August 2012 stop-work-order and subsequent termination of the Ebola portion of the Ebola-Marburg U.S. government contract due to lack of available U.S. government funding.

Research and development expenses were $72.9 million for 2013, compared to $52.4 million for the prior year, a $20.5 million increase. The increase was primarily due to an increase in the Company's DMD program and proprietary research costs offset by a decrease in costs under the Company's government Ebola and Marburg contracts.

General and administrative expenses for 2013 were $31.6 million, compared to $14.6 million for 2012, an increase of $17.0 million. The increase was primarily due to increased personnel costs as well as increased professional service costs compared to the prior year.

The Company had cash, cash equivalents and restricted investments related to its letters of credit of $264.9 million as of December 31, 2013 compared to $187.7 million as of December 31, 2012, an increase of $77.2 million. The increase in cash and cash equivalents was primarily due to $125 million in proceeds from the issuance of approximately 3.4 million shares of common stock under the At-the-Market (ATM) equity financing that was put in place in July 2013 and $18.9 million in proceeds from the exercise of warrants and stock options, offset by cash used to fund the Company's ongoing operations.

The warrant liability is primarily affected by changes in the company's stock price during each financial reporting period which causes the warrant liability to fluctuate as the market price of the Company's stock fluctuates.

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

For 2014, the Company anticipates that loss from operations, excluding stock-based compensation, will be in the $110 to $120 million range. This guidance is largely based on continuing development and scale-up manufacturing for eteplirsen and the Company's follow-on DMD drugs, as well as increased investment in research with our platform technology.

Recent Corporate Developments

Duchenne Muscular Dystrophy Program

-- Announced new pulmonary function data through Week 120 from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with Duchenne muscular dystrophy (DMD). Results through more than two years of treatment showed stable pulmonary function in the Intent-to-Treat (ITT) study population (N=12). These data are consistent with previously reported 120-week clinical data showing a general stabilization of walking ability in eteplirsen-treated patients evaluable on the 6-minute walk test (6MWT).

-- Announced 6MWT data through Week 120 from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with DMD. Results through more than two years showed a continued stabilization of walking ability in eteplirsen-treated patients evaluable on the 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.

Infectious Disease Programs

-- Announced positive safety results from a Phase I multiple ascending dose study of AVI-7288 in healthy volunteers. AVI-7288, which uses Sarepta's advanced and proprietary PMO*plus*™ chemistry, is the company's lead drug candidate for the treatment of Marburg virus infection. Sarepta has been developing AVI-7288 under a Department of Defense contract managed by the Medical Countermeasure Systems BioDefense Therapeutics (MCS-BDTX) Joint Product Management Office.

Corporate Updates

-- Announced Arthur "Art" Krieg, M.D., was named senior vice president and chief scientific officer. In this role, Dr. Krieg will lead the company's drug discovery and early-stage research activities.

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 36644302. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 2013 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com. 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 March 13, 2014 by calling 888.843.7419 or 630.652.3042 and entering access code 36644302.

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.

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 including statements relating to 2014 financial guidance and the potential and timing for obtaining clarity on a pivotal trial design for eteplirsen and, more generally, its path forward. 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 regarding the Company's future revenue, operating loss, cash reserves and expenses, expectations regarding future success, funding from government and other sources and other statements relating to the company's future operations, financial performance, business plans and development of product candidates. 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 or subsequent clinical trials may fail to replicate safety and efficacy data for a product candidate, including eteplirsen; any of Sarepta's drug candidates may fail in development, may not receive required regulatory approvals (including potentially under expedited approval pathways that may be available), or may not become commercially viable due to delays or other reasons; scale-up of manufacturing of drug product may not be achieved on a timely basis depending on our clinical trial and commercialization needs; development of any of Sarepta's drug candidates being developed under agreements with the U.S. government may not result in funding from the U.S. government in the anticipated amounts or on a timely basis, if at all; Sarepta may need additional funds to conduct

research and development efforts; and those risks identified under the heading "Risk Factors" in Sarepta's most recently filed Annual Report on Form 10-K and Quarterly Report on Form 10-Q with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta.

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. You should not place undue reliance on forward-looking statements. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the official corporate documents filed with the SEC. The forward-looking statements included in this press release are based on the beliefs and expectations of Sarepta's management as of the data of this press release. 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)
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		***Year Ended December 31,***	
	2013	***2012***	***2013***	***2012***
Revenues from grants and research contracts	$2,626	$7,336	$14,219	$37,329
Operating expenses:				
Research and development	25,076	12,834	72,909	52,402
General and administrative	10,399	4,868	31,594	14,630
Operating loss	(32,849)	(10,366)	(90,284)	(29,703)
Other non-operating income (loss):				
Interest income and other, net	45	83	326	354
Gain (loss) on change in warrant valuation	23,984	(51,784)	(22,027)	(91,938)
Net loss	$(8,820)	$(62,067)	$(111,985)	$(121,287)
Net loss per share – basic and diluted	$(0.23)	$(2.36)	$(3.31)	$(5.14)
Shares used in per share calculations – basic and diluted	37,596	26,313	33,850	23,602

Sarepta Therapeutics, Inc.
(A Development-Stage Company)
Reconciliation of GAAP to non-GAAP net loss
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		***Year Ended December 31,***	
	2013	***2012***	***2013***	***2012***
Net loss - GAAP	$(8,820)	$(62,067)	$(111,985)	$(121,287)
Research and development:				
Stock-based compensation expense	1,479	390	3,888	1,173
Restructuring expense	17	53	414	69
Total research and development non-GAAP adjustments[2]	1,496	443	4,302	1,242
General and administrative:				

Stock-based compensation expense	2,173	848	7,239	1,905
Restructuring expense	21	79	350	116
Total general and administrative non-GAAP adjustments[2]	2,194	927	7,589	2,021
Other non-operating loss:				
Gain (loss) on change in warrant valuation non-GAAP adjustment	23,984	(51,784)	(22,027)	(91,938)
Net loss - non-GAAP[1]	$ (29,114)	$ (8,913)	$ (78,067)	$ (26,086)
Non-GAAP net loss per share - basic and diluted	$ (0.77)	$ (0.34)	$ (2.31)	$ (1.11)
Shares used in per share calculations - basic and diluted	37,596	26,313	33,850	23,602

[1] Non-GAAP operating loss differs from non-GAAP net loss due to $45 and $83 of net interest income for the three months ended December 31, 2013 and December 31, 2012, respectively, and due to $326 and $354 of net interest income for the twelve months ended December 31, 2013 and December 31, 2012, respectively (in thousands).

[2] 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 $3,690 and $1,370 for the three months ended December 31, 2013 and 2012, respectively. Total non-GAAP operating expense adjustments were $11,891 and $3,263 for the twelve months ended December 31, 2013 and 2012, respectively (in thousands).

Sarepta Therapeutics, Inc.
(A Development-Stage Company)
Balance Sheet Highlights
(in thousands)
(unaudited)

	December 31 2013	***December 31, 2012***
Cash and cash equivalents	$ 256,965	$ 187,661
Restricted investments	7,897	-
Total assets	291,569	204,993
Total liabilities	44,377	81,314
Total stockholders' equity	$ 247,192	$ 123,679

Source: Sarepta Therapeutics, Inc.

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