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

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New Drug Application for eteplirsen planned for submission to FDA by year end;

Multiple eteplirsen clinical studies in broader population of DMD patients to begin dosing later this year;

Investigational New Drug applications for two additional DMD drug candidates targeting different genetic subpopulations to be submitted to FDA in third quarter;

Well capitalized with $233.1 million in cash and other investments at quarter end, with an additional $94.5 million raised post-quarter end

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 2014-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today reported financial results for the three months ended March 31, 2014, and provided an update of recent corporate developments.

"The path toward an NDA filing and a potential accelerated approval of eteplirsen has been laid out for us and we are busy preparing for the important clinical and regulatory milestones toward achieving this goal," said Chris Garabedian, president and chief executive officer of Sarepta. "We are also preparing to advance our broader DMD program beyond eteplirsen in the U.S., and will begin the global clinical development program on our next exon-skipping drugs, as well as seek EMA guidance on requirements for a potential eteplirsen submission in the EU, later this year."

Financial Results

For the first quarter of 2014, Sarepta reported a non-GAAP net loss of $20.7 million, or $0.55 per share, compared to a non-GAAP net loss of $13.0 million for the first quarter of 2013, or $0.41 per share. The incremental loss is primarily the result of an increase of $9.1 million in non-GAAP operating expenses due to corporate growth, offset by an increase of $1.6 million in contract revenue.

On a GAAP basis, the net loss for the first quarter of 2014 was $28.3 million, or $0.75 per share (including $4.4 million of stock-based compensation and restructuring expenses), compared with a net loss of $42.1 million for the first quarter of 2013, or $1.32 per share (including $2.1 million of stock-based compensation and restructuring expenses). The decrease in net loss is primarily due to a decrease of $23.7 million in loss on change in warrant valuation and an increase of $1.6 million in contract revenue offset by an increase of $11.3 million in operating expenses. The fluctuation in the fair value of the Company's outstanding warrant liability is primarily affected by the fluctuation of the Company's stock price during each financial reporting period.

Revenue for the first quarter of 2014 was $6.1 million, up from $4.5 million for the first quarter of 2013. The $1.6 million increase was primarily due to the timing of activities in connection with the Marburg portion of the July 2010 U.S. government contract. Revenue from the Company's European Union SKIP-NMD agreement supporting development of an exon 53 skipping therapeutic and the Company's Children's National Medical Center agreement also increased in the first quarter of 2014. These increases were partially offset by a decrease in revenue from the August 2012 intramuscular agreement with the U.S. government, which was completed in the third quarter of 2013.

Non-GAAP research and development expenses were $19.0 million for the first quarter of 2014, compared to $13.0 million for the first quarter of 2013, an increase of $6.0 million. GAAP research and development expenses were $20.9 million for the first quarter of 2014 (including $1.9 million of stock-based compensation and restructuring expenses), compared to $13.8 million for the first quarter of 2013 (including $0.8 million of stock-based compensation and restructuring expenses), an increase of $7.1 million.

Non-GAAP general and administrative expenses were $7.8 million for the first quarter of 2014, compared to $4.8 million for the first quarter of 2013, an increase of $3.0 million. GAAP general and administrative expenses were $10.3 million for the first quarter of 2014 (including $2.5 million of stock-based compensation expense), compared to $6.1 million for the first quarter of 2013 (including $1.3 million of stock-based compensation and restructuring expenses), 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 (DMD).

The Company had cash, cash equivalents, short-term investments and restricted investments related to its letters of credit of $233.1 million as of March 31, 2014 compared to $264.9 million as of December 31, 2013, a decrease of $31.8 million. The decrease was primarily driven by the use of cash to fund the Company's ongoing operations in the first quarter 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."

Recent Corporate Developments

Duchenne Muscular Dystrophy Program

-- Based on new guidance from the U.S. Food and Drug Administration (FDA), announced plans to submit a New Drug Application (NDA) to the FDA with additional safety and efficacy data and analysis by the end of 2014 for the approval of eteplirsen for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

-- Announced plans to initiate several clinical studies of eteplirsen based on the new FDA guidance. Planned studies with eteplirsen in exon-51 amenable genotypes include a historically controlled clinical trial with predefined efficacy endpoints for ambulatory patients between the ages of 7 to 16 years who can walk a minimum distance, and two additional clinical trials that will evaluate safety and biomarkers in DMD patients younger than 7 years and DMD patients who have advanced in their disease progression to a point where they cannot walk a minimum distance or have become non-ambulant.

-- Additionally, announced plans to initiate a placebo-controlled study with one or more of the Company's follow-on DMD exon-skipping drug candidates by the end of the year.

-- Presented clinical data through Week 120 from the Phase IIb open-label extension study of eteplirsen in patients with DMD at the Muscular Dystrophy Association Clinical Conference and the American Academy of Neurology Annual Meeting.

Corporate Updates

-- Announced the company priced an underwritten public offering of an aggregate of 2,650,000 shares of its common stock at a price to the public of $38.00 per share. In addition, Sarepta has granted the underwriters a 30-day option to purchase up to an additional 397,500 shares of common stock on the same terms and conditions as the initial shares sold to the underwriters. The aggregate net proceeds from the offering to the company was approximately $94.5 million, after deducting the underwriting discount and estimated offering expenses payable by Sarepta, but excluding any exercise of the underwriters' option. The offering closed on April 29, 2014.

Conference Call

The conference call may be accessed by dialing 800-708-4540 for domestic callers and 847-619-6397 for international callers. The passcode for the call is 37204717. Please specify to the operator that you would like to join the "Sarepta First 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 available through May 22, 2014 by calling 888-843-7419 or 630-652-3042 and entering access code 37204717.

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. 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 the company's future operations, financial performance, business plans and development of product candidates including; the timing and acceptance of an NDA submission for eteplirsen in the treatment of DMD; the timing of and the Company's ability to conduct additional studies and submit additional data, analysis and other information to the FDA necessary for the FDA to make regulatory determinations for eteplirsen and other follow-on exons; and the potential regulatory approval of eteplirsen on an accelerated pathway; and our plans to advance our DMD program beyond the US. 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; scale-up of manufacturing may not be successful and any of Sarepta's drug candidates may fail in development or may not receive required regulatory approvals (including potentially under an accelerated pathway); Sarepta may need additional funds to conduct research and development efforts; risks specific to obtaining FDA approval for eteplirsen including: we may not be able to comply with all FDA requests; the FDA may determine that our NDA submission for eteplirsen does not qualify for filing, even with additional information; the results of our ongoing and new clinical trials may not be positive; there may be delays in our projected timelines relating to an NDA submission, initiating clinical trials, or making a product commercially available; and those risks identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the fiscal year ended December 31, 2013 filed with the Securities and Exchange Commission as well as other SEC filings made by Sarepta, which you are encouraged to review. 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. 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 March 31,	
	2014	**2013**
Revenues from grants and research contracts	$6,088	$4,474
Operating expenses:		
Research and development	20,906	13,762
General and administrative	10,303	6,127
Operating loss	(25,121)	(15,415)
Other non-operating income (loss):		
Interest income and other, net	99	237

Loss on change in warrant valuation	(3,251)	(26,906)
Net loss	$(28,273)	$(42,084)
Net loss per share – basic and diluted	$(0.75)	$(1.32)
Shares used in per share calculations – basic and diluted	37,821	31,813

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 March 31,	
	2014	**2013**
Net loss – GAAP	$(28,273)	$(42,084)
Research and development:		
Stock-based compensation expense	1,873	530
Restructuring expense	9	264
Total research and development non-GAAP adjustments[1]	1,882	794
General and administrative:		
Stock-based compensation expense	2,469	1,141
Restructuring expense	-	198
Total general and administrative non-GAAP adjustments[1]	2,469	1,339
Other non-operating loss:		
Loss on change in warrant valuation non-GAAP adjustment	3,251	26,906
Net loss – non-GAAP	$(20,671)	$(13,045)
Non-GAAP net loss per share – basic and diluted	$(0.55)	$(0.41)
Shares used in per share calculations – basic and diluted	37,821	31,813

[1] 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 $4,351 and $2,133 for the three months ended March 31, 2014 and 2013, respectively (in thousands).

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

	March 31, 2014	***December 31, 2013***
Cash, cash equivalents and short-term investments	$ 225,192	$ 256,965
Restricted investments	7,897	7,897
Total assets	275,389	291,569
Total liabilities	46,427	44,377
Total stockholders' equity	$ 228,962	$ 247,192

Source: Sarepta Therapeutics, Inc.

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