Sarepta Therapeutics Announces Third Quarter 2016 Financial Results and Recent Corporate Developments

CAMBRIDGE, Mass.--(BUSINESS WIRE)—October 27, 2016-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage developer of innovative RNA-targeted therapeutics, today reported financial results for the three and nine months ended September 30, 2016.

"We are thrilled that the first patient has been infused with EXONDYS 51. We are pleased with the early stages of the launch and that multiple insurance carriers are providing coverage for patients to have access to EXONDYS 51. We plan on providing a corporate update at the 35th Annual JP Morgan Healthcare Conference, after EXONDYS 51 has been available for a full quarter," said Edward Kaye, Sarepta's chief executive officer.

"We believe our recent financing puts us in a strong financial position to execute on both our internal and external clinical development programs and global manufacturing development plans. We continue to evaluate the sale of the priority review voucher as a potential source of non-dilutive financing to help support these efforts and advance potential therapies for patients with DMD," said Sandy Mahatme, Sarepta's chief financial officer.

Financial Results

For the third quarter of 2016, Sarepta reported a net loss of $56.7 million, or $1.18 per share, compared to a net loss of $51.9 million for the third quarter of 2015, or $1.25 per share. The incremental loss of $4.8 million was primarily the result of increased expenses related to the launch of EXONDYS 51.

Excluding $10.8 million of stock-based compensation expense and restructuring expenses, non-GAAP net loss for the third quarter of 2016 was $45.9 million, or $0.95

per share, compared to a non-GAAP net loss excluding $5.7 million of stock-based compensation expense of $46.3 million for the third quarter of 2015, or $1.11 per share.

No revenue was recognized for the three months ended September 30, 2016 and 2015.

Research and development expenses were $34.3 million for the third quarter of 2016, compared to $36.7 million for the third quarter of 2015, a decrease of $2.4 million. Non-GAAP research and development expenses (excluding $3.4 million of stock-based compensation and restructuring expenses) were $30.9 million for the third quarter of 2016, compared to $34.0 million (excluding $2.6 million of stock-based compensation expense) for the third quarter of 2015, a decrease of $3.1 million.

General and administrative expenses were $22.2 million for the third quarter of 2016, compared to $15.1 million for the third quarter of 2015, an increase of $7.1 million. Non-GAAP general and administrative expenses (excluding $7.4 million of stock-based compensation and restructuring expenses) were $14.8 million for the third quarter of 2016, compared to $12.0 million (excluding $3.1 million of stock-based compensation expense) for the third quarter of 2015, an increase of $2.8 million.

The Company had $406.6 million in cash, cash equivalents, short-term investments and restricted cash as of September 30, 2016 compared to $204.0 million as of December 31, 2015, an increase of $202.6 million. The increase was driven by the net proceeds received from the Company’s public offerings in June and September 2016, offset by the use of cash to fund the Company’s ongoing operations.

Use of Non-GAAP Measures

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 stock-based compensation and restructuring expenses.

Stock-based compensation expenses represent non-cash charges related to equity awards granted by Sarepta. Although these are recurring charges to operations, management believes the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within management's control. Therefore, management believes that excluding these charges from non-GAAP research and development expenses, non-GAAP general and administrative expenses, non-GAAP net loss and non-GAAP net loss per share facilitates comparisons of the Company’s operational performance in different periods.

Restructuring related expenses have been excluded from non-GAAP research and development expenses, non-GAAP general and administrative expenses, non-GAAP net loss and non-GAAP net loss per share as the Company believes that the adjustments for these items represent more closely the sustainability of the Company’s operating performance and understanding of its financial results.

The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. The Company also believes these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating the Company’s performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or 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, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. 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

--Sarepta Therapeutics Announces FDA Accelerated Approval of EXONDYS 51™ (eteplirsen) injection, an Exon Skipping Therapy to Treat Duchenne Muscular Dystrophy (DMD) Patients Amenable to Skipping Exon 51

--Sarepta Therapeutics and Summit Enter Into Exclusive License and Collaboration Agreement for European Rights to Summit's Utrophin Modulator Pipeline for the Treatment of Duchenne Muscular Dystrophy

--Catabasis Pharmaceuticals and Sarepta Therapeutics Announce a Joint Research Collaboration in Duchenne Muscular Dystrophy

--Sarepta Therapeutics Announces First Patient Dosed in Phase III Clinical Trial of SRP-4045 and SRP-4053 for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 45 or 53 Skipping

--Sarepta Therapeutics Announces Favorable USPTO Decisions in Exon 51 and Exon 53 Composition of Matter Patent Interference Cases against BioMarin Pharmaceutical

Corporate Updates

--Sarepta Therapeutics Announces Pricing of $345 Million Public Offering of Common Stock

Conference Call

The Company will be hosting a conference call at 8:00 a.m. EDT, to discuss these financial results and other corporate updates. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 7029987. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2016 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.

About EXONDYS 51™

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of EXONDYS 51 in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

Important Safety Information

Adverse reactions observed in patients (N=8) treated with 30 or 50 mg/kg/wk of EXONDYS 51 with incidence ≥ 25% and higher than in the placebo group (N=4) (Study 1) were: balance disorder (38%), vomiting (38%) and contact dermatitis (25%). The most common adverse reactions were balance disorder and vomiting.

The following events were reported in ≥ 10% of patients treated with EXONDYS 51 for up to 208 weeks (N=88) and occurred more frequently than placebo in a controlled trial for 24 weeks (Study 1): vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

For the full prescribing information please refer to U.S. Full Prescribing Information at www.EXONDYS51.com.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly

advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit us at www.sarepta.com.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. 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. Forward-looking statements in this press release include the Company's plans to provide a corporate update at the 35th Annual JP Morgan Healthcare Conference, our belief that our recent financing puts us in a strong financial position to execute on both our internal and external clinical development programs and global manufacturing development plan, our continued evaluation of a sale of the priority review voucher and other statements regarding the Company's business plans.

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: the commercial launch in the US for EXONDYS 51 may not be successful in part or at all for various reasons including the actual market size and drug supply needed may not be consistent with the company's expectations, EXONDYS 51 may not be accepted by patients and prescribed by physicians to the degree expected by the Company, there may be a significant number of insurance carriers that delay coverage, may not cover or significantly limit coverage of EXONDYS 51, our manufacturing, sales, distribution and specialty pharmacy network may not be efficient in getting EXONDYS 51 to the market and economic, competitive, reimbursement and regulatory conditions could negatively impact the commercial launch of EXONDYS 51; the confirmatory and other studies for EXONDYS 51 may not yield data consistent with prior results or demonstrate a benefit that supports continued or full regulatory approval by the FDA; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval

of our pipeline of exon-skipping products; the results of our ongoing research and development efforts and clinical trials for our product candidates and our technologies may not be positive or consistent with prior results or demonstrate a safe treatment benefit or support an NDA filing, positive advisory committee recommendation or marketing approval by the FDA or other regulatory authority; the USPTO, other agencies or courts may make decisions against Sarepta that negatively impact the EXONDYS 51 commercialization and/ or that result in the erosion of the protections offered by Sarepta's patent estate; Sarepta may not be able to execute on its business plans including meeting its expected or planned regulatory milestones and timelines, clinical development plans and bringing product candidates to market for various other reasons including possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner and regulatory, court or agency decisions, including any FDA decision on whether to provide a full approval of EXONDYS 51, may negatively impact or limit Sarepta's activities; and those risks identified under the heading "Risk Factors" in Sarepta's 2015 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 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 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 Company's filings with the SEC. We caution investors not to place considerable reliance on the forward-looking statements contained in 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.

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. 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 September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Revenues from grants and research contracts	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	34,349	36,673	117,523	105,018
General and administrative	22,184	15,090	60,812	50,714
Operating loss	(56,533)	(51,763)	(178,335)	(155,732)
Other income (loss):				
Interest (expense) income and other, net	(209)	(176)	(478)	383
Net loss	$ (56,742)	$ (51,939)	$ (178,813)	$ (155,349)
Net loss per share - basic and diluted	$ (1.18)	$ (1.25)	$ (3.83)	$ (3.75)
Shares used in per share calculation basic and diluted	48,254	41,565	46,709	41,416

Sarepta Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Net Loss

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Net loss - GAAP	$ (56,742)	$ (51,939)	$ (178,813)	$ (155,349)
Research and development:				
Stock-based compensation expense	2,674	2,631	7,527	7,639
Restructuring Expense	770	-	1,783	-
Total research and development non-GAAP adjustments [1]	3,444	2,631	9,310	7,639
General and administrative:				
Stock-based compensation expense	6,899	3,052	15,566	18,130
Restructuring Expense	494	-	640	-
Total general and administrative non-GAAP adjustments [1]	7,393	3,052	16,206	18,130
Net loss - non-GAAP	$ (45,905)	$ (46,256)	$ (153,297)	$ (129,580)
Non-GAAP net loss per share - basic and diluted	$ (0.95)	$ (1.11)	$ (3.28)	$ (3.13)
Shares used in per share calculations - basic and diluted	48,254	41,565	46,709	41,416

[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 $10,837 and $5,683 for the three months ended September 30, 2016 and 2015, respectively. Total non-GAAP operating expense adjustments were $25,516 and $25,769 for the nine months ended September 30, 2016 and 2015, respectively.

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	September 30, 2016	December 31, 2015
Cash, cash equivalents and short-term investments	$ 395,140	$ 192,491
Restricted investments	11,479	11,478
Total assets	487,310	273,782
Total liabilities	78,686	83,435
Total stockholders' equity	$ 408,624	$ 190,347

Source: Sarepta Therapeutics, Inc.

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