Sarepta Therapeutics Announces Third Quarter 2017 Financial Results and Recent Corporate Developments

-- Third quarter 2017 EXONDYS 51® (eteplirsen) total net revenues of $46 million --

-- Company raises annual 2017 revenue guidance to between $150 million and $155 million --

CAMBRIDGE, Mass., October 25, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, today reported financial results for the third quarter of 2017.

“Driven by continued exceptional execution, physician adoption, and adherence, EXONDYS 51 achieved strong performance in the third quarter,” said Douglas Ingram, Sarepta’s president and chief executive officer. “For the third straight quarter, we have been in a position to raise our annual revenue guidance. We are proud that EXONDYS 51 is on track to be one of the most successful ultra-rare disease launch in history, but we are prouder still that EXONDYS 51 performance and the advancement of our pipeline in the third quarter furthered our mission to improve the lives of children with Duchenne muscular dystrophy (DMD).”

During the quarter, the Company announced results of the 4053-101 study for golodirsen, a phosphorodiamidate morpholino oligomer (PMO) designed to treat exon 53 skipping amenable DMD patients. Golodirsen results were highly statistically significant on all three biological endpoints reported, RT-PCR, Western blot and immunohistochemistry. Golodirsen exhibited a 100 percent response rate in all subjects as measured by RT-PCR, precisely excising exon 53 and creating in reading frame RNA transcript in all patients. The full data set presented at the 2017 Annual Congress of the World Muscle Society by Dr. Francesco Muntoni, a lead investigator in the 4053-101 study, showed a 10.7 fold mean increase in dystrophin production, along with the proper localization of the protein in the muscle.

Additionally, in the quarter the Company appointed Dr. Guriq Basi as chief scientific officer to facilitate the rapid expansion of Sarepta's current and next-generation chemistry platforms, including the PMO and next-generation peptide-conjugated PPMO platforms.

"We intend to move with a sense of urgency to translate our innovative science and impressive pipeline into potentially life-saving and life-enhancing medicines," continued Mr. Ingram. "As we look to the rest of the year and into 2018, we will focus on continued performance of EXONDYS 51 and our numerous near term milestones, including the anticipation of first patients dosed this year in two of our gene therapy collaboration programs with Nationwide Children's Hospital, initiating our PPMO 51 trial in patients, obtaining the readout from our collaborative program for utrophin up-regulation with Summit Therapeutics, preparing to meet with the FDA in early 2018 regarding the pathway for approval of golodirsen, and continuing preparation for a hearing with the European CHMP regarding a European marketing authorization for eteplirsen."

Financial Results

For the third quarter of 2017, on a GAAP basis, Sarepta reported a net loss of $47.7 million, or $0.78 per share, compared to a net loss of $56.7 million for the same period of 2016, or $1.18 per share. On a non-GAAP basis, the net loss for the third quarter of 2017 was $12.4 million, or $0.20 per share, compared to a net loss of $45.9 million for the same period of 2016, or $0.95 per share. The decrease in net loss for the quarter was primarily driven by increased net revenues offset by increases in costs and expenses.

Net Revenues

For the third quarter of 2017, the Company recognized net revenues of $46.0 million which reflects sales from EXONDYS 51 in the U.S. No revenue was recognized for the same period of 2016.

Cost and Operating Expenses

Cost of sales was $3.1 million in the third quarter of 2017, which relates to sales of EXONDYS 51 following its commercial launch in the U.S. There was no cost of sales recognized for the same period of 2016. Prior to the approval of EXONDYS 51, the Company expensed such manufacturing and material costs as research and development expenses.

Research and development expenses were $34.2 million for the third quarter of 2017, compared to $34.3 million for the same period of 2016, which is relatively consistent from period to period. The change was

primarily driven by lower manufacturing expenses due to the capitalization of inventory following the approval of EXONDYS 51, partially offset by increased patient enrollment in the Company's ongoing clinical trials, a ramp up of preclinical studies for the Company's PPMO platform and other follow-on exons and slight increases in professional fees and compensation and other personnel expenses. Non-GAAP research and development expenses were $32.4 million for the third quarter of 2017, compared to $30.9 million for the same period of 2016, an increase of $1.5 million.

Selling, general and administrative expenses were $28.2 million for the third quarter of 2017, compared to $22.2 million for the same period of 2016, an increase of $6.0 million, which was primarily driven by increases in professional services due to global commercial expansion and on-going litigation and compensation and other personnel expenses. Non-GAAP selling, general and administrative expenses were $23.1 million for the third quarter of 2017, compared to $14.8 million for the same period of 2016, an increase of $8.3 million.

As a result of the execution of the settlement and license agreements with BioMarin Pharmaceuticals (BioMarin) in July 2017, the Company recorded $25.6 million in litigation and license charges. Additionally, the Company recognized an amortization of in-licensed rights of $0.8 million for the third quarter of 2017, primarily due to the BioMarin transactions.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had $618.4 million in cash, cash equivalents, restricted cash and investments as of September 30, 2017 compared to $329.3 million as of December 31, 2016, an increase of $289.1 million. The increase is primarily driven by the net proceeds from the Company's equity offering and term loan, proceeds from the sale of the Company's Priority Review Voucher (PRV) and collection of accounts receivable related to EXONDYS 51 sales offset by up-front payments of $35.0 million related to the Company's license and settlement agreements with BioMarin and a milestone payment of $22.0 million to Summit Therapeutics, and 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 selling, general and administrative expenses, non-GAAP other income adjustments, non-GAAP income tax expense, 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, restructuring expenses, and other items.

1. Stock-based compensation 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 facilitates comparisons of the Company's operational performance in different periods.

2. Restructuring expenses

Restructuring expenses have been excluded as the Company believes that adjusting for these items more closely represents the Company's ongoing operating performance and financial results.

3. Other items

Management evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relates to the Company's ongoing business operations, and (c) whether the Company expects the items to continue on a regular basis. These other items include the aforementioned gain from the sale of the Company's PRV and associated income taxes, upfront license and milestone payments to Summit, EXONDYS 51 litigation and license charges and amortization of in-licensed rights.

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 selling, general and administrative expenses, non-GAAP other income adjustments, non-GAAP income tax expense, 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

-- Sarepta Therapeutics to Present at the 22nd International Annual Congress of the World Muscle Society

-- Sarepta Therapeutics Appoints Guriqbal S. Basi, Ph.D. as Chief Scientific Officer

-- Sarepta Therapeutics to Ring Nasdaq Stock Market Closing Bell in Recognition of World Duchenne Awareness Day

-- Sarepta Therapeutics Announces Positive Results in Its Study Evaluating Gene Expression, Dystrophin Production, and Dystrophin Localization in Patients with Duchenne Muscular Dystrophy (DMD) Amenable to Skipping Exon 53 Treated with golodirsen (SRP-4053)

-- Sarepta Therapeutics Announces its Partner, Genethon, Published New Micro-Dystrophin Gene Therapy Data in Nature Communications

-- Sarepta Therapeutics Announces Pricing of $325 Million Public Offering of Common Stock

Conference Call

The Company will be hosting a conference call at 4:30 p.m. Eastern Time, to discuss these financial results and provide a corporate update. The conference call may be accessed by dialing 844-534-7313 for domestic callers and +1-574-990-1451 for international callers. The passcode for the call is 1598174. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2017 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 About EXONDYS 51

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in 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 further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat 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 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," "may," "intends," "prepares," "looks," "poten-

tial," "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 and projections, business plans, priorities and development of product candidates including: EXONDYS 51 being on track to be one of the most successful ultra-rare disease launch in history; the continued exceptional execution, physician adoption of EXONDYS 51 and adherence; Sarepta's mission to improve the lives of children with DMD being advanced by EXONDYS 51 performance and advancement of Sarepta's pipeline; Dr. Guriq Basi facilitating the rapid expansion of Sarepta's current and next-generation chemistry platforms, including PMO and PPMO; our plan to move with a sense of urgency to translate Sarepta's innovative science and impressive pipeline into potentially life-saving and life-enhancing medicines; Sarepta's anticipation that EXONDYS 51 net revenues for 2017 will be in the range of $150 to $155 million; and Sarepta's plans for the rest of the year and 2018, including focusing on continued performance of EXONDYS 51, the anticipation of first patients dosed this year in two of Sarepta's gene therapy collaboration programs with Nationwide Children's Hospital, initiating Sarepta's PPMO 51 trial in patients, obtaining the readout from Sarepta's collaborative program for utrophin up-regulation with Summit Therapeutics, preparing to meet with the FDA in early 2018 regarding the pathway for approval of golodirsen, and continuing preparation for a hearing with the European CHMP regarding a European marketing authorization for eteplirsen.

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 meet expectations with respect to EXONDYS 51 sales or attain the net revenues we anticipate for 2017, profitability or positive cash-flow from operations; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 in a timely manner or at all; we may not be able to obtain regulatory approval for eteplirsen in jurisdictions outside of the U.S. including from the European Medicines Agency; our data for golodirsen (SRP-4053) may not be sufficient for a filing for or obtaining regulatory approval; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of golodirsen (SRP-4053) or any of our other product candidates; the results of our ongoing research and development efforts, including those with strategic partners, and clinical trials for golodirsen (SRP-4053) and our other product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact our business; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, for various 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, such as decisions by the European CHMP on eteplirsen or the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2016 and most recent Quarterly Report on Form 10-Q 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, except to the extent required by applicable law or SEC rules.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website 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,	
	2017	**2016**	**2017**	**2016**
Revenues:				
Product, net	45,954	-	97,307	-
Total revenues	45,954	-	97,307	-
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,078	-	3,807	-
Research and development	34,239	34,349	122,266	117,523
Selling, general and administrative	28,176	22,184	90,461	60,812
Amortization of in-licensed rights	780	-	837	-
EXONDYS 51 litigation and license charges	25,588	-	28,427	-
Total cost and expenses	91,861	56,533	245,798	178,335
Operating loss	(45,907)	(56,533)	(148,491)	(178,335)
Other income (loss):				
Gain from sale of intangible asset	-	-	125,000	-
Interest income (expense) and other, net	184	(209)	703	(478)
Loss before income tax expense	(45,723)	(56,742)	(22,788)	(178,813)
Income tax expenses	2,011	-	3,902	-
Net loss	$ (47,734)	$ (56,742)	$ (26,690)	$ (178,813)
Net loss per share - basic and diluted	$ (0.78)	$ (1.18)	$ (0.47)	$ (3.83)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	61,528	48,254	57,166	46,709

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,	
	2017	**2016**	**2017**	**2016**
Net loss - GAAP	$ (47,734)	$ (56,742)	$ (26,690)	$ (178,813)
Research and development:				
Milestone payment	-	-	22,000	-
Stock-based compensation expense	1,812	2,674	5,881	7,527
Restructuring expense	10	771	184	1,784
Total research and development non-GAAP adjustments	1,822	3,445	28,065	9,311
Selling, general and administrative:				
Stock-based compensation expense	5,110	6,899	17,218	15,566
Restructuring expense	3	493	2,589	639
Total selling, general and administrative non-GAAP adjustments	5,113	7,392	19,807	16,205
Amortization of intangible asset non-GAAP adjustment	780	-	837	-
EXONDYS 51 litigation and license charges non-GAAP adjustment	25,588	-	28,427	-
Gain from sale of intangible asset non-GAAP adjustment	-	-	(125,000)	-
Income tax expense non-GAAP adjustment	2,011	-	3,902	-
Net loss non-GAAP	$ (12,420)	$ (45,905)	$ (70,652)	$ (153,297)
Non-GAAP net loss per share - basic and diluted	$ (0.20)	$ (0.95)	$ (1.24)	$ (3.28)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	61,528	48,254	57,166	46,709

Sarepta Therapeutics, Inc.

Balance Sheet Highlights

(in thousands)

(unaudited)

	As of September 30, 2017	As of December 31,2016
Cash, cash equivalents, restricted cash and investments	$ 618,414	$ 329,324
Total assets	$ 800,281	$ 424,104
Total liabilities	$ 103,125	$ 87,413
Total stockholders' equity	$ 697,156	$ 336,691

Source: Sarepta Therapeutics, Inc.

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