



Sarepta Therapeutics Announces Fourth Quarter 2017 and Full-Year 2017 Financial Results and Recent Corporate Developments

-- Fourth quarter 2017 EXONDYS 51® (eteplirsen) total net revenues of \$57.3 million --

-- Cash and investment balance of \$1.1 billion as of December 31, 2017 --

CAMBRIDGE, Mass., March 1, 2018 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, today reported financial results for the three and twelve months ended December 31, 2017.

"In 2017, Sarepta advanced our ambitious vision to make a profound difference in the lives of those with Duchenne muscular dystrophy (DMD) and furthered our position as an important global genetic medicine company in rare disease. We accelerated our industry-leading DMD pipeline composed of some 16 programs across our RNA-targeted, gene therapy and gene-editing platforms; raised substantial resources to invest in our programs; and delivered on our commitment to execute a successful launch for EXONDYS 51, our first genetic medicine therapy for DMD," said Douglas Ingram, Sarepta's president and chief executive officer.

Mr. Ingram continued, "The exceptional 2017 performance of EXONDYS 51 and the advancement of our multi-platform pipeline reflects our leadership position in DMD and our commitment to turning our vision into reality. As we track into 2018, our commitment to the DMD community is unwavering, exemplified by our continued focus on the launch of EXONDYS 51, multiple important data read outs over the year, and our progress in bringing new therapies to the DMD community with a sense of urgency."

Financial Results

For the fourth quarter of 2017, on a GAAP basis, Sarepta reported a net loss of \$24.0 million, or \$0.37 per share, compared to a net loss of \$88.5 million for the same period of 2016, or \$1.62 per share. On a non-GAAP basis, the net loss for the fourth quarter of 2017 was \$18.0 million, or \$0.28 per share, compared to a net loss of \$38.6 million for the same period of 2016, or \$0.71 per share.

For the year ended December 31, 2017, on a GAAP basis, Sarepta reported a net loss of \$50.7 million, or \$0.86 per share, compared to a net loss of \$267.3 million for the same period of 2016, or \$5.49 per share. On a non-GAAP basis, the net loss for 2017 was \$88.7 million, or \$1.51 per share, compared to a net loss of \$191.9 million for the same period of 2016, or \$3.94 per share.

Net Revenues

For the three and twelve months ended December 31, 2017, the Company recorded net product revenues of \$57.3 million and \$154.6 million, respectively, which reflects sales from EXONDYS 51 compared to net revenues of \$5.4 million for fourth quarter of 2016. The Company did not achieve any product revenues for the first three quarters of 2016. The increase primarily reflects increasing demand for EXONDYS 51 in the U.S.

Cost and Operating Expenses

Cost of sales (excluding amortization of in-licensed rights)

For the three and twelve months ended December 31, 2017, cost of sales (excluding amortization of in-licensed rights) were \$3.5 million and \$7.4 million, respectively, compared to \$0.1 million for the same periods of 2016. The increase primarily reflects royalty payments to BioMarin Pharmaceuticals (BioMarin) as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as higher inventory costs related to increasing demand for EXONDYS 51 during 2017. Prior to the approval of EXONDYS 51, the Company expensed related manufacturing and material costs as research and development expenses.

Research and development

Research and development expenses were \$44.4 million for the fourth quarter of 2017, compared to \$70.7 million for the same period of 2016, a decrease of \$26.3 million. The decrease was primarily driven by an up-front payment of \$40.0 million to Summit (Oxford) Ltd. (Summit) in the fourth quarter of 2016 partially offset by increased patient enrollment in the Company's on-going late stage clinical trials and a ramp up of preclinical studies for the Company's PPMO platform and other follow-on exons. Non-GAAP research and development expenses were \$41.8 million for the fourth quarter of 2017, compared to \$27.8 million for the same period of 2016, an increase of \$14.0 million. The increase was primarily due to increased patient enrollment in the Company's on-going late-stage clinical trials and a ramp up of preclinical studies for the Company's PPMO platform and other follow-on exons.

Research and development expenses were \$166.7 million for 2017, compared to \$188.3 million for the same period of 2016, a decrease of \$21.6 million. The decrease was primarily driven by lower manufacturing expenses due to the capitalization of inventory following the approval of EXONDYS 51 and \$40.0 million and \$7.0 million up-front payments, respectively, to Summit and University of Western Australia in 2016. The decreases were partially offset by a \$22.0 million payment to Summit in 2017 as a result of achieving the milestone of the last patient being dosed in the safety arm cohort to the PhaseOut DMD study, increased patient enrollment in the Company's ongoing late-stage clinical trials and a ramp up of preclinical studies for the Company's PPMO platform and other follow-on exons and increases in professional fees and compensation and other personnel expenses. Non-GAAP research and development expenses were \$136.0 million for 2017, consistent with the same period of 2016.

Selling, general and administration

Selling, general and administrative expenses were \$32.2 million for the fourth quarter of 2017, compared to \$22.9 million for the same period of 2016, an increase of \$9.3 million. Non-GAAP selling, general and administrative expenses were \$27.3 million for the fourth quarter of 2017, compared to \$16.1 million for the same period of 2016, an increase of \$11.2 million. The year-over-year increases for both GAAP and non-GAAP selling, general and administrative expenses for the fourth quarter were primarily driven by increases in professional services due to global expansion and compensation and other personnel expenses.

Selling, general and administrative expenses were \$122.7 million for 2017, compared to \$83.7 million for the same period of 2016, an increase of \$39.0 million. Non-GAAP selling, general and administrative expenses were \$97.9 million for 2017, compared to \$60.7 million for the same period of 2016, an increase of \$37.2 million. The year-over-year increases for both GAAP and non-GAAP selling, general and administration expenses for the full year were primarily driven by increases in professional services due to global expansion, legal expenses and compensation and other personnel expenses.

EXONDYS 51 litigation and license charges and amortization of in-licensed rights

As a result of the execution of the settlement and license agreements with BioMarin in July 2017, the Company recorded \$28.4 million in litigation and license charges. Additionally, the Company recognized an amortization of in-licensed rights of \$1.1 million during 2017, primarily due to the BioMarin transactions.

Other Income (Loss)

Gain from sale of Priority Review Voucher

In connection with the completion of the sale of the Priority Review Voucher (PRV) in March 2017, the Company recorded a gain of \$125.0 million from sale of PRV for 2017.

Interest expense and other, net

For the three and twelve months ended December 31, 2017, the Company recorded \$2.7 million and \$2.0 million, interest expense and other, net, respectively, compared to less than \$0.1 million and \$0.5 million, respectively, for the same periods of 2016. The year-over-year increases for both periods were primarily driven by accrued interest expense related to the convertible note that the Company issued in November 2017.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had \$1.1 billion in cash, cash equivalents, restricted cash and investments as of December 31, 2017 compared to \$329.3 million as of December 31, 2016, an increase of \$760.5 million. The increase is primarily driven by the net proceeds from the Company's equity and debt offerings, proceeds from the sale of the Company's 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 Pre-Announces Fourth Quarter 2017 Revenue and Provides Full-Year 2018 Revenue Guidance for EXONDYS 51® (eteplirsen), Representing Approximately 100 Percent Year-over-Year Growth

- Sarepta Therapeutics Announces Publication of Long-Term Pulmonary Function of Eteplirsen-Treated Patients Compared to Natural History of Duchenne Muscular Dystrophy in The Journal of Neuromuscular Diseases
- Sarepta Therapeutics Elects Biopharmaceutical Veteran, Michael W. Bonney, to its Board of Directors
- Sarepta Therapeutics Announces Exercise of Initial Purchasers' Option to Purchase Additional Convertible Senior Notes Due 2024
- Sarepta Therapeutics Prices \$475 Million of Convertible Senior Notes Due 2024
- Sarepta Therapeutics Announces Proposed Offering of \$375 Million of Convertible Senior Notes Due 2024
- Sarepta Therapeutics Announces FDA Clearance of IND for the Company's PPMO Exon 51 Candidate, SRP-5051
- Sarepta Therapeutics and Nationwide Children's Hospital Announce FDA Clearance of IND for Micro-Dystrophin Gene Therapy Program for the Treatment of Duchenne Muscular Dystrophy
- Sarepta Therapeutics and Nationwide Children's Hospital Announce U.S. Food and Drug Administration (FDA) Clearance of the IND Application for the GALGT2 Gene Therapy Program
- Sarepta Therapeutics Signs Exclusive Global Collaboration with Duke University for Gene Editing CRISPR/Cas9 Technology to Develop New Treatments for Duchenne Muscular Dystrophy (DMD)

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 7396848. Please specify to the operator that you would like to join the "Sarepta Fourth Quarter and Full-Year 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.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

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 $\geq 10\%$ 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.

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 medicine 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,” “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 and projections, business plans, priorities and development of product candidates including: Sarepta’s ambitious vision to make a profound difference in the lives of those with DMD; Sarepta being an important global genetic medicine company in rare disease; the exceptional 2017 performance of EXONDYS 51 and the advancement of Sarepta’s multi-platform pipeline reflecting Sarepta’s leadership position in DMD and its commitment to turning its vision into reality; Sarepta’s commitment to the DMD community being unwavering; Sarepta’s plans for 2018, including continuing to focus on the launch of EXONDYS 51, having multiple important data read outs, and making progress in bringing new therapies to the DMD community with a sense of urgency; and Sarepta’s full-year 2018 revenue guidance for EXONDYS 51, representing approximately 100 percent year-over-year growth.

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 2018, 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 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, PPMO, gene therapy 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, PPMO, gene therapy 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 expectations with respect to EXONDYS 51 sales, meeting our expected or planned regulatory milestones and timelines, research and 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, 2017 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.
Consolidated Statements of Operations
(unaudited, in thousands, except per share amounts)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	2016	2017	2016
Revenues:				
Product, net	\$ 57,277	\$ 5,421	\$ 154,584	\$ 5,421
Total revenues	57,277	5,421	154,584	5,421
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,546	101	7,353	101
Research and development	44,441	70,749	166,707	188,272
Selling, general and administrative	32,221	22,937	122,682	83,749
EXONDYS 51 litigation and license charges	—	—	28,427	—
Amortization of in-licensed rights	216	29	1,053	29
Total cost and expenses	80,424	93,816	326,222	272,151
Operating loss	(23,147)	(88,395)	(171,638)	(266,730)
Other income (loss):				
Gain from sale of Priority Review Voucher	—	—	125,000	—
Interest expense and other, net	(2,693)	(57)	(1,990)	(535)
Loss before income tax expense	(25,840)	(88,452)	(48,628)	(267,265)
Income tax (benefit) expense	(1,842)	—	2,060	—
Net loss	\$ (23,998)	\$ (88,452)	\$ (50,688)	\$ (267,265)
Net loss per share - basic and diluted	\$ (0.37)	\$ (1.62)	\$ (0.86)	\$ (5.49)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	64,277	54,619	58,818	48,697

Sarepta Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Net Loss

(unaudited, in thousands, except share and per share amounts)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	2016	2017	2016
Net loss - GAAP	\$ (23,998)	\$ (88,452)	\$ (50,688)	\$ (267,265)
Research and development:				
Up-front and milestone payments	—	40,785	22,000	40,785
Stock-based compensation expense	2,661	1,972	8,542	9,499
Restructuring expense	4	230	188	2,013
Total research and development non-GAAP adjustments	2,665	42,987	30,730	52,297
Selling, general and administrative:				
Stock-based compensation expense	4,705	4,897	21,923	20,463
Restructuring expense	243	1,909	2,832	2,549
Total selling, general and administrative non-GAAP adjustments	4,948	6,806	24,755	23,012
Amortization of intangible asset non-GAAP adjustment	216	29	1,053	29
EXONDYS 51 litigation and license charges non-GAAP adjustment	—	—	28,427	—
Other income:				
Gain from sale of Priority Review Voucher	—	—	(125,000)	—
Total other income non-GAAP adjustments	—	—	(125,000)	—
Income tax (benefit) expense non-GAAP adjustments	(1,842)	—	2,060	—
Net loss - non-GAAP	<u>\$ (18,011)</u>	<u>\$ (38,630)</u>	<u>\$ (88,662)</u>	<u>\$ (191,927)</u>
Non-GAAP net loss per share - basic and diluted	\$ (0.28)	\$ (0.71)	\$ (1.51)	\$ (3.94)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	64,277	54,619	58,818	48,697

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(unaudited, in thousands, except share and per share data)

	As of December 31, 2017	As of December 31, 2016
Assets		
Current Assets:		
Cash and cash equivalents	\$ 599,691	\$ 122,420
Short-term investments	479,369	195,425
Accounts receivable	29,468	5,228
Inventory	83,605	12,813
Restricted investment	—	10,695
Other current assets	36,511	26,895
Total Current Assets	<u>1,228,644</u>	<u>373,476</u>
Property and equipment, net of accumulated depreciation of \$18,022 and \$30,346 as of December 31, 2017 and 2016, respectively	43,156	37,801
Intangible assets, net of accumulated amortization of \$4,145 and \$3,134 as of December 31, 2017 and 2016, respectively	14,355	8,076
Investments and other assets	21,809	4,751
Total Assets	<u>\$ 1,307,964</u>	<u>\$ 424,104</u>
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	\$ 8,467	\$ 29,690
Accrued expenses	68,982	31,016
Current portion of long-term debt	6,175	10,108
Deferred revenue	3,316	3,303
Other current liabilities	1,392	1,305
Total Current Liabilities	<u>88,332</u>	<u>75,422</u>
Long-term debt	424,876	6,042
Deferred rent and other	5,539	5,949
Total Liabilities	<u>518,747</u>	<u>87,413</u>
Stockholders' Equity:		
Preferred stock, \$.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, \$.0001 par value, 99,000,000 shares authorized; 64,791,670 and 54,759,234 issued and outstanding at December 31, 2017 and 2016, respectively	6	5
Additional paid-in capital	2,006,598	1,503,126
Accumulated other comprehensive loss	(379)	(120)
Accumulated deficit	(1,217,008)	(1,166,320)
Total Stockholders' Equity	<u>789,217</u>	<u>336,691</u>
Total Liabilities and Stockholders' Equity	<u>\$ 1,307,964</u>	<u>\$ 424,104</u>

Source: Sarepta Therapeutics, Inc.

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