SAREPTA
THERAPEUTICS

FIRST QUARTER 2017 FINANCIAL RESULTS & RECENT CORPORATE DEVELOPMENTS

April 27, 2017

FORWARD-LOOKING STATEMENTS

In order to provide Sarepta's investors with an understanding of its current results and future prospects, forward-looking statements will be made during this conference call. Any statements made by Sarepta 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 by management relating to Sarepta's future operations, financial performance and projections, business plans, priorities and research and development programs including: The timing of Sarepta's 10-Q filing for Q1 2017; Sarepta's goals and ability to execute these goals including helping as many DMD patients as possible, expanding EXONDYS 51 access globally, continuing to make progress on and the prospects of the commercial launch of EXONDYS 51 in the United States, expanding into Ex-U.S. territories, and building an innovative pipeline; the update to Sarepta's full-year revenue guidance and Sarepta's belief it is well positioned for future growth and being in a position to successfully achieve its goals for the remainder of the year; the mechanism of action and potential safety and benefits of EXONDYS 51, including through use with steroids; the U.S. and E.U. market size for and potential beneficiaries of EXONDYS 51; Sarepta's strong commitment and plans for generating clinically significant data through its own pipeline and through collaborations with third parties, the status of, projected timelines and milestones for these studies, including timing for PMO entering the clinic, patient enrollment, last patient to complete a study, data availability, readouts and analyses, and finalization of work with regulatory authorities impacting these studies such as agreeing on protocols for dystrophin measurements with FDA that Sarepta plans to use for its products including PMOs and PPMOs and Sarepta plans to complete an ADME study required by EMA; Sarepta's belief regarding the impact that its published data can have to support patient access to EXONDYS 51; The timing of and plans to work with EMA through their review process of Sarepta's MAA, Sarepta plans to conduct an ADME study and complying with EMA data and analyses requests until a final determination is made; the timing of and Sarepta's plans and efforts to support a potential launch of EXONDYS 51 in Europe including its IP efforts, launching a managed access program and completing related agreements; the design and potential efficacy and safety of PPMO's, their potential application in DMD and other diseases and Sarepta's PPMO development plans and timing of the same; the potential benefits of recent senior hires; the natural history of DMD and Sarepta's beliefs regarding ambulant and non-ambulant patients access to EXONDYS 51 and its progress in securing reimbursement for non-ambulatory patients; Sarepta's expectations regarding the impact of port use by patients, re-authorizations, Sarepta education programs on genetic testing, sale trends continuing and discussions with payers on prescriptions, START Forms, patients starting on therapy and reimbursement landscape for EXONDYS 51; Sarepta looking forward to one day serving DMD patients globally and its belief in its ability to execute and build toward its goal of becoming the lead in DMD and other neuromuscular diseases and its strategy to achieve this goal; Sarepta's plans in connection with its collaborations with Summit and Nationwide and the potential efficacy of the products and technologies under these collaborations as well as the patients that could benefit from these technologies; and the planned resignation of Sarepta's CEO and his future plans and role with Sarepta including plans for smooth transition and focus on key initiatives for the Company.

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 EXONDYS 51 in jurisdictions outside of the U.S. including from the European Medicines Agency for various reasons including any inability on our end to satisfactorily respond to their requests during the review process for our MAA; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of any of our product candidates; the results of our ongoing research and development efforts and clinical trials for our product candidates and product candidates being developed through third party collaborations may not be positive or consistent with prior results or demonstrate a safe treatment benefit; the planned resignation and transition of our current CEO may have a negative impact on the Company and its business plans; 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 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 or most recently filed 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.

AGENDA

Welcome
- Ian Estepan, Executive Director, Corporate Affairs

Clinical & Corporate Update
- Edward Kaye, M.D., Chief Executive Officer

Financial Results
- Sandy Mahatme, Chief Financial Officer

Commercial Update
- Bo Cumbo, Head of Global Commercial

Looking Ahead
- Edward Kaye, M.D., Chief Executive Officer

Q&A Session

THANK YOU