

# SAREPTA THERAPEUTICS Q1 2018 EARNINGS CONFERENCE CALL 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 "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements by management relating to our future operations, financial performance and projections, business plans, priorities and research and development programs including: the promise of gene therapy to dramatically change the course of disease becoming a reality and our intention to play a central role in shaping this reality; Sarepta remaining on track to achieve its full-year guidance of \$295 to \$305 million; our plan to engage in a policy-level discussion with the FDA to discuss how to efficiently bring ultra-rare exon PPMOs to patients with DMD; our plan to build out a strategy for the use of our PMO and PPMO technology in other rare diseases over the course of the year; the potential benefits of the partnership with Myonexus and expected payments in connection with the agreement with Myonexus; LGMD's potential patient population; our plan to seek a re-examination of our MAA for eteplirsen and to request that a SAG be convened; our expectation that the re-examination process will likely be completed by year-end 2018; expected milestones and timelines, including commencing the first clinical trial for MYO-101 in mid-2018 and reporting gene expression data in late-2018 to early 2019, completing a rolling NDA submission by year-end 2018, seeking accelerated approval based on an increase in dystrophin protein as a surrogate endpoint, with a target approval in mid-2019, presenting our micro-dystrophin safety and expression data from at least two patients enrolled in our current gene therapy clinical trial on June 19, 2018, announcing progress later in the year on our SAD study for SRP-5051, launching up to 6 different gene therapy programs within a 2-year window of time, and possibly multiple PMO and PPMO based programs over the same time period; the potential benefits of our product candidates and technologies, including in collaboration with strategic partners, such as PPMO's potential for significantly improved efficacy with less frequent dosing for patients, and PPMO offering utility in a broad range of other diseases and the potential of our first six PPMO candidates to treat up to 43% of the DMD population; our expectation that the average age of patients will not change dramatically until newborn screening for Duchenne becomes standard medical practice, which would identify hundreds of new patients eligible for EXONDYS 51 and other exon-skipping or Gene Therapy products; our plan to continue to strengthen our global presence in Europe by solidifying our relationships with KOLs, partnering with advocacy groups and advancing our distribution network during the re-examination period with the EMA; our plan for the potential of combination therapy with our RNA programs; and our expected operational and executional success as we now have one of the deepest rare disease pipelines in biotech.*

*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 granted a re-examination of our MAA for eteplirsen, a SAG may not be convened, and even if a re-examination and a related SAG are granted, the CHMP may render a negative opinion and we may not be able to obtain regulatory approval for eteplirsen from the European Medicines Agency; our data for golodirsen may not be sufficient for a filing for or obtaining regulatory approval; the expected benefits and opportunities related to the agreement with Myonexus may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership with Myonexus may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement, including any inability by us to fulfill our financial commitments to Myonexus, and even if the agreement results in new commercialized products, we may not achieve any significant revenues from the sale of such products; 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, re-search 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 the USPTO with respect to patents that cover our product candidates; potential strategic transactions may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products and retention of key employees; 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 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.*

