

# SAREPTA THERAPEUTICS Q4 & FULL-YEAR 2017 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: Sarepta having the ability, through a ruthless focus on execution, to fulfill its commitments; our goal to improve and extend the lives of individuals with DMD, profoundly improve of all patients with DMD, and emerge as one of the most important leaders around the globe in precision genetic medicine; Sarepta having all the elements to make its goal a reality; our revenue guidance for 2018; our expectation for a nearly 100 percent year-over-year growth rate versus 2017; our team being engaged and continuing to execute well, with progress in securing reimbursement, and persistent commitment from prescribing physicians, patients and their caregivers; our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design, enrollment rates and results of such trials, including the potential consistency of data produced by these trials with prior results; the positive dystrophin results from our 4053-101 study offering potential to treat 8% of the DMD community and validating our precision medicine/RNA-splicing platform and our focus on scientific excellence through continuous methodological improvements; the application of the FDA draft guidance from February 2018 for the development of treatments for DMD and related dystrophinopathies to our product candidates; our expectation regarding having an oral explanation in late April and the completion of the review of our application by the CHMP in mid-2018; our belief that we have compiled a robust data package and a strong response to the CHMP 180-day questions; the potential benefits of our technologies, including PPMO's potential to increase cell-penetration, with the goal of producing greater quantities of dystrophin with less frequent dosing, PPMO's potential to treat 13% of DMD patients who have exon 51 amenable mutations, PPMO being a promising next-generation therapy platform for DMD and having potential utility in a broad range of other diseases, the potential of our first 6 PPMO candidates to treat up to 43% of the DMD population, and GALGT2 gene therapy program's potential to preserve muscle function regardless of genetic mutation; our plan to bring PPMO therapies to the extremely rare exon mutations; our ESSENCE study remaining on track and the expectation to complete enrollment in the near term; our expectation for a solid 2018 and beyond, and our plans for 2018, including continuing the successful launch for EXONDYS 51, building out a strategy for the use of our PMO and PPMO technology in other rare diseases, reporting preliminary data from our micro-dystrophin gene therapy program by mid-2018, dosing a second patient in the GALGT2 gene therapy program early in the second quarter and reporting data in the second half of 2018; our bolstered balance sheet laying the foundation for a successful 2018 and beyond and providing the resources to accelerate our R&D, expand our talent base and execute on our strategic plan; our plan to accelerate the development of our gene therapy and PPMO programs and to utilize our resources to advance our industry leading pipeline for DMD and pursue multiple disease targets for underserved and life-threatening rare diseases; our planned efforts to provide access to EXONDYS 51 to all amenable patients and to ensure physicians understand the importance of identifying appropriate patients and starting them on EXONDYS 51; our expectations regarding future age demographic for EXONDYS 51, port use by patients, compliance staying relatively high, and the permanent J code helping ease future reauthorizations; our plan to continue to build thoughtful operational infrastructure to prepare for EXONDYS 51 launches in other key markets and future approvals of other product candidates; and our expectation that 2018 will be the year of transformation, with a steady stream of catalysts and milestones, all as we continue to serve our community with eteplirsen and advance our pipeline with urgency.*

*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 EMA; our data for golodirsen and for other product candidates 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 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 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.*

