

# SAREPTA THERAPEUTICS Q3 2018 EARNINGS CONFERENCE CALL, OCTOBER 24, 2018

## 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 remaining on track to achieve its full-year guidance of \$295 to \$305 million; the expectation to see modest contribution from our MAP through 2018, with increasing contribution in 2019 and beyond; our plan to evaluate the CHMP's guidance and take scientific advice from the EMA later in 2019; our goal to bring all our therapies to the greatest number of patients around the world who could benefit from them; the potential of casimersen, golodirsen and Exondys 51 to serve nearly 30% of the DMD community in the U.S.; the theoretical probability of success of each of our gene therapy programs, their potential market opportunity and the expected accelerated timelines for approval; the opportunity to rapidly follow on our success with micro-dystrophin with new therapeutic areas and new constructs and the potential of the success we build with each program to read through to the next program; the potentially significant read-through from our micro-dystrophin program to our LGMD programs; our goal with Brammer to have more gene therapy commercial capacity in about two years than all of the gene therapy capacity that currently exists in the world; the potential benefits of our relationship with Paragon; our plan to continue to forge strong partnerships with the leaders in gene therapy over the coming months; expected milestones and timelines, including having dosing and safety insight regarding our exon 51 PPMO program by the first quarter of 2019, filing INDs for another 5 PPMO programs, which along with our first could serve approximately 43% of the DMD community, dosing all of the patients in the first cohort of our 2E program this year, with biomarker results in the first quarter of 2019, completing our rolling FDA submission for golodirsen by year end, with a target approval in 2019, submitting our NDA for casimersen by mid-2019 with a target approval by the end of 2019 to first quarter of 2020, having a meeting with the FDA with respect to our gene therapy micro dystrophin program in the 4th quarter and commencing a pivotal trial by the end of 2018, commencing a pivotal trial for MPS IIIA by the end of 2018, commencing our LGMD 2E pivotal trial next year; beginning dosing in CMT patients in the first half of 2019, and dosing in the Pompe program in the first half of 2019; the potential benefits of our product candidates and technologies, including in collaboration with strategic partners, such as the potential of our micro-dystrophin gene therapy program to be a transformative, one-time therapy; our expectation to have some number of years of enormous growth if our micro-dystrophin program is successful, as we treat the greatest percentage possible of the prevalent population of DMD around the world and then treating the incident population; potentially positioning investors for an ROI that dwarfs industry standards; our expectation that our cost of sales in Q4 2018 will be consistent with Q3; our belief that our recent investments lay the foundation for building shareholder value for many years to come; and our intention to be the leaders of the revolution that precision genetic medicine is driving.*

*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; the expected benefits and opportunities related to our recent transactions may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; our recent partnerships may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreements; the expected benefits and opportunities related to the agreements with Brammer and Paragon may not be realized or may take longer to realize than expected; Sarepta's dependence on its manufacturers to produce its product candidates, including any inability on Sarepta's part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; Sarepta may not be able to eventually obtain regulatory approval for eteplirsen, or any other product candidates, from EMA; there is no assurance that we will be able to rely on the recent FDA guidance on gene therapy in rare disease to expedite the development of our gene therapy-based product candidate; our data for golodirsen and casimersen may not be sufficient for a filing for or obtaining regulatory approval; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, some of which may be outside of our control, 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 Sarepta's 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.*

