



Sarepta Therapeutics Announces FDA Approval of VYONDYS 53™ (golodirsen) Injection for the Treatment of Duchenne Muscular Dystrophy (DMD) in Patients Amenable to Skipping Exon 53

-- VYONDYS 53 is Sarepta's second RNA exon-skipping treatment for DMD approved in the U.S. --

-- Commercial distribution of VYONDYS 53 in the U.S. will commence immediately --

-- Information for patients and clinicians is available at www.SareptAssist.com --

CAMBRIDGE, Mass., Dec. 12, 2019 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has approved VYONDYS 53™ (golodirsen). VYONDYS 53 is an antisense oligonucleotide from Sarepta's phosphorodiamidate morpholino oligomer (PMO) platform, indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation amenable to exon 53 skipping. This indication is based on a statistically significant increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53, which is reasonably likely to predict clinical benefit for those patients who are exon 53 amenable. Consistent with the accelerated approval pathway, the continued approval of VYONDYS 53 may be contingent on confirmation of a clinical benefit in this post-marketing confirmatory trial.

Sarepta's placebo-controlled, post-marketing confirmatory trial to support the VYONDYS 53 accelerated approval – titled ESSENCE – is currently enrolling and expected to conclude by 2024.

Hypersensitivity reactions, including rash, pyrexia (fever), pruritis, urticaria (hives), dermatitis, and skin exfoliation have occurred in patients who were treated with VYONDYS 53. Renal toxicity was observed in animal studies. Although not observed in the clinical studies with VYONDYS 53, renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. The most common adverse reactions that occurred in at least 20% of VYONDYS 53-treated patients and more frequently than in placebo-treated patients were headache (41%), pyrexia (41%), fall (29%), abdominal pain (27%), nasopharyngitis (27%), cough (27%), vomiting (27%), and nausea (20%).

Following a New Drug Application (NDA) submission to and review by the Division of Neurology Products (the Review Division) for VYONDYS 53, which the Review Division recommended for approval, the Office of Drug Evaluation 1 issued a complete response letter (CRL) in August of 2019. Thereafter, Sarepta made

a formal dispute resolution request as outlined in relevant FDA Guidance. With the support of the Review Division, the matters raised in the CRL were rapidly evaluated and resolved by Dr. Peter Stein, Director of the Office of New Drugs (OND). OND granted the Company's appeal and Sarepta re-submitted its NDA to the Review Division, which worked expeditiously to review and approve VYONDYS 53.

"Today is monumental for Sarepta and, more importantly, for the DMD community," said Doug Ingram, president and chief executive officer, Sarepta. "VYONDYS 53, our second approved exon-skipping RNA therapy for DMD, may treat up to 8% of the DMD community, representing those patients who have a confirmed exon 53 amenable mutation. Along with EXONDYS 51® (eteplirsen), we now offer treatment options for approximately 20% of those with DMD in the U.S."

Ingram continued, "In the span of four months, we commenced and completed the formal dispute resolution process culminating in the grant of our appeal, resubmitted our NDA and obtained an approval – a great benefit to DMD patients awaiting treatment. This unprecedented timing could not have been achieved without the commitment of the Review Division under the leadership of Dr. Billy Dunn, and the Office of New Drugs, which expeditiously heard and granted our appeal. Along with the DMD community, we owe our gratitude to both the Review Division and the OND for their objective, evidence-based approach to this review, for their fairness, and for the sense of urgency with which they addressed and resolved the CRL and granted this approval."

"With the approval of VYONDYS 53, up to another 8% of Duchenne families will have a therapy to treat this devastating disease," said Pat Furlong, founding president and chief executive officer, Parent Project Muscular Dystrophy (PPMD). "For 25 years, PPMD has been working with researchers, clinicians, industry, and the Duchenne community to find treatments for all people living with Duchenne. And while we need to ensure that these approved therapies are accessible for patients, today we celebrate this approval and thank Sarepta for their continued leadership in the fight to end Duchenne."

VYONDYS 53 is priced at parity to EXONDYS 51, the price of which has not increased since its launch in 2016. Patients and physicians can access more information at www.SareptAssist.com or by calling 1-888-727-3782.

About VYONDYS 53

VYONDYS 53 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. VYONDYS 53 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping,"

of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

VYONDYS 53 is approved under accelerated review based on an increase in dystrophin production in skeletal muscle of patients amenable to exon 53 skipping. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

VYONDYS 53 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for VYONDYS 53

Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy.

Renal toxicity was observed in animals who received golodirsen. Although renal toxicity was not observed in the clinical studies with VYONDYS 53, renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Renal function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of renal function in DMD patients. Measurement of glomerular filtration rate (GFR) by 24-hour urine collection prior to initiation of therapy is recommended. Monthly monitoring for proteinuria by dipstick urinalysis and monitoring of serum cystatin C every three months is recommended. In the case of a confirmed dipstick proteinuria of 2+ or greater or elevated serum cystatin C, a 24-hour urine collection to quantify proteinuria and assess GFR should be performed.

Adverse reactions observed in at least 20% of treated patients and greater than placebo were (VYONDYS 53, placebo): headache (41%, 10%), pyrexia (41%, 14%), fall (29%, 19%), abdominal pain (27%, 10%), nasopharyngitis (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

For further information, please see the full [Prescribing Information](#).

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 mg 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 is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 6 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit www.sarepta.com.

Forward-Looking Statement

This press release contains "forward-looking statements." 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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding the immediate commencement of commercial distribution of VYONDYS 53 in the U.S.; VYONDYS 53's continued approval for its indication potentially being contingent upon verification of a clinical benefit in confirmatory trials; the potential benefits and risks of VYONDYS 53; VYONDYS 53's potential to treat up to another 8% of those living with DMD; the potential of EXONDYS 51 and VYONDYS 53 to treat up to 20% of those with DMD in the U.S.; exon skipping's intention to allow for production of an internally truncated dystrophin protein; and our mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the planned commercial launch in the U.S. for VYONDYS 53 may not be successful for various reasons including the actual market size and drug supply needed may not be consistent with the company's expectations and its executed commercial readiness plans, the degree to which VYONDYS 53 is accepted by patients and prescribed by physicians, manufacturing limitations that may not be anticipated or resolved for in a timely manner or at all, the efficiency of our manufacturing, sales, distribution and specialty pharmacy network in getting VYONDYS 53 to the market and future economic, competitive, reimbursement and regulatory conditions that could negatively impact the commercial launch of VYONDYS 53; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 and VYONDYS 53 in a timely manner or at all; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of our product candidates; the results of our ongoing research and development efforts and clinical trials for our products and product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit or support an NDA or a BLA filing, positive advisory committee recommendation or marketing approval by the FDA or other regulatory authority; 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, including the commercialization of VYONDYS 53, for various reasons, including factors outside of our control, such as possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner or at all, 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 and 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, 2018, 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. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2018 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the SEC as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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.

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