Sarepta Therapeutics Announces FDA Approval of AMONDYS 45™ (casimersen) Injection for the Treatment of Duchenne Muscular Dystrophy (DMD) in Patients Amenable to Skipping Exon 45

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-- AMONDYS 45 is Sarepta's third RNA exon-skipping treatment for DMD approved in the U.S. --
-- Commercial distribution of AMONDYS 45 in the U.S. will commence immediately --
-- Information for patients and clinicians is available at www.SareptAssist.com --

CAMBRIDGE, Mass., Feb. 25, 2021 (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 AMONDYS 45 (casimersen). AMONDYS 45 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 45 skipping. This indication is based on a statistically significant increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45, which is reasonably likely to predict clinical benefit for those patients who are exon 45 amenable. Consistent with the accelerated approval pathway, the continued approval of AMONDYS 45 may be contingent on confirmation of a clinical benefit in confirmatory trials.

The ESSENCE trial – a placebo-controlled confirmatory trial to support the AMONDYS 45 approval – is ongoing and expected to conclude in 2024.

Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. In the clinical trial, the most common adverse reactions observed in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in placebo were (AMONDYS 45, placebo): upper respiratory tract infections (65%, 55%), cough (33%, 26%), fever (33%, 23%), headache (32%, 19%), joint pain (21%, 10%), and pain in mouth and throat (21%, 7%).

"This is an important day for Sarepta and, far more importantly, for the patients that we serve. After years of scientific commitment, investment and development, the approval of AMONDYS 45, Sarepta's third approved RNA therapy, offers treatment to the 8% of the DMD community who have a confirmed exon 45 amenable mutation," said Doug Ingram, president and chief executive officer, Sarepta. "Along with our other approved RNA therapies, we can now offer treatment options for nearly 30% of Duchenne patients in the U.S. And our commitment to bring therapies to the greatest percentage of the DMD community as soon as possible continues."

"Decades of research and commitment have fueled and now accelerate our progress towards new treatments for Duchenne," said Marissa Penrod, founder of Team Joseph and parent of an 18-year old with Duchenne. "The extraordinary diligence and persistence of the Duchenne community – patients and families, clinicians and researchers – have led us to today's approval, where we now have exon-skipping treatments for almost a third of those with Duchenne."

AMONDYS 45 is priced at parity with Sarepta's other approved exon-skipping treatments. Patients and physicians can access more information at www.SareptAssist.com or by calling 1-888-727-3782.

About AMONDYS 45
AMONDYS 45 (casimersen) 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 45 skipping. AMONDYS 45 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 45 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 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

AMONDYS 45 is approved under accelerated review based on an increase in dystrophin production in skeletal muscle of patients amenable to exon 45 skipping. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

AMONDYS 45 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for AMONDYS 45
Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse reactions observed in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were (AMONDYS 45, placebo): upper respiratory tract infections, including upper respiratory infection, pharyngitis, nasopharyngitis and rhinitis (65%, 55%), cough (33%, 26%), pyrexia (33%, 23%), headache (32%, 19%), arthralgia (21%, 10%), and oropharyngeal pain (21%, 7%).

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45, and that were reported at a rate at least 5% more frequently in the AMONDYS 45 group than in the placebo group, were: ear pain, nausea, ear infection, post-traumatic pain, and dizziness and light-headedness.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics
At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit **www.sarepta.com** or follow us on **Twitter**, **LinkedIn**, **Instagram** and **Facebook**.

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 AMONDYS 45 in the U.S.; AMONDYS 45's continued approval potentially being contingent upon confirmation of a clinical benefit in confirmatory trials; the expectation to conclude the ESSENCE trial by 2024; AMONDYS 45's potential to treat 8% of the DMD community who have a confirmed exon 45 amenable mutation; Sarepta being able to offer treatment options for nearly 30% of Duchenne patients in the U.S.; the potential benefits and risks of AMONDYS 45; our commitment to bring therapies to the greatest percentage of the DMD community as soon as possible; and exon skipping's intention to allow for production of an internally truncated dystrophin protein.

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 AMONDYS 45 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 AMONDYS 45 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 AMONDYS 45 to the market, the response to COVID-19 and future economic, competitive, reimbursement and regulatory conditions that could negatively impact the commercial launch of AMONDYS 45; we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; 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 AMONDYS 45, 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, COVID-19 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, 2019, 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 2019 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.*

Source: Sarepta Therapeutics, Inc.

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