Wave Life Sciences Announces Fast Track Designation from U.S. FDA for Suvodirsen

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Suvodirsen is an investigational stereopure oligonucleotide in development for the treatment of DMD patients amenable to exon 51 skipping

Clinical dystrophin data from interim analysis of ongoing OLE study on track for 4Q 2019

DYSTANCE 51 global Phase 2/3 trial is underway

CAMBRIDGE, Mass., Sept. 16, 2019 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to suvodirsen for the treatment of Duchenne muscular dystrophy (DMD) in patients amenable to exon 51 skipping. The designation was based on comprehensive *in vitro* and *in vivo* nonclinical data that support the potential for suvodirsen to address a significant unmet medical need.

"Our goal for Wave's Duchenne programs is to urgently develop therapies that restore functional dystrophin to levels that have the potential to result in meaningful clinical benefit," said Michael Panzara, MD, MPH, Chief Medical Officer of Wave Life Sciences. "We are thrilled to have received Fast Track designation from the FDA for suvodirsen, a decision which we believe reflects its potential to provide a meaningful option for those living with this dreadful genetic disease. We look forward to continuing to work with regulatory authorities with the hope of bringing suvodirsen to the Duchenne community as quickly as possible."

Fast Track designation is granted by the FDA for products that are intended for the treatment of serious or life-threatening disease or conditions, which demonstrate the potential to address an unmet medical need. The designation offers the opportunity for frequent interactions with the FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval, as well as eligibility for rolling submission of a New Drug Application (NDA).

Suvodirsen is currently being evaluated in an ongoing open-label extension (OLE) study for DMD patients amenable to exon 51 skipping. Wave remains on track to deliver an interim analysis of dystrophin expression from muscle biopsies in boys receiving suvodirsen in the OLE study in the fourth quarter of 2019. Pending positive clinical dystrophin expression data, the company expects to file for an accelerated approval of suvodirsen in the U.S. in the second half of 2020. Suvodirsen is also currently being studied in DYSTANCE 51, a global Phase 2/3, multicenter, randomized, double-blind, placebo-controlled trial that will evaluate the efficacy and safety of suvodirsen. Results from the DYSTANCE 51 trial are intended to support global regulatory filings for suvodirsen. The study is also the first ever selected by the FDA for its Complex Innovative Trial Design (CID) pilot program.

Suvodirsen has also been granted orphan drug designation for the treatment of DMD by the FDA and the European Commission, as well as rare pediatric disease designation by the FDA. In addition to suvodirsen, Wave continues to advance WVE-N531, its preclinical candidate to treat DMD in boys amenable to exon 53 skipping. The company is also exploring exon targets beyond those targeted by suvodirsen and WVE-N531, including exons 44, 45, 52, 54 and 55.

About Suvodirsen
Suvodirsen is an investigational stereopure oligonucleotide currently being evaluated in an ongoing open-label extension (OLE) study for the treatment of boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. Suvodirsen is also being a studied in DYSTANCE 51, a global Phase 2/3, multicenter, randomized, double-blind, placebo-controlled trial that will evaluate the efficacy and safety of suvodirsen.

Approximately 13% of DMD patients have genetic mutations that are amenable to treatment with an exon 51 skipping therapy. Exon-skipping technology has the potential to induce cellular machinery to 'skip over' a targeted exon and restore the reading frame, resulting in the production of internally truncated, but functional dystrophin protein.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (DMD) is a fatal X-linked genetic neuromuscular disorder caused predominantly by out-of-frame deletions in the *dystrophin* gene, resulting in absent or defective dystrophin protein. Dystrophin protein is needed for normal muscle maintenance and operation. Because of the genetic mutations in DMD, the body cannot produce functional dystrophin, which results in progressive and irreversible loss of muscle function, including the heart and lungs. Worldwide, DMD affects approximately one in 5,000 newborn boys.

About Wave Life Sciences
Wave Life Sciences (NASDAQ: WVE) is a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases. Wave aspires to develop best-in-class medicines across multiple therapeutic modalities using PRISM™, the company's proprietary discovery and drug development platform that enables the precise design, optimization and production of stereopure oligonucleotides. Driven by a resolute sense of urgency, the Wave team is targeting a broad range of genetically defined diseases so that patients and families may realize a brighter future. To find out more, please visit www.wavelifesciences.com and follow Wave on Twitter @WaveLifeSci.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, the anticipated benefits of having received Fast Track designation for suvodirsen, the expected timing and plans to report interim data from the ongoing OLE, the intention to use the results from the OLE and Phase 2/3 trials to seek various regulatory approvals for suvodirsen globally, and the anticipated timing of such regulatory filing in the United States. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release. These risks and uncertainties include but are not limited to the following: Wave's current and planned clinical trials, other studies for suvodirsen and Wave's other product candidates may not be successful or may take longer and be more costly than anticipated; product candidates that appeared promising in earlier research and clinical trials may not demonstrate safety and/or efficacy in later-stage or larger-scale clinical trials; and the other risk factors discussed under the heading "Risk Factors" contained in Wave's Annual Report on Form 10-K for the year ended December

31, 2018 filed with the Securities and Exchange Commission (SEC), as well as in other filings Wave makes with the SEC from time to time. All statements contained in this press release are made only as of the date of this press release, and Wave undertakes no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

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