Wave Life Sciences Announces Initiation of DYSTANCE 51, a Phase 2/3 Clinical Trial of Suvodirsen

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Global study designed to assess the efficacy and safety of suvodirsen in DMD patients amenable to exon 51 skipping

Results intended to support regulatory approvals globally

CAMBRIDGE, Mass., June 27, 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 the initiation of DYSTANCE 51, the Phase 2/3 efficacy and safety clinical trial of suvodirsen, an investigational stereopure oligonucleotide intended for boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping.

“The initiation of the DYSTANCE 51 Phase 2/3 study represents a significant milestone for Wave and our DMD clinical program, as well as an important development for the families affected by Duchenne,” said Michael Panzara, MD, MPH, Chief Medical Officer of Wave Life Sciences. “We are grateful to be working in collaboration with the U.S. Food and Drug Administration, global regulators and our partners from the Duchenne community on this innovative and exciting trial. We are proud to have worked with patient groups to incorporate their feedback into the trial design to better address the needs of boys and families living with DMD.”

"We are pleased that Wave Life Sciences has incorporated important input from the Duchenne community in designing their Phase 2/3 clinical trial of suvodirsen. This global study is designed around the needs of families while measuring a robust set of biomarker and functional endpoints. PPMD looks forward to learning more about this stereopure exon-skipping program when Wave presents at our 25th Annual Conference later this week," said Pat Furlong, founding President and CEO of Parent Project Muscular Dystrophy (PPMD).

DYSTANCE 51 is a global study and is expected to include clinical trial sites in the United States, Europe, Australia, Canada and Japan. Results from this trial are intended to support global regulatory filings for suvodirsen.

DYSTANCE 51 Phase 2/3 Clinical Trial Design
DYSTANCE 51 is a global Phase 2/3, multicenter, randomized, double-blind, placebo-controlled clinical trial that will evaluate the efficacy and safety of suvodirsen in ambulatory boys who are between 5 and 12 years of age (inclusive) with a genetically confirmed diagnosis of DMD amenable to exon 51 skipping. The trial will test two dose levels of suvodirsen and is expected to enroll approximately 150 patients. The primary efficacy endpoints of the clinical trial will measure change in dystrophin protein level and change in the North Star Ambulatory Assessment (NSAA) score. In addition to the NSAA, the trial will include other functional outcome measures as secondary efficacy endpoints.

The DYSTANCE 51 clinical trial is the first study ever selected by the U.S. Food and Drug Administration (FDA) for its pilot program for complex innovative trial designs. As a participant in the pilot program, the company has held multiple meetings with FDA staff to discuss the design elements of the trial, including the potential use of Bayesian methods to adapt the trial with the aim of maximizing efficiency while ensuring robust clinical results.

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. Data from the company’s completed Phase 1 clinical trial of suvodirsen demonstrated a favorable safety and tolerability profile after single doses, supporting its continued clinical development, including the launch of DYSTANCE 51 and continuation of the OLE study. 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 second half of 2019.

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.

Suvodirsen has 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. Pending positive clinical dystrophin expression data, the company expects to file for an accelerated approval of suvodirsen in the United States in the second half of 2020.

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 countries in which the Phase 2/3 clinical trial for suvodirsen is expected to be conducted, the expected timing and plans to report interim data from the ongoing OLE, the belief that the safety and tolerability data from the Phase 1 clinical trial support initiation of the Phase 2/3 clinical trial, the anticipated benefits of participating in FDA’s complex innovative trial designs pilot program, the intention to use the results of the OLE and Phase 2/3 trials to seek various regulatory approvals globally and the anticipated timing of such regulatory filing in the U.S. 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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