# Wave Life Sciences to Present Suvodirsen Phase 1 Safety and Tolerability Data and Phase 2/3 Clinical Trial Design at the Muscular Dystrophy Association Clinical and Scientific Conference

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CAMBRIDGE, Mass., March 25, 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 four poster presentations highlighting new data and progress for its Duchenne muscular dystrophy (DMD) programs will be shared at the 2019 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference themed, “Progress in Motion,” April 13-17, 2019 in Orlando, Fla. This year the MDA annual conference will gather both healthcare providers and researchers together into one conference to discuss the latest in neuromuscular disease research and care.

Wave will present two posters focused on suvodirsen (WVE-210201), the company’s investigational stereopure oligonucleotide for boys with DMD who are amenable to exon 51 skipping:

- Safety and Tolerability of Suvodirsen (WVE-210201) in Patients with Duchenne Muscular Dystrophy: Results from a Phase 1 Clinical Trial
- Design of a Phase 2/3 Randomized Controlled Trial of Suvodirsen (WVE-210201) in Patients with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

Two additional posters providing preclinical data on Wave’s DMD programs will also be presented:

- Potency of Stereopure Antisense Oligonucleotides in Cellular Free-Uptake Model Predicts Exon Skipping and Dystrophin Protein Restoration in mdx23 Mice
- Investigational Stereopure Exon-Skipping Antisense Oligonucleotides Targeting Exons 51 and 53 in Duchenne Muscular Dystrophy

Conference organizers will announce poster numbers and schedules.

**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. Globally, DMD affects approximately one in 5,000 newborn boys.

**About Suvodirsen (WVE-210201)**

Suvodirsen is an investigational stereopure oligonucleotide that has been shown to induce skipping of exon 51 of *dystrophin* pre-mRNA in preclinical studies and is intended for the treatment of Duchenne muscular dystrophy (DMD). 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. Wave preclinical *in vitro* experiments using gymnotic delivery (free uptake) of suvodirsen in DMD patient-derived myoblasts demonstrated efficient exon 51 skipping and dystrophin protein restoration. Preclinical western blot studies of suvodirsen demonstrated 52% dystrophin protein restoration compared with normal skeletal muscle tissue lysates. Suvodirsen has been granted orphan drug designation for the treatment of DMD by the U.S. Food and Drug Administration (FDA) and the European Commission, as well as rare pediatric disease designation by the FDA. In addition, the planned Phase 2/3 clinical trial of suvodirsen was the first study ever selected for the FDA pilot program for complex innovative trial designs (CID).

**About Suvodirsen (WVE-210201) Clinical Trials**

Suvodirsen was studied in a global, multicenter, double-blind, placebo-controlled Phase 1 clinical trial designed to evaluate the safety, tolerability and plasma concentrations of single ascending doses of suvodirsen administered intravenously in Duchenne muscular dystrophy patients with gene mutations amenable to exon 51 skipping. In December 2018, the company announced that, based on results from four ascending dose cohorts in the Phase 1 clinical trial, the safety and tolerability profile of suvodirsen supports initiation of a Phase 2/3 clinical trial.

Suvodirsen is currently being studied in an ongoing multi-dose, open-label extension (OLE) study which was initiated in August 2018 with patients from the Phase 1 clinical trial. The company 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. Data from this analysis are intended to be an important component of a submission to the U.S. Food and Drug Administration for accelerated approval in the United States.

Wave anticipates initiating a global, placebo-controlled Phase 2/3 efficacy and safety clinical trial of suvodirsen in DMD patients amenable to exon 51 skipping in 2019. The trial is designed to measure clinical efficacy and dystrophin expression, and Wave intends to use the results of this trial to seek regulatory approvals globally.

**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 expected timing of an interim analysis of dystrophin expression from muscle biopsies in boys receiving suvodirsen in the Phase 1 OLE study, Wave's intention to potentially use the interim analysis data to support an accelerated approval in the U.S., the expected timing of initiation for and the anticipated endpoints of a suvodirsen Phase 2/3 clinical trial, and Wave's intention to potentially use the results of the Phase 2/3 trial to seek regulatory approvals globally. 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, including, without limitation, the risks and uncertainties described in the section entitled "Risk Factors" in Wave's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as amended, and in other filings Wave makes with the SEC from time to time. Wave undertakes no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

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