Wave Life Sciences to Present at Parent Project Muscular Dystrophy 2019 Annual Conference

June 12, 2019

CAMBRIDGE, Mass., June 12, 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 Michael Panzara, MD, MPH, Chief Medical Officer, will present at the Parent Project Muscular Dystrophy (PPMD) 2019 Annual Conference in Orlando, Fla. on Saturday, June 29, 2019 at 12:00 p.m. ET. The presentation will include an overview of the ongoing clinical development of suvodirsen, the company's investigational stereopure oligonucleotide for boys with Duchenne muscular dystrophy who are amenable to exon 51 skipping.

Suvodirsen is currently being studied in an open label extension (OLE) study, initiated in August 2018 with patients from the Phase 1 clinical trial. Wave expects 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. 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.

The presentation slide deck will be available for a limited time on the investor relations page of the Wave Life Sciences corporate website at http://ir.wavelifesciences.com. A live stream of the presentation will be available on the PPMD website during the conference.

During PPMD, Wave will also present two posters with previously presented data and information on suvodirsen and DYSTANCE 51, the planned Phase 2/3 efficacy and safety clinical trial in DMD patients amenable to exon 51 skipping.

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 a completed Phase 1 clinical trial of suvodirsen demonstrated a favorable safety and tolerability profile, supporting the continued clinical development of the investigational therapy in DMD.

In July 2019, Wave expects to initiate DYSTANCE 51, a global, multicenter, randomized, double-blind, placebo-controlled Phase 2/3 efficacy and safety clinical trial of suvodirsen in DMD patients amenable to exon 51 skipping. The company intends to use the results of this trial to seek regulatory approvals globally.

The DYSTANCE 51 Phase 2/3 clinical trial was the first study ever selected by the U.S. Food and Drug Administration (FDA) for the agency's pilot program for complex innovative trial designs. In addition, 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.

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 expected timing of initiation of the Phase 2/3 clinical trial for suvodirsen in DMD, the plans to report interim efficacy data from the ongoing open-label extension study with patients from the Phase 1 clinical trial of suvodirsen in DMD, the belief that the safety and tolerability data from the Phase 1 clinical trial support initiation of a Phase 2/3 clinical trial, Wave's intention to use the results of the OLE and Phase 2/3 trials to seek various regulatory approvals for suvodirsen. 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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Source: Wave Life Sciences