



## Wave Life Sciences Announces Positive Phase 1 Results for WVE-210201 in Duchenne Muscular Dystrophy (DMD)

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**WVE-210201 safety and tolerability profile supports Phase 2/3 clinical trial initiation in boys with DMD amenable to exon 51 skipping**

**Delivery of interim efficacy data from ongoing open-label extension study expected in H2 2019**

CAMBRIDGE, Mass., Dec. 06, 2018 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (NASDAQ: WVE), a biotechnology company focused on delivering transformational therapies for patients with serious, genetically-defined diseases, today announced that the safety and tolerability data from the WVE-210201 Phase 1 clinical trial in boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping support initiation of a Phase 2/3 clinical trial. Based on results from four ascending dose cohorts in the Phase 1 trial and pending final analysis, Wave has selected a dose for its planned Phase 2/3 clinical trial of WVE-210201, which it intends to initiate in 2019. In parallel, the independent Safety Monitoring Committee of the Phase 1 clinical trial has endorsed moving forward with a higher dose of WVE-210201 to be studied in a fifth cohort. Wave plans to present the results from the WVE-210201 Phase 1 clinical trial, as well as details of the Phase 2/3 study design, at upcoming scientific meetings.

"These results mark an important milestone for the Duchenne community and toward our goal of inducing meaningful, natural dystrophin expression in boys with DMD who are amenable to exon 51 skipping," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "This is an exciting time at Wave as we continue to partner with the patient and medical communities to advance our lead program in DMD. We thank all of the boys and their families who are participating in this Phase 1 trial and its open-label extension and are grateful for their trust and commitment."

WVE-210201 is also currently being studied in an ongoing multi-dose, open-label extension (OLE) study initiated in August 2018 and available to patients as they complete the Phase 1 clinical trial. The company remains on track to deliver an interim analysis of dystrophin expression from muscle biopsies in boys receiving WVE-210201 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.

"PPMD continues to be optimistic about the progress the team at Wave Life Sciences is making with their stereopure exon 51 skipping program for Duchenne. We are pleased to see WVE-210201 advance on the clinical development path and look forward to more updates from the Wave team," said Pat Furlong, founding President and CEO of Parent Project Muscular Dystrophy (PPMD).

Wave anticipates initiating a global, placebo-controlled Phase 2/3 efficacy and safety clinical trial of WVE-210201 in DMD patients amenable to exon 51 skipping in 2019. The Phase 2/3 trial is designed to measure clinical efficacy and dystrophin expression, and the company intends to use the results of this trial to seek regulatory approvals globally.

### 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 in the heart and lungs. Globally, DMD affects approximately one in 5,000 newborn boys.

### About WVE-210201

WVE-210201 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 WVE-210201 in DMD patient-derived myoblasts demonstrated efficient exon 51 skipping and dystrophin protein restoration. Preclinical Western blot studies of WVE-210201 demonstrated 52% dystrophin protein restoration compared with normal skeletal muscle tissue lysates. WVE-210201 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.

### About WVE-210201 Clinical Trials

WVE-210201 is being studied in an ongoing global, multicenter, double-blind, placebo-controlled Phase 1 clinical trial designed to evaluate the safety, tolerability and plasma concentrations of single ascending doses of WVE-210201 administered intravenously in Duchenne muscular dystrophy patients with gene mutations amenable to exon 51 skipping. The trial is expected to enroll up to 40 patients, including ambulatory and non-ambulatory patients between 5 and 18 years of age. As patients complete the Phase 1 trial, they have the option to enroll in an ongoing open-label extension study in which they receive WVE-210201.

### About Wave Life Sciences

Wave Life Sciences is a biotechnology company focused on delivering transformational therapies for patients with serious, genetically-defined diseases. Its chemistry platform enables the creation of highly specific, well characterized oligonucleotides designed to deliver superior efficacy and safety across multiple therapeutic modalities. The company's pipeline is initially focused on neurological disorders and extends across several other therapeutic areas. For more information, please visit [www.wavelifesciences.com](http://www.wavelifesciences.com).

### 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 a WVE-210201 Phase 2/3 clinical trial, the plans to present the WVE-210201 Phase 1 clinical trial results and details of the Phase 2/3 study design at upcoming scientific meetings, the expected timing of an interim analysis of dystrophin expression from muscle biopsies in boys receiving WVE-210201 in the OLE study, the belief that the safety and tolerability data from the WVE-210201 Phase 1 clinical trial supports initiation of a Phase 2/3 clinical trial, and Wave's intention to use the results of that 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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