# Wave Life Sciences Receives US Orphan Drug and Rare Pediatric Disease Designations for WVE-210201

August 16, 2018

**Investigational WVE-210201 in development for treatment of Duchenne muscular dystrophy patients amenable to exon 51 skipping**

CAMBRIDGE, Mass., Aug. 16, 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 U.S. Food and Drug Administration (FDA) has granted both orphan drug designation and rare pediatric disease designation for WVE-210201 for the treatment of Duchenne muscular dystrophy (DMD). The European Commission previously granted orphan drug designation for WVE-210201 in July 2018.

“Our team is motivated by a sense of urgency and compassion for the patients, families and caregivers affected by Duchenne muscular dystrophy and other serious, life-threatening conditions with high areas of unmet need,” said Michael Panzara, MD, MPH, Neurology Franchise Lead of Wave Life Sciences. “We are very pleased to receive these two important designations from the FDA and believe they further reinforce the potential of WVE-210201 to help boys suffering from DMD.”

The Orphan Drug Act provides for economic incentives to encourage the development of drugs intended to treat, diagnose or prevent rare diseases and conditions affecting fewer than 200,000 people in the United States. In determining orphan drug designation, the FDA’s Office of Orphan Products Development evaluates preclinical and clinical data to identify products as promising for rare disease. If market approval is granted by the FDA for WVE-210201 for the treatment of DMD, orphan drug designation would entitle Wave to seven years of market exclusivity in the United States. Additional incentives may include tax credits related to clinical trial expenses, exemption from prescription drug user fees and FDA assistance in clinical trial design.

Rare pediatric disease designation by the FDA is granted in the case of serious or life-threatening diseases affecting fewer than 200,000 people in the United States in which the serious or life-threatening manifestations are primarily in individuals 18 years of age and younger. The designation provides regulatory incentives for companies to develop and market therapies that treat these conditions. The sponsor of a drug for a rare pediatric disease may be eligible for a priority review voucher upon approval of the drug that can be used to obtain a priority review of a subsequent marketing application.

"These designations from U.S. regulators represent the significant progress being made by Wave Life Sciences and our community is grateful to Wave for their ongoing commitment to the Duchenne muscular dystrophy community," said Pat Furlong, founding President and CEO of Parent Project Muscular Dystrophy. "We look forward to their exon 51 skipping program advancing and Wave’s other initiatives in DMD."

WVE-210201 is currently 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 DMD 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 the ages of 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 continued treatment with WVE-210201.

**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 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 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 as compared with normal skeletal muscle tissue lysates.

**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.

**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, statements regarding the potential of WVE-210201 to help boys suffering from DMD; the future performance and results of WVE-210201 in clinical trials; the potential of our preclinical data to predict the behavior of WVE-210201 in humans; the expected patient enrollment for our Phase 1 clinical trial of WVE-210201; and the anticipated benefits to Wave from receiving U.S. orphan drug and rare pediatric disease designations for WVE-210201. 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 our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as amended, and in other filings we make with the SEC from time to time. We undertake no obligation to update the information contained in

this press release to reflect subsequently occurring events or circumstances.

**Investor Contact:**
Jillian Connell
617-949-2981
jconnell@wavelifesci.com

**Media Contact:**
José Juves
617-949-4708
jjuves@wavelifesci.com

**Patient Contact:**
Wendy Erler
617-949-2898
werler@wavelifesci.com

Source: Wave Life Sciences