Wave Life Sciences Receives Positive Opinion for Orphan Drug Designation in the European Union for WVE-210201

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

Patients currently enrolling in global Phase 1 clinical trial

CAMBRIDGE, Mass., July 02, 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 European Medicines Agency (EMA) Committee for Orphan Medical Products (COMP) issued a positive opinion recommending WVE-210201 for designation as an orphan medicinal product for the treatment of Duchenne muscular dystrophy (DMD).

"This positive opinion from the EMA's Committee for Orphan Medical Products, based on our *in vivo* data supporting medical plausibility and the potential for significant benefit of WVE-210201, marks another milestone in our efforts to try to improve the lives of boys suffering from Duchenne muscular dystrophy," said Michael Panzara, MD, MPH, Neurology Franchise Lead of Wave Life Sciences. "We remain keenly focused on advancing WVE-210201 in our ongoing global Phase 1 clinical trial."

To be considered for Orphan Drug Designation in the EU, companies must provide data that demonstrate the plausibility for use of the investigational therapy in the treatment of the disease and establish that the drug has the potential to provide relevant advantages or a major contribution to patient care over existing therapies. The positive opinion issued by COMP is anticipated to be adopted by the European Commission (EC) at the end of July 2018.

Orphan Drug Designation by the EC provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and where no satisfactory treatment is available. Among the incentives available to medicines designated as orphan drugs by the EC are ten-year market exclusivity in the EU after product approval, eligibility for conditional marketing authorization, protocol assistance from the EMA at reduced fees during the product development phase and direct access to centralized marketing authorization in the EU.

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. Safety data from the trial are expected in the third quarter of 2018.

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 nonclinical 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 pre-clinical *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 likelihood and timing of adoption by the EC of the COMP's positive opinion for orphan drug designation for WVE-210201 in the EU; the potential of WVE-210201 to provide relevant advantages or a major contribution to patient care over existing therapies; the future performance and results of WVE-210201 in clinical trials; the potential of our *in vivo* preclinical data to predict the behavior of WVE-210201 in humans in clinical trials; the expected patient enrollment, duration and data readouts for our Phase 1 clinical trial of 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.

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