Wave Life Sciences Announces Discontinuation of Suvodirsen Development for Duchenne Muscular Dystrophy

December 16, 2019

No change from baseline in dystrophin observed in Phase 1 open-label extension study

Wave to host investor conference call at 8:00 a.m. ET today

CAMBRIDGE, Mass., Dec. 16, 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 its decision to discontinue development of suvodirsen for patients with Duchenne muscular dystrophy (DMD) who have mutations amenable to exon 51 skipping, based on its interim analysis of the Phase 1 open-label extension (OLE) study. The results showed no change from baseline in dystrophin expression, as measured by western blot, with either the 3.5 mg/kg or 5 mg/kg doses of suvodirsen. No safety concerns or emerging safety signals were observed.

As a result of this decision, the company is immediately discontinuing the two suvodirsen trials, the OLE study and the Phase 2/3 DYSTANCE 51 trial. Patients will have a final follow-up visit, but no further doses will be administered, and patients will no longer undergo muscle biopsies. In addition, Wave is suspending further development of WVE-N531 for patients with mutations amenable to exon 53 skipping.

“We set out to restore meaningful levels of dystrophin in patients with Duchenne, and we failed to achieve this goal,” said Michael Panzara, MD, MPH, Chief Medical Officer of Wave Life Sciences. “These results are not what we expected, particularly given the promising data from our preclinical models, and we commit to further analyzing and understanding the results to aid in future research. I would like to extend our gratitude to the patients and families for their courage and participation in this program, as well as to our colleagues at Duchenne centers throughout the world whose advice and dedication made the execution of these studies possible. We will share additional findings from the suvodirsen development program so that the Duchenne community may benefit from its contributions to this study.”

The interim analysis was from a global multicenter OLE study of suvodirsen in patients who previously enrolled in a Phase 1 safety and tolerability study. A total of 36 patients enrolled in the OLE and received the target doses of either 3.5 mg/kg or 5 mg/kg. A biopsy of the deltoid muscle was performed prior to initial dosing in the OLE and, as of data cut-off for this interim analysis, follow-up deltoid muscle biopsies were available for 27 of 36 patients. Within the 5 mg/kg weekly infusion arm, 10 patients received follow-up muscle biopsies at 12 weeks and nine patients received follow-up biopsies at 22 weeks. Within the 3.5 mg/kg weekly infusion arm, eight patients received follow-up muscle biopsies at 22 weeks. Biopsies from both timepoints were analyzed as part of this interim analysis.

“The suvodirsen results are unexpected and deeply disappointing to us, and undoubtedly will be to the patients we aim to serve. We are grateful to be part of the Duchenne community and our organization has been shaped by their strength and resilience,” said Paul Bolno, MD, CEO of Wave Life Sciences. “While we did not achieve dystrophin restoration in this study, there is a rising tide in nucleic acid therapeutics, and we are fully committed to advancing genetic medicines in diseases of the central nervous system, eye and liver. We will work to rapidly incorporate our learnings, so that we can seek to deliver on the promise of our current and future pipeline. As previously announced, we look forward to reporting topline clinical data from our PRECISION-HD2 trial in Huntington’s disease by the end of 2019.”

Revised Cash Guidance
As a result of the decisions announced above, Wave now expects that its existing cash and cash equivalents, together with expected and committed cash from existing collaborations, will enable Wave to fund its operating and capital expenditure requirements into the third quarter of 2021.

Investor Conference Call and Webcast
Wave management will host an investor conference call today at 8:00 a.m. ET. The conference call may be accessed by dialing +1 (866) 220-8068 for participants based in the United States or +1 (470) 495-9153 for participants based outside the United States, and entering conference ID 9967079. The live webcast may be accessed by visiting the “For Investors & Media” section of the Wave Life Sciences website at www.ir.wavelifesciences.com. Following the webcast, a replay will be available on the website.

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 Suvodirsen
Suvodirsen is an investigational stereopure oligonucleotide previously in development as a treatment for patients with Duchenne muscular dystrophy (DMD) who have genetic mutations amenable to exon 51 skipping. Wave initiated clinical development of suvodirsen in November 2017 and completed a Phase 1 safety and tolerability study in early 2019. Based on an interim analysis from a Phase 1 Open Label Extension (OLE) study conducted in December 2019, Wave discontinued development of suvodirsen.

Approximately 13% of DMD patients have mutations amenable to treatment with an exon 51 skipping therapy. Exon-skipping technology is intended 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 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^{TM}, 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 concerning our goals, beliefs, expectations, strategies, objectives and plans, and other statements that are not necessarily based on historical facts, including statements regarding the following, among others: our commitment to advancing genetic medicines in diseases of the central nervous system, eye and liver; our intent to rapidly incorporate our learnings from the Phase 1 OLE study; our ability to deliver on the promise of our current and future pipeline; our plans to report topline clinical data from the PRECISION-HD2 trial; the future performance and results of our programs in clinical trials and in preclinical development; the potential benefits of PRISM and our stereopure oligonucleotides compared with stereorandom oligonucleotides; the benefit of nucleic acid therapeutics generally; and the anticipated duration of our cash runway. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including the following: our ability to finance our drug discovery and development efforts and to raise additional capital when needed; the ability of our preclinical programs to produce data sufficient to support our clinical trial applications and the timing thereof; our ability to continue to build and maintain the company infrastructure and personnel needed to achieve our goals; the clinical results of our programs, which may not support further development of product candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing future clinical trials and regulatory processes; the effectiveness of PRISM; the continued development and acceptance of oligonucleotides as a class of medicines; our ability to demonstrate the therapeutic benefits of our candidates in clinical trials, including our ability to develop candidates across multiple therapeutic modalities; our dependence on third parties, including contract research organizations, contract manufacturing organizations, collaborators and partners; our ability to manufacture or contract with third parties to manufacture drug material to support our programs and growth; our ability to obtain, maintain and protect intellectual property; our ability to enforce our patents against infringers and defend our patent portfolio against challenges from third parties; and competition from others developing therapies for similar uses, as well as the information under the caption “Risk Factors” contained in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) 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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Source: Wave Life Sciences