Wave Life Sciences Provides Timing Update on PRECISION-HD Clinical Programs

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Topline data readout from Huntington's disease clinical trials now expected by year end 2019

CAMBRIDGE, Mass., April 10, 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 provided an update on the timing of the topline data readout from its ongoing PRECISION-HD program, which consists of two global Phase 1b/2a clinical trials evaluating investigational therapies WVE-120101 and WVE-120102 for patients with Huntington's disease (HD). The company now expects to report topline clinical data from the PRECISION-HD program by the end of the year. The reason for the revised timeline is operational, resulting from slower than anticipated patient enrollment because of the logistics of screening and scheduling across global sites. This update is not due to a preclinical or clinical safety finding and the PRECISION-HD clinical program remains blinded. The company expects the topline clinical data will include a summary of clinical safety results, the degree of mutant huntingtin protein lowering in cerebrospinal fluid (CSF) and the ratio of total huntingtin versus mutant huntingtin protein in CSF to assess wild-type huntingtin protein.

"When we initiated these exciting clinical trials to explore the potential of an allele-selective approach to treating Huntington's disease, we set aggressive timelines for ourselves in recognition of the enormous need in the global Huntington's disease community. While we are disappointed that enrollment is not as fast as we anticipated, we are pleased that patient and physician interest in the trials remains high," said Michael Panzara, MD, MPH, Chief Medical Officer at Wave Life Sciences. "Like others in the community, we are focused on advancing these trials quickly and are eagerly looking forward to the first clinical results from the program."

About WVE-120101 and WVE-120102

WVE-120101 and WVE-120102 are investigational stereopure antisense oligonucleotides designed to selectively target the mutant huntingtin (HTT) mRNA transcript of SNP rs362307 (SNP1) and SNP rs362331 (SNP2), respectively. SNPs, or single nucleotide polymorphisms, are naturally occurring variations within a given genetic sequence and in certain instances can be used to distinguish between two related copies of a gene where only one is associated with the expression of a disease-causing protein. In vitro studies in patient-derived cell lines have shown that WVE-120101 and WVE-120102 selectively reduce levels of mutant HTT mRNA transcript and protein, while leaving wild-type, or healthy, HTT mRNA transcript and protein relatively intact. The healthy transcript is required to produce healthy HTT protein, which is critical for neuronal function, as evidenced by multiple preclinical studies indicating that long-term suppression of healthy HTT protein may have detrimental consequences. Wave's allele-specific approach may also enable the company to address the pre-manifest, or asymptomatic, HD patient population in the future.

About Huntington's Disease

Huntington's disease (HD) is a debilitating and ultimately fatal autosomal dominant neurological disorder, characterized by cognitive decline, psychiatric illness and chorea. HD causes nerve cells in the brain to deteriorate over time, affecting thinking ability, emotions and movement. HD is caused by an expanded cytosine-adenine-guanine (CAG) triplet repeat in the huntingtin (HTT) gene that results in production of mutant HTT protein. Accumulation of mutant HTT causes progressive loss of neurons in the brain. Wild-type, or healthy, HTT protein is critical for neuronal function, and suppression may have detrimental long-term consequences. Approximately 30,000 people in the United States have symptomatic HD and more than 200,000 others are at risk for inheriting the disease. There are currently no approved disease-modifying therapies available.

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 the topline data readout from the company's PRECISION-HD program, and the type of data expected to be included in the readout. 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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