



## Wave Life Sciences to Present Preclinical In Vivo and In Vitro Data for SNP3-Targeting Huntington's Disease Program at CHDI Foundation's 15th Annual HD Therapeutics Conference

February 18, 2020

CAMBRIDGE, Mass., Feb. 18, 2020 (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 that it will present preclinical data from its SNP3-targeting Huntington's disease (HD) program at the CHDI Foundation's 15th Annual Huntington's Disease Therapeutics Conference, being held February 24-27, 2020, in Palm Springs, California. The poster presentation will include preclinical *in vivo* and *in vitro* data for Wave's investigational allele-selective stereopure oligonucleotides designed to preferentially target the mutant huntingtin (mHTT) mRNA transcript associated with a single nucleotide polymorphism (SNP3).

Wave's HD pipeline includes its Phase 1b/2a investigational oligonucleotides targeting SNP1 (WVE-120101) and SNP2 (WVE-120102), as well as its SNP3-targeting oligonucleotides. All of these oligonucleotides are designed to be allele-selective and preferentially lower mHTT protein while keeping the level of healthy or wild-type HTT (wtHTT) protein relatively intact. The wtHTT protein is important for neuronal function, and there is increasing evidence that it may be neuroprotective in an adult brain. Additionally, a dominant gain of function in mHTT protein and a concurrent loss of function of wtHTT protein may be important components of the pathophysiology of HD.

### Details of Data Presentation

- Poster: Stereopure oligonucleotides for the selective silencing of mutant huntingtin  
Wednesday, February 26, 2020  
1:00-4:00 p.m. PST

### About Wave Life Sciences' Clinical Research in Huntington's Disease

Wave is developing WVE-120101 and WVE-120102, which are investigational stereopure antisense oligonucleotides designed to selectively target the mutant huntingtin (mHTT) mRNA transcript of SNP rs362307 (SNP1) and SNP rs362331 (SNP2), respectively. SNPs are naturally occurring variations within a given genetic sequence and in certain instances can be used to distinguish between two related copies of a genetic transcript where only one is associated with the CAG expansion that leads to expression of mHTT protein.

WVE-120101 and WVE-120102 are currently being investigated in early manifest HD patients in the Phase 1b/2a PRECISION-HD1 and PRECISION-HD2 studies, respectively. In December 2019, Wave announced initial data from PRECISION-HD2 showing mHTT target engagement and that WVE-120102 was generally safe and well tolerated across all cohorts. Based on these data, Wave announced the addition of a higher dose cohort to each study with the intention to explore higher doses. Results from PRECISION-HD1 and PRECISION-HD2, including from the next cohort in each study (32mg), are expected in the second half of 2020.

In addition to WVE-120101 and WVE-120102, Wave expects to initiate clinical development of a SNP3-targeting candidate in the second half of 2020. Between its SNP1, SNP2 and SNP3 programs, Wave has the potential to address up to 80% of the HD patient population. Wave's allele-selective 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 (mHTT) protein. Accumulation of mHTT protein causes progressive loss of neurons in the brain. HD may also be caused by a reduction in wild-type, or healthy, HTT (wtHTT) protein that occurs concurrently with production of mHTT protein. wtHTT 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](http://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 importance of wtHTT protein preservation in treating HD; Wave's intention to explore higher doses in its PRECISION-HD1 and PRECISION-HD2 studies and the expected timing for future data readouts; the expected timing for the initiation of clinical development for Wave's SNP3-targeting candidate; the potential addressable HD patient population covered by Wave's three HD programs; and that Wave's allele-selective approach may enable it to address the pre-manifest, or asymptomatic, HD patient population in the future. 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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