Wave Life Sciences to Develop Programs in Rare, Genetic Eye Diseases

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Research will focus on inherited retinal diseases associated with RHO, USH2A, ABCA4 and CEP290

Initial development candidate expected in H2 2019

CAMBRIDGE, Mass., Oct. 09, 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 plans to design and advance stereopure oligonucleotide therapeutics for the potential treatment of rare, inherited eye diseases. Wave's research in ophthalmology will initially focus on the following four inherited retinal diseases which commonly lead to progressive vision loss typically starting in childhood or adolescence: retinitis pigmentosa due to a P23H mutation in the RHO gene, Stargardt disease, Usher syndrome type 2A and Leber congenital amaurosis 10. The company expects to announce its first ophthalmology development candidate in the second half of 2019.

"We have long believed that oligonucleotides have the potential to be particularly effective and durable in the eye and are energized by our latest research that provides additional validation of our precisely designed stereopure oligonucleotides," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "Our aim is to move quickly to develop long-acting, intravitreally injected, disease-modifying therapies to address the enormous need across a spectrum of rare, genetically-defined eye diseases."

Wave's decision to expand its therapeutic pipeline into ophthalmology is supported by its data presented at the 14th Annual Meeting of the Oligonucleotide Therapeutics Society on October 1, 2018 in Seattle, Washington. The data demonstrate that a single intravitreal injection of stereopure oligonucleotide in the eye of non-human primates resulted in greater than 95% knockdown of a target RNA in the retina for at least four months. Based on these data, the company is working to design development candidates that could achieve a therapeutic effect with only two doses per year.

Inherited retinal dystrophies are a wide range of heterogeneous, rare eye disorders characterized by progressive loss of vision and/or eventual blindness caused by inherited genetic mutations. These conditions affect approximately 200,000 people in the U.S. There are currently no cures or treatments for this category of diseases, except for one approved disease-modifying therapeutic in the U.S. for patients with biallelic RPE65 mutation-associated retinal dystrophy.

Wave's research will assess four genetic targets, RHO P23H, USH2A, ABCA4 and CEP290 to address four rare, inherited retinal diseases. The company estimates that approximately 10,000 U.S. patients could potentially be treated by Wave's approach to addressing these four diseases:

Inherited Retinal Disease	Target
Retinitis pigmentosa due to a P23H mutation in the RHO gene impacts how the retina responds to light. Initial symptoms in early to late childhood include problems with night vision with blind spots developing in the peripheral vision over time. These blind spots converge to produce tunnel vision as the disease continues to progress, causing many patients to become legally blind in adulthood.	RHO P23H
Usher syndrome type 2A is characterized by hearing loss, starting at birth, followed by progressive vision loss beginning in adolescence or adulthood with problems with night vision and later peripheral blind spots. It is caused by a mutation in the USH2A gene.	USH2A
Stargardt disease, caused by a mutation in the ABCA4 gene, is characterized by progressive build-up of fatty yellow pigment (lipofuscin) in the cells underlying the macula. Over time, this accumulation damages the cells that are responsible for clear central vision, causing blurry sight, difficulty with night vision or impaired color vision in some patients.	ABCA4
Leber congenital amaurosis 10 is caused by autosomal recessive mutations in the CEP290 gene, which encodes a protein required for the survival and proper function of photoreceptor cells. Affected infants are often blind at birth. Other symptoms may include crossed eyes; rapid, involuntary eye movements; unusual sensitivity to light; clouding of the lenses of the eyes; and/or a cone shape to the front of the eye.	CEP290

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, the identity of the first four inherited retinal diseases and the first four targets that Wave expects to focus on; the expected timing of Wave's initial ophthalmology development candidate; the belief that Wave's stereopure oligonucleotides are particularly well-suited to address inherited retinal diseases; the belief that Wave's recent ophthalmology data disclosed at OTS supports the potential for Wave's oligonucleotides to deliver a therapeutic effect with less frequent dosing than other technologies; and the potential of Wave's preclinical data to predict the behavior of Wave's oligonucleotides in humans. 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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