



Wave Life Sciences to Highlight RNA Base Editing Modality and Alpha-1 Antitrypsin Deficiency Program at Upcoming Scientific and Industry Meetings

March 15, 2022

Presentation at OPT 2022 to highlight AATD program, including reduction of liver aggregates with repeat AImEr dosing out to 19 weeks

Multiple presentations and panel discussions at 3rd Annual RNA Editing Summit to highlight Wave's leadership in therapeutic RNA base editing

CAMBRIDGE, Mass., March 15, 2022 (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 highlight its therapeutic A-to-I(G) RNA base editing oligonucleotides ("AIMers") at two upcoming scientific and industry meetings. Preclinical data for Wave's Alpha-1 antitrypsin deficiency (AATD) AImEr program, including editing efficiency, AAT protein restoration and reduction of liver aggregates with repeat AImEr dosing out to 19 weeks, will be shared in a Featured Session at the 7th Annual Oligonucleotide & Precision Therapeutics (OPT) Congress, taking place March 15-16, 2022 in Boston, MA and virtually. The company's progress toward, and leadership in, advancing a therapeutic RNA base editing platform capability and pipeline will be highlighted in five sessions at the 3rd RNA Editing Summit, taking place April 5-7 in Boston, MA.

"The Wave team continues to make rapid progress on developing a versatile therapeutic editing capability with potential to address diseases of many different tissues and cell types. Our GalNAc-conjugated AImEr program for subcutaneous delivery in AATD continues to advance, with IND-enabling studies scheduled to begin in the third quarter of this year. In parallel, we are building out a pipeline of additional AImEr programs which also use GalNAc to reach hepatocytes," said Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences. "We also continue to advance our research in unconjugated AIMers for delivery to the central nervous system and other organs that are not reachable with other editing approaches. Our AIMers achieve high editing efficiencies, durable effects, and are highly specific, reinforcing the benefits of Wave's proprietary chemistry for establishing potential best-in-class RNA editing therapeutics. We look forward to sharing a comprehensive update on our AIMers across these two meetings."

AIMers are designed to correct single base mutations in an RNA transcript, thereby avoiding permanent changes to the genome that occur with DNA-targeting approaches. Rather than using an exogenous editing enzyme, AIMers recruit proteins that exist in the body, called ADAR enzymes, which naturally edit certain adenine (A) bases to inosine (I), which cells read as guanine (G). This approach redirects a natural system for therapeutic purposes, relies on simplified delivery, and avoids the risk of irreversible off-target effects of DNA-targeting approaches. AIMers are short in length, fully chemically modified, and use novel chemistry, including proprietary PN backbone modifications and chiral control, that make them distinct from other ADAR-mediated editing approaches. Recently, data highlighting Wave's foundational AImEr technology were published in [Nature Biotechnology](#).

Wave's upcoming AImEr presentations include:

7th Annual Oligonucleotide & Precision Therapeutics Congress

- **Tuesday, March 15 at 5:05 p.m. EDT**
Chemically Optimized Stereopure Oligonucleotides Direct ADAR-Mediated RNA Editing (Paloma Giangrande, PhD, Vice President, Platform Discovery Sciences, Biology, at Wave Life Sciences)
Session: Emerging Oligo Therapies

3rd RNA Editing Summit

- **Tuesday, April 5 at 12:10 p.m. EDT**
Panel Discussion: Chemical Modifications of RNA Editing (Chandra Vargeese, PhD, Chief Technology Officer and Head of Platform Discovery Sciences at Wave Life Sciences)
Session: Improving Fundamental Mechanisms of RNA-Editing in Order to Prepare for Therapeutic Application
- **Wednesday, April 6 at 1:40 p.m. EDT**
AIMers: Correcting Driver Mutations for the Treatment of Alpha-1 Antitrypsin Deficiency (AATD) and Beyond (Paloma Giangrande, PhD, Vice President, Platform Discovery Sciences, Biology, at Wave Life Sciences)
Session: Disease Specific Case Studies: Using RNA Editing Therapeutically Across Diseases
- **Wednesday, April 6 at 5:10 p.m. EDT**
Panel Discussion: The Future Possibilities of RNA Editing (Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences)
Session: Disease Specific Case Studies: Using RNA Editing Therapeutically Across Diseases
- **Thursday, April 7 at 10:50 a.m. EDT**
Unlocking Therapeutic RNA Editing (Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences)
Session: Delving into Delivery
- **Thursday, April 7 at 11:20 a.m. EDT**
Morning Q&A and Panel Discussion – Improving Delivery Performance (Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences)
Session: Delving into Delivery

About AIMers

Adenosine deaminases acting on RNA (ADAR) enzymes are naturally occurring enzymes in humans which catalyze adenine (A) to inosine (I) changes in repetitive elements, microRNAs (miRNAs), and protein encoding transcripts. Wave's A-to-I RNA base editing oligonucleotides ("AIMers") are designed to recruit these endogenous ADAR enzymes to direct efficient and highly specific editing of RNA transcripts. Because I is read as G (guanine) by translational machinery, sequence-directed editing with ADAR has the potential to revert transcripts with single G-to-A point mutations that cause genetic diseases.

It is estimated that there are more than 32,000 pathogenic single nucleotide polymorphisms, of which about 50% may be ADAR amenable. In addition, A-to-I(G) editing could potentially address non-genetic diseases through modulation of post-translational modifications or protein-protein interactions.

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, our expectations for our GalNAc-conjugated A-to-I(G) RNA base editing oligonucleotides (AIMers) and the anticipated therapeutic benefits thereof; our expectations regarding the ability of our AIMers to address diseases of many different tissues and cell types; our research of unconjugated AIMers for delivery to the central nervous system and other organs that are not reachable by other editing approaches; the potential benefits of our AIMers compared with other RNA base editing approaches; the potential benefits of PRISM, including our AIMers and our stereopure oligonucleotides compared with stereorandom oligonucleotides; and the anticipated timing of future development milestones for our lead AIMer program. 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 and actual results may differ materially from those indicated by these forward-looking statements as a result of these risks, uncertainties and important factors, 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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