



## Wave Life Sciences to Highlight Leading RNA Editing Capability and Multimodal Discovery and Development Platform at ASGCT 26th Annual Meeting

May 16, 2023

*Presentations include new data showing WVE-006 improved markers of liver disease in mice; WVE-006 is a first-in-class RNA editing therapeutic for alpha-1 antitrypsin deficiency (AATD) which is designed to restore healthy, wild-type AAT protein*

*Best-in-class exon skipping clinical data for WVE-N531 in Duchenne muscular dystrophy (DMD) also highlighted in oral presentation*

*Poster presentations will focus on Wave's pioneering application of RNA editing to upregulate gene expression, as well as Wave's emerging best-in-class siRNA formats*

CAMBRIDGE, Mass., May 16, 2023 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage RNA medicines company committed to delivering life-changing treatments for people battling devastating diseases, today announced it will highlight its leading RNA editing capability and its multimodal discovery and development platform at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, taking place May 16-20, 2023, in Los Angeles, California.

New preclinical data for WVE-006, Wave's investigational, GalNAc-conjugated RNA editing candidate for the treatment of alpha-1 antitrypsin deficiency (AATD), will be presented in an oral abstract session. These data demonstrate that treatment with WVE-006 improved several markers of liver disease in mice, including a decrease in liver inflammation and hepatocyte turnover and reduction in the size of PAS-D positive globules. WVE-006 is uniquely designed to address both liver and lung disease related to AATD and is poised to be the industry's first RNA editing candidate to enter clinical trials, with CTA submissions by Wave expected in the second half of 2023.

Additionally, Wave will present preclinical data in a poster session regarding the ability of ADAR-mediated RNA editing to upregulate gene expression. Wave is pioneering this novel editing application with potential to address diseases with large patient populations, independent of genetic mutation status.

"The ingenuity of our scientists has resulted in a best-in-class RNA medicines platform and the industry's most advanced and promising RNA editing capability. We are proud to be highlighting two innovative applications of our RNA editing oligonucleotides – correction of single point mutations and upregulation of gene expression – as well as our first editing clinical candidate, WVE-006, at this year's ASGCT meeting," said Chandra Vargeese, PhD, Chief Technology Officer at Wave Life Sciences. "Additionally, we will present on the impact of our novel chemistry in DMD, which translated to the greatest level of exon skipping ever seen in the clinic with our WVE-N531 program and has the potential to result in clinically meaningful, functional dystrophin protein in our upcoming Phase 2 study."

Presentation details are as follows:

- **Wednesday, May 17 at 4:30-4:45 p.m. PDT**  
RNA base editing for the treatment of Alpha-1 antitrypsin deficiency (Prashant Monian, PhD, Senior Scientist II, Biology at Wave Life Sciences)  
Nucleic Acid Therapeutics: Oral Abstract Session
- **Wednesday, May 17 at 4:45-5:00 p.m. PDT**  
Phosphoryl guanidine (PN)-containing oligonucleotides support exon skipping in skeletal muscle in mice and boys with DMD (Pachamuthu Kandasamy, Vice President, Medicinal Chemistry at Wave Life Sciences)  
Nucleic Acid Therapeutics: Oral Abstract Session
- **Wednesday, May 17 from 12:00-2:00 p.m. PDT**  
PN modification of stereopure GalNAc-siRNAs enhances durability of human HSD17B13 silencing in transgenic mouse model (Wei Liu, PhD, Senior Scientist II, Biology at Wave Life Sciences)  
Wednesday Poster Session (#478)
- **Thursday, May 18 from 12:00-2:00 p.m. PDT**  
Application of ADAR-mediated RNA editing to modulate gene expression (Chikdu Shivalila, PhD, Director, Biology at Wave Life Sciences)  
Thursday Poster Session (#1219)

Wave's presentations from ASGCT will be available on the Investor Relations section of Wave's website at the conclusion of the meeting and can be accessed by visiting: [ir.wavelifesciences.com/scientific-presentations](http://ir.wavelifesciences.com/scientific-presentations).

### About Wave Life Sciences

Wave Life Sciences (Nasdaq: WVE) is a clinical-stage RNA 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](https://twitter.com/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 regarding the potential benefits of WVE-006 for the treatment of AATD; our expectations for the timing for our clinical trials, including the timing of CTA submissions for WVE-006; the potential benefit of ADAR-mediated RNA editing to upregulate gene expression; and our beliefs on the impact of our splicing and siRNA modalities, including the potential benefits of our WVE-N531 DMD 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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