

# Wave Life Sciences to Present New Data on Its DMD Exon 53 Skipping Program and Discovery Platform at Upcoming Meetings

September 20, 2018

CAMBRIDGE, Mass., Sept. 20, 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 presentations at two upcoming meetings highlighting new data on the company's Duchenne muscular dystrophy (DMD) exon 53 skipping program and the company's proprietary chemistry platform.

#### 23rd International Annual Congress of the World Muscle Society

A poster entitled "Identification of Potent, Muscle-Targeting Investigational Stereopure Oligonucleotides for Exon 53 DMD Therapy" (Poster #127) will be presented on October 3, 2018 at 4:00 pm ART at the 23<sup>rd</sup> International Annual Congress of the World Muscle Society in Mendoza, Argentina. The poster will include new data from a stereopure exon 23 skipping oligonucleotide demonstrating substantial and durable dystrophin protein restoration across multiple muscle tissues, including heart, in the *mdx* 23 mouse model of DMD. Wave's exon 53 skipping program is in preclinical development and leverages the company's ongoing neuromuscular research and *in vivo* data supporting the development of WVE-210201, the company's lead exon 51 skipping investigational oligonucleotide in DMD.

WVE-210201 is currently being studied in a global Phase 1 clinical trial in DMD patients amenable to exon 51 skipping. As patients complete the Phase 1 trial, they have the option to enroll in an ongoing open-label extension study in which they continue to receive WVE-210201.

#### 14th Annual Meeting of the Oligonucleotide Therapeutics Society (OTS)

A presentation entitled "Stereochemical Control of Antisense Oligonucleotides Enhances Target Efficacy" will take place on October 3, 2018 at 11:00 am PDT at the 14<sup>th</sup> Annual Meeting of the Oligonucleotide Therapeutics Society in Seattle, Washington. The presentation will be given by Chandra Vargeese, PhD, Senior Vice President and Head of Discovery at Wave.

Wave will also present the following three posters at this meeting:

- "Optimized, Stereopure Antisense Oligonucleotides Achieve Broad Tissue Distribution and Excellent Exposure, Enabling Potent and Durable Knockdown of Nuclear Malat1 in Mice and Nonhuman Primates" (Poster #030)
- "A Bicyclic Synthetic Ligand for ASGR is a Safe Alternative to GalNAc for Effective Hepatocyte-specific Delivery in Mouse Models" (Poster #092)
- "Cellular Free-Uptake Models for Optimized, Stereopure Antisense Oligonucleotides Predict Exon Skipping Efficiency and Dystrophin Protein Restoration in Mice" (Poster #119)

Data from the presentation and the three posters highlight advances in Wave's novel chemistry platform and its ability to precisely design, optimize and manufacture stereopure oligonucleotides.

## **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 <a href="https://www.wavelifesciences.com">www.wavelifesciences.com</a>.

## **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 potential for Wave's preclinical data, including data from Wave's exon 23 skipping oligonucleotide in the *mdx* 23 mouse model, to predict the behavior of Wave's compounds, including its exon 53 skipping program, in humans; Wave's ability to leverage the learnings from its ongoing neuromuscular research to apply to its new neuromuscular compounds; the potential of Wave's DMD programs to help boys suffering from DMD; the future performance and results of Wave's DMD compounds in clinical trials. 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.

#### **Investor Contact:**

Graham Morrell 781-686-9600 gmorrell@wavelifesci.com

#### **Media Contact:**

617-949-4708 <u>jjuves@wavelifesci.com</u>

# **Patient Contact:**

Wendy Erler 617-949-2898 werler@wavelifesci.com



Source: Wave Life Sciences