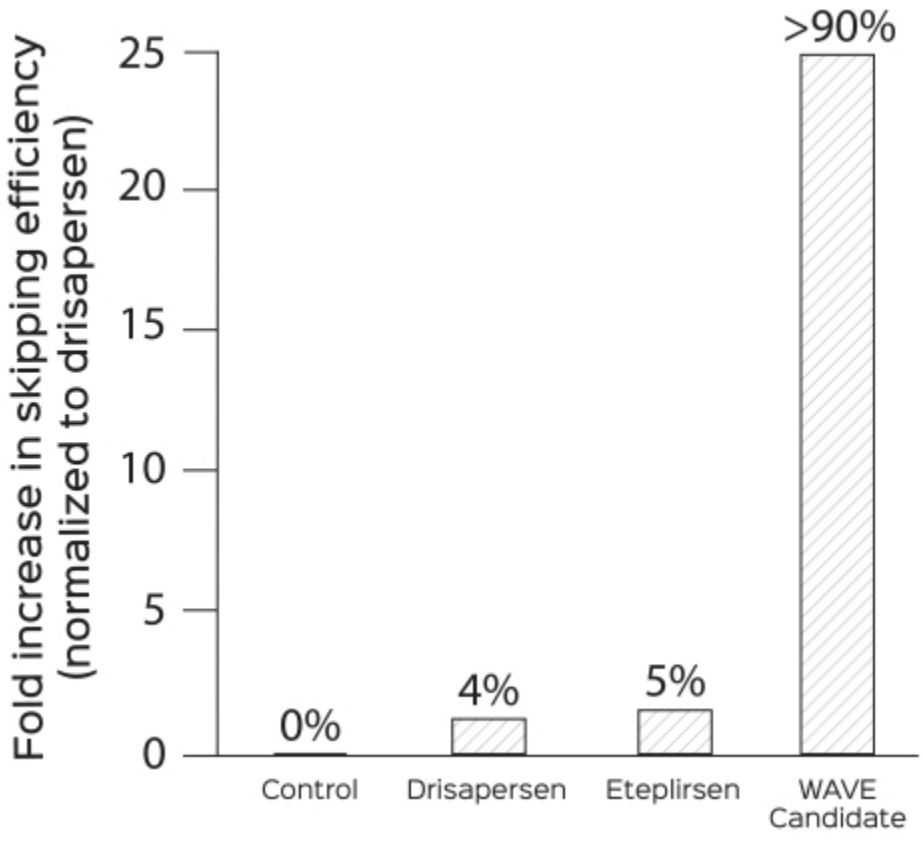
WAVE Life Sciences to Advance Next-Generation Nucleic Acid Therapies to Address Unmet Need in Duchenne Muscular Dystrophy

May 9, 2016

- *WAVE advances exon 51 DMD candidate; initiation of first clinical trial planned for 2H 2017; candidates for other exons expected to follow*
- *Preclinical data demonstrates 25-fold enhanced exon-skipping efficiency and broader distribution, including to heart and diaphragm, compared to other exon-skipping therapies*
- *WAVE to present data at upcoming Parent Project Muscular Dystrophy (PPMD) Annual Connect Conference in June 2016*
- *Extends current research collaboration with University of Oxford; renowned researcher Matthew Wood to accelerate additional exon-skipping targets*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 9, 2016-- WAVE Life Sciences Ltd. (NASDAQ:WVE), a genetic medicines company focused on developing stereopure nucleic acid therapies for patients impacted by rare diseases, today reaffirmed its commitment to advance next-generation nucleic acid therapies to address the significant unmet need of patients diagnosed with Duchenne Muscular Dystrophy (DMD). WAVE's DMD program is based on preclinical data demonstrating an approximate 25-fold improvement in exon-skipping efficiency compared to drisapersen and eteplirsen, suggesting the potential for improved potency and an enhanced ability to restore the production of functional dystrophin. In addition, WAVE's proprietary muscle targeting technology has demonstrated substantial improvement in distribution to critical tissues in animal models, including skeletal muscle, diaphragm, and heart.

This Smart News Release features multimedia. View the full release here: http://www.businesswire.com/news/home/20160509005436/en/



Free uptake assay measuring DMD mRNA by RT-qPCR at equal concentrations of compound in patient-derived cells; fraction of transcript skipped (%)(Graphic: Business Wire)

"We recognize the acute need of the Duchenne community for therapeutic options to address this devastating disease, and also appreciate regulators' requirements for strong, well-validated, scientific evidence. Our goal is to fulfill both of these needs by bringing forward optimally designed drugs through robust clinical trials," said Paul Bolno, M.D., MBA, President and CEO of WAVE Life Sciences. "Based on the strong preclinical data we've seen to date, we are highly encouraged that we are on track to develop exon-skipping medicines that maximize potency with a favorable safety profile. In addition, we plan to conduct rigorous, well-designed clinical trials that explore various predictive biomarkers and evaluate comprehensive endpoints."

WAVE's planned clinical trials will include both ambulatory and non-ambulatory patients in order to evaluate preservation of walking ability, heart and lung function.

WAVE's initial DMD candidate skips exon 51 in the dystrophin gene. The company will initiate IND-enabling studies in 2016 and intends to initiate its first clinical trial of the candidate in the second half of 2017. WAVE will also leverage its novel stereopure chemistry platform to advance therapies targeting additional DMD-related exons to expand the potential impact of its promising approach to a broader group of patients.

WAVE will present data at the upcoming Parent Project Muscular Dystrophy's (PPMD) Annual Connect Conference being held June 26 – 29, 2016 in Orlando, Florida.

"We are excited whenever a new company enters the Duchenne space," said Pat Furlong, Founding President and CEO of PPMD. "Individuals with Duchenne need and deserve options, and we are hoping this will become another tool in our arsenal."

As part of its commitment to developing better medicines for DMD, WAVE has extended its ongoing research collaboration with the University of

Oxford to advance stereopure nucleic acid therapies for DMD across exons. Through the collaboration, renowned researcher Matthew Wood, M.D., Ph.D., Professor of Neuroscience, Department of Physiology, Anatomy and Genetics, Medicine Sciences Division, University of Oxford, and his team will continue to work with WAVE to use the company's proprietary platform to enhance oligonucleotide approaches, including exon-skipping, to address the rare genetic muscle disease.

"The data we have seen to date using WAVE's novel approach to exon-skipping in DMD is very promising. I believe that academia and industry, working together, may be on the verge of a veritable medical revolution where we can potentially effectively and durably treat genetically based diseases such as DMD," said Professor Wood. "Collaborations between academia and industry are critical now more than ever in order to collectively harness the latest scientific advancements to rapidly progress therapies for patients. We look forward to expanding our collaboration with the WAVE team and advancing the potential and benefits of stereopure oligonucleotide approaches for DMD."

About Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is a fatal X-linked genetic neuromuscular disorder caused by mutations in the gene that encodes for the protein dystrophin. Dystrophin is needed for muscle maintenance and operation. Because of the genetic mutations, the body cannot produce functional dystrophin. Without dystrophin, muscles cannot work properly, and patients experience progressive loss of strength and serious medical complications involving the heart and lungs. DMD affects approximately one in 3,500 newborn boys around the world; approximately 13% have mutations in Exon 51. There are currently no approved disease-modifying drugs available to treat DMD. Exon-skipping drugs are designed to "skip over" the mutated exon, enabling the gene to once again code for and produce functional dystrophin.

About WAVE Life Sciences

At WAVE Life Sciences, we are driven by an unwavering passion and commitment to deliver on our mission of confronting challenging diseases by developing transformational therapies and empowering patients. We are utilizing our innovative and proprietary synthetic chemistry drug development platform to design, develop and commercialize stereopure nucleic acid therapeutics that precisely target the underlying cause of rare and other serious genetically defined diseases. Given the versatility of our chemistry platform, WAVE's deep, diverse pipeline spans multiple modalities including antisense, exon-skipping, and single-stranded RNAi. For more information, please visit www.wavelifesciences.com and follow us on Twitter at @WAVELifeSci and LinkedIn.

Forward Looking Statements

This press release contains forward-looking statements concerning our goals, beliefs, expectations, strategies, objectives and plans, and other statements that are not necessarily based on historical facts, including statements regarding the following: our commencing of IND-enabling studies and clinical trials involving our exon 51 candidate; the future performance and results of our exon 51 candidate in clinical trials; our identification of future candidates targeting DMD and their therapeutic potential; the ability of industry and academia to effectively treat DMD; and the potential of our stereopure approach and nucleic acid therapeutics. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including the following: the ability of our IND-enabling studies to produce data sufficient to support the filing of an IND for our exon 51 candidate and the timing thereof; our ability to demonstrate the therapeutic benefits of our exon 51 candidate in clinical trials; the success of our platform in identifying additional viable DMD candidates; the continued development and acceptance of nucleic acid therapeutics as a class of drugs; and competition from others developing therapies for similar uses, as well as the information under the caption "Risk Factors" contained in our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) and in other filings we make with the SEC from time to time. We undertake no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

View source version on businesswire.com: http://www.businesswire.com/news/home/20160509005436/en/

Source: WAVE Life Sciences Ltd.

Media:
Feinstein Kean Healthcare
Liz Melone, 617-256-6622
liz.melone@fkhealth.com
or
Investors:
Westwicke Partners
Chris Brinzey, 339-970-2843
chris.brinzey@westwicke.com