



WAVE Life Sciences Expands Stereopure Synthetic Chemistry Platform Capabilities, Augments Patent Portfolio with Addition of Single-Stranded RNAi (ssRNAi)

June 8, 2015

Licenses co-exclusive rights from Max Planck Institute for Biophysical Chemistry to develop and commercialize ssRNAi therapeutics

Advances overall strategy to address wide range rare genetic diseases utilizing RNAi, antisense, exon-skipping and other oligonucleotide modalities

Cambridge, Mass., Singapore— On June 8, 2015, WAVE Life Sciences, a preclinical biopharmaceutical company using its proprietary synthetic chemistry drug development platform to design, develop and commercialize a broad pipeline of first-in-class or best-in-class nucleic acid therapeutics candidates, licensed co-exclusively certain patent rights originating from research performed at the Max Planck Institute for Biophysical Chemistry (MPI-BC) and at the University of Massachusetts Medical School (UMMS), to develop and commercialize single-stranded RNA interference (ssRNAi) therapeutics. Utilizing its novel stereopure synthetic chemistry platform, WAVE is advancing a pipeline of nucleic acid therapies across multiple oligonucleotide modalities, including RNAi, antisense and exon-skipping. The addition of ssRNAi expands the breadth of the company's platform capabilities and further enhances its strong intellectual property (IP) position in the oligonucleotide space.

"Unlike other companies in this field, WAVE has the ability to develop therapies across a broad spectrum of nucleic acid therapeutic modalities, creating a truly unique, next-generation genetic medicine company," said Paul Bolno, M.D., MBA, president and chief executive officer at WAVE Life Sciences. "The addition of ssRNAi capabilities afforded by this co-licensing agreement adds another critical modality to our toolbox of therapeutic approaches, and enriches our unprecedented latitude to pursue a pipeline that can address a wide range of rare genetic diseases to realize our ultimate goal of impacting numerous patients' lives."

WAVE acquired the co-exclusive license for certain patent rights based on the discovery by Dr. Thomas Tuschl and other scientists at the MPI-BC, and by Dr. Phillip D. Zamore and other scientists at the University of Massachusetts Medical School, surrounding the features of single-stranded RNA molecules required to mediate target-specific silencing by RNAi. MI, a wholly owned subsidiary and technology transfer agency of Max-Planck-Gesellschaft zur Foerderung der Wissenschaften e.V. (MPG), the parent non-profit scientific research organization of MPI-BC, is the licensor of the patent rights. In addition to WAVE, ISIS Pharmaceuticals is the other co-exclusive licensee of the patent rights.

"RNAi holds tremendous promise to prevent proteins from driving disease; however, double-stranded approaches are largely limited to addressing liver-linked conditions. ssRNAi offers the promise to target a plethora of diseases beyond the liver," said Dr. Bolno. "We're excited to build on the tremendous work done by Dr. Tuschl, Dr. Zamore and their teams in order to create chirally pure, rationally designed ssRNAi nucleic acid therapies capable of precisely targeting underlying disease biology to yield safe and effective therapies for patients."

"We're gratified to be able to play a central role in helping advance the field of oligonucleotide therapeutics, particularly at this moment in time as the space continues to rapidly evolve and gain momentum," said Dr. Joern Erselius, Managing Director of Max Planck Innovation GmbH. "Dr. Tuschl and colleague's discoveries are a key foundational element to ssRNAi drug development. We look forward to seeing WAVE's endeavors unfold as the company translates this groundbreaking science into novel medical solutions."

"We are pleased to see yet another example of the potential commercial application of the inventive work of Dr. Zamore and his lab, and we look forward to working with WAVE as the company further develops its chiral nucleic acid technologies using ssRNAi as potential therapeutics," said Dr. James P. McNamara, Executive Director, Office of Technology Management, University of Massachusetts Medical School.

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