Ra Pharmaceuticals Receives Development Milestone Payment Under Agreement with Merck

December 6, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 6, 2018-- Ra Pharmaceuticals, Inc. (NASDAQ:RARX) today announced that it has received a development milestone payment under its collaboration agreement with Merck, known as MSD outside the US and Canada. The milestone payment is associated with the companies' collaboration for a non-complement cardiovascular target with a large market opportunity. Initiated in 2013, this collaboration leverages Ra Pharma's Extreme Diversity™ platform aimed at producing macrocyclic peptides that have the diversity and specificity of antibodies, while retaining the pharmacologic attributes of small molecules.

"Our Extreme Diversity platform allows us to create drug-like peptides with high bioavailability, stability, cell permeability, and potency, potentially filling unmet needs associated with monoclonal antibody, biologic, and peptide therapies," said Doug Treco, PhD, President and Chief Executive Officer of Ra Pharma. "As our collaboration with Merck has demonstrated, this chemistry, and the large libraries of drug-like molecules it enables, allow us to rapidly identify orally-available lead peptides, and we are pleased to have delivered these peptides to Merck."

"The advancement of our collaboration with Merck, in parallel with the clinical progress of zilucoplan, our lead internal pipeline candidate in development for generalized myasthenia gravis, paroxysmal nocturnal hemoglobinuria, and other complement-mediated diseases, underscores the power and productivity of our platform for drug discovery," Dr. Treco added.

Under the terms of the agreement, Ra Pharmaceuticals is eligible to earn up to $59 million in additional milestone payments from Merck based upon the achievement of development, regulatory, and commercialization milestones. Ra Pharma is also eligible to receive low-to-mid single digit percentage royalties on any future sales of compounds resulting from the collaboration.

About the Extreme Diversity™ Platform

Ra Pharma's proprietary Extreme Diversity™ platform generates highly specific and stable peptide-like molecules with the potential for greatly increased bioavailability, improved cell permeability, and the opportunity to address protein-protein interactions and other previously undruggable targets. The platform combines *in vitro* display technology, a completely defined translation system, and a wide variety of non-natural amino acids to produce novel drug-like peptides. Unlike certain other display technologies, *in vitro* display does not require the use of a bacterial or yeast host, and it can produce libraries of 10 to 100 trillion members, allowing for the rapid discovery of highly potent candidate molecules.

About Ra Pharmaceuticals

Ra Pharmaceuticals is a clinical stage biopharmaceutical company focusing on the development of next-generation therapeutics for complement-mediated diseases. The Company discovers and develops peptides and small molecules to target key components of the complement cascade. For more information, please visit: www.rapharma.com.

Forward-Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding anticipated milestone payments, the potential safety, efficacy and regulatory and clinical progress of our product candidates, including without limitation zilucoplan and the product candidates under development through our collaboration with Merck, and the potential of our Extreme Diversity platform. All such forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include the risks that Ra Pharma's product candidates, including zilucoplan and those product candidates under development with Merck, will not successfully be developed or commercialized, in the timeframe we expect or at all; the risk that we do not receive earned milestone payments in the timeframe expected, or at all; the risk that topline results as of February 7, 2018 from the Company's global Phase 2 clinical program evaluating zilucoplan for the treatment of PNH may not be indicative of final study results; the risk that USAN does not approve the name zilucoplan; as well as the other factors discussed in the "Risk Factors" section in Ra Pharma's most recently filed Annual Report on Form 10-K, and the other risks detailed in Ra Pharma's subsequent filings with the Securities and Exchange Commission. There can be no assurance that the actual results or developments anticipated by Ra Pharma will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, Ra Pharma. All information in this press release is as of the date of the release, and Ra Pharma undertakes no duty to update this information unless required by law.

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