Ra Pharmaceuticals Earns Clinical Development Milestone for Oral Macrocyclic Peptide Candidate Targeting Cardiovascular Indication Under Agreement with Merck

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Marks the second compound from Ra's Extreme Diversity™ platform to enter clinical trials

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 21, 2019-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced that it has earned a clinical development milestone under its collaboration agreement with Merck, known as MSD outside the U.S. and Canada. The milestone is associated with the dosing of the first patient in a Phase 1 clinical trial evaluating an investigational orally-available macrocyclic peptide for a non-complement cardiovascular target with a large market opportunity from the companies' collaboration.

"I'm extremely excited to have reached this significant milestone, which notably marks the second compound from Ra's Extreme Diversity™ platform to enter human clinical testing," said Doug Treco, Ph.D., President and Chief Executive Officer of Ra Pharma. "The continued progress of our collaboration with Merck, in parallel with the advancement of our internal pipeline, including zilucoplan, our lead clinical candidate in development for the treatment of multiple tissue-based complement-mediated disorders, underscores the robust and broad potential of our platform technology across a range of therapeutic areas."

Initiated in 2013, the Merck collaboration leverages Ra Pharma's Extreme Diversity platform aimed at producing macrocyclic peptides that are designed to have the diversity and specificity of antibodies, while retaining the pharmacologic attributes of small molecules, with the potential to allow for the rapid identification, design, and development of drug-like peptides with high stability, bioavailability, cell permeability, and potency.

Under the terms of the agreement, Ra Pharma is eligible to earn up to $56 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, Inc.

Ra Pharmaceuticals is a clinical-stage biopharmaceutical company focused on leading the field of complement biology to bring innovative and accessible therapies to patients with rare 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 Statements

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 Ra Pharma's collaboration agreement with Merck and potential payments thereunder, the potential, safety, efficacy, and regulatory and clinical progress of Ra Pharma's product candidates, including zilucoplan, and its Extreme Diversity platform, and bringing innovative and accessible therapies to patients with rare diseases. 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, without limitation, the risk that Ra Pharma's product candidates, including zilucoplan, will not successfully be developed or commercialized, in the timeframe it expects or at all, Ra Pharma's ability to maintain and establish collaborations, 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, as well as 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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