Ra Pharmaceuticals Receives Orphan Drug Designation from the U.S. FDA for Zilucoplan for the Treatment of Myasthenia Gravis

September 4, 2019

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 4, 2019-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to zilucoplan for the treatment of myasthenia gravis. Ra Pharma is developing zilucoplan, a self-administered macrocyclic peptide inhibitor of complement component 5 (C5), for the treatment of generalized myasthenia gravis (gMG) and other rare, tissue-based complement-mediated diseases.

“gMG is a chronic and debilitating neuromuscular disease that affects more than 60,000 patients in the U.S. who have limited treatment options,” said Doug Treco, Ph.D., President and Chief Executive Officer of Ra Pharma. “We’ve designed zilucoplan, a macrocyclic peptide inhibitor of C5, as an easy-to-use, self-administered subcutaneous treatment option to address the underlying cause of gMG through targeted complement control. With site activations underway, we are on track to initiate our single, pivotal, 12-week, Phase 3 trial of zilucoplan for the treatment of gMG in the second half of this year.”

About Orphan Drug Designation

The U.S. Food and Drug Administration (FDA) grants Orphan Drug Designation status to products that treat rare diseases, providing incentives to sponsors developing drugs or biologics. The FDA defines rare diseases as those affecting fewer than 200,000 people in the U.S. at the time of designation. Orphan Drug Designation qualifies the sponsor for certain development incentives, including tax credits for qualified clinical testing.

About Myasthenia Gravis

Myasthenia gravis (MG) is a chronic, autoimmune, neuromuscular disease characterized by weakness and fatigue of skeletal muscles. Patients with MG present with muscle weakness that becomes increasingly severe with repeated use and recovers with rest. Weakness can be localized to specific muscles, such as those responsible for eye movements, but often progresses to affect a broader range, including head, limb, and respiratory muscles. This progression is often described as the generalized, or severe, form of the disease. gMG is estimated to affect approximately 60,000 people in the U.S. alone.

About Zilucoplan

Ra Pharma is developing zilucoplan and zilucoplan extended release (XR) for generalized myasthenia gravis (gMG), immune-mediated necrotizing myopathy (IMNM), and other tissue-based complement-mediated disorders with high unmet medical need. The product candidate is designed for convenient subcutaneous (SC) self-administration. Zilucoplan is an investigational, synthetic, macrocyclic peptide discovered using Ra Pharma's powerful proprietary drug discovery technology. The peptide is designed to bind complement component 5 (C5) with sub-nanomolar affinity and allosterically inhibit its cleavage into C5a and C5b upon activation of the classical, alternative, or lectin pathways.

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 ability to expand patient access to important therapies, the potential, safety, efficacy, and regulatory and clinical progress of Ra Pharma's product candidates, including without limitation zilucoplan and zilucoplan XR, beliefs regarding clinical trial data, and statements regarding trial design, timeline, and enrollment of Ra Pharma's ongoing and planned clinical programs, including without limitation the Phase 3 trial of zilucoplan for the treatment of gMG. 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 zilucoplan XR, will not successfully be developed or commercialized, in the timeframe we expect or at all; the risk that Ra Pharma may fail to enroll patients in its clinical trials, which may cause delays or other adverse effects; the risk that Ra Pharma may be unable to obtain orphan drug designation or to maintain the benefits associated with orphan drug status, including market exclusivity; 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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