Ra Pharmaceuticals Announces Dosing of First Patient in Global Phase 3 Pivotal Study of Zilucoplan for gMG

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Top-line data from the Phase 3 RAISE study expected in early 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 2, 2019-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced the initiation of dosing in the RAISE study, its global, pivotal, Phase 3 clinical trial evaluating zilucoplan for the treatment of generalized myasthenia gravis (gMG).

“In a Phase 2 clinical trial, zilucoplan achieved rapid, clinically meaningful, and statistically significant reductions in primary and key secondary efficacy endpoints, with a durable treatment effect that was sustained at 24 weeks for patients enrolled in the long-term extension study,” said James F. Howard, M.D., Distinguished Professor of Neuromuscular Disease, Department of Neurology, University of North Carolina School of Medicine. “We look forward to building on these data to support zilucoplan’s potential as a simple, convenient, self-administered complement inhibitor for a broad spectrum of patients with gMG.”

The single, pivotal, randomized, double-blind, placebo-controlled Phase 3 trial is designed to evaluate the efficacy of a once-daily, subcutaneously (SC) self-administered dose of 0.3 mg/kg of zilucoplan versus placebo. The trial is expected to enroll approximately 130 patients with gMG who are acetylcholine receptor (AChR)-antibody-positive, regardless of their prior therapies. The primary endpoint is the change in the MG Activities of Daily Living (MG-ADL) score from baseline to week 12. Following completion of the Phase 3 clinical trial, patients will have the option to enroll in the RAISE-XT study, an open-label, long-term extension study. Top-line results from this Phase 3 trial are expected in early 2021.

“We’re pleased to have dosed the first patient in the RAISE study, a critical milestone in our mission to expand patient access to convenient complement inhibition. With feedback from the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), and Japan’s Pharmaceuticals and Medical Devices Agency (PMDA) incorporated into the global Phase 3 trial design, we look forward to advancing zilucoplan through late-stage clinical development in gMG,” said Doug Treco, Ph.D., President and Chief Executive Officer of Ra Pharma. “With additional indications, including immune-mediated necrotizing myopathy (IMNM) and amyotrophic lateral sclerosis (ALS) recently added to the pipeline, we believe zilucoplan, if successfully developed and approved in these indications, has the potential to support the creation of a leading complement-focused neurology franchise.”

The initiation of the Phase 3 gMG clinical trial follows the successful completion of a Phase 2, multi-center, randomized, double-blind, placebo-controlled clinical trial evaluating zilucoplan for the treatment of gMG. Rapid, clinically meaningful, and statistically significant improvements in the pre-specified primary and key secondary endpoints were observed for both zilucoplan dose groups tested versus placebo at 12 weeks. Treatment with zilucoplan was well-tolerated in the study, consistent with results observed in the previously-completed Phase 1 and Phase 2 studies. The majority of adverse events (AEs) reported were mild and were not considered by the investigators to be related to study drug. There were no serious AEs observed related to treatment with zilucoplan.

About gMG

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), amyotrophic lateral sclerosis (ALS), 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. The U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to zilucoplan for the treatment of MG.

About Ra Pharmaceuticals, Inc.

Ra Pharmaceuticals, Inc. 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 anticipated timing of release, and statements regarding trial design, timeline, and enrollment of Ra Pharma's ongoing and planned clinical programs,

including without limitation the Phase 3 clinical trial of zilucoplan for the treatment of gMG, building a leading complement-focused neurology franchise, 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 the risks that Ra Pharma's product candidates, including zilucoplan, will not successfully be developed or commercialized, in the timeframe we expect or at all; 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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