Ra Pharmaceuticals Announces Acceptance of gMG Phase 2 and Open-Label, Long-Term Extension Data for Emerging Science Dual Presentation at the 2019 AAN Annual Meeting

April 1, 2019

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 1, 2019-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced that data from its Phase 2 clinical trial of zilucoplan for the treatment of generalized myasthenia gravis (gMG) have been selected for an Emerging Science dual oral and poster presentation at the 2019 American Academy of Neurology (AAN) Annual Meeting in Philadelphia, PA, from May 4 to 10, 2019. The presentation will feature data from the Phase 2, randomized, double-blind, placebo-controlled clinical trial, as well as data from the open-label, long-term extension study.

Details of the presentation are as follows:

Abstract Title: Zilucoplan, a Subcutaneously Self-Administered Peptide Inhibitor of Complement Component 5 (C5), for the Treatment of Generalized Myasthenia Gravis: Results of a Phase 2 Randomized, Double-Blind, Placebo-Controlled Trial and Open-Label Long-Term Extension
Session Title: Emerging Science
Date/Time: Tuesday, May 7, Poster Presentation: 11:30 a.m.-12:45 p.m. E.T., Data Blitz Oral Presentation: 12:06 p.m. E.T.

About Zilucoplan

Ra Pharma is developing zilucoplan for generalized myasthenia gravis (gMG), paroxysmal nocturnal hemoglobinuria (PNH), and other complement-mediated disorders. The product candidate is designed for convenient, once-daily subcutaneous self-administration. Zilucoplan is a synthetic, macrocyclic peptide discovered using Ra Pharma's powerful proprietary drug discovery technology. The peptide binds complement component 5 (C5) with sub-nanomolar affinity and allosterically inhibits its cleavage into C5a and C5b upon activation of the classical, alternative, or lectin pathways.

About Ra Pharmaceuticals

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 upcoming presentations, development of product candidates, 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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