Ra Pharmaceuticals Reports Second Quarter 2018 Financial Results and Announces Early Completion of Enrollment in gMG Phase 2 Program

August 8, 2018

Surpassed target enrollment in Phase 2 trial of RA101495 SC for gMG

Top-line data now expected around year-end 2018

Regulatory progress on Phase 3 PNH program

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 8, 2018-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced financial results for the second quarter ended June 30, 2018 and highlighted recent progress in advancing its pipeline programs, including RA101495 SC for the treatment of generalized myasthenia gravis (gMG), paroxysmal nocturnal hemoglobinuria (PNH), and complement-mediated renal diseases such as atypical hemolytic uremic disorder (aHUS), and its first-in-class, orally bioavailable, small molecule C5 inhibitor program.

“Enthusiasm from neurologists and their patients for a convenient, self-administered, subcutaneous (SC) C5 inhibitor has resulted in the rapid recruitment of 44 patients in our Phase 2 clinical trial of RA101495 SC for the treatment of gMG, not only allowing us to complete enrollment ahead of schedule, but also to surpass our original enrollment target of 36 patients. We now expect to report top-line data around year-end 2018. The acceleration of our gMG Phase 2 program highlights RA101495 SC’s potential as a convenient and accessible subcutaneous treatment option designed to deliver everyday complement control to more patients with gMG,” said Doug Treco, PhD, Chief Executive Officer of Ra Pharma.

Dr. Treco continued, “We are also pleased to report that we have made significant progress in discussions with several regulatory authorities regarding our planned global Phase 3 studies of RA101495 SC for the treatment of PNH and, to date, have met with the Medicines and Healthcare products Regulatory Agency in the United Kingdom (MHRA), Health Canada, and the US Food and Drug Administration (FDA) to discuss the design of our Phase 3 registrational studies. The MHRA and Health Canada have indicated that a single arm Phase 3 design in both naïve and switch patients is acceptable, and discussions with the FDA are ongoing. We plan to meet with the European Medicines Agency (EMA) in the fourth quarter of 2018 to discuss our global Phase 3 program and, pending the successful outcome of these discussions, anticipate initiating our Phase 3 clinical trials during the first half of 2019.”

“We also completed dosing in our Phase 1b study designed to evaluate the pharmacokinetic (PK) profile of RA101495 SC in patients with renal impairment and expect to report top-line data by the end of the third quarter of 2018. We believe these data will allow us to proceed with the development of RA101495 SC in complement-mediated renal diseases, such as aHUS,” said Ramin Farzaneh-Far, MD, Chief Medical Officer of Ra Pharma.

Second Quarter 2018 Highlights and Recent Developments

- In August, Ra Pharma completed enrollment in the Phase 2 trial evaluating RA101495 SC for the treatment of gMG. The multi-center, randomized, double-blind, placebo-controlled trial enrolled a total of 44 patients. At the outset of the 12-week treatment period, patients were randomized in a 1:1:1 ratio and received daily SC doses of 0.1 mg/kg of RA101495, 0.3 mg/kg of RA101495, or matching placebo. The Company plans to report top-line data around year-end 2018.
- Dialogue advanced with global regulatory agencies regarding the design of the Phase 3 registrational program for RA101495 SC in both naïve and switch PNH patients.
    - Ra Pharma has met with the MHRA and Health Canada. Both agencies have indicated that a single arm Phase 3 design in both naïve and switch patients is acceptable.
    - Ra Pharma has met with the FDA, and discussions are ongoing.
    - Ra Pharma plans to meet with the EMA in the fourth quarter of 2018.

  Pending the successful outcome of these discussions, Ra Pharma anticipates initiating the Phase 3 clinical trials during the first half of 2019.
- Dosing has been completed in the Phase 1b PK study evaluating RA101495 SC in patients with renal impairment, which is designed to enable development in complement-mediated renal diseases such as aHUS. The Company remains on track to report top-line data by the end of the third quarter 2018.
- Data was presented on two programs at the 23rd Congress of the European Hematology Association, June 14-17, 2018 in Stockholm.
    - **Oral, small molecule complement inhibitor program:** This presentation described pre-clinical PK data demonstrating that compounds in the series display dose-dependent oral exposure and low clearance values, *ex vivo* and *in vitro* assays demonstrating selective engagement of complement C5, and a PK/PD relationship

informing the level of exposure required to achieve complete complement inhibition. Collectively, these data confirmed, for the first time in an *in vivo* setting, the feasibility of oral, small molecule inhibition of complement C5.

- **Phase 2 trial of RA101495 SC in PNH:** This presentation described the results of the Phase 2, global, multi-center, open-label, dose-finding study. These data demonstrated that RA101495 SC appears safe and well-tolerated in patients with PNH, with the ability to rapidly and robustly reduce lactate dehydrogenase (LDH) to the levels seen in patients receiving eculizumab, and which are associated with improved long-term outcomes.

- An overview of the Phase 2 clinical trial design evaluating RA101495 SC for the treatment of gMG was presented at the 4th Congress of the European Academy of Neurology, June 16-19, 2018, in Lisbon. The Phase 2, multi-center, randomized, double-blind, placebo-controlled trial is designed to evaluate the safety, tolerability, and preliminary efficacy of RA101495 SC in patients with gMG. The trial enrolled 44 patients. The presentation also highlighted previously reported Phase 1 healthy volunteer data for RA101495 SC.

Second Quarter 2018 Financial Results

For the second quarter of 2018, the Company reported a net loss of $15.7 million, or a net loss of $0.49 per share (basic and diluted), compared to a net loss of $12.7 million, or a net loss of $0.56 per share for the same period in 2017.

Research and development (R&D) expenses for the second quarter of 2018 were $12.3 million, compared to $10.5 million for the same period in 2017. The increase in R&D expenses for the second quarter of 2018 was primarily due to clinical development costs associated with our lead program, RA101495 SC.

General and administrative (G&A) expenses for the second quarter of 2018 were $3.8 million, compared to $2.3 million for the same period in 2017. The increase in G&A expenses for the second quarter of 2018 was primarily due to employee-related costs due to the increase in G&A headcount to support the growth of the Company.

As of June 30, 2018, Ra Pharma reported total cash and cash equivalents of $95.1 million. The Company expects that its cash and cash equivalents will be sufficient to fund operations through the end of 2019.

About RA101495 SC

Ra Pharma is developing RA101495 SC for paroxysmal nocturnal hemoglobinuria (PNH), generalized myasthenia gravis (gMG), atypical hemolytic uremic syndrome (aHUS), and lupus nephritis (LN). The product is designed for convenient, once-daily subcutaneous self-administration. RA101495 SC 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. By binding to a region of C5 corresponding to C5b, RA101495 SC is designed to disrupt the interaction between C5b and C6 and prevent assembly of the membrane attack complex (MAC). This activity may define an additional, novel mechanism for the inhibition of C5 function.

About RA101495 SC Phase 2 gMG Clinical Program

The Phase 2, multi-center, randomized, double-blind, placebo-controlled trial is designed to evaluate the safety, tolerability, and preliminary efficacy of RA101495 SC in patients with gMG. The trial has enrolled [44] patients and will include a screening period of up to four weeks. At the outset of the 12-week treatment period, patients were randomized in a 1:1:1 ratio and will receive daily, subcutaneous doses of 0.1 mg/kg of RA101495 SC, 0.3 mg/kg of RA101495 SC, or matching placebo. The primary efficacy endpoint is change in Quantitative Myasthenia Gravis (QMG) score from baseline to week 12. All patients will have the opportunity to receive RA101495 SC in a long-term extension study.

About RA101495 SC Phase 2 PNH Clinical Program

The global, dose-finding Phase 2 program was designed to evaluate the safety, tolerability, preliminary efficacy, pharmacokinetics, and pharmacodynamics of RA101495 SC in patients with PNH. The study evaluated RA101495 SC in three cohorts. The first cohort included eculizumab-naïve patients, the second cohort included patients switching from eculizumab to RA101495 SC, and the third cohort included patients who were currently treated with eculizumab but had evidence of an inadequate response. Patients in all three cohorts were eligible for entry into a long-term extension study following the completion of the initial 12-week studies. The primary efficacy endpoint was the change in LDH from baseline to the mean level from week 6 to week 12.

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 the potential safety, efficacy and regulatory and clinical progress of our product candidates, including without limitation RA101495 SC, planned meetings with regulatory authorities, statements regarding trial design, timeline and enrollment of our ongoing and planned clinical programs, including without limitation our Phase 3 studies of RA101495 SC for the treatment of PNH, and anticipated timelines for the release of clinical data . 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 RA101495 SC, will not successfully be developed or commercialized, in the timeframe we expect or at all; the risk that topline results as of February 7, 2017 from the Company's global Phase 2 clinical program evaluating RA101495 SC for the treatment of PNH may not be indicative of final study results; 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.

Ra Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(Unaudited)
(in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
Operating expenses:				
Research and development	$ 12,305	$ 10,464	$ 25,717	$ 19,476
General and administrative	3,821	2,348	7,133	4,817
Total operating expenses	16,126	12,812	32,850	24,293
Loss from operations	(16,126)	(12,812)	(32,850)	(24,293)
Other income (expense), net	380	149	606	270
Net loss	$ (15,746)	$ (12,663)	$ (32,244)	$ (24,023)
Net loss per common share – basic and diluted	$ (0.49)	$ (0.56)	$ (1.08)	$ (1.06)
Weighted average number of common shares outstanding – basic and diluted	32,307	22,575	29,789	22,562

Ra Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(Unaudited)
(In thousands)

	June 30, 2018	**December 31, 2017**
Assets		
Cash and cash equivalents	$ 95,057	$ 70,381
Prepaid expenses and other current assets	3,092	2,496
Property and equipment, net	5,709	5,606
Other noncurrent assets	1,681	1,714
Total assets	$ 105,539	$ 80,197
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 7,993	$ 8,285
Deferred rent	460	329
Noncurrent liabilities	2,166	2,399
Stockholders' equity	94,920	69,184
Total liabilities and stockholders' equity	$ 105,539	$ 80,197

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Source: Ra Pharmaceuticals, Inc.

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