Ra Pharmaceuticals Reports First Quarter 2019 Financial Results and Provides Corporate Update

May 9, 2019

Phase 2 gMG long-term extension data show durability of zilucoplan treatment effect, with sustained improvements in primary and secondary endpoints observed at 24 weeks

Initiation of a single, pivotal, 12-week Phase 3 clinical trial in gMG on track for second half of 2019

Zilucoplan XR achieved rapid and sustained pharmacodynamic inhibition of complement C5 in non-human primates, supporting once weekly or less frequent dosing

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 9, 2019-- Ra Pharmaceuticals, Inc. (Nasdaq:RARX) today announced financial results for the first quarter ended March 31, 2019, and provided an update on recent corporate and clinical developments.

“Zilucoplan’s competitive profile is underscored by the recently presented results from our Phase 2 long-term extension (LTE) study, in which clinically meaningful reductions in primary and secondary endpoints were sustained in patients treated with zilucoplan for 24 weeks,” said Doug Treco, Ph.D., President and Chief Executive Officer of Ra Pharma. “As an accessible and convenient self-administered therapy, zilucoplan offers the potential to deliver complement inhibition to a broad population of patients with generalized myasthenia gravis (gMG).”

Dr. Treco continued: “Building on this momentum, we continue to progress our zilucoplan life-cycle extension program. The recent presentation of pre-clinical data for the extended release (XR) zilucoplan program, in which rapid and sustained pharmacodynamic inhibition of complement component 5 (C5) was achieved with once-weekly subcutaneous (SC) dosing, supports our patient-centric approach to the development of convenient therapies. With initiation of a Phase 2 trial for our second neuromuscular indication for zilucoplan expected in the second half of this year, as well as initiation of Phase 1 studies for the zilucoplan XR program and our oral small molecule C5 inhibitor program expected in the first half of 2020, we are positioned to build meaningful value as we advance our mission of expanding access to next-generation therapies for patients with complement-mediated diseases.”

First Quarter 2019 Highlights and Recent Developments

- In April 2019, Ra Pharma announced the successful completion of End-of-Phase 2 interactions with the U.S. Food and Drug Administration (FDA) for its Phase 3 clinical trial of zilucoplan in gMG. Based on feedback provided by the FDA, the Company plans to initiate a single, pivotal, 12-week, Phase 3, randomized, double-blind, placebo-controlled trial evaluating the efficacy of a once-daily, SC self-administered dose of 0.3 mg/kg of zilucoplan versus placebo in the second half of 2019.
- In May 2019, Ra Pharma presented data from its Phase 2 clinical trial and open-label LTE study of zilucoplan in patients with gMG at the 2019 American Academy of Neurology Annual Meeting in Philadelphia, PA. In the Phase 2 clinical trial, both zilucoplan doses achieved rapid, clinically meaningful, and statistically significant reductions in pre-specified primary and secondary endpoints versus placebo at week 12. In the LTE study, sustained responses were observed for all four efficacy endpoints after 24 weeks at the 0.3 mg/kg dose of zilucoplan. Placebo patients crossing over to the 0.3 mg/kg dose of zilucoplan after 12 weeks experienced rapid, clinically meaningful, and statistically significant improvements for all four efficacy endpoints from weeks 12 to 24. Treatment with zilucoplan had a favorable safety and tolerability profile in the study, consistent with previously-completed Phase 1 and Phase 2 studies. There were no serious adverse events observed related to treatment with zilucoplan.
- In December 2018, Ra Pharma completed dosing in a Phase 1 ethno-bridging study in healthy subjects of Japanese and non-Japanese descent, enrolling 16 subjects in a multi-dose cohort and 20 subjects in a single-dose cohort. Ra Pharma today reports positive results from this study, showing that the pharmacokinetic and pharmacodynamic profile of zilucoplan was consistent and similar across both groups. These results support development of zilucoplan for the Japanese market without the need for dose modification.
- In April 2019, Ra Pharma presented pre-clinical data for the poly(D,L-lactic-co-glycolic acid) XR formulation of zilucoplan at the 6th Annual Peptides Congress in London, UK. Weekly SC doses of the PLGA XR formulation of zilucoplan in non-human primates rapidly achieved and maintained target drug concentrations for 14 days, sustaining near complete pharmacodynamic inhibition throughout this period.

First Quarter 2019 Financial Results

For the first quarter of 2019, the Company reported a net loss of $19.0 million, or a net loss of $0.45 per share (basic and diluted), compared to a net loss of $16.5 million, or a net loss of $0.61 per share, for the same period in 2018.

Research and development (R&D) expenses for the first quarter of 2019 were $15.3 million, compared to $13.4 million for the same period in 2018. The increase in R&D expenses for the first quarter was primarily due to increased headcount and employee-related costs to support increased research and development activities.

General and administrative (G&A) expenses for the first quarter of 2019 were $4.8 million, compared to $3.3 million for the same period in 2018. The increase in G&A expenses for the first quarter was primarily due to increased headcount and employee-related costs and increased expenses related to our pre-commercial activities.

There was no revenue earned in the first quarter of 2019 or the same period in 2018.

As of March 31, 2019, Ra Pharma reported total cash and cash equivalents of $191.6 million. The Company expects that its cash and cash equivalents will be sufficient to fund operating expenses and capital expenditures through at least the first quarter of 2021.

About Zilucoplan

Ra Pharma is developing zilucoplan and zilucoplan extended release (XR) for generalized myasthenia gravis (gMG) and other tissue-based, complement-mediated disorders with high unmet medical need. The product candidate is designed for convenient, once-daily, subcutaneous (SC) 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 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 our zilucoplan XR and oral small molecule C5 inhibitor programs, beliefs regarding clinical trial data, 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 and the XR and oral C5 small molecule inhibitor programs, pending End-of-Phase 2 discussions with regulatory agencies in the first half of 2019, and the expectation that Ra Pharma's cash and cash equivalents will be sufficient to fund operating expenses and capital expenditures through at least the first quarter of 2021. 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.

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

	Three Months Ended March 31,	
	2019	**2018**
Operating expenses:		
Research and development	$ 15,282	$ 13,412
General and administrative	4,807	3,312
Total operating expenses	20,089	16,724
Loss from operations	(20,089)	(16,724)
Other income (expense), net	1,050	226
Benefit from income taxes	-	-
Net loss	$ (19,039)	$ (16,498)
Net loss per common share – basic and diluted	$ (0.45)	$ (0.61)
Weighted average number of common shares outstanding – basic and diluted	42,174	27,242

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

	March 31, 2019	December 31, 2018
Assets		
Cash and cash equivalents	$ 191,647	$ 209,822
Prepaid expenses and other current assets	3,911	2,585
Property and equipment, net	5,015	5,165
Operating Lease right-of-use assets, net	2,864	-
Other noncurrent assets	1,632	1,648
Total assets	$ 205,069	$ 219,220
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 8,686	$ 9,722
Operating lease liabilities	1,042	-
Deferred rent	-	479
Noncurrent liabilities	4,087	1,901
Stockholders' equity	191,254	207,118
Total liabilities and stockholders' equity	$ 205,069	$ 219,220

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