Aerie Pharmaceuticals Announces Positive Topline Results of Netarsudil Ophthalmic Solution in Pilot Phase 2 Study Supporting Clinical Development in Japan

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Announces Opening of Tokyo Branch Office and Two Key Hires in Japan

DURHAM, N.C.--(BUSINESS WIRE)--Jan. 4, 2019-- Aerie Pharmaceuticals, Inc. (NASDAQ:AERI), an ophthalmic pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with open-angle glaucoma, retina diseases and other diseases of the eye today announced the topline results of its pilot Phase 2 study of netarsudil ophthalmic solution in a Japanese-American population. The study was designed in accordance with the requirements of Japan's PMDA (Pharmaceuticals and Medical Devices Agency) to support the potential regulatory submission of netarsudil ophthalmic solution in Japan. Netarsudil ophthalmic solution 0.02% is known by the name Rhopressa® in the United States, where it is currently marketed. This pilot study was initially designed as a larger Phase 2 trial to be conducted in the United States, enrolling Japanese subjects and Japanese-American subjects that are within second generation. Due to scarcity of qualified subjects in the United States, the enrollment of this study was limited to approximately 40 subjects across three study arms.

The primary objectives of the study were to evaluate (1) the ocular hypotensive activity of two different dose concentrations of netarsudil ophthalmic solution (0.02% and 0.04%) relative to placebo over a 28-day period, for a total of three arms all dosed in the evening, and (2) the ocular and systemic safety of netarsudil ophthalmic solution relative to placebo over that period. The ranges of unmedicated baseline IOP (intraocular pressure) at 8am in the study were greater than or equal to 15 mmHg (millimeters of mercury) to less than 35 mmHg for subjects with open-angle glaucoma, and greater than or equal to 22 mmHg to less than 35 mmHg for subjects with ocular hypertension.

The results, which are outlined in the supporting slide presentation to this press release, demonstrated that netarsudil ophthalmic solution 0.02% lowered IOP in mean diurnal IOP by a range of 5.0 to 5.3 mmHg for subjects with an average baseline IOP of 18.3 mmHg. The netarsudil ophthalmic solution 0.04% arm lowered IOP in mean diurnal IOP by a range of 5.2 mmHg to 6.6 mmHg for subjects with average baseline IOP of 20.2 mmHg. The placebo arm lowered IOP in mean diurnal IOP by a range of 2.0 to 2.5 mmHg for subjects with an average baseline pressure of 19.6 mmHg. Both netarsudil arms showed higher levels of IOP reduction as compared to placebo to a statistically significant degree at Day 28. The safety findings were consistent with previous netarsudil trials.

Aerie expects to initiate a Phase 2 clinical trial in Japan in the first quarter of 2019 structured, as agreed with the PMDA, consistently with this pilot study with the addition of a 0.01% concentration of netarsudil.

"We are delighted to see that netarsudil reduced pressures in these lower-baseline subjects at consistent levels to what was demonstrated in our previous Phase 3 trials in the United States. These pilot study results, while representing a relatively low number of subjects, may hold great promise for the Japanese glaucoma market. Studies in Japan have shown that Japanese glaucoma patients experience IOPs that are generally lower than those experienced in the United States and Europe, essentially what is known as low-tension glaucoma. Also, the netarsudil IOP-reducing performance in this study compares favorably to that of a twice-daily Rho kinase inhibitor currently marketed in Japan," said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer at Aerie.

Dr. Anido added, "The Japanese glaucoma market is one of the largest in the world at approximately $1 billion annually, and we believe there continues to be substantial unmet need in this very important market for Aerie. With that, we are pleased to announce the opening of our Japan branch office in Tokyo, and the addition of two well-respected industry leaders to our Tokyo team. Yasuhide Fukushima joins as our Head of Strategy and Professional Affairs in Japan; he previously held a related position in Alcon Japan, Inc.Kenji Aso, M.D., Ph.D., joins as our Head of Clinical; he previously held a related position in Japan Bayer Yakuhin, Ltd. Both are reporting to Theresa Heah, M.D., M.B.A., Aerie's Vice President of Clinical Research, Medical and Professional Affairs. We are fortunate to have such exceptional talent leading the way as we execute our global strategy."

About Aerie Pharmaceuticals, Inc.

Aerie is an ophthalmic pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with open-angle glaucoma, retina diseases and other diseases of the eye. Aerie's first product, Rhopressa® (netarsudil ophthalmic solution) 0.02%, a once-daily eyedrop approved by the U.S. Food and Drug Administration (FDA) for the reduction of elevated intraocular pressure (IOP) in patients with open-angle glaucoma or ocular hypertension, was launched in the United States in April 2018. In clinical trials of Rhopressa®, the most common adverse reactions were conjunctival hyperemia, cornea verticillata, instillation site pain, and conjunctival hemorrhage. More information about Rhopressa®, including the product label, is available at www.rhopressa.com. Aerie's advanced-stage product candidate, Rocklatan™ (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005%, a fixed-dose combination of Rhopressa® and the widely-prescribed PGA (prostaglandin analog) latanoprost, achieved its 3-month primary efficacy endpoint in two Phase 3 registration trials, Mercury 1 and Mercury 2, and also showed safety and efficacy throughout 12 months in Mercury 1. Aerie submitted the Rocklatan™ New Drug Application (NDA) in May 2018 and, in July 2018, the FDA set the PDUFA (Prescription Drug User Fee Act) goal date for the completion of the FDA's review of the Rocklatan ™ NDA for

March 14, 2019. Aerie continues to focus on global expansion and the development of additional product candidates and technologies in ophthalmology, including for wet age-related macular degeneration and diabetic macular edema. More information is available at www.aeriepharma.com.

Forward-Looking Statements

This press release contains forward-looking statements for purposes of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as “predicts,” “believes,” “potential,” “proposed,” “continue,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “could,” “might,” “will,” “should,” “exploring,” “pursuing” or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Forward-looking statements include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things: our expectations regarding the commercialization and manufacturing of Rhopressa® and Rocklatan™ or any future product candidates, including the timing, cost or other aspects of the commercial launch of Rhopressa® and Rocklatan™ or any future product candidates; our commercialization, marketing, manufacturing and supply management capabilities and strategies; the success, timing and cost of our ongoing and anticipated preclinical studies and clinical trials for Rhopressa®, with respect to regulatory approval outside of the United States or additional indications, and Rocklatan™ or any future product candidates, including statements regarding the timing of initiation and completion of the studies and trials, such as statements in this press release regarding our expected clinical trial in Japan of netarsudil ophthalmic solution; our expectations regarding the effectiveness of Rhopressa®, Rocklatan™ or any future product candidates and results of our clinical trials; the timing of and our ability to request, obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, as applicable, Rhopressa® and Rocklatan™ or any future product candidates, including the expected timing of, and timing of regulatory and/or other review of, filings for, as applicable, Rhopressa® and Rocklatan™ or any future product candidates; the potential advantages of Rhopressa® and Rocklatan™ or any future product candidates; our plans to pursue development of additional product candidates and technologies within and beyond ophthalmology, including development of Rhopressa® and Rocklatan™ for additional indications and other therapeutic opportunities; our plans to explore possible uses of our existing proprietary compounds beyond glaucoma, including development of our retina program; our ability to protect our proprietary technology and enforce our intellectual property rights; and our expectations regarding strategic operations, including our ability to in-license or acquire additional ophthalmic products, product candidates or technologies. By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, industry change and other factors beyond our control, and depend on regulatory approvals and economic and other environmental circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. We discuss many of these risks in greater detail under the heading “Risk Factors” in the quarterly and annual reports that we file with the Securities and Exchange Commission (SEC). In particular, FDA approval of Rhopressa® does not constitute FDA approval of Rocklatan™, and there can be no assurance that we will receive FDA approval for Rocklatan™ or any future product candidates. FDA approval of Rhopressa® also does not constitute regulatory approval of Rhopressa® in jurisdictions outside the United States and there can be no assurance that we will receive regulatory approval for Rhopressa® in jurisdictions outside the United States. Our receipt of a Prescription Drug User Fee Act (PDUFA) goal date notification for Rocklatan™ does not constitute FDA approval of the Rocklatan™ New Drug Application (NDA), and there can be no assurance that the FDA will complete its review by the PDUFA goal date of March 14, 2019, that the FDA will not require changes or additional data that must be made or received before it will approve the NDA, if ever, or that the FDA will approve the NDA. In addition, the clinical trial results discussed in this press release are preliminary and the outcome of such clinical trials may not be predictive of the outcome of later clinical trials. Forward-looking statements are not guarantees of future performance and our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this press release. Any forward-looking statements that we make in this press release speak only as of the date of this press release. We assume no obligation to update our forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

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