October 27, 2016

Aerie Pharmaceuticals Reports Positive Topline Efficacy Results of Rocket 4 Phase 3 Trial of RhopressaTM (netarsudil ophthalmic solution) 0.02%

- Study Successfully Achieves Primary Efficacy Endpoint -

- Company Separately Announces Withdrawal of Rhopressa™ NDA, with the Expectation of Refiling in January 2017 -

Conference Call and Webcast Today, October 27, at 5:00 p.m. ET

IRVINE, Calif.--(BUSINESS WIRE)-- Aerie Pharmaceuticals, Inc. (NASDAQ:AERI), a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of glaucoma and other diseases of the eye, today reported the successful 90-day topline efficacy results of its Rocket 4 Phase 3 clinical trial of product candidate Rhopressa™, a novel once-daily eye drop being tested for its ability to lower intraocular pressure (IOP) in patients with glaucoma or ocular hypertension. The Rocket 4 trial is designed to provide adequate six-month safety data for European regulatory filing purposes. Rocket 4 is not necessary for NDA filing purposes.

Rocket 4 enrolled a total of approximately 700 patients and is a two-arm six-month trial, which includes a 90-day efficacy readout evaluating once-daily Rhopressa™ for non-inferiority to twice-daily timolol. The range for the primary endpoint includes patients with baseline IOPs from above 20 mmHg (millimeters of mercury) to below 25 mmHg.

The study achieved its primary efficacy endpoint demonstrating non-inferiority of once-daily Rhopressa™ compared to twice-daily timolol.

Separately, Aerie announced that it has withdrawn the Rhopressa™ NDA (new drug application) that was submitted to the FDA in the third quarter of 2016. The filing was withdrawn as the result of a third party manufacturing facility in Tampa, Florida not being ready for pre-approval inspection by the FDA. The drug product contract manufacturer has advised Aerie and the FDA that it expects to be prepared for FDA inspection in January 2017, and Aerie expects to resubmit the Rhopressa™ NDA filing at that time.

Management will host a conference call and provide accompanying slides to discuss the clinical results and the Rhopressa™ NDA at 5:00 p.m. ET today.

Rhopressa™ Phase 3 Highlights for Rocket 4

- Rhopressa™ dosed once-daily achieved its primary efficacy endpoint demonstrating non-inferiority compared to twice-daily timolol for patients with baseline IOPs ranging from above 20 to below 25 mmHg.
- Rhopressa™ also demonstrated non-inferiority compared to timolol at the pre-specified secondary endpoint range of above 20 mmHg to below 27 mmHg, and also at a range of above 20 mmHg to below 28 mmHg.
- The Rocket 4 efficacy results for Rhopressa™ demonstrated a consistent level of IOP lowering across all baseline IOPs in the trial, and throughout the 90-day efficacy period.
- The most common Rhopressa™ adverse event was hyperemia, or eye redness, which was reported in approximately 40 percent of patients, 85 percent of which was scored as mild. Other adverse events, which have also been observed in previous Rhopressa™ clinical trials, were reported in 5 percent to 12 percent of patients. There were no drug-related systemic or serious adverse events.

"These successful Rocket 4 topline efficacy results once again confirm that Rhopressa™ is a highly efficacious and well-tolerated drug. In fact, Rhopressa™ performed better at the higher baseline ranges in Rocket 4 than in both Rocket 1 and Rocket 2. This is in addition to Rhopressa™ having demonstrated non-inferiority to latanoprost for baseline IOP ranges of above 20 mmHg to below 25 mmHg in the recent readout from the Mercury 1 clinical trial," said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer at Aerie.

Dr. Anido continued, "Rocket 4 is designed to provide adequate six-month Rhopressa™ safety data for our filing with the European regulatory authorities. We expect this trial to be completed in the second quarter of 2017. Updating on Roclatan™, patient enrollment in the second Phase 3 registration trial, Mercury 2, is well underway and we remain on target for the 90-day topline efficacy readout in the second quarter of 2017."

Dr. Anido added, "Regarding the withdrawal of the Rhopressa™ NDA, we plan on refiling the NDA in January of 2017, which is when we and the FDA have been advised by our contract manufacturer that they expect to be prepared for FDA inspection. As part of a 60-day review by the FDA, we also received a few minor routine inquiries, none of which were related to safety or efficacy of Rhopressa™."

Richard A. Lewis, M.D., Aerie's Chief Medical Officer, commented, "We have accumulated a wealth of information on how Rhopressa™ performs, with nearly 2,000 patients dosed and approximately 150 investigator sites engaged in our Phase 3 clinical trials. It is very clear to me that, if approved, this novel IOP-lowering product that targets the diseased tissue has the potential to be a meaningful new therapy for patients with glaucoma or ocular hypertension."

About Rhopressa™

Rhopressa™ (netarsudil ophthalmic solution) 0.02%, is a novel eye drop that we believe, if approved, would become the only once-daily product available that, based on Aerie's preclinical studies, specifically targets the trabecular meshwork, the eye's primary fluid drain and the diseased tissue responsible for elevated IOP in glaucoma. Preclinical studies have also demonstrated that Rhopressa™ lowers episcleral venous pressure, which contributes approximately half of IOP in healthy subjects. Further, based on Aerie's preclinical studies, Rhopressa™ provides an additional mechanism that reduces fluid production in the eye and therefore lowers IOP. Biochemically, Rhopressa™ has been shown in Aerie studies to inhibit both Rho Kinase (ROCK) and norepinephrine transporter (NET). Recent preclinical studies have also shown that Rhopressa™ may have disease-modifying properties, including an anti-fibrotic effect on the trabecular meshwork and the potential to increase perfusion of the trabecular meshwork. Preclinical research is also currently underway to evaluate the potential neuroprotective benefits of Rhopressa™.

The results of two Phase 3 registration trials (Rocket 2 and Rocket 1) for Rhopressa™ were included in a NDA filing submitted to the FDA in the third quarter of 2016 that was withdrawn in October 2016 due to the contract drug product manufacturer not being prepared for pre-approval inspection by the FDA, and is expected to be resubmitted in January 2017. Rocket 2 will represent the pivotal trial, and Rocket 1 will be supportive. There are two additional Phase 3 clinical trials currently underway for Rhopressa™, named Rocket 3 and Rocket 4. Rocket 3 is a 12-month safety-only study in Canada that is not needed for the NDA filing. Rocket 4 is designed to provide adequate six-month safety data for regulatory filing purposes in Europe, and is also not needed for the NDA filing.

Conference Call / Web Cast Information

Aerie management will host a live conference call and webcast at 5:00 p.m. Eastern Time today to discuss the Rhopressa™ Phase 3 efficacy results from Rocket 4.

The live webcast and a replay may be accessed by visiting Aerie's website at http://investors.aeriepharma.com. In addition, key data slides from the Rhopressa™ Rocket 4 study will be discussed on the conference call and are posted to Aerie's website. Please connect to Aerie's website at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call 1-888-734-0328 (U.S.) or 1-678-894-3054 (international) to listen to the live conference call. The conference ID number for the live call is 8732488. Please dial in approximately 10 minutes prior to the call. Telephone replay will be available approximately two hours after the call. To access the replay, please call 1-855-859-2056 (U.S.) or 1-404-537-3406 (international). The conference ID number for the replay is 8732488. The telephone replay will be available until November 3, 2016.

About Aerie Pharmaceuticals, Inc.

Aerie is a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with glaucoma and other diseases of the eye. Aerie's two lead product candidates are once-daily IOP-lowering therapies with novel mechanisms of action to treat patients with glaucoma or ocular hypertension. The NDA filing for Rhopressa™ (netarsudil ophthalmic solution) 0.02% was originally submitted in the third quarter of 2016 and is expected to be resubmitted in January 2017. The second product candidate, Roclatan™ (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005%, which is a fixed dose combination of Rhopressa™ and widely prescribed PGA latanoprost, currently has two Phase 3 registration trials underway, named Mercury 1 and Mercury 2. If these trials are successful, a Roclatan™ NDA filing is expected to take place near year-end 2017. Aerie is also focused on the development of additional product candidates and technologies in ophthalmology.

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: the success, timing and cost of our ongoing and anticipated preclinical studies and clinical trials for our current product candidates, including statements regarding the timing of initiation and completion of the studies and trials; our expectations regarding the clinical effectiveness of our product candidates and results of our clinical trials; the timing of and our ability to request, obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates, including the expected resubmission of the NDA filing for Rhopressa™ discussed in this press release; our expectations regarding the commercialization of our product candidates; our expectations related to the use of proceeds from our equity and debt financings; our estimates regarding anticipated capital requirements and our needs for additional financing; the potential advantages of our product candidates; our plans to pursue development of our product candidates for additional indications and other therapeutic opportunities; our plans to explore possible uses of our existing proprietary compounds beyond glaucoma; and our ability to protect our proprietary technology and enforce our intellectual property rights. By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics and industry change, and depend on regulatory approvals and economic 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, the topline Rocket 4 data presented herein is preliminary and based solely on information available to us as of the date of this press release and additional information about the results may be disclosed at any time. In addition, the preclinical research discussed in this press release is preliminary and the outcome of such preclinical studies may not be predictive of the outcome of later clinical trials. Any future clinical trial results may not demonstrate safety and efficacy sufficient to obtain regulatory approval related to the preclinical research findings discussed in this press release. 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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