



June 15, 2015

## **Aerie Pharmaceuticals Receives Positive Feedback from FDA**

### **-Rocket 2 Primary Endpoint Range Modified upon FDA Agreement-**

**Conference Call and Webcast Today, June 15, at 5:00 p.m. ET**

IRVINE, Calif.--(BUSINESS WIRE)-- Aerie Pharmaceuticals, Inc. (NASDAQ:AERI) (the "Company"), 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, today reported that the U.S. Food and Drug Administration (FDA) has agreed in written and verbal communications that Aerie may change the primary endpoint range of its second Phase 3 registration trial of Rhopressa<sup>TM</sup>, named Rocket 2. With this agreement, the Company is changing the primary endpoint range to include patients with baseline intraocular pressures (IOPs) ranging from above 20 mmHg (millimeters of mercury) to below 25 mmHg. The former range for the primary endpoint of above 20 mmHg to below 27 mmHg will now represent a secondary endpoint range for Rocket 2.

### **Highlights**

- | The Rocket 2 primary endpoint range is now changed to the same range where the Phase 3 registration trial results of Rocket 1 demonstrated success, ranging from above 20 mmHg to below 25 mmHg. In the Rocket 1 trial, in this range, Rhopressa<sup>TM</sup> demonstrated non-inferiority to timolol, and numerical superiority over timolol at the majority of time points. According to the Baltimore Eye Survey, nearly 80 percent of newly diagnosed glaucoma patients have unmedicated baseline IOPs below 26 mmHg.
- | The FDA also agreed that the Company may use a hierarchically-based statistical approach in determining whether this three-arm trial is adequately powered at the revised primary endpoint range. Using this methodology, the Company believes that the new primary endpoint range is adequately powered, and there is no need to recruit additional patients into Rocket 2. Three-month efficacy results for Rocket 2 are expected by the end of the third quarter of 2015.
- | An additional Rhopressa<sup>TM</sup> Phase 3 registration trial, named Rocket 4, is expected to commence in the third quarter of 2015, along with the first Roclatan<sup>TM</sup> Phase 3 registration trial, named Mercury 1.

"We are extremely pleased with the outcome of our communications with the FDA. If Rocket 2 results resemble those of Rocket 1, we believe we may have a much greater opportunity for success in meeting the clinical endpoint of non-inferiority to timolol. We are also very appreciative of the thoughtful guidance provided by the FDA, and believe their feedback will prove very useful as our programs progress. Looking ahead, we expect to commence our next Phase 3 registration trial for Rhopressa<sup>TM</sup>, named Rocket 4, in the third quarter of 2015. Rocket 4 is expected to be established with a primary endpoint range of above 20 mmHg to below 25 mmHg," said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer.

Dr. Anido continued, "We are also preparing to commence our first Phase 3 registration trial for Roclatan<sup>TM</sup>, named Mercury 1, in the third quarter of 2015. We believe the apparent synergies we observed in Rocket 1 for Rhopressa<sup>TM</sup> trial patients previously on prostaglandin analogues such as latanoprost may explain why the Roclatan<sup>TM</sup> Phase 2b results were so impressive. If successful in the Phase 3 registration trials, we believe this once-daily quadruple-action product candidate has the potential to be the most efficacious eye drop in the market for patients with glaucoma and ocular hypertension."

### **Triple-Action Rhopressa<sup>TM</sup>**

Rhopressa<sup>TM</sup> is a novel triple-action eye drop that we believe, if approved, would become the only once-daily product available that specifically targets the trabecular meshwork (TM), the eye's primary fluid drain and the diseased tissue

responsible for elevated IOP in glaucoma. Preclinical results have demonstrated that Rhopressa™ also lowers episcleral venous pressure (EVP), which contributes approximately half of IOP in healthy subjects. Further, Rhopressa™ provides an additional mechanism that reduces fluid production in the eye and therefore lowers IOP. Biochemically, Rhopressa™ is known to inhibit both Rho Kinase (ROCK) and norepinephrine transporter (NET).

There were originally three Phase 3 registration trials for Rhopressa™. "Rocket 1" was a 90-day efficacy trial, the results of which were initially reported on April 23, 2015, "Rocket 2" is a 12-month safety trial with a 90-day interim efficacy readout, and "Rocket 3" is a safety-only study being conducted in Canada. In Rocket 1, for the primary endpoint range of above 20 mmHg to below 27 mmHg, Rhopressa™ did not demonstrate non-inferiority to timolol. However, Rhopressa™ did demonstrate non-inferiority to timolol at all ranges below 26 mmHg. As a result, the Company plans to commence in the third quarter of 2015 an additional Phase 3 registration trial, named "Rocket 4". Based on the current clinical trial status, the Company may submit a New Drug Application filing in the second half of 2016.

### **Quadruple-Action Roclatan™**

Roclatan™ is a once-daily eye drop that combines our triple-action Rhopressa™ with latanoprost, a prostaglandin analogue that is the most widely prescribed glaucoma drug. If approved, we believe that Roclatan™ would be the first glaucoma product to lower IOP through all known mechanisms: (i) increasing fluid outflow through the TM, the eye's primary drain, (ii) increasing fluid outflow through the uveoscleral pathway, the eye's secondary drain, (iii) reducing fluid production in the eye, and (iv) reducing EVP.

A successful 28-day Phase 2b clinical trial for Roclatan™ was completed in June 2014. Roclatan™ achieved its primary efficacy endpoint on day 29 and demonstrated statistical superiority over the product's individual components at all time points. We believe that Roclatan™, if approved, would be the only glaucoma product that covers the full spectrum of known IOP-lowering mechanisms, giving it the potential to provide a greater IOP-lowering effect than any currently approved glaucoma product. The first Phase 3 registration trial for Roclatan™, "Mercury 1," is expected to commence in the third quarter of 2015. The Company expects to commence two additional Mercury trials in 2016.

### **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 Company's clinical trials and provide a general business update.

The live webcast and a replay may be accessed by visiting the Company's website at <http://investors.aeriepharma.com>. Please connect to the Company'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 (888) 734-0328 (U.S.) or (678) 894-3054 (international) to listen to the live conference call. The conference ID number for the live call is 67259440. 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 (855)-859-2056 (U.S.) or (404) 537-3406 (international). The conference ID number for the replay is 67259440. The telephone replay will be available until June 21, 2015.

### **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. The Company is currently conducting a Phase 3 registration trial in the United States named Rocket 2, where the primary efficacy endpoint is to demonstrate non-inferiority of IOP lowering for Rhopressa™ compared to timolol, along with a Phase 3 registration safety-only trial, named Rocket 3, in Canada. The Company recently completed its initial Phase 3 registration trial, named Rocket 1, the three-month efficacy results of which were initially reported in April 2015, and expects to commence a fourth Phase 3 registration trial, named Rocket 4, in the third quarter of 2015. The Company also completed in 2014 a Phase 2b clinical trial in which Roclatan™ met the primary efficacy endpoint, demonstrating the statistical superiority of Roclatan™ to each of its components, and plans to commence the first Phase 3 registration trial for Roclatan™, named Mercury 1, in the third quarter of 2015.

### **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 obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates; our expectations regarding the commercialization of our product candidates; our expectations related to the use of proceeds from our initial public offering and the issuance and sale of our senior secured convertible notes and the issuance and sale of shares of our common stock in connection with our "at-the-market" sales agreement; 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. 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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