



Aerie Pharmaceuticals Reports Topline Result from Rhopressa® Mechanism of Action Study

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Clinical confirmation of the effect of Rhopressa® on trabecular meshwork outflow facility in patients with open-angle glaucoma or ocular hypertension

DURHAM, N.C.--(BUSINESS WIRE)--Aug. 15, 2018-- 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 topline efficacy results from a double-masked, randomized, placebo-controlled study (AR-13324-CS206; NCT03233308) designed to evaluate the effect of Rhopressa® (netarsudil ophthalmic solution) 0.02% on aqueous humor dynamics in patients with open-angle glaucoma or ocular hypertension. While several pre-clinical models and a previous study in healthy volunteers examined the effect of Rhopressa® on trabecular meshwork outflow facility, this study is the first performed on glaucoma patients to confirm that it lowers intraocular pressure (IOP) primarily through this mechanism.

The study enrolled patients who were diagnosed with open-angle glaucoma or ocular hypertension in both eyes, with unmedicated baseline IOP between 20 and 30 mmHg (millimeters of mercury). The primary endpoint of the study was the mean change from baseline in the mean diurnal outflow facility, measured using previously published methods.¹

In the study, Rhopressa® produced a statistically significant increase in trabecular outflow facility of approximately 35 percent over baseline. A complete analysis of the study data will be presented at a future scientific congress.

"Elevated IOP in open-angle glaucoma is due to dysfunction of the trabecular outflow pathway. This study confirms that in patients with elevated IOP, Rhopressa® lowers IOP by increasing trabecular outflow facility," said Casey C. Kocczynski, Ph.D., Chief Scientific Officer, Aerie Pharmaceuticals. "We believe this will give physicians additional confidence that Rhopressa® targets the tissue responsible for elevated IOP in their patients, and it will help them understand their options for combining this novel trabecular outflow drug with other therapies that lower IOP through different mechanisms."

This topline finding is consistent with the published results of a study of similar design in healthy volunteers (AR-13324-CS102; NCT02406287) in which Rhopressa® was also shown to exert a statistically significant effect on trabecular outflow facility.¹

About Rhopressa®

Rhopressa® (netarsudil ophthalmic solution) 0.02%, is a novel once-daily eye drop approved for the reduction of elevated intraocular pressure (IOP) in patients with open-angle glaucoma and ocular hypertension. A Rho kinase (ROCK) inhibitor, Rhopressa® is specifically designed to increase outflow of aqueous humor (the fluid inside the eye) through the trabecular meshwork, the main fluid drain of the eye.

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 full product label, is available at www.rhopressa.com.

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 full product label, is available at www.rhopressa.com. Aerie's advanced-stage product candidate, Roclatan™ (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 Roclatan™ 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 Roclatan™ 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," "opportunities" 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 commercial launch and sales of Rhopressa[®] and Roclatan[™] and any future product candidates, if approved; our commercialization, marketing, manufacturing and supply management capabilities and strategies; third-party payer coverage and reimbursement of Rhopressa[®] and Roclatan[™] and any future product candidates, if approved; the glaucoma patient market size and the rate and degree of market adoption of Rhopressa[®] and Roclatan[™] and any future product candidates, if approved, by eye-care professionals and patients; the timing cost or other aspects of the commercial launch of Rhopressa[®] and Roclatan[™] and any future product candidates, if approved; the success, timing and cost of our ongoing and anticipated preclinical studies and clinical trials for Rhopressa[®], with respect to regulatory approval outside the United States, and Roclatan[™] and any future product candidates, including statements regarding the timing of initiation and completion of the studies and trials; our expectations regarding the effectiveness of Rhopressa[®], Roclatan[™] and any future product candidates and results of our clinical trials and any potential preclinical studies; 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[®], Roclatan[™] and any future product candidates in the United States, Canada, Europe, Japan and elsewhere, including the expected timing of, and regulatory and/or other review of, filings for, as applicable, Rhopressa[®], Roclatan[™] and any future product candidates; the potential advantages of Rhopressa[®], Roclatan[™] and any future product candidates; our plans to pursue development of additional product candidates and technologies in ophthalmology, including development of Rhopressa[®] and Roclatan[™] for additional indications, our preclinical retina programs and other therapeutic opportunities; our plans to explore possible uses of our existing proprietary compounds beyond glaucoma and ophthalmology; our ability to protect our proprietary technology and enforce our intellectual property rights; and our expectations regarding collaborations, licensing, acquisitions and 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 Roclatan[™], and there can be no assurance that we will receive FDA approval for Roclatan[™] or for 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 Rhopressa[®] will obtain regulatory approval in other jurisdictions. Our receipt of a Prescription Drug User Fee Act (PDUFA) goal date notification for Roclatan[™] does not constitute FDA approval of the Roclatan[™] 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 research discussed in this press release is preliminary and the outcome of such studies may not be predictive of the outcome of later studies. 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.

¹ Kazemi A, McLaren JW, Kopczyński CC, Heah TG, Novack GD, Sit AJ. The effects of netarsudil ophthalmic solution on aqueous humor dynamics in a randomized study in humans. *J Ocul Pharmacol Ther.* 2018 Feb 22. doi: 10.1089/jop.2017.0138.

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