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Aerie Pharmaceuticals Reports Positive Roclatan™ (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005% Phase 3 12-month Topline Safety Results

Roclatan™ Successfully Demonstrates a Positive Safety Profile and Efficacy Levels Consistent with Previously Reported Results

Conference Call and Webcast Today, July 19, 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 patients with glaucoma and other diseases of the eye, today reported the successful 12-month safety results of the Company's "Mercury 1" Phase 3 registration trial for its fixed-dose combination product candidate, Roclatan™. Mercury 1 is a 12-month safety and efficacy trial which included a 90-day efficacy endpoint. As previously reported, both Mercury 1 and Mercury 2, the Company's second Phase 3 registration trial of Roclatan™, achieved their 90-day primary efficacy endpoints of demonstrating statistical superiority over each of its components at all measured time points, including Aerie product candidate Rhopressa™ (netarsudil ophthalmic solution) 0.02%, and market-leading prostaglandin analogue (PGA) latanoprost, all of which were dosed once daily in the evening.

The purpose of the 12-month Mercury 1 study is to provide adequate safety data for an expected NDA (new drug application) submission to the FDA in the first half of 2018. While not primary endpoints, the study also included measurements of intraocular pressure (IOP) at 8 a.m., 10 a.m. and 4 p.m. at months six, nine and twelve. The 12-month safety and efficacy results of Mercury 1 were consistent with the 90-day results from the Mercury 1 and Mercury 2 trials, each of which evaluated patients with maximum baseline IOPs ranging from above 20 to below 36 mmHg (millimeters of mercury). Management will host a conference call with accompanying slides to discuss these results at 5:00 p.m. Eastern Time (ET) today. The accompanying slides will be available at Aerie's website, aeriepharma.com.

Roclatan™ 12-Month Safety and Efficacy Highlights for Mercury 1

- Safety results for Roclatan™ for the 12-month period were consistent with those observed for the 90-day efficacy period in the trial. There were no new adverse events that developed following the initial 90-day period, and there were no drug-related serious or systemic adverse events.
- As expected, the most common adverse event for Roclatan™ was conjunctival hyperemia, or eye redness, which was observed in approximately 60 percent of patients, of which approximately 70 percent was determined to be mild by biomicroscopy. As observed in previous trials, hyperemia was sporadic, with only approximately 10 percent of patients with hyperemia across each physician visit during the 12-month period.
- The other Roclatan™ adverse events observed during the 12-month trial are consistent with those observed during the initial 90-day efficacy period, including conjunctival hemorrhages (subconjunctival petechiae) and cornea verticillata.
- In addition to the primary efficacy endpoint at 90 days, IOPs were measured at 8 a.m., 10 a.m., and 4 p.m. at months six, nine and twelve. Roclatan™ IOP lowering exceeded that of both latanoprost and Rhopressa™ in a range from 1 to 3 mmHg. Levels of IOP lowering were consistent with those observed in the Mercury 1 and Mercury 2 90-day efficacy results for all arms of the study. Roclatan™ also demonstrated consistent levels of IOP lowering across the 12-month study period.
- Roclatan™ reduced mean diurnal IOPs to 16 mmHg or lower in 60 percent of patients, a significantly higher percentage than observed in the two comparator arms.
- The Rhopressa™ arm of the study performed consistently with previous Phase 3 trials from both a safety and efficacy perspective. Rhopressa™ also demonstrated consistent levels of IOP lowering across the 12-month study period. At baseline IOPs below 25 mmHg, Rhopressa™ IOP lowering was similar to latanoprost at month 12.

"With these positive 12-month Mercury 1 data, we have again demonstrated the consistent and well-understood performance of Roclatan™ and Rhopressa™ from both a safety and efficacy perspective. Roclatan™ has the potential to become the most efficacious IOP-lowering therapy to enter the market, if approved, bolstered by an overall favorable safety and tolerability profile. We continue to expect to submit our Roclatan™ NDA (new drug application) in the first half of 2018," said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer at Aerie.

Dr. Anido continued, "This data readout represents the last in our series of Phase 3 trials for both Roclatan™ and Rhopressa™ for approval in the U.S., and we are now actively engaged in preparations for the expected Rhopressa™ commercialization next year."

Richard A. Lewis, M.D., Aerie's Chief Medical Officer, added, "As a clinician, I am very excited about the responder analysis data for Roclatan™ showing such a profound drop in IOP. We now have a robust understanding of the Roclatan™ safety profile and expect that clinicians will be highly satisfied with the 12-month safety and efficacy data."

About Roclatan™

Roclatan™ is a once-daily eye drop that combines Rhopressa™, as described below, with latanoprost, a widely prescribed PGA. Based on the Company's preclinical studies and clinical trials to date, Aerie believes that Roclatan™, if approved, would be the first glaucoma product to lower IOP through all known mechanisms: (i) increasing fluid outflow through the trabecular meshwork, 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 episcleral venous pressure (EVP). By covering the full spectrum of known IOP-lowering mechanisms, Roclatan™ has the potential to provide a greater IOP-lowering effect than any currently approved glaucoma product.

The first Phase 3 registration trial for Roclatan™, named Mercury 1, is a 12-month safety and efficacy trial, which was just completed and is the subject of this press release. Mercury 1 had a successful 90-day efficacy readout in September 2016. The second Phase 3 registration trial, named Mercury 2, is a 90-day efficacy trial, which reported successful primary efficacy results in May 2017. The topline 90-day efficacy readouts for both Mercury 1 and Mercury 2 demonstrated that Roclatan™ was statistically superior to each of its components, thus achieving their primary clinical endpoints. Aerie expects to submit a Roclatan™ NDA to the U.S. Food and Drug Administration (FDA) in the first half of 2018. A third Phase 3 registration trial, named Mercury 3, is expected to commence in Europe in the third quarter of 2017. Mercury 3 is not necessary for approval in the U.S., but rather to facilitate regulatory approval and commercialization in Europe.

About Rhopressa™

Rhopressa™ (netarsudil ophthalmic solution) 0.02%, is a novel eye drop that the Company believes, if approved, would become the only once-daily product available that, based on Aerie's preclinical and clinical studies to date, specifically targets the trabecular meshwork, the eye's primary fluid drain and the diseased tissue responsible for elevated IOP in glaucoma. Preclinical and clinical 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™ may provide an additional mechanism that reduces fluid production in the eye and therefore lowers IOP. Biochemically, the active ingredient in Rhopressa™, netarsudil, 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 of netarsudil on trabecular meshwork cells and the potential to increase perfusion of the trabecular meshwork.

The results of two Phase 3 registration trials (Rocket 2 and Rocket 1) for Rhopressa™ were included in the NDA submission to the FDA in February 2017. There were two additional Phase 3 registration trials for Rhopressa™, named Rocket 3 and Rocket 4. Rocket 3 was a small 12-month safety-only study in Canada that was not necessary for the NDA submission and for which enrollment has been discontinued. Rocket 4, which was successfully completed in April 2017, was designed to provide adequate six-month safety data for regulatory filing purposes in Europe, and was also not necessary for the NDA submission. The 90-day efficacy results from Rocket 4 and Mercury 1, the initial Phase 3 registration trial for Aerie product candidate Roclatan™ (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005%, were also included in the Rhopressa™ NDA submission as supportive. The FDA has set the Prescription Drug User Fee Act (PDUFA) goal date for the completion of the FDA's review of the Rhopressa™ NDA for February 28, 2018.

Conference Call / Webcast Information

Aerie management will host a live conference call and webcast at 5:00 p.m. ET today to discuss the Roclatan™ Phase 3 12-month safety and efficacy results from Mercury 1, including a review of the associated slides that are posted on Aerie's website, aeriepharma.com.

The live webcast and a replay may be accessed by visiting Aerie'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 48191718. 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 48191718. The telephone replay will be available until July 26, 2017.

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 current product candidates are once-daily intraocular pressure lowering therapies with novel mechanisms of action to treat patients with glaucoma or ocular hypertension. The NDA for Rhopressa™ (netarsudil ophthalmic solution) 0.02% was submitted to the FDA in February 2017, and, in May 2017, the FDA set the PDUFA goal date for the completion of the FDA's review of the Rhopressa™ NDA for February 28, 2018. Aerie's 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, achieved its primary efficacy endpoint in two Phase 3 registration trials, named Mercury 1 and Mercury 2, and also achieved successful 12-month safety and efficacy results in Mercury 1. The Roclatan™ NDA submission is expected to take place in the first half of 2018. 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 and potential future 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 FDA or other regulatory authority approval of, or other action with respect to, our product candidates, including the expected timing of, and timing of regulatory and/or other review of, filings for our product candidates; our expectations regarding the commercialization and manufacturing of our product candidates; the potential advantages of our product candidates; our plans to pursue development of additional product candidates and technologies in ophthalmology, including 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; 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 or 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, the topline Mercury 1 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. The receipt of the PDUFA goal date notification does not constitute FDA approval of the Rhopressa™ NDA, and there can be no assurance that the FDA will complete its review by the PDUFA goal date, that the FDA will not require changes or additional data, whether as a result of recommendations, if any, made by any FDA advisory committee or otherwise, 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 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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Aerie Pharmaceuticals
Richard Rubino, 908-947-3540
rrubino@aeriepharma.com
or
Burns McClellan, Inc., on behalf of Aerie Pharmaceuticals
Investors
Ami Bavishi, 212-213-0006
abavishi@burnsmc.com
or
Media
Justin Jackson, 212-213-0006
jjackson@burnsmc.com

Source: Aerie Pharmaceuticals, Inc.