Aerie Pharmaceuticals Initiates First-in-Human Clinical Trial of AR-13503 Sustained Release Intravitreal Implant in Patients with Neovascular Age-Related Macular Degeneration and Diabetic Macular Edema

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-- Study will evaluate the safety and preliminary efficacy of AR-13503 SR Implant as monotherapy and in combination with aflibercept --

DURHAM, N.C.--(BUSINESS WIRE)--Aug. 20, 2019-- Aerie Pharmaceuticals, Inc. (NASDAQ:AERI) (Aerie), 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, retinal diseases and other diseases of the eye, today announced the commencement of patient dosing in a first-in-human clinical trial of AR-13503 Sustained Release (SR) Implant in patients with neovascular age-related macular degeneration (nAMD) or diabetic macular edema (DME).

This multi-arm, 24-week study (AR-13503-CS201) is being conducted in two sequential stages. The first phase is a multicenter, open label, dose escalation study of the safety and tolerability of a single intravitreal injection of AR-13503 SR Implant, using two doses, in up to 12 patients. The second phase, enrolling up to 90 patients, is a multicenter, single-masked, randomized, parallel group study of the safety and preliminary efficacy of low- or high-dose AR-13503 SR Implant, dosed as monotherapy and in combination with aflibercept (EYLEA®; Regeneron), compared to aflibercept alone. Aflibercept is a vascular endothelial growth factor (VEGF) inhibitor approved for the treatment of retinal diseases. More information about the study is available at www.clinicaltrials.gov under the study designation NCT03835884.

“We are excited to be initiating human clinical evaluation of a novel treatment pathway for common, sight-threatening retinal diseases,” said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer. “Because AR-13503 may address several distinct components of the disease process in nAMD and DME, we believe the AR-13503 SR Implant has the potential to be an important addition to the treatment armamentarium in retina, particularly by helping expand the options for individualizing therapy. This study is designed to confirm our pre-clinical observations that AR-13503 could be useful as either monotherapy or in combination with VEGF inhibitors, and to help us select an appropriate dose for later stage trials. It will also provide the first clinical data for our polyesteramide bio-erodible polymer implant technology, which allows us to deliver small-molecule drugs to the back of the eye while extending treatment duration.”

About AR-13503 SR Implant

The AR-13503 SR Implant is a bio-erodible polyesteramide polymer implant that provides controlled release of AR-13503, a proprietary, small-molecule inhibitor of both Rho kinase and Protein kinase C, over a sustained period. It is designed to be administered approximately once every six months by intravitreal injection. Preclinical studies suggest that AR-13503 may inhibit angiogenesis, preserve the blood retinal barrier, and reduce retinal fibrosis in retinal diseases such as nAMD and DME, while potentially reducing the treatment burden associated with more frequent intravitreal injections.

About Retinal Disease

The retina is a thin membrane lining the back of the eye composed of highly-specialized cells that convert visible light into electrical impulses that reach the brain through the optic nerve. There are a variety of diseases, including progressive disorders such as age-related macular degeneration (AMD) and diabetic macular edema (the macula is the central portion of the retina), that can damage the retina and cause visual impairment or permanent blindness. In the United States, AMD is the leading cause of significant visual acuity loss in people over age 50 and diabetic retinopathy is the most common cause of irreversible blindness among working-age people. As a result, the retinal disease market is the largest therapeutic category in ophthalmology, with revenues in the United States reaching approximately $6.0 billion in 2018. Because current medical therapies are not adequately effective for many patients and do not stop disease progression, new treatment options are needed.

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, retinal diseases and other diseases of the eye. Aerie's first product, Rhopressa® (netarsudil ophthalmic solution) 0.02%, a once-daily eye drop 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, corneal verticillata, instillation site pain, and conjunctival hemorrhage. More information about Rhopressa®, including the product label, is available at www.rhopressa.com. Aerie’s second product for the reduction of IOP in patients with open-angle glaucoma or ocular hypertension, Rocklatan® (netarsudil and latanoprost ophthalmic solution) 0.02%/0.005%, the first and

only fixed-dose combination of Rhopressa® and the widely-prescribed PGA (prostaglandin analog) latanoprost, has been approved by the FDA and was launched in the United States in the second quarter of 2019. In clinical trials of Rocklatan®, the most common adverse reactions were conjunctival hyperemia, corneal verticillata, instillation site pain, and conjunctival hemorrhage. More information about Rocklatan®, including the product label, is available at www.rocklatan.com. 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® and Rocklatan®, with respect to regulatory approval outside of the United States or additional indications, and any future product candidates, including statements regarding the timing of initiation and completion of the studies and 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®, Rocklatan®, AR-13503, AR-1105 or any future product candidates, including the expected timing of, and timing of regulatory and/or other review of, filings for, as applicable, Rhopressa®, Rocklatan®, AR-13503, AR-1105 or any future product candidates; the potential advantages of Rhopressa® and Rocklatan®, AR-13503, AR-1105 or any future product candidates; our plans to pursue development of additional product candidates and technologies within and beyond ophthalmology; 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® and Rocklatan® do not constitute FDA approval of AR-1105, AR-13503 or any future product candidates and there can be no assurance that we will receive FDA approval for AR-1105, AR-13503 or any future product candidates. In addition, the initiation of the clinical trial discussed in this press release does not constitute FDA approval of AR-13503 and the outcome of clinical trials for AR-13503, including those discussed in this press release, may not be sufficient to submit an NDA with the FDA or to receive FDA approval. FDA approval of Rhopressa® and Rocklatan® do not constitute regulatory approval of Rhopressa® and Rocklatan® in jurisdictions outside the United States and there can be no assurance that we will receive regulatory approval for Rhopressa® and Rocklatan® in jurisdictions outside the United States. 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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