Building a Major Ophthalmic Pharmaceutical Company

Aerie Pharmaceuticals, Inc.
OIS @ ASRS
August 8, 2016
Important Information

Any discussion of the potential use or expected success of our product candidates is subject to our product candidates being approved by regulatory authorities. In addition, any discussion of clinical trial results for Rhopressa™ (netarsudil ophthalmic solution) 0.02% relate to the results in its first Phase 3 registration trials, Rocket 1 and Rocket 2, and for Roclatan™ (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005% relate to the results in its Phase 2b clinical trial.

The information in this presentation is current only as of its date and may have changed or may change in the future. We undertake no obligation to update this information in light of new information, future events or otherwise. We are not making any representation or warranty that the information in this presentation is accurate or complete.

Certain statements in this presentation are “forward-looking statements” within the meaning of the federal securities laws. Words such as “may,” “will,” “should,” “would,” “could,” “believe,” “expects,” “anticipates,” “plans,” “intends,” “estimates,” “targets,” “projects,” “potential” or similar expressions are intended to identify these forward-looking statements. These statements are based on the Company’s current plans and expectations. Known and unknown risks, uncertainties and other factors could cause actual results to differ materially from those contemplated by the statements. In evaluating these statements, you should specifically consider various factors that may cause our actual results to differ materially from any forward-looking statements. In particular, the preclinical research discussed in this presentation is preliminary and the outcome of such preclinical studies may not be predictive of the outcome of later 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 presentation. These risks and uncertainties are described more fully in the quarterly and annual reports that we file with the SEC, particularly in the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” Such forward-looking statements only speak as of the date they are made. We undertake no obligation to publicly update or revise any forward-looking statements, whether because of new information, future events or otherwise, except as otherwise required by law.
Aerie – Building a Major Ophthalmic Pharmaceutical Company

Glaucoma / Ocular Hypertension

- **Rhopressa™** (netarsudil ophthalmic solution) 0.02%
  - Inhibits ROCK, NET, lowers IOP, targets diseased tissue
  - Potential for disease modification
  - NDA filing expected Q3 2016
- **Roclatan™** (netarsudil/latanoprost ophthalmic solution) 0.02%/0.005%
  - Fixed combination of Rhopressa™ and latanoprost

Retinal Diseases

- **AR-13154**
  - Inhibits ROCK, PDGFR, JAK, targets multiple disease processes
  - Significant lesion size reduction in models of wet AMD, DR
  - Potential for monotherapy and adjunctive use with anti-VEGF
- **Drug Delivery**

Data on File
ROCK Inhibition Addresses Multiple Disease Processes Associated with AMD and DR

- Age-Related Macular Degeneration (AMD)\(^1\)
  - Angiogenesis/Vascular Leakage
  - Inflammation
  - Fibrosis

- Diabetic Retinopathy (DR)\(^2\)
  - Angiogenesis/Vascular Leakage
  - Inflammation

Clinical Experience with Anti-VEGF Treatments Supports Need to Treat Additional Drivers of Disease\(^3\)

ROCKi Library Screened for Multi-Kinase Inhibitors Targeting Pathology of AMD/DR

Aerie Kinase Library Screen

- 182 Aerie compounds screened against 469 human kinases
- Selected wide variety of structures and known activities
- Best lead compounds:
  - Hit multiple known or expected AMD/DR targets
  - Avoid kinases needed for cell survival

Relationship tree of human kinases. TK, TKL, STE, CK1, AGC, CAMK, CMGC, Other: Kinase superfamilies
AR-13154 Identified as Inhibitor of ROCK, PKC, PDGF Receptor and JAK Kinases

Panel of 182 ROCK inhibitors

Lin et al., ARVO 2016 Abstract 287

*182 internal compounds screened @500nM; Fasudil and Y-27632 screened @5 μM
*Only includes > 70% inhibition at screened concentration
Preclinical and Clinical Validation of Additional Kinase Targets Inhibited by AR-13154

- Angiogenesis/Vascular Leakage
  - PDGFR: preclinical models, including CNV
  - PDGFR: clinical validation in AMD (Phase 2 - Fovista®)
  - PKC: preclinical models of diabetic vascular leakage

- Inflammation
  - JAK: preclinical models, leukocyte adhesion/trafficking

- Fibrosis
  - PDGFR: multiple preclinical models, including AMD
Laser-induced choroidal neovascularization (CNV) in rats

Compounds delivered by intravitreal injection

ROCK/JAK2/PDGFRβ Inhibitor AR-13154 Numerically More Effective than Eylea® in Rat Model of AMD
Topical AR-13154(S) Provides Added Efficacy to Eylea® in Proliferative Diabetic Retinopathy Model

- Oxygen-induced retinopathy model of PDR (mouse)
- 0.06% AR-13154(S) delivered topically from P12 to P17
- Eylea delivered IP
- Confirms AR-13154(S) potential as effective adjunct to anti-VEGF therapies

Lin et al., ARVO 2016 Abstract 287
AR-13154 Next Steps

- Pair AR-13154(S) with sustained delivery system
  - Evaluating multiple technologies
  - Bioerodible implant/formulation
  - Targeting IVT injection every 3 – 6 months

- Establish long-term efficacy, PK in preclinical models

- Initiate IND-enabling toxicology, CMC in 1Q2017
AR-13154 Potential Advantages

- Addresses multiple disease processes
  - May improve long-term outcomes in AMD
  - May provide greater efficacy in DR

- Mechanisms of action compatible with anti-VEGF therapy
  - Potential use as monotherapy or adjunctive therapy to anti-VEGFs

- Potential for 3 – 6 month duration of effect
  - Reduces risks associated with IVT injection
  - Reduces burden on physicians and patients
Building a Major Ophthalmic Pharmaceutical Company