Developing Breakthrough Treatments For Dry Eye Disease

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CEO & Founder
## Corporate Highlights

| COMPANY | Privately held clinical stage biotech founded in 2016 as a spinout of GSK  
| Pharma partner in Laboratoire Théa  
| Nimble, highly experienced team with 200+ years of combined experience in ophthalmic drug development (Allergan, Pfizer, GSK) |

| MISSION | Develop drugs for treatment of dry eye disease and other ophthalmic inflammatory diseases |

| SCIENCE | First-in-class small molecules (DED)  
| Excellent preclinical data and safety packages, initially developed at GSK  
| AXR-159 completed a positive Phase 2a clinical trial  
| IND filing for AXR-270 in Q4 2019 |

| MARKET | Dry eye disease market is >$3B despite suboptimal therapies  
| Xiidra acquired by Novartis for $3.4B upfront, $1.9B in milestones in 2019 |
## Pipeline

<table>
<thead>
<tr>
<th>Indication</th>
<th>Target</th>
<th>Pre-clinical</th>
<th>IND-enabling</th>
<th>Phase 1/2a</th>
<th>Phase 2b</th>
<th>Phase 3</th>
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</thead>
<tbody>
<tr>
<td><strong>AXR-159 (eyedrop)</strong></td>
<td>DED</td>
<td>α4β1, α4β7</td>
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<tr>
<td><strong>AXR-270 (cream)</strong></td>
<td>Acute DED, Blepharitis</td>
<td>GR</td>
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AXR-159 Mechanism of Action

Multi-point interruption of the immune cycle in DED

Epithelial Immune Cycle in Dry Eye

1. Epithelial Stress
2. APC Activation & Migration to Lymph Node
3. T cell Homing to Ocular Surface
4. Ocular Surface Epithelial Pathology

Activity Based on Available Evidence:
- AXR-159 Only
- AXR-159 & Lifitegrast

Steroid-like Reduction of Corneal Staining and Inflammation in Sjögren’s Mouse Model

**Corneal Fluorescein Score**

![Graph showing corneal fluorescein score over time with vehicle and dexamethasone (0.1%) treatments.]

**IL-1b**

![Graph showing relative expression of IL-1b with vehicle and dexamethasone (0.1%) treatments.]

* compared to baseline; # compared to vehicle
Phase 2a POC Study Design

- Randomized, double-masked, vehicle-controlled
- 3 US sites
- Moderate-severe DED
- N=102; 1:1 randomization, 2 groups
- 50 mg/mL TID for 12 weeks; 2-week vehicle run-in
- Outcome measures: Safety, Tolerability, Systemic Pharmacokinetics, and Pharmacodynamics
  - CFS, LGS, Schirmer’s, TBUT; OSDI, SANDE, VAS; tear biomarkers
Demonstration of Safety and Efficacy in Phase 2a Study

- Improvement in signs and symptoms, including total ocular surface staining
- Target / responder population identified → increases probability of success in Phase 3
- Safe and well tolerated
- Opportunity for best-in-class dry eye therapeutic

![Responder Subpopulation, Day 14 CFB Total Staining Score](chart.png)

- Total Cornea/Calibra: P=0.03
- Total/Calibra: P=0.01
- Total/Oxford: P=0.02

Vehicle | AXR-159
Faster Onset and Superior Acute Efficacy Than Xiidra

AXR-159 (pre-specified responder group) comparison with Xiidra

Inferior Corneal Staining Score (CFB Drug – Vehicle)

Day 14  Day 42  Day 84

Xiidra (OPUS-1)  Xiidra (OPUS-2)  Xiidra (OPUS-3)  AXR-159
Scientific Advisory Board

World leading experts in ocular surface disease and drug development

Penny A. Asbell, MD, FACS, MBA, FARVO
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James and Margaret Elkins Chair
Ocular Surface Center
Cullen Eye Institute
Baylor College of Medicine
Future Plans

Advance portfolio in dry eye disease, blepharitis

Prepare for BID dose-ranging study

IND filing in 2019; initiate POC study in early 2020

Build new once-daily delivery platform: goal to replace eye drops

Seasoned team positioned to take AXR-159 and AXR-270 to approval

Looking for additional funding to advance our ambitious plans