Gemini Therapeutics

Ophthalmology Innovation Summit (OIS)
October 25\textsuperscript{th}, 2018 Chicago, IL

James McLaughlin
Co-Founder, President and CEO
Who We Are

Gemini is a **product engine** company which is using the process of **redefining AMD with precision medicine** to create a pipeline of **first-in-class therapeutics** for unmet clinical needs in the eye and throughout the body.
Unlocking the Potential of Precision Medicine with a Multimodal Engine

Committed to treating **genetically defined patient populations** orphaned within common diseases (e.g. AMD)

The future is multimodal

Our pipeline includes rProteins, mAbs and gene therapies

Novel first-in-class therapies

MOA matched to genotype (e.g. a new class of therapeutics which **restore complement regulation**)

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**gemini THERAPEUTICS**
Team

James McLaughlin
President and CEO, Co-Founder

Scott Lauder, PhD
CTO

Sandra Rojas-Caro, MD
CMO

Claude Knopf
CBO

Eric Sullivan
SVP Finance

Suresh Katti, PhD
VP Research

Walter Strapps, PhD
VP Gene Therapy

Amilcar “Mick” Ribeiro
VP Clinical Operations

Johanna Seddon, MD
Sr. Medical Advisor
Functionally Consequential CFH Variants are Linked to AMD

1. **Genetic studies** have consistently shown CFH is one of the genes most closely associated with AMD.

2. **Risk variants** are clustered in important functional regions.

3. Assays show some variants result in **significant loss-of-function**.
Severe Clinical Phenotypes are Associated with CFH Variants

52-year Old Woman with Significant CFH Loss of Function Variant

Her Family is Also Affected (Patient = Red Arrow)

Redefining a Disease with Precision Therapeutics Requires New Translational Tools

**Today**
- Patients are not genotyped
- No pharmacologic biomarkers
- No precision therapeutics have been developed in AMD

**Gemini**
- Genetic screening process and registry
- Diagnostics
- Pioneering natural history studies
- Functional characterization suite
- Pharmacologic biomarkers
- Multimodal technologies for intervention

**2020**
- Thousands of patients genotyped with custom diagnostics
- Pioneering intraocular pharmacologic biomarkers
- New natural history data on rare variants with Gemini-sponsored studies
Unlocking the Potential of Precision Medicine
Adding Genotype to the Definition of Disease

- CFH-AMD
- Gene B-AMD
- New Target
- New Target

AMD all comers
Natural History Studies: Gathering Unprecedented Phenotypic Data

In 2018, we are initiating the largest and most complete prospective longitudinal study of high-risk genetically defined dry AMD conducted to date.

Scheduled Clinical Assessments

- Medical History
- Visual Function Testing
- Anatomic Ocular Assessments
- Multi-Modal Ocular Imaging
- Quality of Life
- Ocular Fluid Biomarkers
Natural History: Genotyping Thousands and Multi-Year Follow-Up on Hundreds

On Site Baseline Visit
Subjects to be stratified into one of three cohorts

GEM-NH-001 (Registry)
C0: AMD-Related Variants

GEM-NH-002 (Natural History)
C1: High-risk CFH variants or compltotype
C2: CFH Common variants
Month 6  Month 18  Month 24

Year 2
All Subjects

Year 4
(All Subjects)

Year 6
All Subjects
End of Study Visit for NH-001

Remote Genetic Screening @ Scale (Thousands of Patients)
A New Class of Therapeutics Focused on Restoring Complement Regulation

1. Traditionally, complement therapeutics have focused on inhibiting the lectin, alternative, classical or terminal pathways.

2. In CFH-AMD patients, there are genetic variants that lead to complement dysregulation.

3. Our approach is to restore regulation of this essential biological system.

4. GEM103, our novel full-length rCFH candidate, is an intravitreally-administered investigational therapeutic currently in preclinical development.
Over 2 Years of R&D has Resulted in a Deep Multimodal Pipeline of First-in-Class Therapeutic Candidates

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<th>Target</th>
<th>Modality</th>
<th>Research</th>
<th>Development</th>
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<tr>
<td>CFH</td>
<td>GEM103 (CFH-NC-GA)</td>
<td>Preclinical Clinical</td>
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Thank You