HMR59 Gene Therapy for Macular Degeneration

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Hemera Biosciences

- Privately held Biotech located in Waltham, MA
- AAV2 Gene Therapy called HMR59
- Intravitreal injection
- Transgene Product is soluble form of CD59 (sCD59)
- Blocks complement at MAC
Therapeutic platform of HMR59 targets:

- **Dry AMD** (Phase 1 | fully enrolled | 6M Data)
- **Wet AMD** (Phase 1 | First Patient 9/2018)
- **Diabetic Eye disease** (Phase 1 Ready)
CD59 blocks MAC

In AMD CD59 and MAC

HMR59 increases sCD59
Evidence Supporting MAC in Dry AMD

- MAC identified in drusen\(^1\)
- MAC elevated in human eyes with AMD\(^2\)

\(^1\)Mullins, et al. FASEB 2000. 14:835
HMR59 Blocks MAC and CNV in Laser CNV Model

73.1% reduction in MAC formation (p=0.002)

62.6% reduction in CNV formation (p<0.001)
sCD59 in Ganglion Cell Layer at 6 Months

Stain of sCD59 after $1.78 \times 10^{11}$ vg intravitreal injection
sCD59 Measurements at 6 Months

**Vitreous**
- 6.2 µg/mL Right Vitreous;
- 7.4 µg/mL Left Vitreous

**Aqueous**
- 6.9 µg/mL Left Aqueous
Phase 1 Clinical Trial: HMR-1001

- 17 Patients Enrolled
- Advanced GA 5-20mm² (mean, 11.12mm²)
- 6-month study with 18-month safety follow-up
- MTD 3.56x10^{11} vg
- NO pretreatment with topical or oral prednisone
- Mild inflammation in 3 of 11 high dose eyes
- No HMR59 treated eyes converted to Wet AMD
- sCD59 measured in aqueous at Month 2
Mean Change in ETDRS VA from Baseline to Month 6

- Mean Observed (10 Matched Control Eyes)
- Mean 11 High Dose

ETDRS Letters
HMR59 Reduces GA Growth at 2 Time Points

**Hemera Biosciences INC.**

**Change Mean 9 High Dose Eyes (mm2)**

- Month 0: 0
- Month 3: 0.4533
- Month 6: 0.9278

**Natural History**

- Month 0: 0
- Month 3: 0.59
- Month 6: 1.18

P = 0.0379

GA Growth (mm2)

21.4%
HMR59 Reduces GA Growth vs. Opposite Eye

- Change Mean 5 High Dose Treated Eyes
- Change Mean 5 High Dose Matching Control

GA Growth (mm2)

Month 0: 0
Month 3: 0.5
Month 6: 1.082

HMR59 reduces GA growth by 8.0%.
HMR59 One-Time Injection vs. Monthly Apellis

- **APL Sham**
  - Day 0: 0.18
  - Month 3: 0.15
  - Month 6: 0.18

- **APL Monthly**
  - Day 0: 0.15
  - Month 3: 0.11
  - Month 6: 0.15

- **HMR59**
  - Day 0: 0.08
  - Month 3: 0.085
  - Month 6: 0.08
HMR-1001 Summary

- HMR59 high dose beneficial compared with natural history at 3 and 6 months
- HMR59 and monthly APL-2 have nearly identical results
- In the same individual HMR59 high dose eye has reduced GA growth compared with opposite untreated eye
- No conversion to Wet AMD
- Proceeding to Phase 2 Dry AMD HMR-2001
Intervention: Single Intravitreal HMR59 vs. Sham at Day 0
Study Population: GA 2.5-10 mm²
Primary Endpoint: Reduction in Growth of GA at 18 months
Secondary Endpoint: BCVA, Conversion to Wet AMD
Start: Q1 2019