OpRegen® Retinal Pigment Epithelium (RPE) Cell Therapy for Patients with Geographic Atrophy (GA): Month 36 Results from the Phase 1/2a Trial

Christopher Riemann, MD, *on behalf of OpRegen Phase 1/2a Study Investigators*
Cincinnati Eye Institute, University of Cincinnati, Cincinnati, OH, USA

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Disclosures

AI:ON Health – Consultant, Stock
Alimera / ANI – Speaker, Consultant
Allergan – Speaker, Consultant
Animal Eye Institute – Consultant
Aniridia Foundation International – Scientific Advisory Council Aniridia North America – Medical and Scientific Advisory Council
Aspicio Therapeutics – Consultant, Stock
Bausch & Lomb – Speaker, Consultant
BMC/Eyetube – Consultant
CSTLII – Speaker
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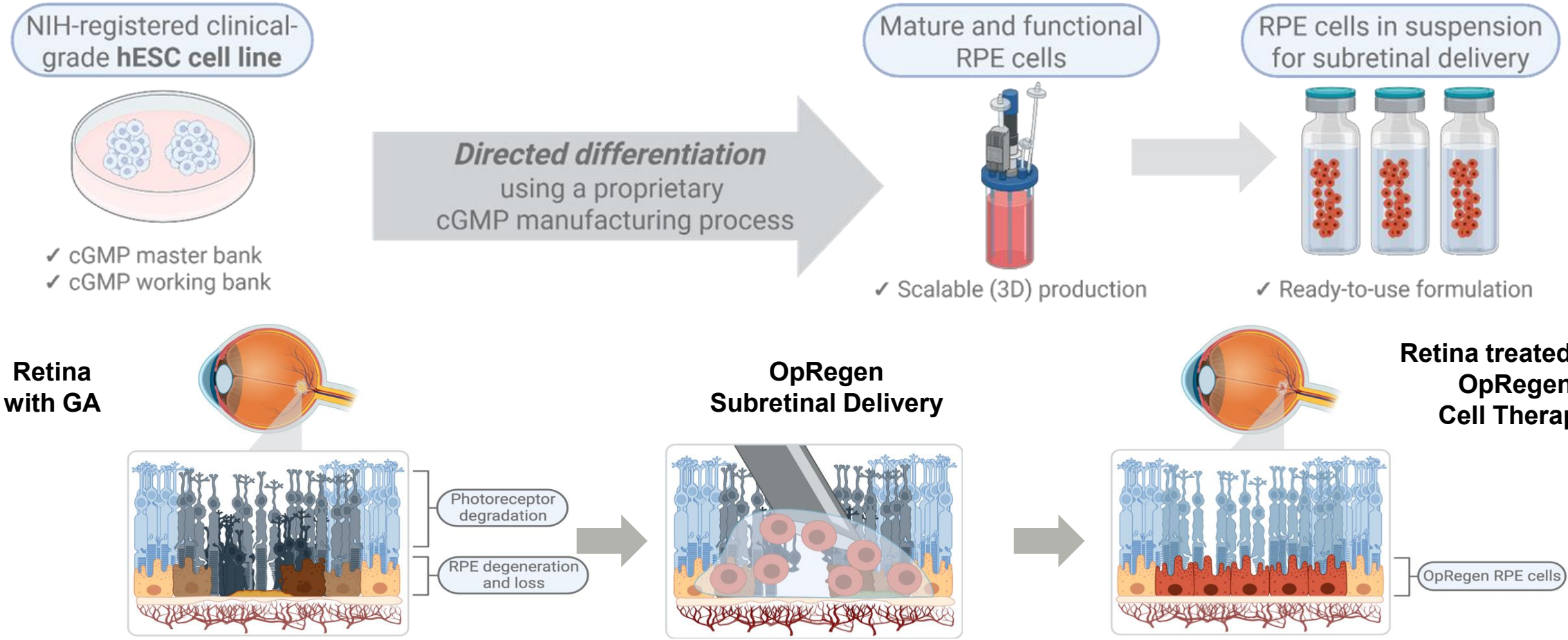
D.O.R.C. – Consultant
ForwardVue Pharma – Board of Directors, Stock
Genentech / Roche – Consultant
Gore – Consultant
Gyroscope & Orbit BioMedical – Consultant, I.P.
Haag Streit AG & Haag Streit Surgical – Consultant
Haag Streit USA – Consultant, Speaker, I.P.
HumanOptics AG – Consultant
Iamc2 – Consultant, Cofounder, I.P.
iVeena – Investor, Stock, Consultant
IvericBio/Astellis – Speaker, Consultant
Janssen / Johnson & Johnson – Consultant, I.P.
Kaleidoscope Engineering – Consultant, I.P.
Lineage / BioTime – Consultant
MedOne – Consultant, Royalties, I.P.
Neuracle – Consultant, Acting Medical Director
Northmark Pharmacy – Ownership
Novartis – Consultant
Oculinea – Investor, Stock
Regeneron – Speaker
Reliance Industries – Speaker, Honoraria, Consultant, I.P.
TrueVision - Consultant, Speaker, Royalties, Stock Options
VEO – Owner / Cofounder
VitaPixel – Owner / Cofounder
Vortex Surgical – Stock, Consultant, Royalties , I.P.
Zeiss – Consultant

Monies for research: AGTC, Alcon, Alimera, Allergan, Arepio, BioTime / Lineage, Chengdu Kanghong, Clearside, Genentech/Roche, Gyroscope, Lineage/BiotimeJanssen / Johnson & Johnson, Lowry-MacTel Registry, Neurotech, Nightstar/Biogen, NotalVision, Novartis, Ophthotec/Iveric, Regeneron, RegenexBio, Spark

OpRegen Cell Therapy – A Suspension of Allogeneic RPE Cells

With the Potential to Counteract RPE Cell Dysfunction & Loss in GA



cGMP, current Good Manufacturing Practice; hESC, human embryonic stem cell.
NIH registry for hESC cell line HAD-C 102 available at https://grants.nih.gov/stem_cells/registry/current.htm?id=428. Figures created with BioRender.com.

Phase I/IIa Study Design (NCT02286089; active)
An Open-Label, Single-Arm, Multi-Center, Dose-Escalation Trial

Key Enrollment Criteria

Patients with bilateral GA secondary to AMD

Cohorts 1-3 (n=12):

- Legally blind (BCVA: ≤20/200)
- GA area: 1.25–17 mm^2

Cohort 4 (n=12):

- **Impaired vision (BCVA: ≥20/250 and ≤20/64)**
- **GA area: ≥4 and ≤11 mm^2**

Single OpRegen Administration

- **Cohort 1 (n=3)** 50,000 cells
- **Cohort 2 (n=3)** Up to 200,000 cells
- **Cohort 3 (n=6)** Up to 200,000 cells
- **Cohort 4 (n=12)** Up to 200,000 cells

Subretinal Delivery

- Vitrectomy/retinotomy (n=5)
- Suprachoroidal cannula using Orbit® SDS (Gyroscope Therapeutics) in Cohort 4 only (n=7)

Perioperative Immunosuppressive Regimen
Tacrolimus 0.01 mg/kg daily up to 6 weeks after surgery
Mycophenolate up to 2.0 g daily at least 3 months after surgery

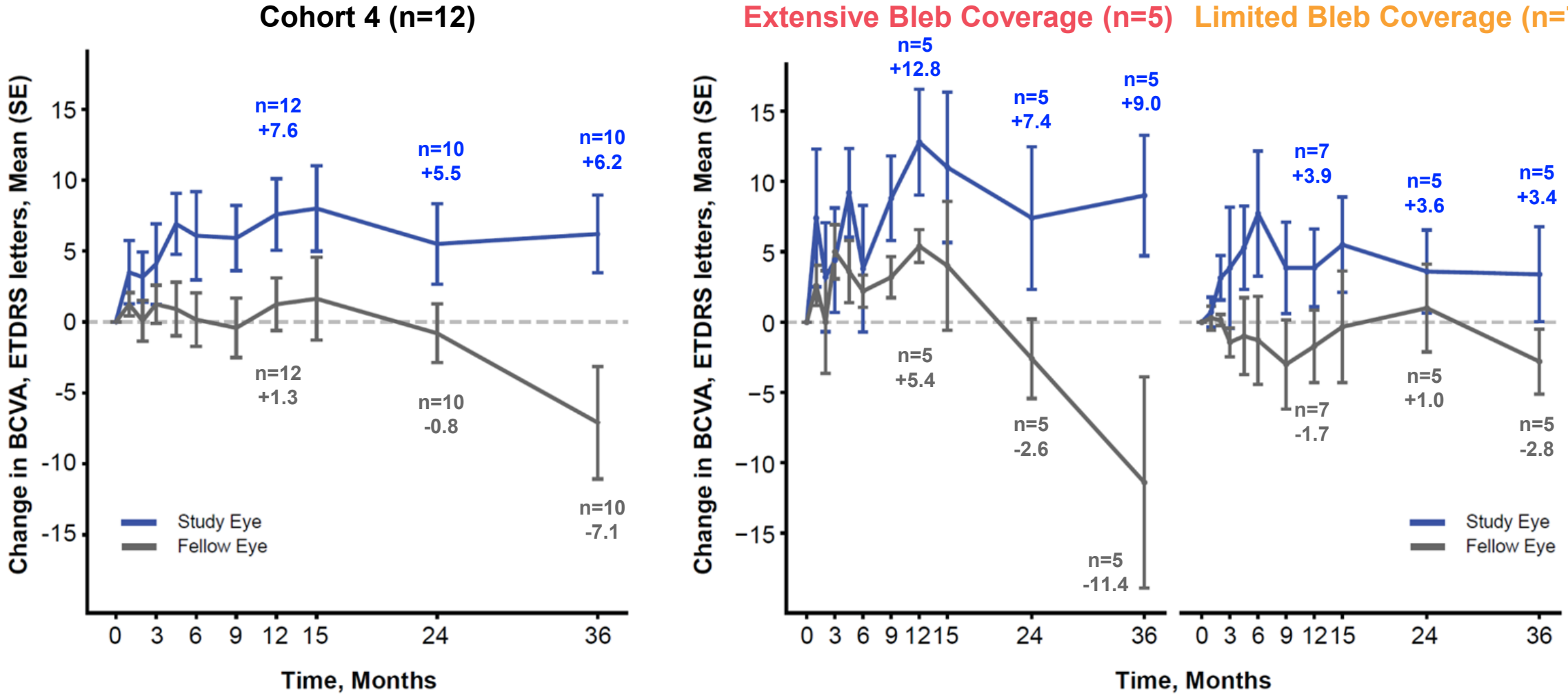
Primary Outcome at 12 Months: Safety/Tolerability

OpRegen cell therapy was generally well-tolerated with no cases of acute or delayed intraocular inflammation or tumor formation[a]

[a]Safety data previously presented (Ho AC et al. ARVO 2022. https://iovs.arvojournals.org/article.aspx?articleid=2780049); NCT02286089
AMD, age-related macular degeneration; BCVA, best corrected visual acuity; GA, geographic atrophy; SDS, subretinal delivery system.

Study Eyes of Cohort 4 Patients (Less Advanced GA) Show Sustained BCVA Gains Through Month 36

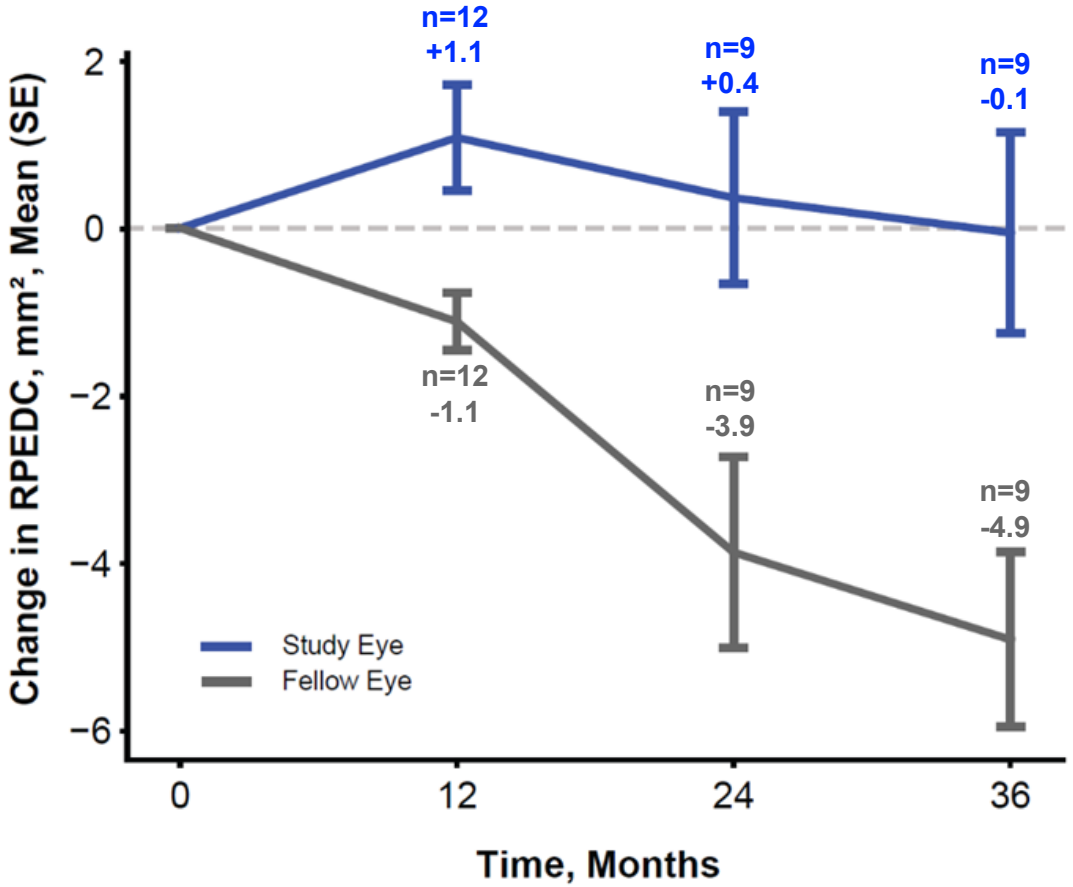
BCVA Gains are Greater Among Eyes with Extensive Coverage of GA by the Surgical Bleb



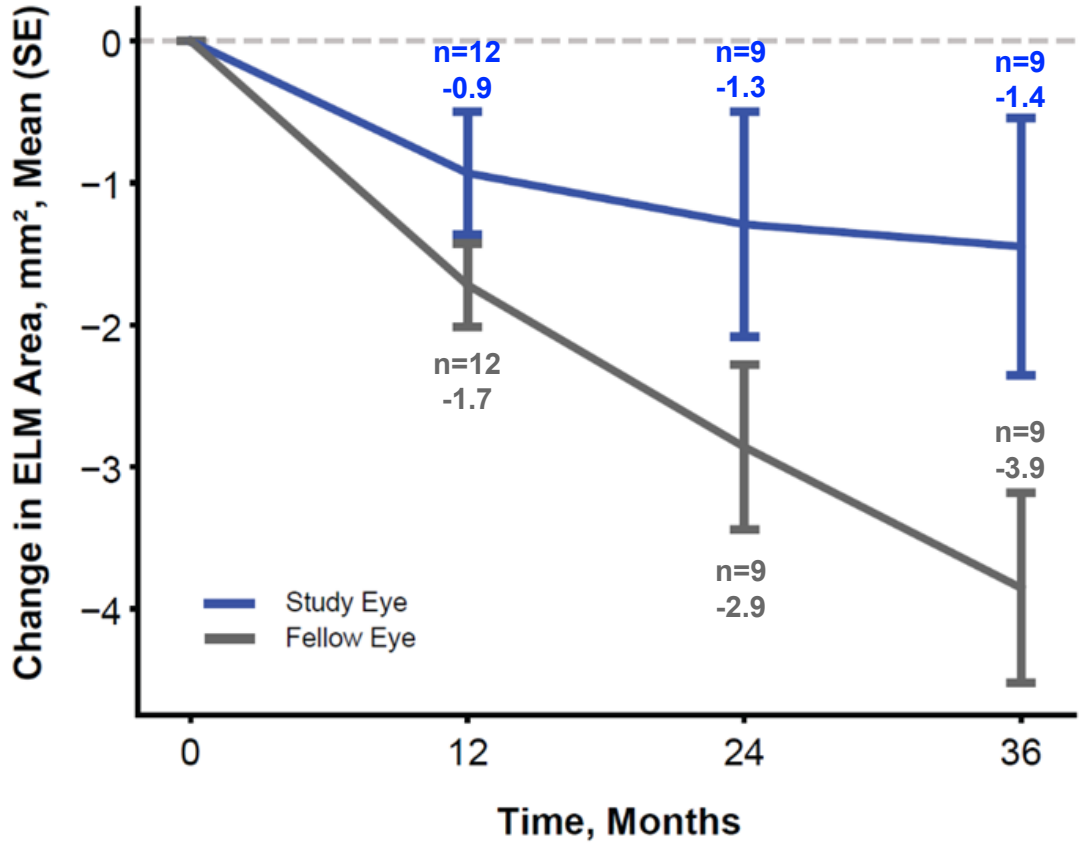
ETDRS, Early Treatment Diabetic Retinopathy Study.

Study Eyes of Cohort 4 Patients (Less Advanced GA) Show Sustained Evidence of Retinal Structural Support by Quantitative OCT Analysis Through Month 36

RPE - Drusen Complex (RPEDC)
Cohort 4 (n=12)



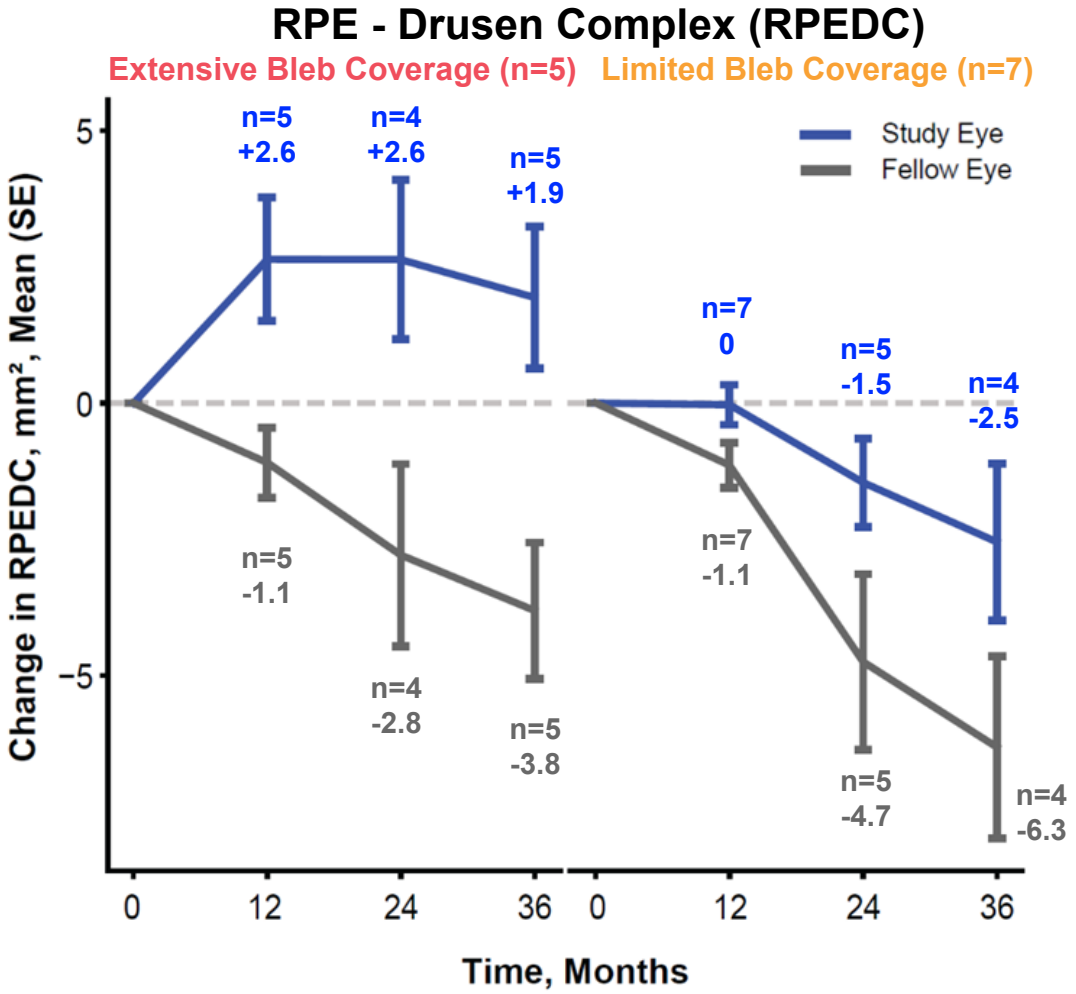
External Limiting Membrane (ELM)
Cohort 4 (N=12)

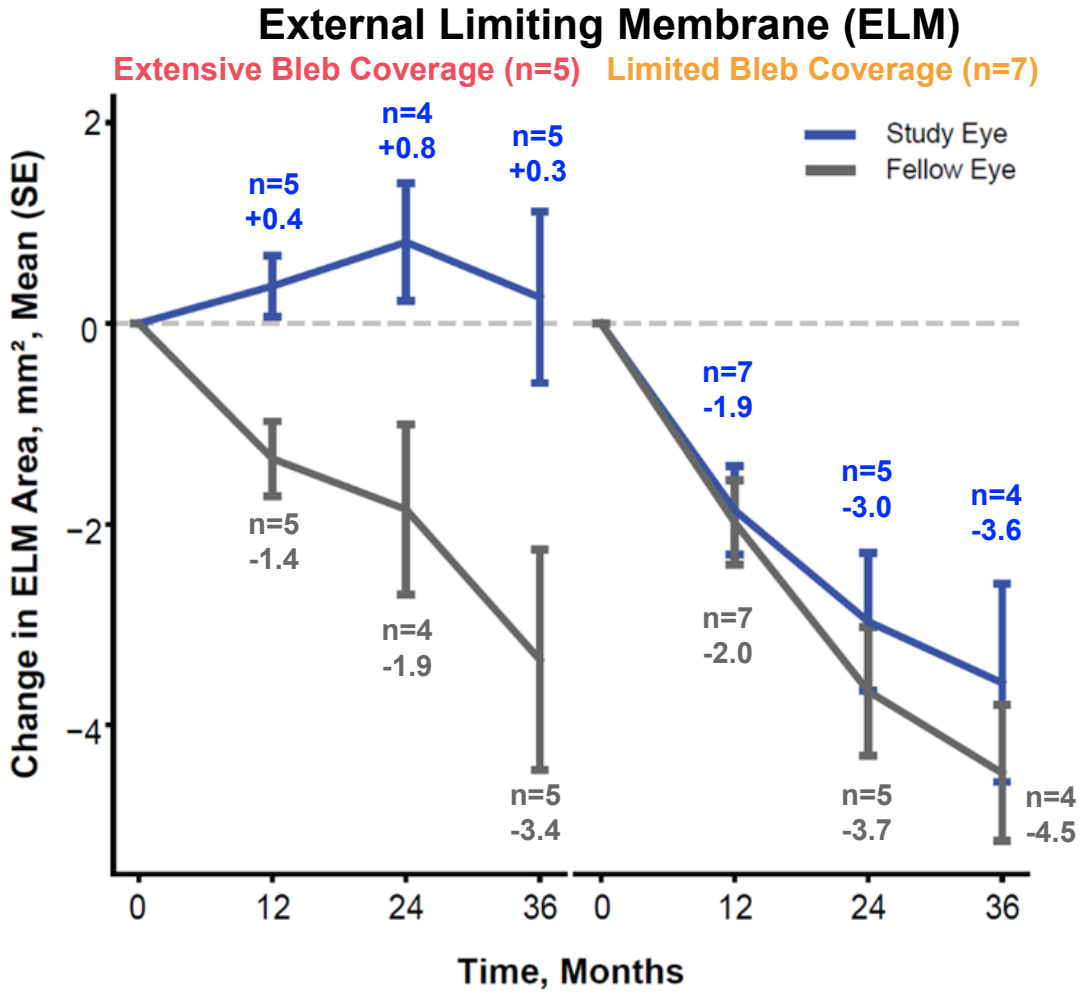


Segmentation result is generated by Genentech EyeNotate OCT segmentation algorithm, reviewed and corrected by a single masked expert grader.

Study Eyes of Cohort 4 Patients (Less Advanced GA) Show Sustained Evidence of Retinal Structural Support through Month 36

Support is Most Evident Among Eyes with Extensive Coverage of GA by the Surgical Bleb





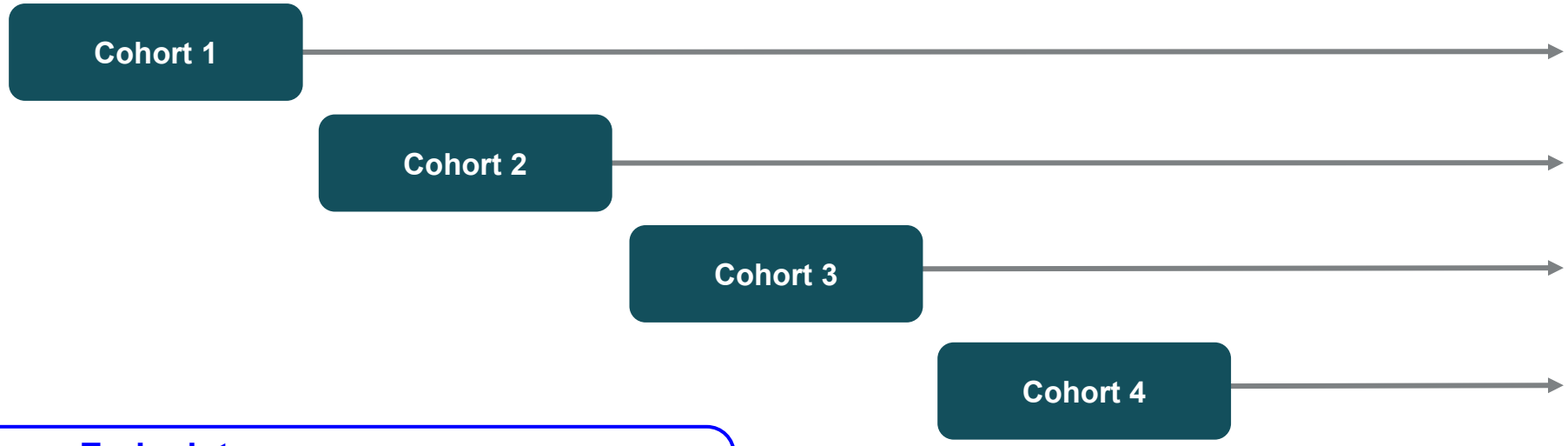
Segmentation result is generated by Genentech EyeNotate OCT segmentation algorithm, reviewed and corrected by a single masked expert grader.

Galette: A Phase 2a Multicenter, Open-Label, Single-Arm Study to Optimize the Subretinal Delivery of Opregen Cell Therapy In GA (NCT05626114; [Recruiting])[1]

Key Enrollment Criteria

- Age ≥50 years
- Diagnosis of GA secondary to AMD
- BCVA score ≥29 letters and ≤60 letters in the study eye as assessed by ETDRS
- Pseudophakic (study eye)
- Ability to undergo vitreoretinal surgical procedure under sedation or general anesthesia

Up to ~60 patients
3-10 patients per cohort
Up to 200,000 cells



Primary Endpoints

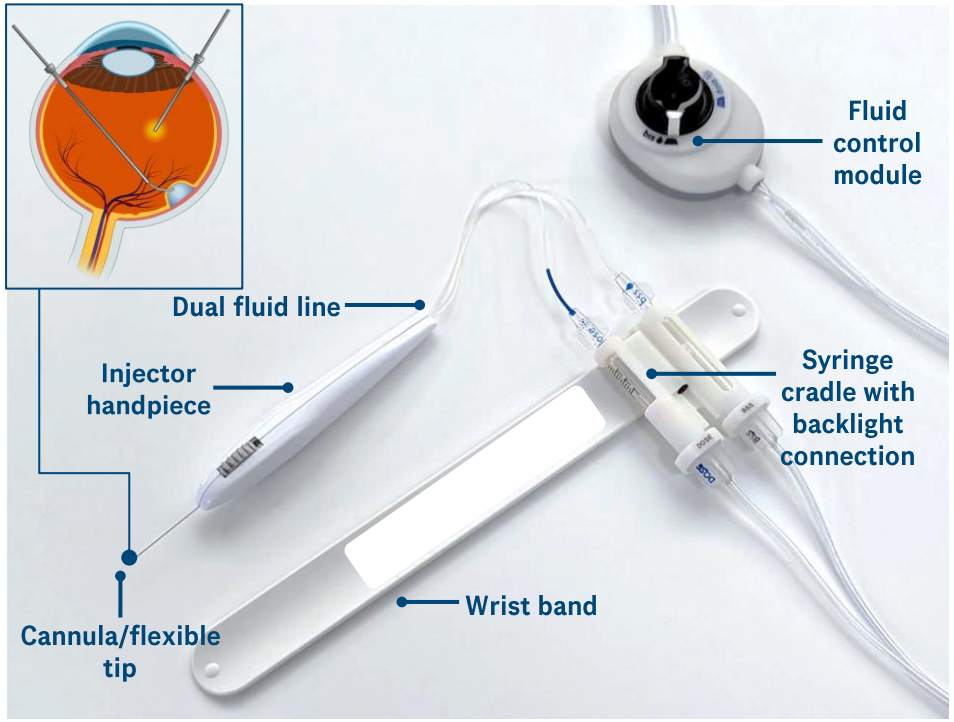
- Success of OpRegen subretinal surgical delivery to target GA regions
- Safety of OpRegen surgical delivery at 3 months

ClinicalTrials.gov Identifier: NCT05626114. Accessed 05/2025 from https://clinicaltrials.gov/study/NCT05626114.

Advanced Subretinal Delivery Devices are Under Development for Evaluation with OpRegen Cell Therapy

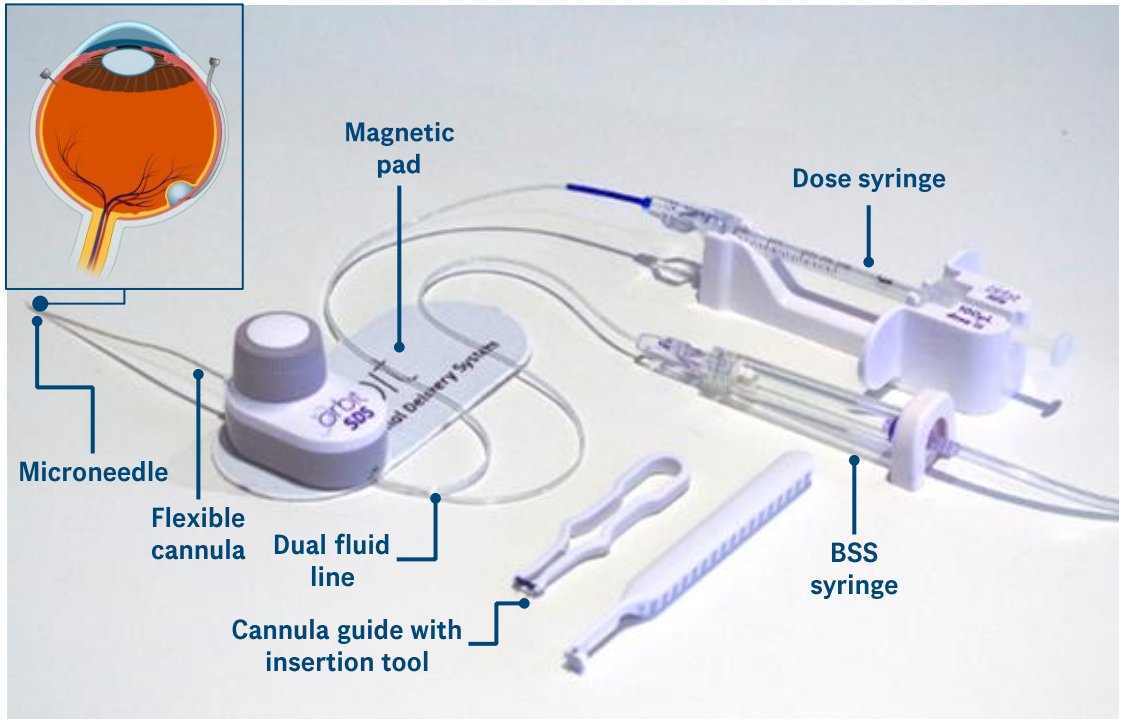
Transvitreal Subretinal Injector (TVSI) with Dual Lumen

Allows delivery of two infusates via a single insertion/retinotomy



Orbit SDS®
Next Generation

Subretinal delivery via transchoroidal approach removes the need for vitrectomy and retinotomy



Conclusions

- Gains in BCVA in patients in Cohort 4 (less advanced GA) measured at Month 12 remain evident through Month 36 following subretinal administration of OpRegen cell therapy
- Improvement in BCVA and outer retinal structure in patients with extensive OpRegen bleb coverage of their GA area was greater than in patients with limited coverage and persisted through Month 36
- These data suggest that OpRegen cell therapy may counteract RPE cell dysfunction and loss in GA by providing support to remaining retinal cells in ways that bear further study, and these effects appear durable through at least 36 months after a single administration
- The Phase IIa GAlette study evaluating the success of subretinal delivery of OpRegen cell therapy to target areas of GA is currently enrolling (NCT05626114)
  - In addition to evaluating other surgical parameters, this study will test proprietary surgical devices in development for subretinal delivery of OpRegen cell therapy with potential advantages over currently available devices and procedures

Ph1/2a Study Investigators, Sites, and Support

Investigators

- Eyal Banin, Hadassah-Hebrew University Medical Center, Jerusalem, Israel
- Adiel Barak, Sourasky Medical Center, Tel Aviv, Israel
- David Boyer, Retina Vitreous Associates Medical Group Los Angeles, CA, USA
- Allen Ho, Mid Atlantic Retina, Philadelphia, PA, USA
- Rita Ehrlich, Rabin Medical Center, Petah Tikva, Israel
- Tareq Jaouni, Hadassah-Hebrew University Medical Center, Jerusalem, Israel
- Richard McDonald, West Coast Retina Group, San Francisco, CA, USA
- Christopher D. Riemann, CEI, Cincinnati, OH, USA
- David Telander, Retinal Consultants Medical Group, Sacramento, CA, USA

Additional Contributors

- Benjamin Reubinoff, Hadassah-Hebrew University Medical Center, Jerusalem, Israel
- Miao Zhang, Genentech, Inc., South San Francisco, CA, USA
- Simon S. Gao, Genentech, Inc., South San Francisco, CA, USA
- Katie M. Litts, Genentech, Inc., South San Francisco, CA, USA
- Ling Ma, Genentech, Inc., South San Francisco, CA, USA
- Gary S. Hogge,* Lineage Cell Therapeutics, Inc., Carlsbad, CA, USA
- Avi Ben-Shabat, Lineage Cell Therapeutics, Inc. (Cell Cure Neurosciences, Ltd.), Jerusalem, Israel

Trial Conduct

- Central Reading Center: Merit CRO (EyeKor), Madison, WI, USA
- Duke Ohashi, Lineage Therapeutics, Inc., Carlsbad, CA, USA
- Yumiko Kawai, Lineage Therapeutics, Inc., Carlsbad, CA, USA
- Jessica Hallinan, Lineage Therapeutics, Inc., Carlsbad, CA, USA
- Joyce Velez, Lineage Therapeutics, Inc., Carlsbad, CA, USA
- Diana Angelini, Lineage Therapeutics, Inc., Carlsbad, CA, USA
- Yana Aisen, Lineage Therapeutics, Inc., Carlsbad, CA, USA

*Former Lineage employee; Gary Hogge contributed to this work while at Lineage Cell Therapeutics, Inc.