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OPGx-BEST1 Gene Therapy Phase 1/2 Study Low Dose Cohort 1 3-month Results

September 9, 2026

Disclosures and Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases, you can identify forward-looking statements by the following words: “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “aim,” “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. Such statements include, but are not limited to, statements related to our continued clinical development, clinical results, preclinical data, and future plans for OPGx-BEST1, including the anticipated timing of dosing completion and topline data from Cohort 2 of the OPGx-BEST1 Phase 1/2 clinical trial; the outcome of our ongoing regulatory interactions with the U.S. Food and Drug Administration (the “FDA”) and our expectations regarding the design of, and potential endpoints for, of any pivotal clinical trial of OPGx-BEST1; our expectations regarding the clinical and therapeutic potential of OPGx-BEST1, including with respect to its ability to improve visual function and retinal structure in patients with BEST1-related retinal disease; our estimates of the BEST1 symptomatic patient population in the U.S. and globally; and our expectations regarding our company, its business prospects, and our results of operations. These forward-looking statements are subject to certain risks and uncertainties posed by many factors and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to: our clinical data related to gene therapies for the treatment of inherited retinal diseases is preliminary and related to a relatively small group of patients, and, as a result, data that initially appears promising may be revised, updated, or invalidated at a later data readout and/or may ultimately not be capable of duplication in additional patients; our gene therapy product candidates are based on a novel technology that is difficult to develop and manufacture, which may result in delays and difficulties in obtaining regulatory approval; our planned clinical trials may face substantial delays, result in failure, or provide inconclusive or adverse results that may not satisfy the FDA requirements to further develop our therapeutic products; delays or difficulties associated with patient enrollment in clinical trials may affect our ability to conduct and complete those clinical trials and obtain necessary regulatory approvals; changes in regulatory requirements could result in increased costs or delays in development timelines; we depend heavily on the success of our product pipeline; if we fail to find strategic partners or fail to adequately develop or commercialize our pipeline products, our business will be materially harmed; we have not generated significant revenue from sales of any products and expect to incur losses for the foreseeable future; our future viability is difficult to assess due to our short operating history and our future need for substantial additional capital, access to which could be limited by any adverse developments that affect the financial services markets; we rely on third parties for material aspects of our business, such as conducting our nonclinical and clinical trials and supplying and manufacturing bulk drug substances, which exposes us to certain risks; and those risks and uncertainties described under the heading “Risk Factors” included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2025 and our subsequent filings with the U.S. Securities and Exchange Commission (the “SEC”). Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this presentation. We undertake no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise. These forward-looking statements are based upon our current expectations and involve assumptions that may never materialize or may prove to be incorrect.

Management Team and KOL Participant

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OPGx-BEST1 Program and Low-Dose Cohort 1 Clinical Data Highlights

OPGx-BEST1 Cohort 1 & Program Summary

- Well-tolerated with no SAEs, no DLTs, and no intraocular inflammation
- Structural and functional improvements at 3 months
- **FDA meeting aligned on potential pivotal endpoint**
  - ≥3 dB microperimetry improvement in ≥5 prespecified loci
  - In conjunction with a RCT using the PGI-S
- Phase 3 and commercial manufacturing on schedule for delivery in early 2027
- Cohort 2 overenrolled at 8 participants, weighted towards earlier stage BVMD
  - 6-month Low-Dose Cohort 1 and 3-month High-Dose Cohort 2 data expected in Q2 2027
- **Pivotal trial to commence dosing in 2027**
- New epidemiology report surveying 150 eye care professionals estimates 23,600 *BEST1* patients in the U.S.

TODAY'S TOPICS

01 Opus & BEST1 Overview

02 Trial Design & Topline Results

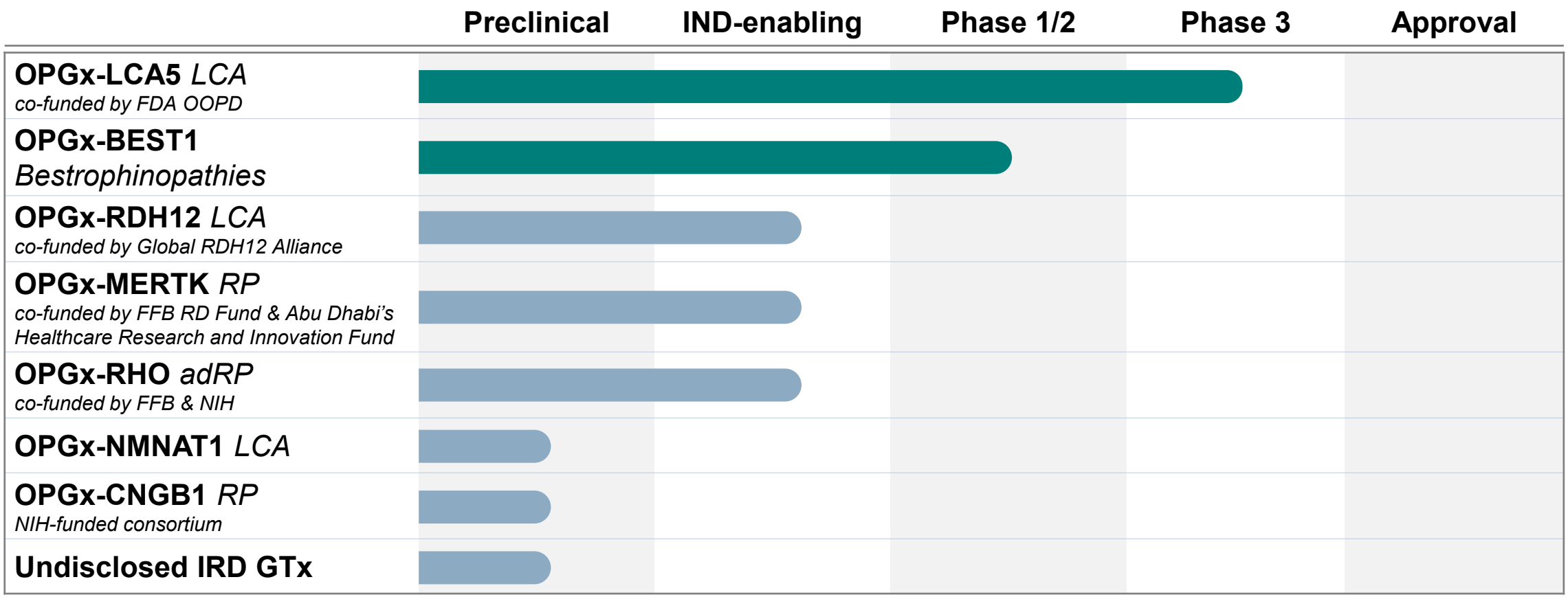
03 Participant Case Studies

04 Program Summary & Next Steps

75% of evaluable participants met the FDA-aligned microperimetry of ≥3 dB improvement in ≥5 prespecified loci

BEST1, bestrophin 1; BVMD, best vitelliform macular dystrophy; dB, decibel; DLT, dose-limiting toxicity; FDA, Food and Drug Administration; PGI-S, patient global impression-severity; RCT, randomized controlled trial; SAE, serious adverse event.

Building a Differentiated Gene Therapy Platform



All gene therapy programs have the potential to qualify for a Priority Review Voucher

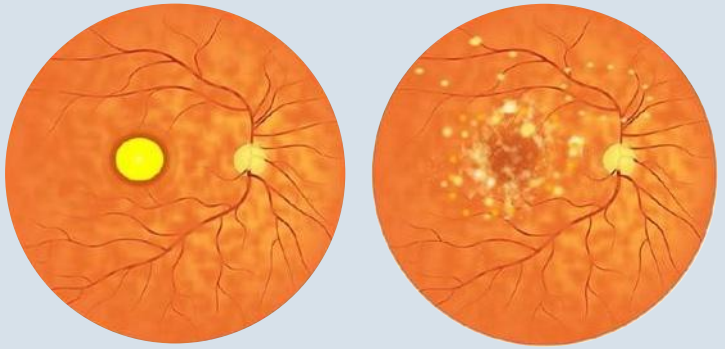
Opus Genetics owns worldwide rights to all gene therapy programs.

adRP, autosomal dominant retinitis pigmentosa; BEST1, bestrophin 1; CNGB1, cyclic nucleotide-gated channel β1; FDA OOPD, Food and Drug Administration Office of Orphan Products Development; FFB, Foundation Fighting Blindness; GTx, gene therapy; LCA5, Leber congenital amaurosis 5; NIH, National Institutes of Health; RD, retinal degeneration; RDH12, retinol dehydrogenase 12; RHO, rhodopsin; RP, retinitis pigmentosa; MERTK, MER proto-oncogene tyrosine kinase; NMNAT1, nicotinamide mononucleotide adenylyltransferase.

BEST1: Group of Inherited Retinal Diseases with a Range of Onset and Slow Rate of Progression and no Approved Treatment Options

Overview & Prevalence	• Mutations in *BEST1* have been associated with at least five clinically distinct retinal degenerative diseases, with onset from childhood to adulthood[1] • Accounts for ~3.5% of all IRDs[1] • **Global prevalence*: ~45,400** patients[2] • **U.S. prevalence: 23,600** patients (**~23,200** BVMD and ~400 ARB)[2]
Clinical Features[1,3]	• Serous retinal detachment • BVMD (most common) is characterized by vitelliform (“egg-yolk”) lesion beneath the macula[1] • Macular atrophy • CNV
Symptoms[3,4]	• Loss of central vision • Metamorphopsia (distorted vision) • Scotoma (blind spot) • Photophobia[4]

Most Common BEST1 Phenotypes



BVMD ARB

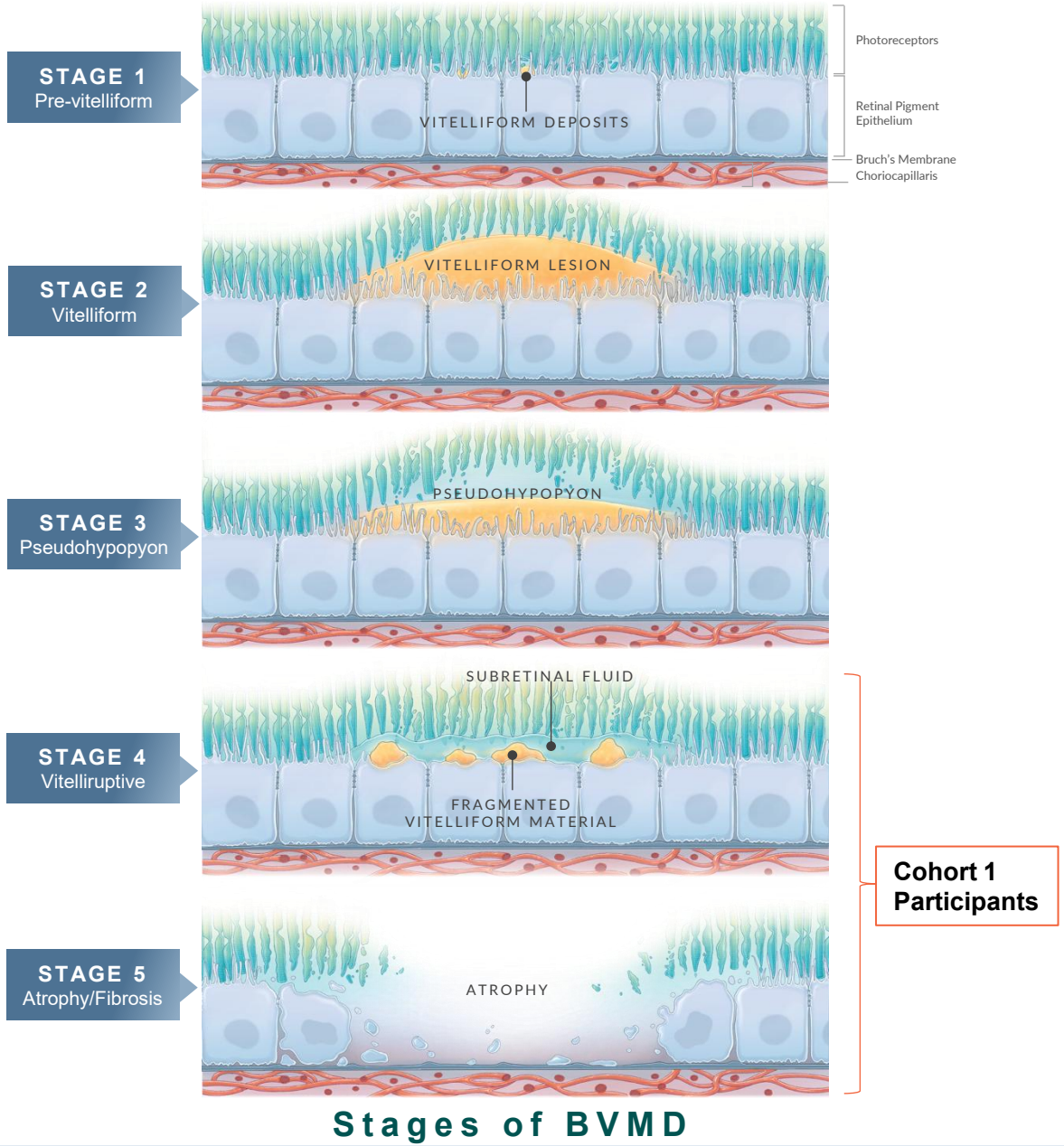
Prevalence	
BVMD 23,200 (98%)[2]	ARB 400 (2%)[2]

*Global prevalence estimate includes United States, EU4 (France, Spain, Germany, & Italy), UK, Middle East/North Africa, and China.
ARB, autosomal recessive bestrophinopathy; BEST1, bestrophin 1; BVMD, best vitelliform macular dystrophy; CNV, choroidal neovascularization; IRD, inherited retinal disease.
1. Amato A, et al. *Saudi J Ophthalmol.* 2023;37(4):287-295. 2. BEST1 Market Landscape Quantitative Market Research, Triangle Insights Group, Q3 2026. 3. Johnson AA, et al. *Prog Retin Eye Res.* 2017;58:45-69.
4. Tripathy K, et al. StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2024.

BEST1 Disease Biology

- ***BEST1* gene encodes for bestrophin-1**, a homopentameric (i.e., 5 identical monomers) Ca^{2+}-activated chloride channel required for RPE maintenance and retinal physiology
- ***BEST1* mutations in BVMD disrupt cellular ion and fluid homeostasis** resulting in electrophysiological abnormalities, RPE dysfunction, and retinal degeneration via:
  - Defective **clearance of toxic waste** products (e.g., lipid deposits)
  - **Impaired RPE-photoreceptor interactions** (e.g., defective phagocytosis)
  - **Build-up of vitelliform material** (toxic waste products) and fluid under the retina

- Vitelliform material occurs early and is the hallmark of BVMD
- Subretinal fluid occurs late in disease and pools in atrophic areas



Stages of BVMD

BEST1, bestrophin 1; BVMD, best vitelliform macular dystrophy; RPE, retinal pigment epithelium.
Guziewicz KE, et al. *Prog Retin Eye Res.* 2017;58:70-88.

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Trial Design & Topline Cohort 1 Results

Sally Tucker, MCOptom, PhD
Chief Medical Officer

OPGx-BEST1: Phase 1/2 Study Overview (BIRD-1)

Design	Adaptive, open-label, dose-exploration, safety and tolerability study of subretinal injection of OPGx-BEST1 in adult participants with BVMD or ARB
Dosing Cohorts	1. **Cohort 1: 1.5x10^9 vg/eye** 2. Cohort 2: 4.5x10^9 vg/eye
Objectives	• **Primary:** Safety and tolerability; identify appropriate dose for Phase 3 • **Secondary:** Efficacy
Study Population	• Minimum of 5 participants at each dose level
Status of Cohort 1 (low dose)	5 participants dosed: • ARB (n=2): Data at 6 months • BVMD (n=3): Data at 3 months
Status of Cohort 2 (high dose)	• Enrollment complete with 8 participants (6 surgeries scheduled) • Dosing expected to be completed in Q4 2026

ARB, autosomal recessive bestrophinopathy; BVMD, best vitelliform macular dystrophy.

Participant Demographics: Low-Dose Cohort 1

	101-101	101-104	102-101	102-102	101-106
Age	63	59	50	45	31
Sex	Female	Female	Male	Male	Male
BEST phenotype	**ARB**	**ARB**	**BVMD**	**BVMD**	**BVMD**
Baseline VA* (study eye†)	1	50	49	61	47
Baseline VA* (fellow eye)	43	65	68	72	56
Follow-up duration	6 months	6 months	3 months	3 months	3 months
Severity of disease	End-stage with significant atrophy	End-stage with significant atrophy	End-stage with sub-foveal, sub-RPE scar	Advanced stage vitello-eruptive with some scarring	Earlier stage without significant atrophy or scarring

*ETDRS letters equivalent calculated from logMAR.
†Worse eye deemed study eye.
ARB, autosomal recessive bestrophinopathy; BEST, bestrophin; BVMD, best vitelliform macular dystrophy; ETDRS, Early Treatment Diabetic Retinopathy Study; RPE, retinal pigment epithelium; VA, visual acuity.

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Study Endpoints

Microperimetry	• Measures pointwise sensitivity of the retina over the lesion • Recent natural history data shows a steady decline over 5 years in BVMD, with most of the decline in the area on the edge of the lesion[1]
BCVA & LLVA	• Methods to measure central fine visual function
Contrast Sensitivity	• Measures visual function and is sensitive in patients with central atrophy (used extensively in geographic atrophy studies)
Autofluorescence	• A 2-dimensional picture of the retina highlighting vitelliform lesions
OCT	• A cross-sectional view of the retina

BCVA, best-corrected visual acuity; BVMD, best vitelliform macular dystrophy; LLVA, low-luminance visual acuity; OCT, optical coherence tomography.

1. Bianco L, et al. *Invest Ophthalmol Vis Sci.* 2026;67(11):1.

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Safety and Efficacy Summary: Highly Encouraging Proof-of-Concept Results from Low-Dose Cohort 1

Safety	• **OPGx-BEST1 was well-tolerated in 5/5 participants,** with no SAEs or DLTs

Structural Endpoints	• **80% of participants (4/5) had structural improvements**:
	  • BVMD: 67% of participants (2/3) had a decrease in vitelliform material
	  • ARB: 100% of participants (2/2) had a decrease in intraretinal fluid

Functional Endpoints	• **100% of participants (5/5) had improvements*** in at least one functional measure:
	  • Microperimetry – 75% of participants (3/4) improved
	  • BCVA – 60% of participants (3/5) improved
	  • LLVA – 40% of participants (2/5) improved
	  • Contrast Sensitivity – 40% of participants (2/5) improved
	• **Earlier-stage participant (101-106)† showed the biggest functional gains,** suggesting a potential benefit from earlier treatment

*Improvements defined as: >5 letters of improvement from baseline and >5 letter improvement from fellow eye in BCVA or LLVA, 0.2 logMAR improvement in contrast sensitivity, and ≥5 loci improving by 3 or more decibels in RPE transitional zone on microperimetry.

†101-106 was the youngest participant with most recent onset of disease and less progressive disease than other participants.

ARB, autosomal recessive bestrophinopathy; BCVA, best-corrected visual acuity; BVMD, best vitelliform macular dystrophy; DLT, dose-limiting toxicity; LLVA, low-luminance visual acuity; logMAR, logarithm of the minimum angle of resolution; RPE, retinal pigment epithelium; SAE, serious adverse event.

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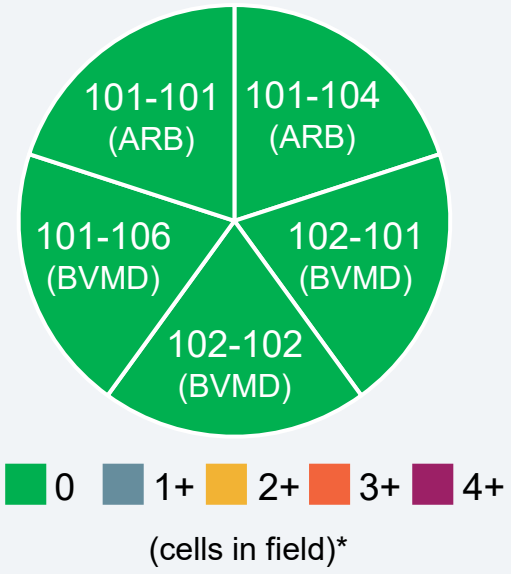
OPGx-BEST1 Demonstrated a Favorable Safety Profile

- Well-tolerated in 100% of participants
  - No intraocular inflammation
  - No serious adverse events
  - No dose-limiting toxicities
  - No treatment-related systemic AEs
  - All ocular treatment-related AEs were mild/moderate in severity
  - No vital sign issues or safety lab findings of note

Independent Data Monitoring Committee recommended the Phase 1/2 Trial advance to Cohort 2 at higher dose

No intraocular inflammation was observed in any patient at any study visit



*Based on the SUN Working Group Grading Scheme.
AE, adverse event.

Participant-Level Improvement by Endpoint*

Endpoint	101-101 ARB	101-104 ARB	102-101 BVMD	102-102 BVMD	101-106 BVMD
BCVA ≥5 letter improvement compared to baseline and fellow eye	✓			✓	✓
LLVA ≥5 letter improvement compared to baseline and fellow eye	✓				✓
Contrast Sensitivity ≥0.2 logMAR improvement from baseline	✓		✓		
Microperimetry ≥5 loci cluster improvement ≥3 dB	NE	✓		✓	✓
OCT IRF / vitelliform material reduced	✓	✓		✓	✓

✓ Improvement

NE: Not evaluable

*Improvement defined as: ≥5 letters of improvement from baseline in BCVA or LLVA and ≥5 letter improvement compared to fellow eye, ≥0.2 logMAR improvement from baseline in contrast sensitivity, and ≥5 loci cluster improvement by ≥3 decibels in Transitional Zone on microperimetry.

ARB, autosomal recessive bestrophinopathy; BCVA, best corrected visual acuity; BVMD, best vitelliform macular dystrophy; dB, decibel; IRF, intraretinal fluid; LLVA, low luminance visual acuity; logMAR, logarithm of the minimum angle of resolution; OCT, optical coherence tomography.

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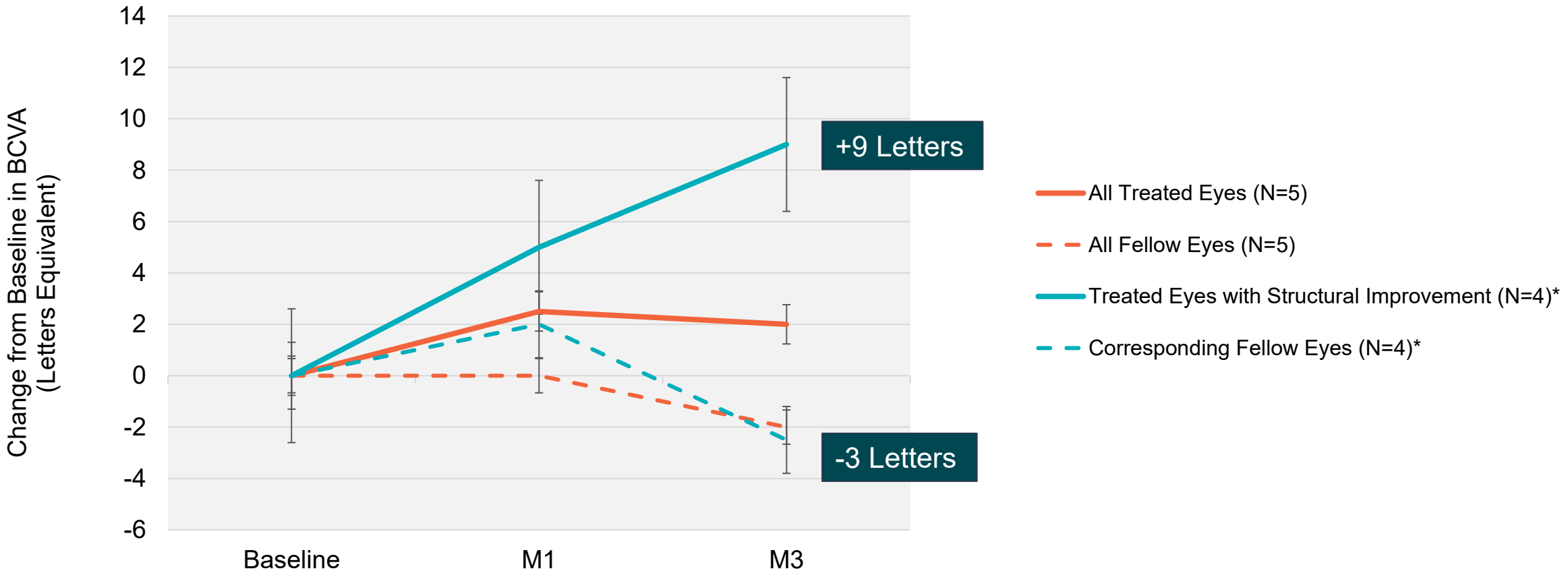
Mean BCVA Improved from Baseline Through 3 Months in Cohort 1



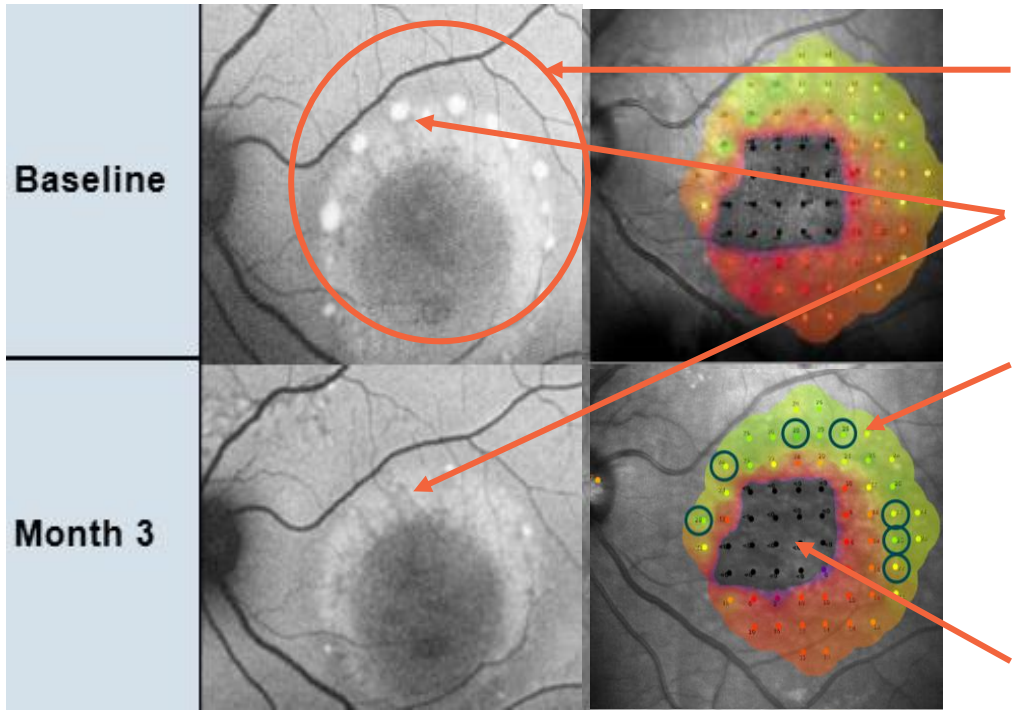
*Excludes Participant 102-101 due to significant foveal atrophy (with a subfoveal, sub-RPE scar) at baseline, likely exclusionary from the pivotal trial. Participant 102-101 had a 0.2 logMAR decrease in vision from baseline, all other participants had an improvement in vision; Participant 102-101 had a meaningful improvement in contrast sensitivity.
Error bars represent the standard error of the mean.
BCVA, best corrected visual acuity; logMAR, logarithm of the minimum angle of resolution; M, month; RPE, retinal pigment epithelium.

Microperimetry Showed BVMD Improvement in the Treated RPE Transitional Zone

Defining the Transitional Zone

- Border area surrounding the atrophic lesion with RPE and photoreceptors that are structurally intact but functionally compromised or at risk of imminent atrophic progression
- Highest potential to be rescued with OPGx-BEST1
- **Treated RPE Transitional Zone** is the area of rescuable photoreceptors around the atrophic lesion where OPGx-BEST1 treatment is administered
- **New natural history data[1]:** Untreated BVMD sensitivity only declines with highest rate detected in the transition zone – No untreated eye met the 3 dB improvement threshold, consistent with fellow eyes in Cohort 1

75% 3/4 evaluable participants* demonstrated **retinal sensitivity improvement†** in the Transitional Zone

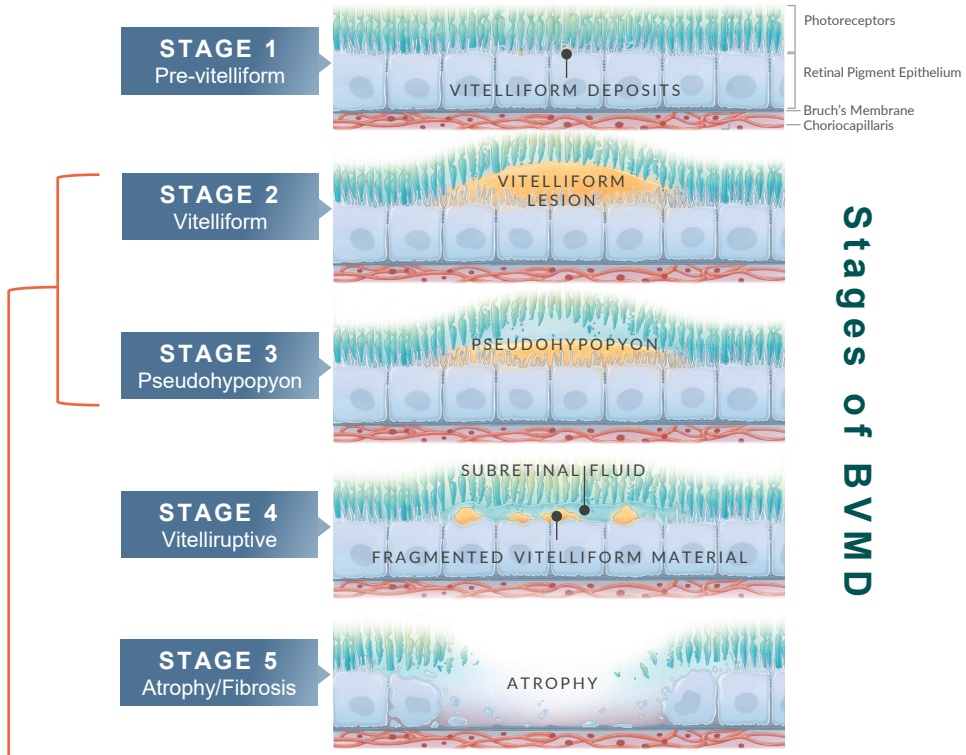


FDA aligned on ≥3 dB change from baseline in ≥5 loci in treated RPE Transitional Zone anchored to a patient reported outcome

*All eyes includes all evaluable patients (n=4); 101-101 could not complete microperimetry due to low vision.
†Improvement is defined as ≥5 loci area within the treated transitional zone improving by ≥3 dB from baseline.
1. Bianco L, et al. *Invest Ophthalmol Vis Sci.* 2026;67(11):1.
BVMD, best vitelliform macular dystrophy; dB, decibel; FDA, Food and Drug Administration; RPE, retinal pigment epithelium.

Structural Improvements Seen in BVMD and ARB Participants

- **BVMD: 67% of participants (2/3) had reduction in vitelliform material on multimodal imaging**
  - All three BVMD participants had improvements in visual function, with highest gains in areas of vitelliform material reduction
- **ARB: 100% of participants (2/2) had reduction in intraretinal fluid on OCT**
  - Both ARB participants had improvements in visual function in areas where intraretinal fluid decreased
- **Functional gains were co-localized to structural improvements**
  - Retinal sensitivity improved in the treated transitional zone at the edge of the lesion where fluid was minimal
  - Fixation moved from outside the lesion to within the lesion in all four evaluable participants
  - Areas of the transitional zone treated within the subretinal bleb had the highest functional gains



Results Inform Future Enrollment

Earlier-stage participants showed the greatest structural and functional improvements, suggesting earlier intervention may yield improved outcomes

ARB, autosomal recessive bestrophinopathy; BVMD, best vitelliform macular dystrophy; OCT, optical coherence tomography.

Post-Treatment Participant Feedback

101-101 ARB	**Able to see on the eye chart for the first time in 30 years**
101-104 ARB	**Wants the second eye treated**
102-101 BVMD	**Happy with the study, mostly stable vision**
102-102 BVMD	**Wants the second eye treated**
101-106 BVMD	**Reports less eye strain after prolonged computer work; colors on TV appear brighter and clearer**

ARB, autosomal recessive bestrophinopathy; BVMD, best vitelliform macular dystrophy.

Participant Case Studies & Next Steps

Ash Jayagopal, PhD, MBA
Chief Scientific and Development Officer

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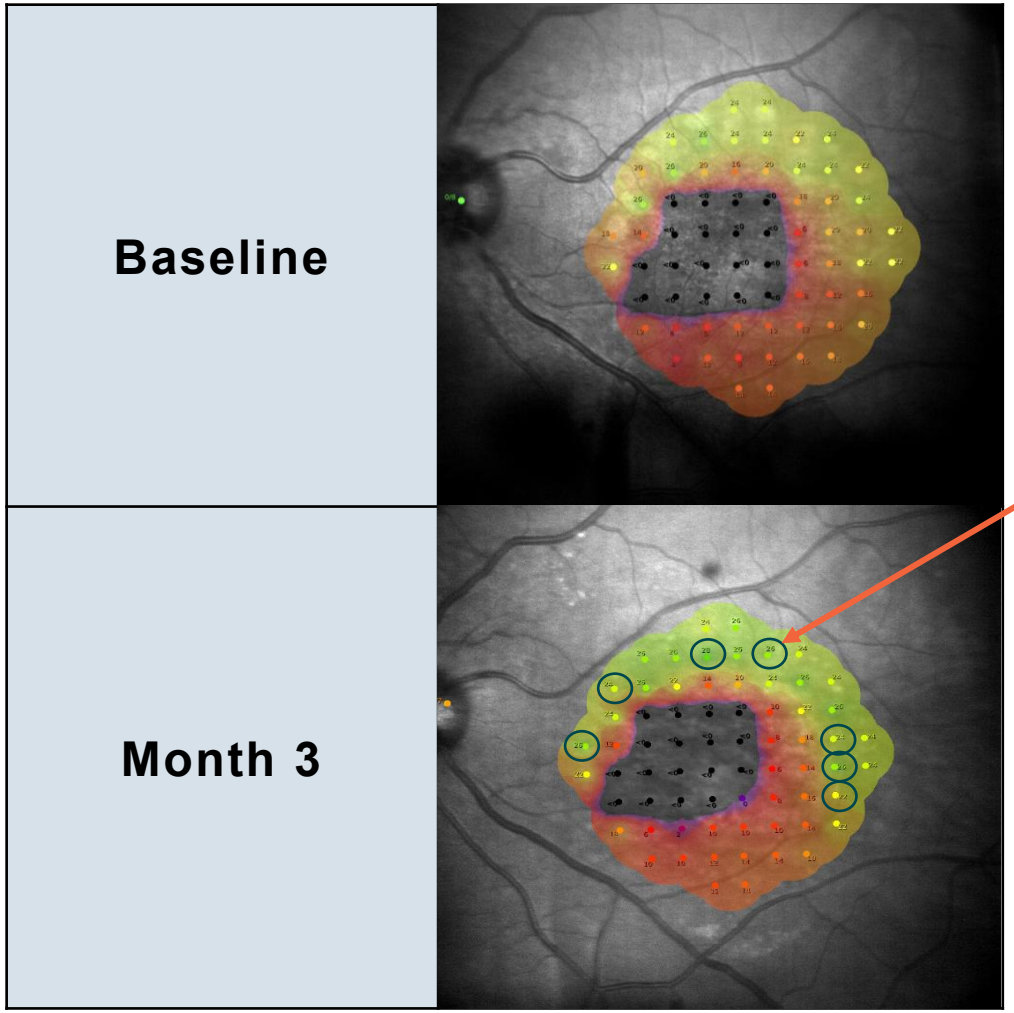
Participant 101-106 (BVMD): Microperimetry Improvements

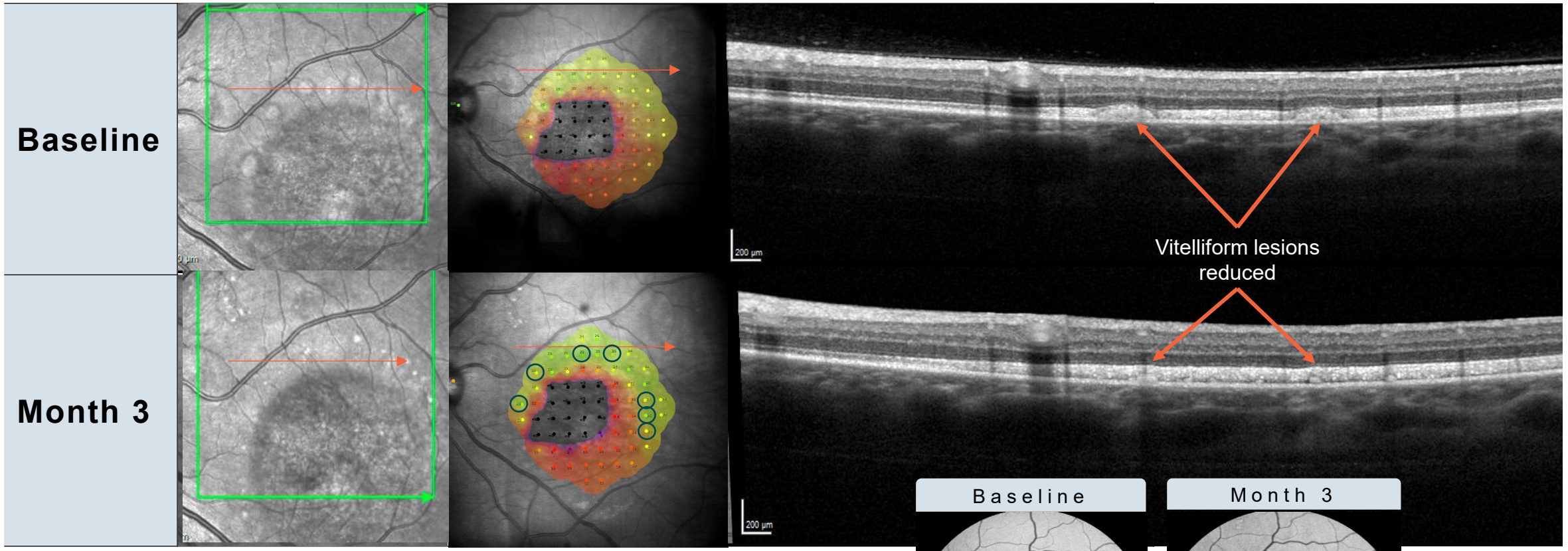


- Threshold for success ≥3 dB
- Average improvement in TTZ = 3.125 dB
  - Fellow eye TZ = 0.5 dB
- 7 loci had ≥3 dB improvement (circled)

This participant met the microperimetry threshold for success of ≥3 dB

BVMD, best vitelliform macular dystrophy; dB, decibel; TTZ, treated transitional zone; TZ, transitional zone.
Treated transitional zone follows the vitelliform arc as seen on fundus autofluorescence and OCT
Microperimetry loci were not pre-specified

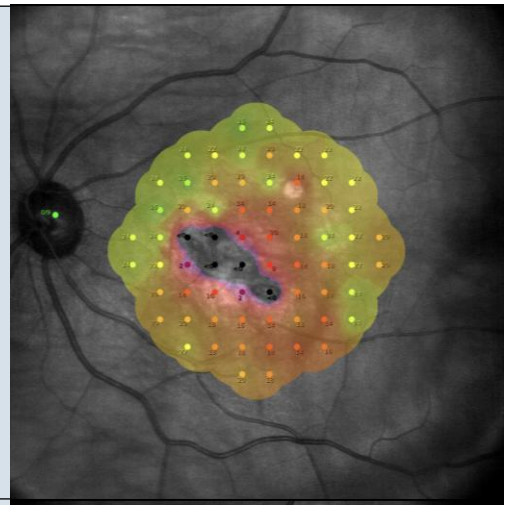
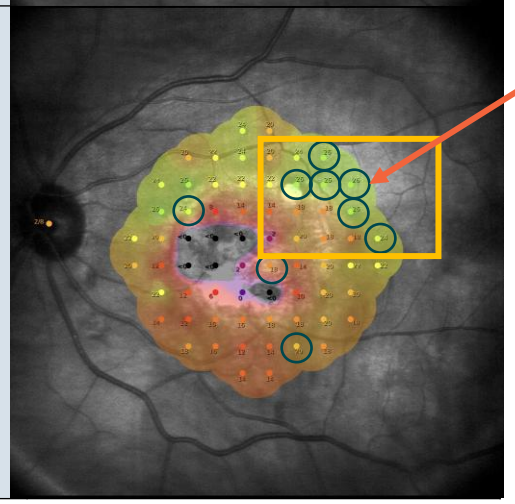
Participant 101-106 (BVMD): Vitelliform Material Reduction Co-localized with Microperimetry Improvements



Lesion reduction consistent with improved function of RPE cells and treatment activity as indicated in microperimetry improvements

BVMD, best vitelliform macular dystrophy; dB, decibel; RPE, retinal pigment epithelium.

Participant 102-102 (BVMD): Microperimetry Improvements

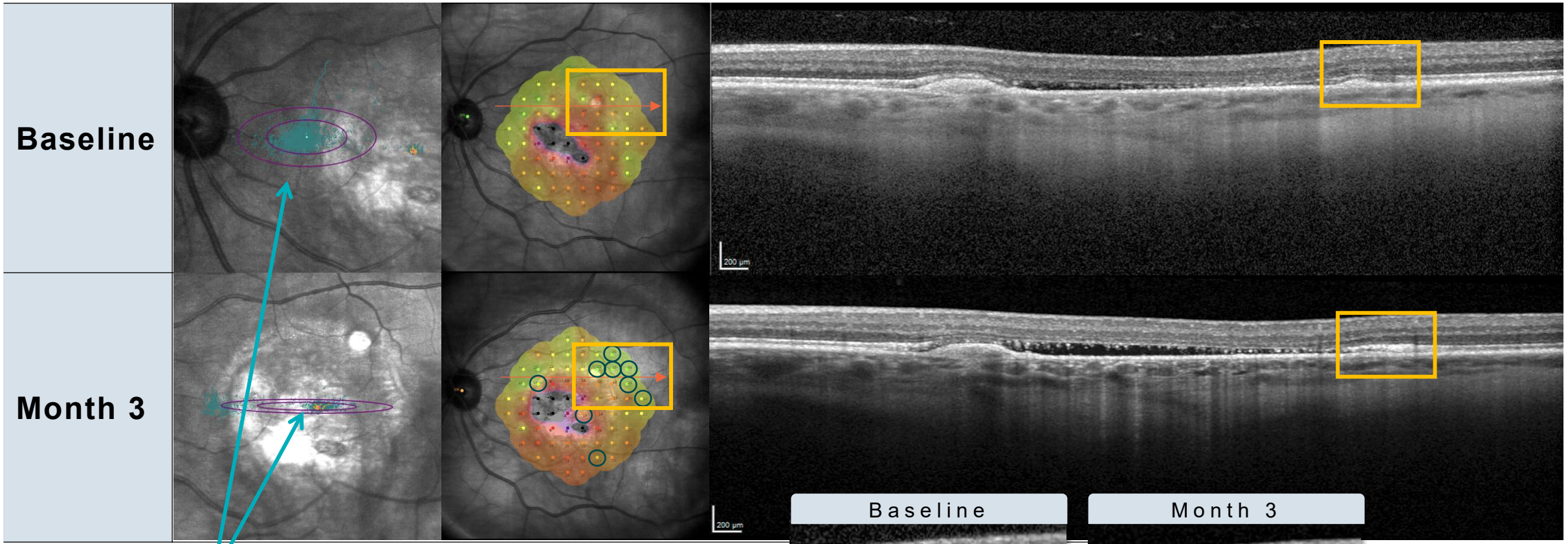
Baseline	
Month 3	

- Threshold for success ≥3 dB
- Average improvement in TTZ = 3.5 dB
  - Fellow eye TZ = 0.5 dB
- 6 loci had ≥3 dB improvement (circled) – clustered within the TTZ

This participant met the microperimetry threshold for success of ≥3 dB

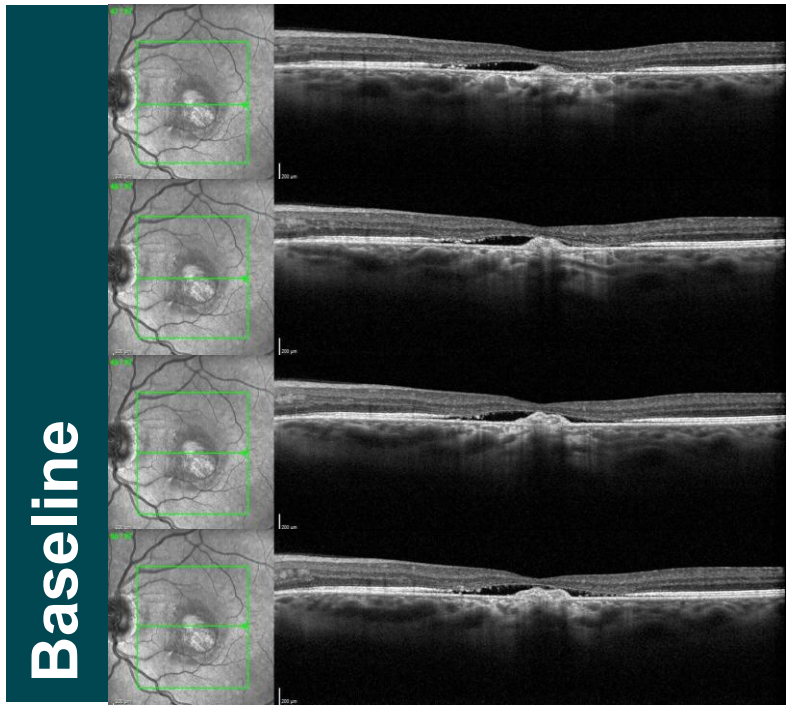
BVMD, best vitelliform macular dystrophy; dB, decibel; TTZ; treated transitional zone; TZ, transitional zone.
Yellow box represents the treated transitional zone
Microperimetry loci were not prespecified

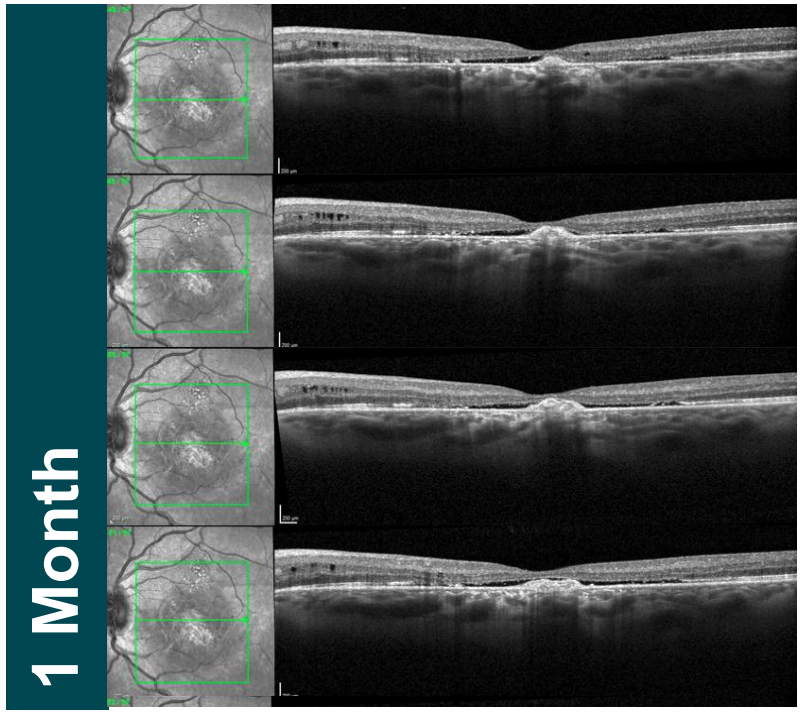
Participant 102-102 (BVMD): Microperimetry Improved in the Treated RPE Transitional Zone

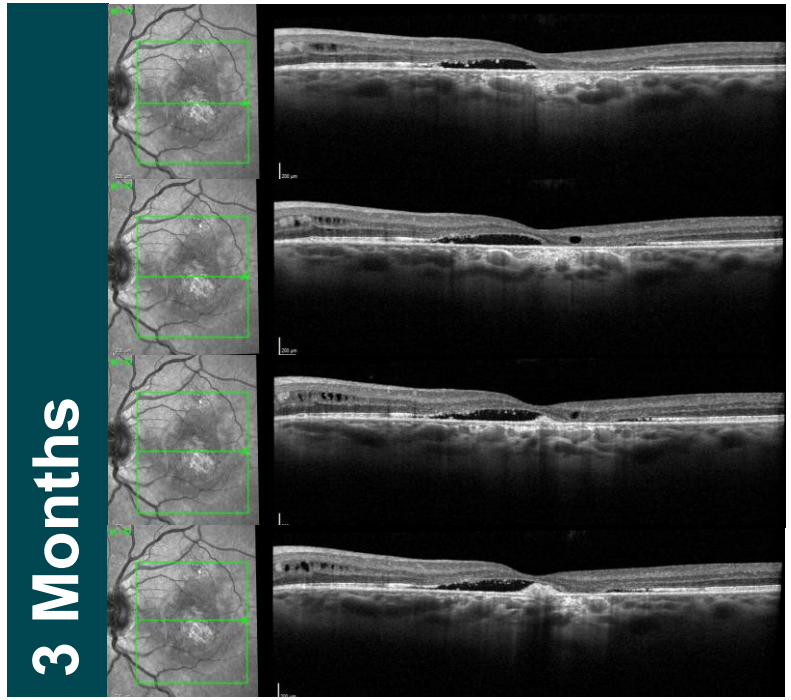


BVMD, best vitelliform macular dystrophy; RPE, retinal pigment epithelium.
Yellow box represents treated transitional zone

Participant 102-101 (BVMD): Photoreceptor and RPE Atrophy Limit Improvement



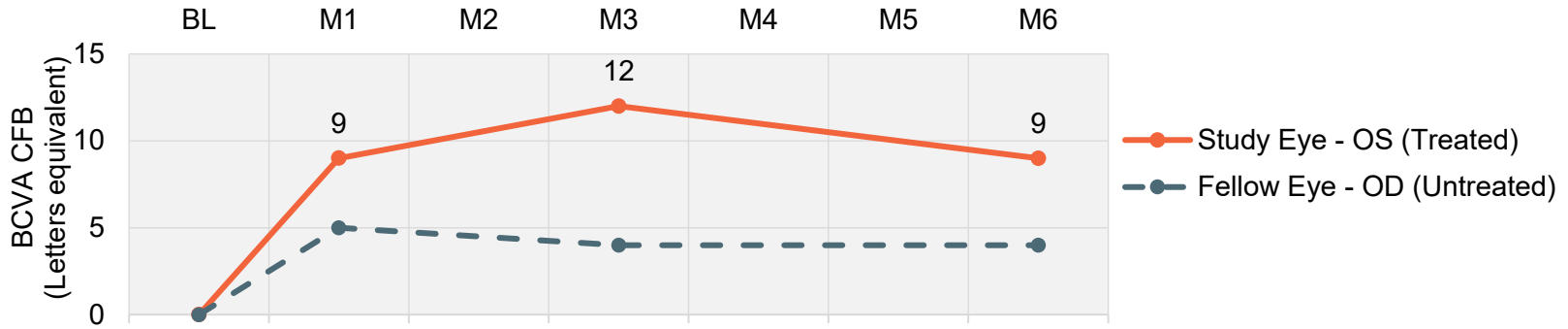


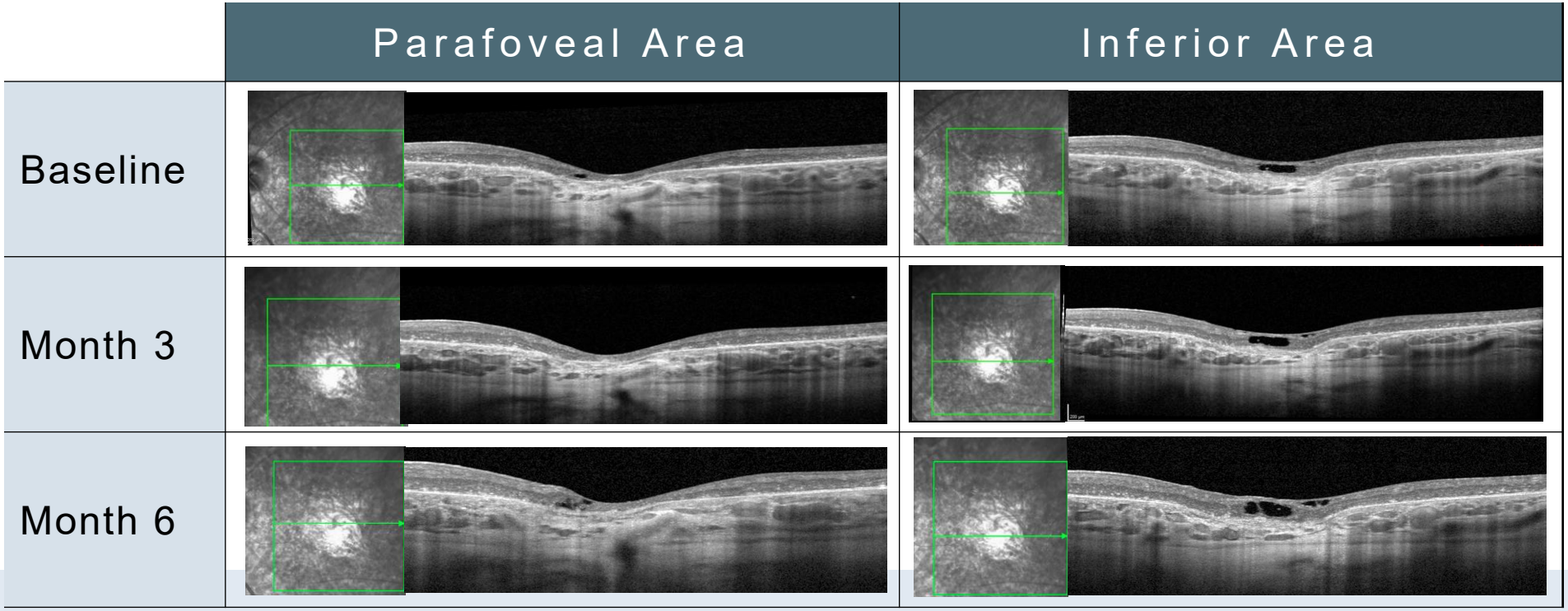


- Most advanced BVMD participant
- Sub-RPE atrophy and scarring, exclusionary from a potential pivotal trial
- Vitelliform material changes over time
- CS improvements* akin to observations in geographic atrophy

Images are consecutive registered OCT rasters at baseline, 1 month, and 3 months.
*CS improvements defined as ≥2 dB change from baseline.
CS, contrast sensitivity; BVMD, best vitelliform macular dystrophy; OCT, optical coherence tomography; RPE, retinal pigment epithelium.

Participant 101-101 (ARB): Functional and Structural Outcomes Through 6 Months





- Functional improvement maintained to 6 months on BCVA, CS, and LLVA
- Unable to perform MP due to poor baseline vision
- IRF decreased at 3 months in the parafoveal area
- Fluctuations observed in both areas between 3 and 6 months

ARB, autosomal recessive bestrophinopathy; BCVA, best-corrected visual acuity; BL, baseline; CFB, change from baseline; CS, contrast sensitivity; IRF, intraretinal fluid; LLVA, low luminance visual acuity; M, month, MP, microperimetry; OD, right eye; OS, left eye.

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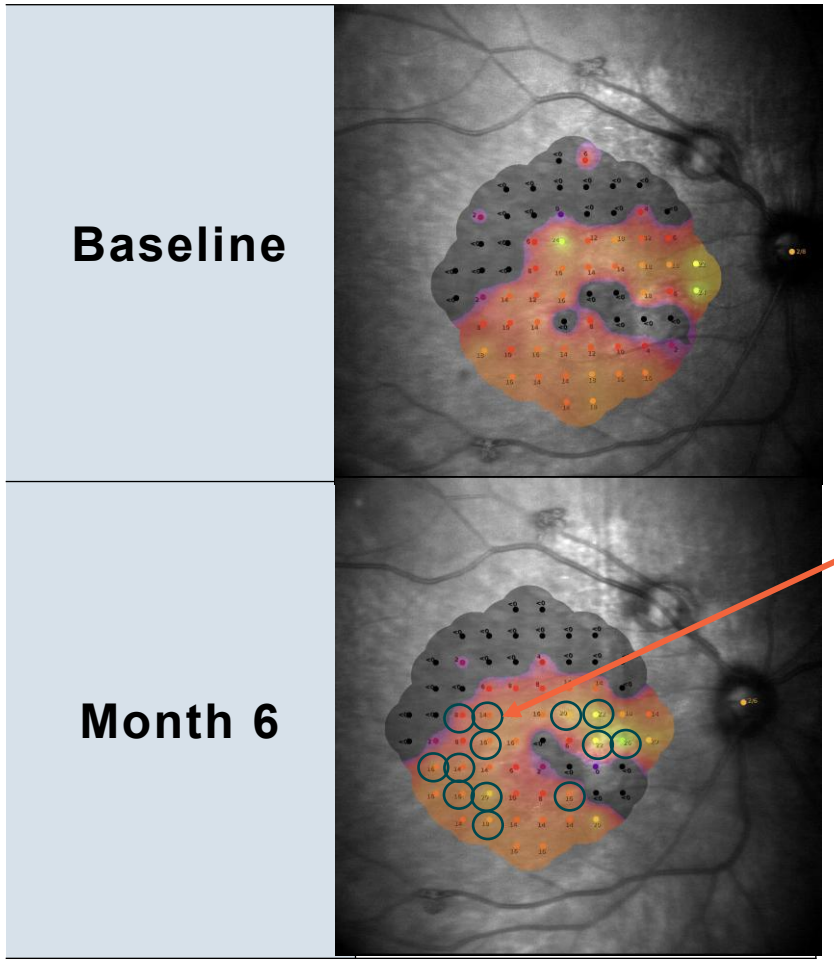
Participant 101-104 (ARB): Microperimetry Improvement



- Threshold for success: ≥3 dB change
- Average improvement in area of IRF = 3.3 dB
  - Fellow eye corresponding area = 2.3 dB
- 13 loci had ≥3 dB improvement (circled) – clustered within the TTZ

This participant met the microperimetry threshold for success of ≥3 dB

Total macular IRF volume = 19.06 µl at baseline, 7.55 µl at 6 months.
Microperimetry loci were not prespecified
ARB, autosomal recessive bestrophinopathy; dB, decibel; IRF, intraretinal fluid; RPE, retinal pigment epithelium; TTZ; treated transitional zone.

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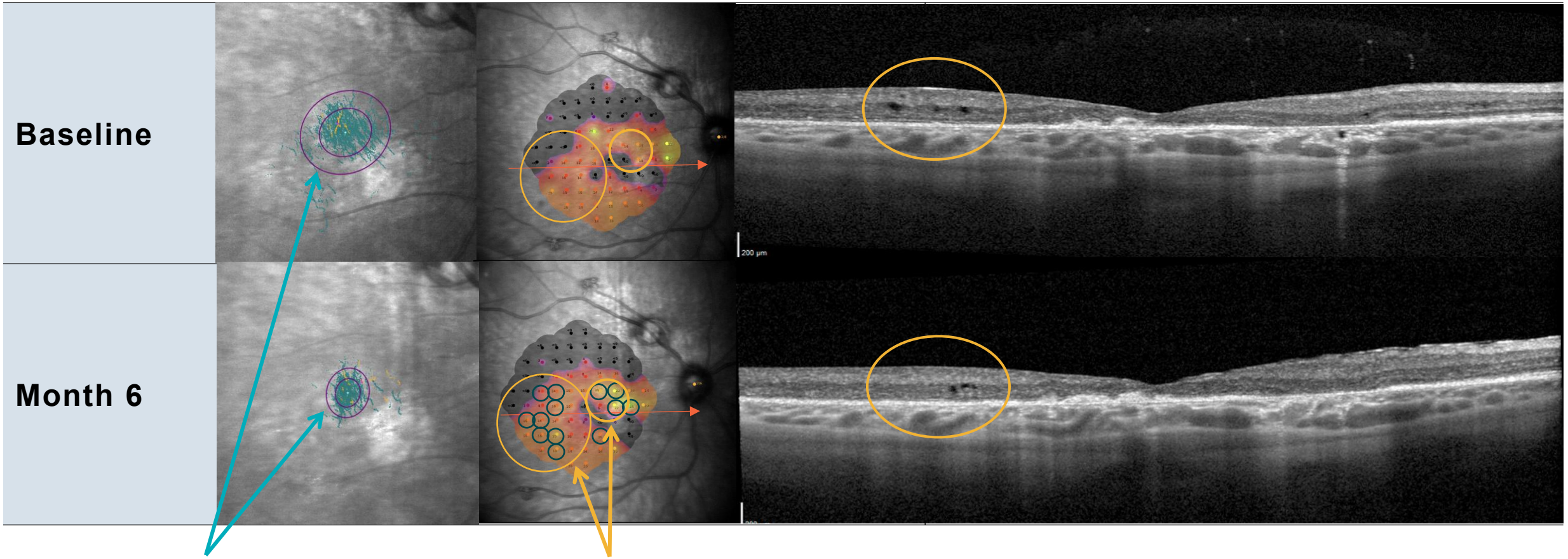
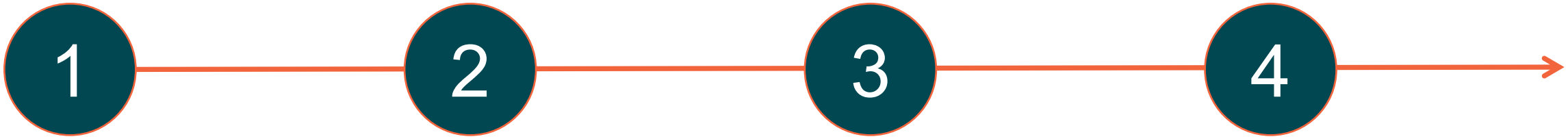
Participant 101-104 (ARB): IRF Improvement Co-localized with Microperimetry Improvement



Yellow circles denote pockets of intraretinal fluid.
Total macular intraretinal fluid volume: Baseline = 19.06 µl, Month 6 = 7.55 µl
ARB, autosomal recessive bestrophinopathy; IRF, intraretinal fluid.

OPGx-BEST1 Advancing Based on Positive Cohort 1 Results

4 milestones cleared on the path to pivotal



Well-tolerated with no SAEs, no DLTs, and no intraocular inflammation

Efficacy observed on both functional and structural endpoints

Positive FDA Type C meeting aligned on potential pivotal endpoint with microperimetry

75% of Cohort 1 evaluable participants met the microperimetry threshold for success of ≥3 dB

CMC, chemistry, manufacturing, and controls; dB, decibel; DLT, dose-limiting toxicity; FDA, Food and Drug Administration; IDMC, independent data monitoring committee; SAE, serious adverse event.

Program Summary and Next Steps

George Magrath, MD
Chief Executive Officer

OPUS GENETICS

Accomplished Significant OPGx-BEST1 Program Milestones

PH 1/2 COHORT 2 STATUS

Dose

$4.5x10^9$ vg/eye

Enrollment

Already **over-enrolled**

- Originally planned for 5 participants
- Over-enrollment of 8 participants

Clinical Development Timeline*

- Dosing expected to be completed Q4 2026
- Topline 3-month data expected Q2 2027
- **Pivotal trial planning initiated, dosing expected in 2027**

REGULATORY STATUS

August 2026 FDA Type C meeting

- Aligned on Phase 3 CMC
- Constructive dialogue on potential endpoint options for Phase 3

Potential Pivotal Endpoints

- **Use of ≥3 dB microperimetry improvement in ≥5 prespecified loci area** and;
  - Randomized controlled trial with a patient-reported outcome
  - BCVA, LLVA, contrast sensitivity may also be acceptable endpoints

Next Regulatory Interaction

- Following early data from Cohort 2

*Clinical development timelines are based on current estimates and are subject to change; data readouts are targeted for ~9-12 months after study initiation.

ARB, autosomal recessive bestrophinopathy; BCVA, best corrected visual acuity; BVMD, best vitelliform macular dystrophy; CMC, chemistry, manufacturing, and controls; dB, decibels; FDA, Food and Drug Administration; LLVA, low luminance visual acuity.

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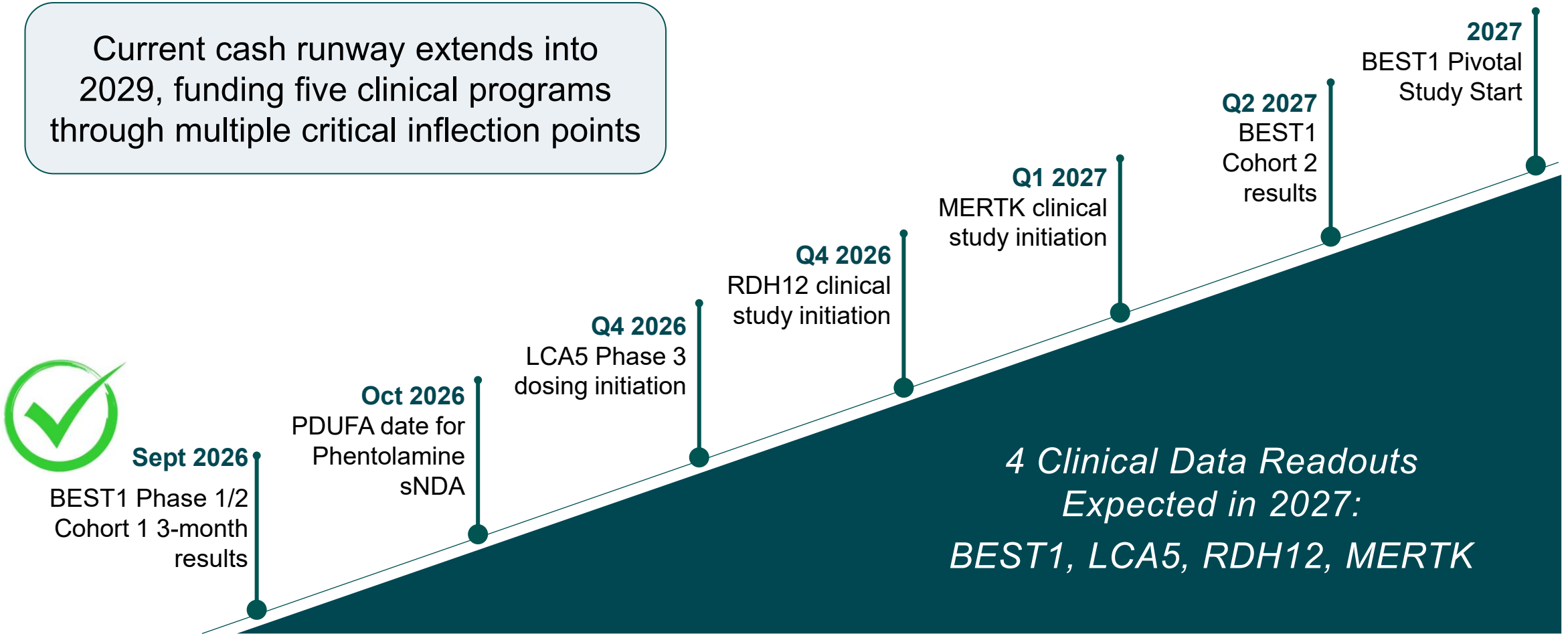
Current Cash to Support Multiple Clinical Inflection Points

Current cash runway extends into 2029, funding five clinical programs through multiple critical inflection points



Clinical development timelines are based on current estimates and are subject to change; data readouts are targeted for ~9-12 months after study initiation.

Phentolamine ophthalmic solution 0.75% is a commercial partnered program; it is FDA-approved for the treatment of pharmacologically-induced mydriasis; an sNDA has been submitted for the treatment of presbyopia.
BEST1, bestrophin 1; LCA5, Leber congenital amaurosis 5; MERTK, MER proto-oncogene tyrosine kinase; PDUFA, Prescription Drug User Fee Act; PRV, Priority Review Voucher; RDH12, retinol dehydrogenase 12; RHO, rhodopsin; sNDA, supplemental New Drug Application.

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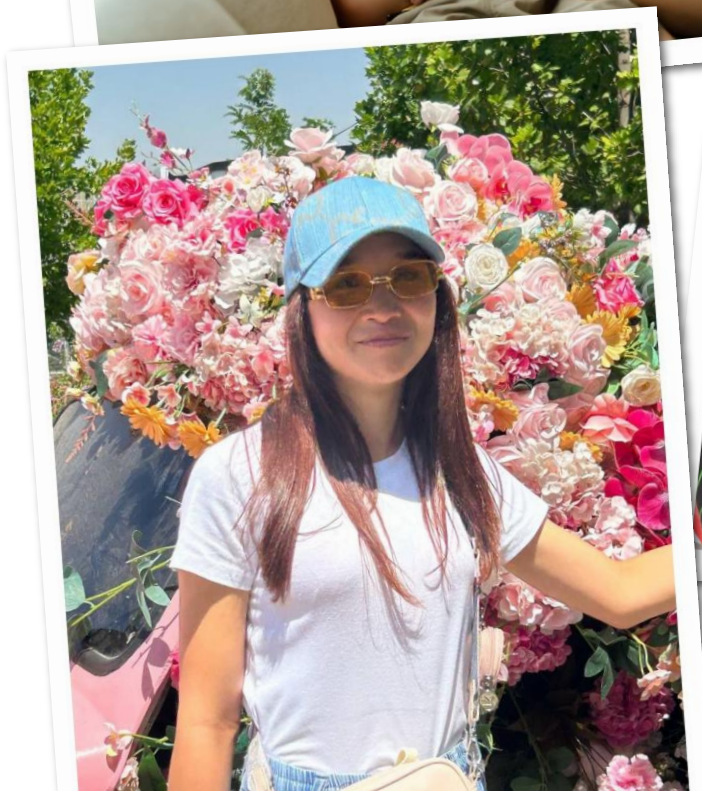
Nasdaq: IRD



Mark E. Pennesi, MD, PhD, FARVO

Leading ophthalmologist, researcher specializing in inherited retinal diseases, and pioneer in gene therapy

- **Current Appointments**
  - Chief Medical Officer, Steve and Debbie Gray Inherited Retinal Degeneration Endowed Chair; Director, Inherited Retinal Degeneration Center – Retina Foundation, Dallas, Texas
  - Adjunct Professor of Ophthalmology – Paul H. Casey Ophthalmic Genetics Division, Casey Eye Institute, Oregon Health & Science University, Portland, Oregon
- **Education**
  - B.S. in Biomedical Engineering, University of Pennsylvania (summa cum laude); Combined MD/PhD, Baylor College of Medicine; Ophthalmology residency, UCSF; Ophthalmic genetics fellowship, Casey Eye Institute/OHSU
- **Research and Awards**
  - Research focuses on developing novel treatments for inherited retinal diseases
  - Author of 170+ peer-reviewed publications; principal or co-principal investigator on numerous first-in-human gene therapy trials
  - Research to Prevent Blindness and the Foundation Fighting Blindness have recognized Dr. Pennesi with career development awards; Additionally, he was the recipient of the 2011 ARVO/Alcon Early Clinician Scientist, the Alcon Young investigator Award in 2014, and the Casey Eye Institute Resident teach award

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Q&A with Opus Management and Dr. Pennesi

George Magrath, MD
Chief Executive Officer

Rob Gagnon, CPA, MBA
Chief Financial Officer

Sally Tucker, MCOptom, PhD
Chief Medical Officer

Ben Yerxa, PhD
President

Ash Jayagopal, PhD, MBA
Chief Scientific & Development Officer

Mark E. Pennesi, MD, PhD, FARVO
Retina Foundation of the Southwest
Dallas, TX

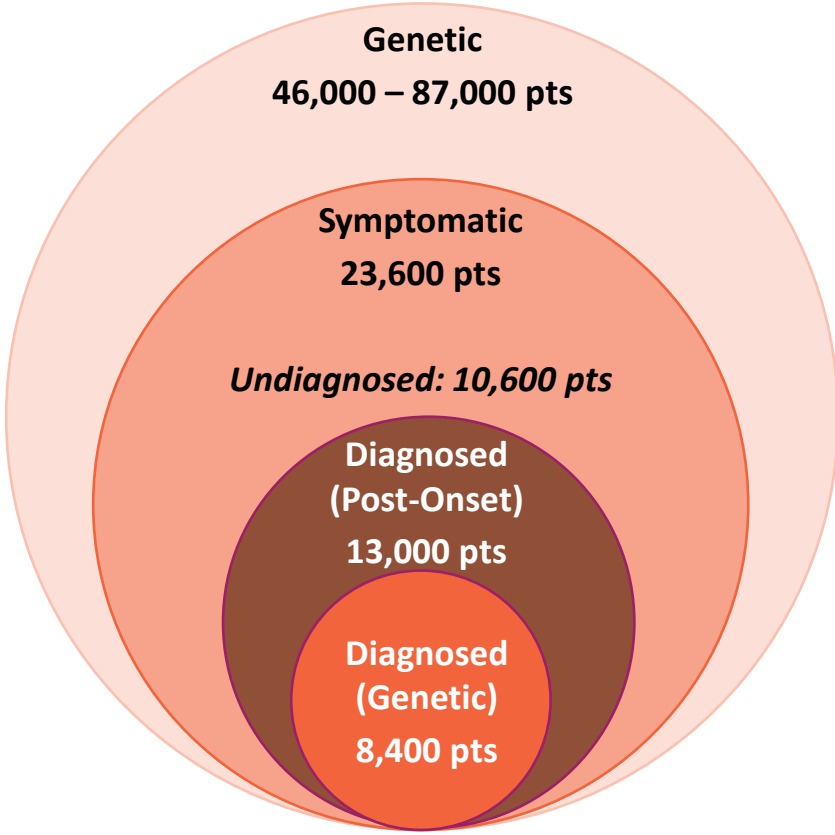
Appendix

BEST1 Market Landscape Quantitative Market Research

BEST1 Patients: Estimated Inputs

Patient Group	Definition	Est. Value
Genetic	BEST1 genotype (with or without symptoms)	**~46,000 – 87,000 patients**
Symptomatic	Diagnosed + undiagnosed (with ocular symptoms)	**~23,600 patients**
Diagnosed (Post-onset)	Diagnosed (with ocular symptoms)	**~13,000 patients**
Diagnosed (Genetically Confirmed)	Diagnosed with genetic confirmation	**~8,400 patients**
Undiagnosed (Post-onset)	Undiagnosed (with ocular symptoms)	**~10,600 patients**

BEST1 Mutation: Estimated Subpopulations



Triangle Insights Group Research, Q3 2026.