



Clinical Momentum and Pipeline Progress at Opus Genetics

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Disclosures and Forward-Looking Statements

Certain statements contained in this presentation are not statements of historical fact and are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements give current expectations or forecasts of future events or our future financial or operating performance. Such statements include, but are not limited to, statements concerning our data from and future enrollment for our clinical trials, our pipeline of additional indications and anticipated regulatory milestones.

In some cases, you can identify forward-looking statements by the following words: “aim,” “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes to identify these forward-looking statements.

These forward-looking statements reflect our management’s beliefs and views with respect to future events, are based on estimates and assumptions as of the date of this presentation and are subject to risks and uncertainties, many of which are beyond our control, that could cause our actual results to differ materially from those in these forward-looking statements, including, without limitation: our gene therapy product candidates are based on a novel technology and manufactured by a third party, 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 U.S. Food and Drug Administration (“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; others may discover, develop, or commercialize products similar to those in our pipeline before or more successfully than we do or develop generic variants of our products even while our product patents remain active, thereby reducing our market share and potential revenue from product sales; we do not currently have any sales or marketing infrastructure in place and we have limited drug research and discovery capabilities; the future commercial success of our products could significantly depend upon several uncertain factors, including third-party reimbursement practices and the existence of competitors with similar products; product liability lawsuits against us or our suppliers or manufacturers could cause us to incur substantial liabilities and could limit commercialization of any product candidate that we may develop; failure to comply with health and safety laws and regulations could lead to material fines; 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 markets; raising additional capital may cause our stockholders to be diluted, among other adverse effects; instability and operational disruptions at government agencies, such as the FDA, may adversely impact our development and commercialization plans by causing delays and requiring the use of additional, unforeseen resources to obtain regulatory approval for trials or products in our pipeline; we operate in a highly regulated industry and face many challenges adapting to sudden changes in legislative reform or the regulatory environment, including due to government shutdowns and disruptions at government agencies, which cause delays, requires the use of additional, unforeseen resources, affects our pipeline stability, and could impair our ability to compete in international markets; we may not receive regulatory approval to market our developed product candidates within or outside of the U.S.; with respect to any of our product candidates that receive marketing approval, we may be subject to substantial penalties if we fail to comply with applicable regulatory requirements; our potential relationships with healthcare providers and third-party payors will be subject to certain healthcare laws and regulations, which could expose us to extensive potential liabilities; 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; we may be unsuccessful in entering into or maintaining licensing arrangements or establishing strategic alliances on favorable terms, which could harm our business; inadequate patent protection for our product candidates may result in our competitors developing similar or identical products or technology, which would adversely affect our ability to successfully commercialize; we may be unable to obtain full protection for our intellectual property rights under U.S. or foreign laws; we may become involved in lawsuits for a variety of reasons associated with our intellectual property rights, including alleged infringement suits initiated by third parties; we are dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy; as we grow, we may not be able to operate internationally or adequately develop and expand our sales, marketing, distribution, and other corporate functions, which could disrupt our operations; the market price of our common stock is expected to be volatile and if we fail to comply with the continued listing standards of Nasdaq, our common stock may be delisted; and factors out of our control related to our securities, such as securities litigation or actions of activist stockholders, could adversely affect our business and stock price and cause us to incur significant expenses.

We discuss many of these risks in greater detail under Part I, Item 1A “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2025 and in subsequent reports filed with or furnished to the Securities and Exchange Commission. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Any forward-looking statement in this presentation speaks only as of the date hereof or as of the date specified herein. We undertake no obligation to publicly update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable laws or regulations.

Building a Differentiated Gene Therapy Platform

	Preclinical	IND-enabling	Phase 1/2	Phase 3	Approval
OPGx-LCA5 LCA <i>co-funded by FDA OOPD</i>					
OPGx-BEST1 <i>Bestrophinopathies</i>					
OPGx-RDH12 LCA <i>co-funded by Global RDH12 Alliance</i>					
OPGx-MERTK RP <i>co-funded by FFB RD Fund & Abu Dhabi's Healthcare Research and Innovation Fund</i>					
OPGx-RHO adRP <i>co-funded by FFB & NIH</i>					
OPGx-NMNAT1 LCA					
OPGx-CNGB1 RP <i>NIH-funded consortium</i>					
Undisclosed IRD GTx					

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 β 1; 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.

OPGx-LCA5 Gene Therapy is Designed to Restore a Key Protein of the Visual Cycle

- Lebercilin is a ciliary protein critical for the function of photoreceptor inner and outer segments¹
- In *LCA5* patients, photoreceptor function is severely impaired due to a lack of functioning lebercilin¹
 - However, photoreceptors can survive through the third decade of life, suggestive of a broad window for therapeutic intervention²
- OPGx-LCA5 is designed to address mutations in the *LCA5* gene, which encodes for lebercilin
 - Clinically-derisked AAV8 vector delivers a functional *LCA5* gene directly to photoreceptor cells, using same promoter technology as LUXTURN[®] (voretigene neparvovec-rzyl)

OPGx-LCA5 Gene Therapy	
Vector	AAV8
Delivery	Single subretinal injection
Clinical Program Status	• Phase 1/2 ongoing • Phase 3: • Enrollment complete • Run-in ongoing • Dosing initiation expected in Q4 2026

Clinical development timelines are based on current estimates and are subject to change.

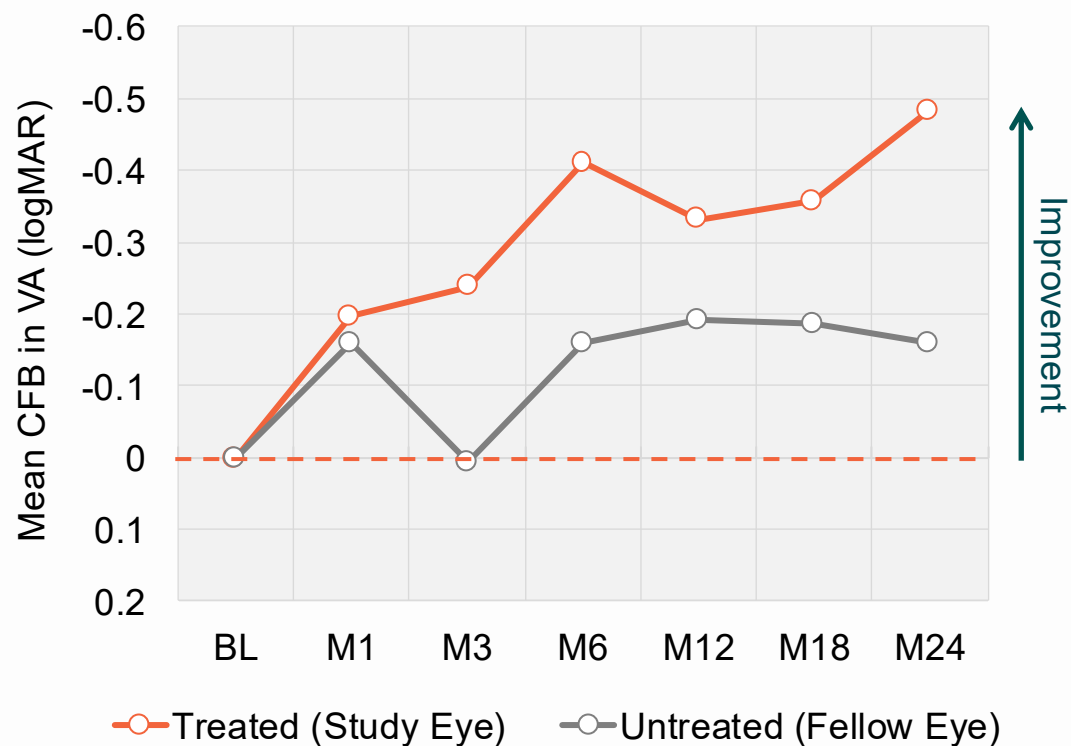
LUXTURN[®] is a registered trademark of Spark Therapeutics, Inc.

AAV8, adeno-associated virus serotype 8; LCA5, Leber congenital amaurosis 5.

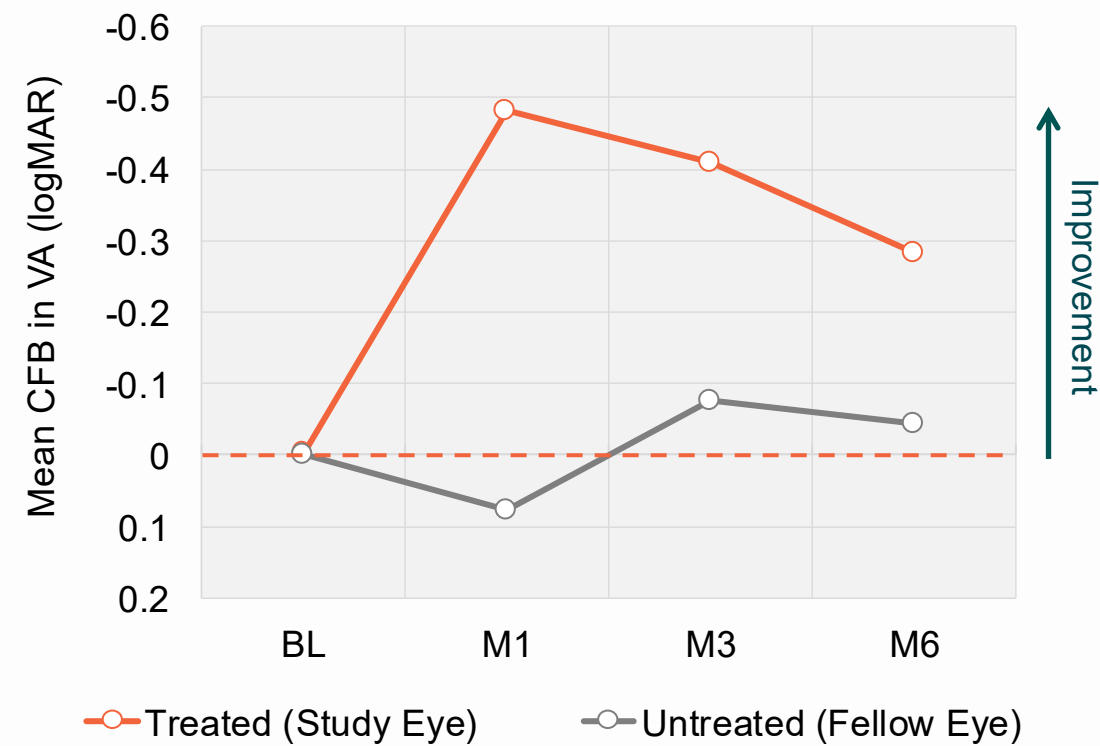
1. Uyhazi KE, et al. *Invest Ophthalmol Vis Sci.* 2020;61:30. 2. Song JY, et al. *Mol Ther.* 2018;26:1581-1593.

Ongoing Phase 1/2 Study: Visual Acuity Maintained in Adults Over 24 Months and Improved Over 6 Months in Pediatric Cohort

Mean Change from Baseline in VA
Adult Cohort (N=3)

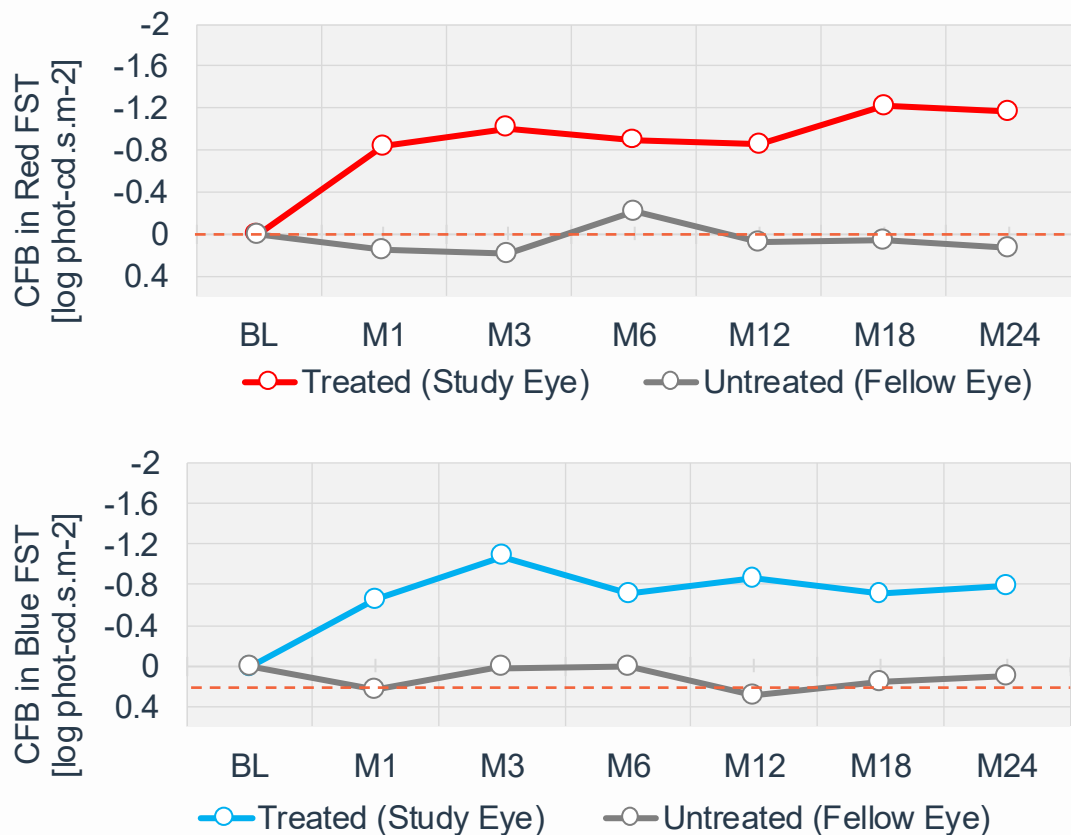


Mean Change from Baseline in VA
Pediatric Cohort (N=3)

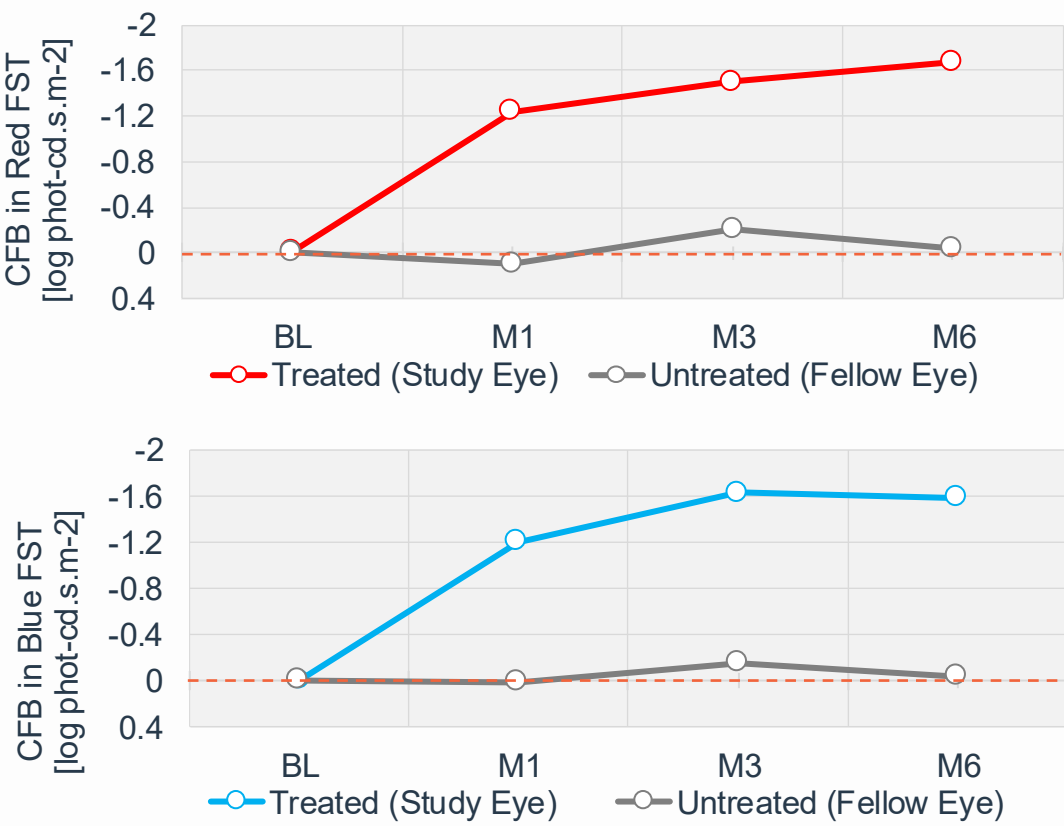


Ongoing Phase 1/2 Study: Full-Field Stimulus Test Demonstrated Durable Vision Improvement

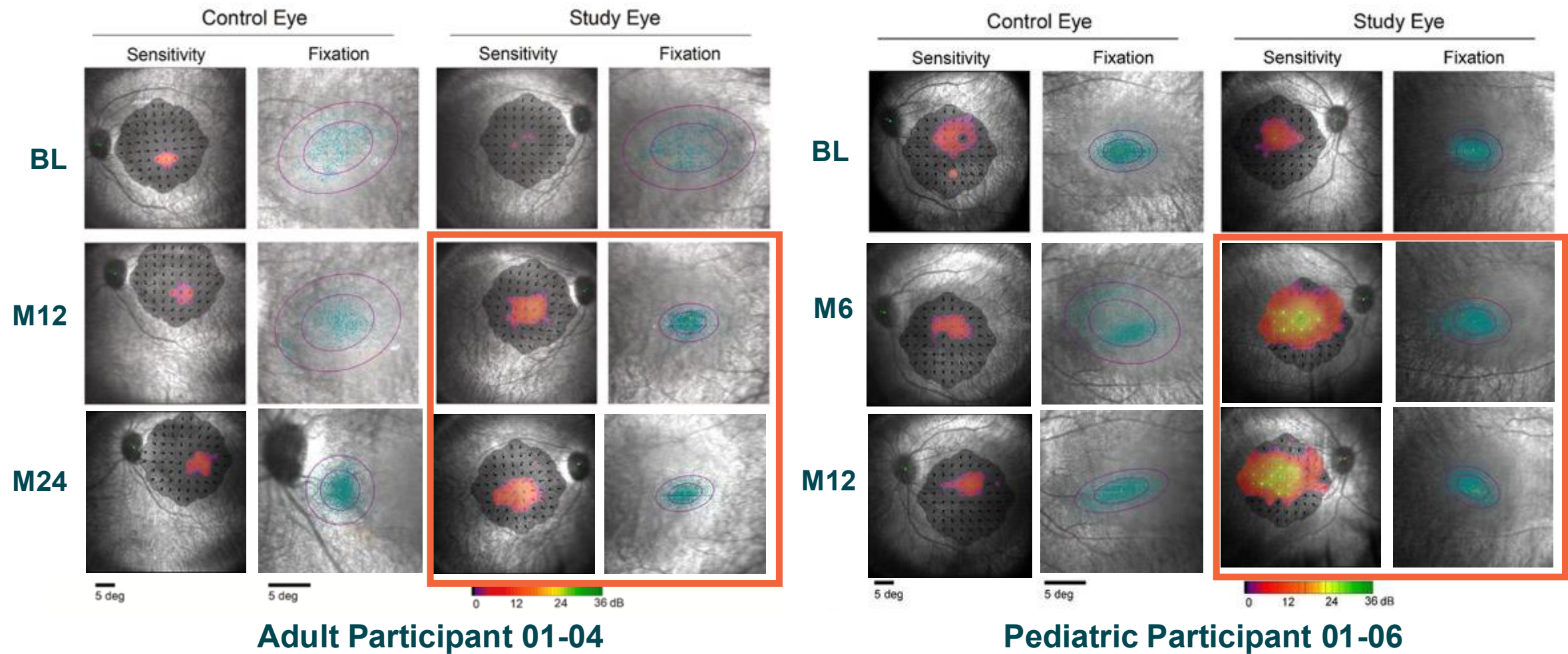
Mean CFB in Red and Blue FST
Adult Cohort (N=3)



Mean CFB in Red and Blue FST
Pediatric Cohort (N=3)



Ongoing Phase 1/2 Study: Microperimetry Data Provides Evidence of Increased Sensitivity and Movement of Fixation Toward the Fovea



OPGx-BEST1 Gene Therapy is Designed to Restore Retinal Ion Homeostasis

- *BEST1* mutations disrupt cellular ion and fluid homeostasis resulting in electrophysiological abnormalities, RPE dysfunction, and retinal degeneration
- Most bestrophinopathies exhibit a slow rate of decline and central photoreceptors usually remain viable for decades, providing a wide therapeutic window
- OPGx-BEST1 is designed to restore retinal ion homeostasis in bestrophinopathies, ameliorating retinal structural and functional deficits
- *BEST1* is targeted using the AAV2 capsid employed in LUXTURNA[®] and an RPE-specific promoter

OPGx-BEST1 Gene Therapy	
Vector	AAV2
Delivery	Single subretinal injection
Clinical Program Status	• Phase 1/2 Cohort 1 (low dose) ongoing with 5 participants dosed • Phase 1/2 Cohort 2 (high dose) enrollment complete; dosing expected in Q4 2026

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.

Ongoing Phase 1/2 Study Safety and Efficacy Summary: Highly Encouraging Proof-of-Concept Results from Low Dose Cohort 1 at Month 3

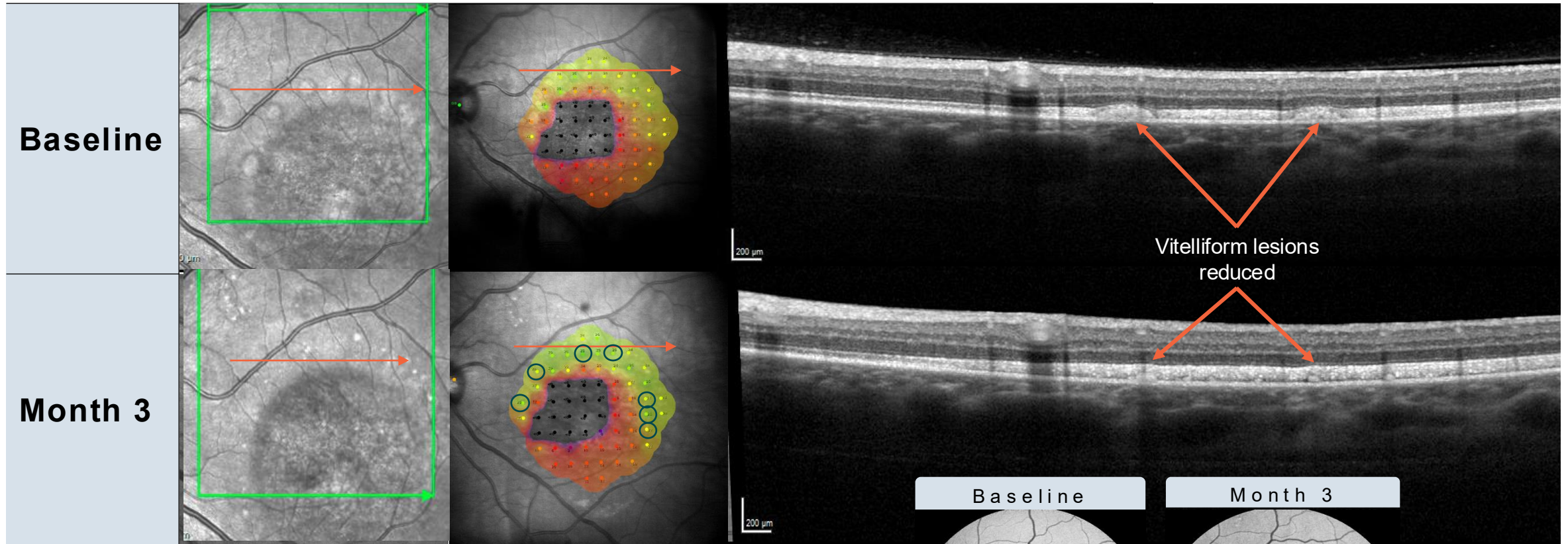
Safety	• OPGx-BEST1 was well-tolerated in 5/5 participants, with no SAEs or DLTs
Structural Endpoints	• 80% of participants (4/5) had structural improvements: • BVMD: 67% of participants (2/3) had a decrease in vitelliform material • ARB: 100% of participants (2/2) had a decrease in intraretinal fluid
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 from baseline in contrast sensitivity, and ≥5 loci cluster improvement by ≥3 decibels in RPE Transitional Zone on microperimetry.

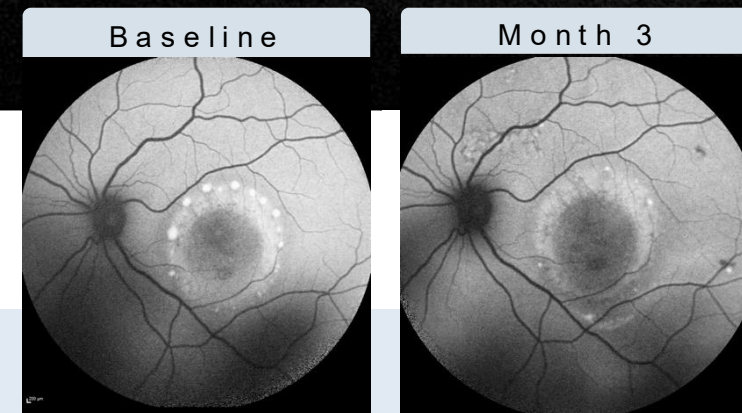
[†]101-106 was the youngest participant with most recent onset of disease and less progressive disease than other participants.

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.

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

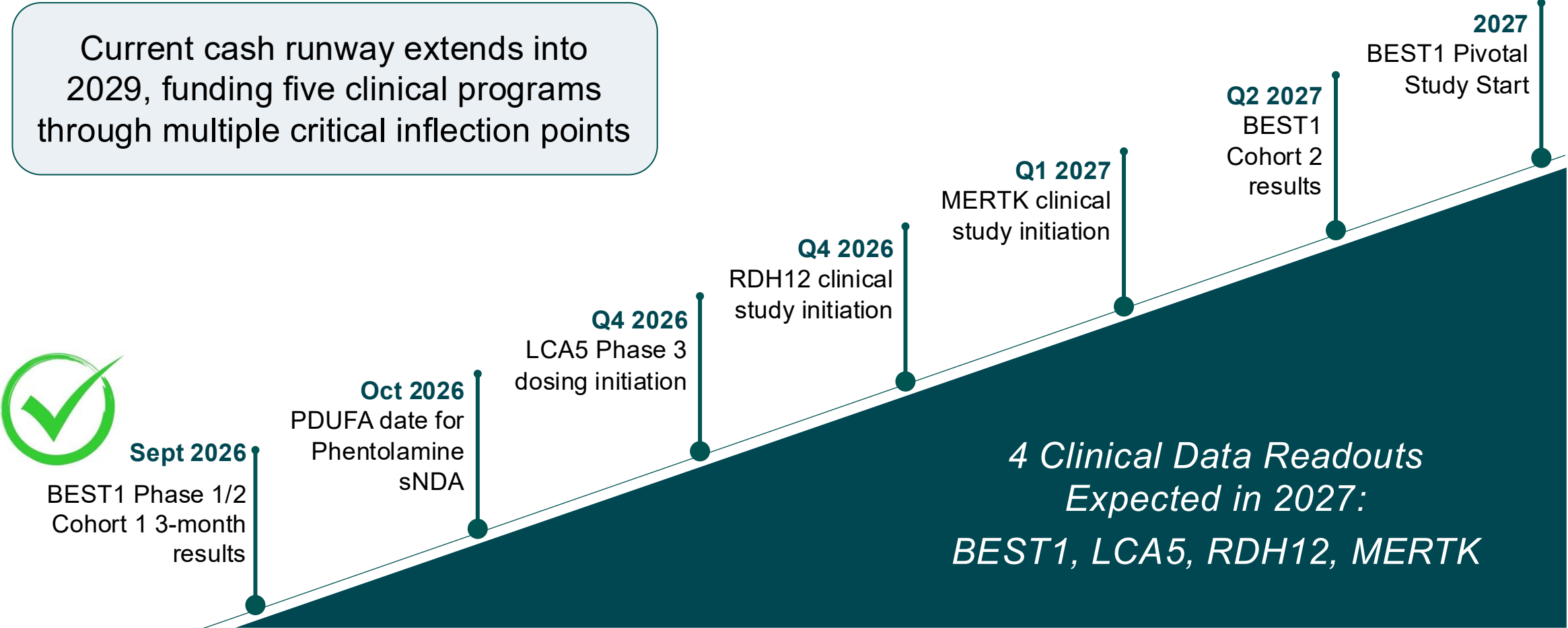


Lesion reduction consistent with improved function of RPE cells and treatment activity as indicated in microperimetry improvements

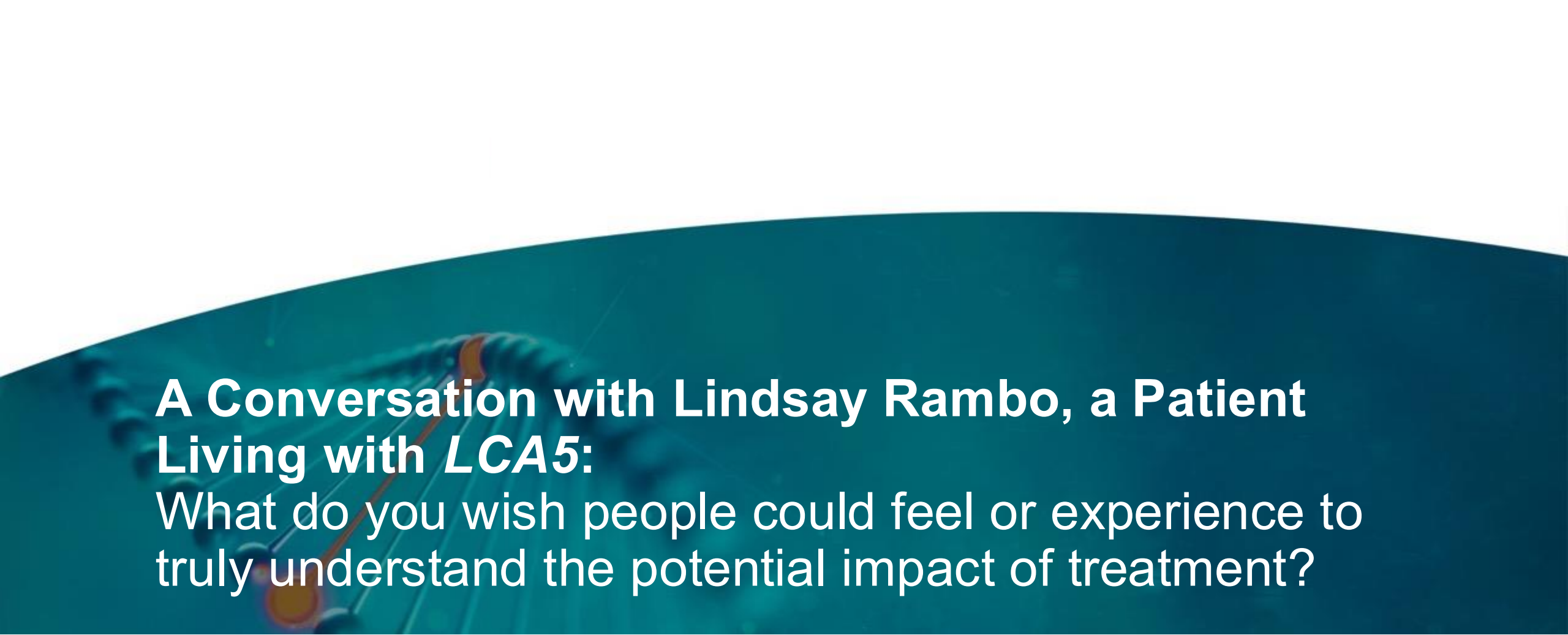


Current Cash to Support Multiple Clinical Inflection Points

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.

A Newton's cradle with several spheres is visible in the background, partially obscured by a dark teal curved shape. The spheres are in motion, creating a sense of dynamic energy.

A Conversation with Lindsay Rambo, a Patient Living with *LCA5*:

What do you wish people could feel or experience to truly understand the potential impact of treatment?



Lindsey Rambo

Thank you

