Financial Disclosures

- Rishi P. Singh has the following disclosures:
  - Consultant (Apellis, Iveric Bio, Eyepoint, Regenxbio, Genentech, Bausch and Lomb, Zeiss, Alcon, Neurotech, Regeneron); Research grants (Janssen)
- This study was funded by Neurotech Pharmaceuticals
- This study includes research conducted on human subjects; institutional review board approval was obtained prior to study initiation

Pooled Functionality Data of Revakinagene Taroretcel-lwey in Patients With Macular Telangiectasia Type 2

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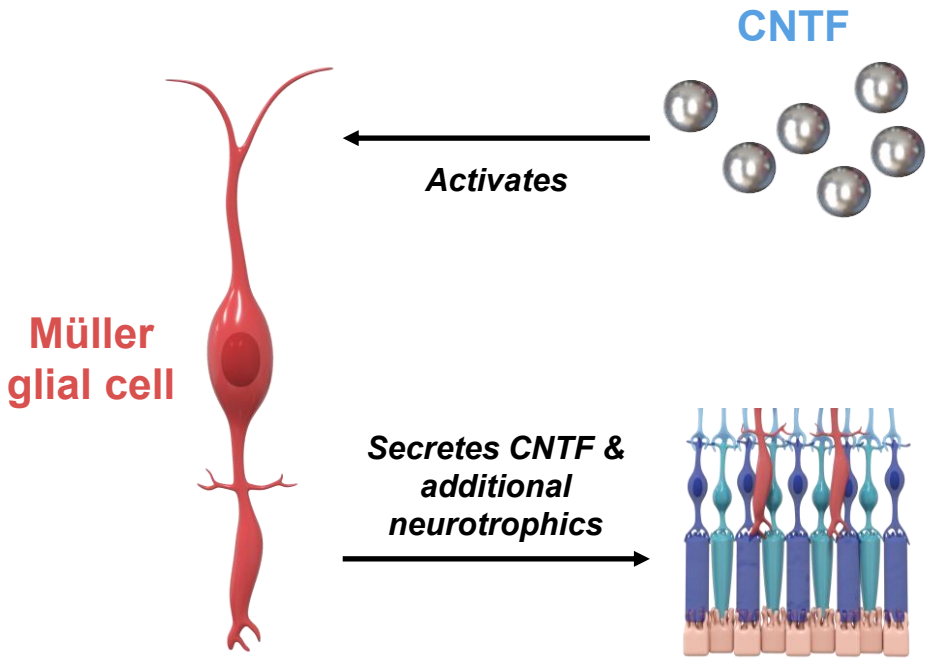
*At the time of study analysis.

Presented at: the 25th Euretina Congress; 4–7 September 2025; Paris, France

Macular Telangiectasia Type 2 (MacTel) and Ciliary Neurotrophic Factor (CNTF)

- MacTel is a bilateral, progressive, retinal neurodegenerative disease
  - Leads to central vision loss and functional impairment[1,2]
  - Characterized by abnormalities in Müller glia, retinal pigment epithelium, and photoreceptors in the central retina,[3] and progressive loss of the EZ[4]
- CNTF, released from Müller cells under pathologic conditions, protects and preserves photoreceptors[5,6]



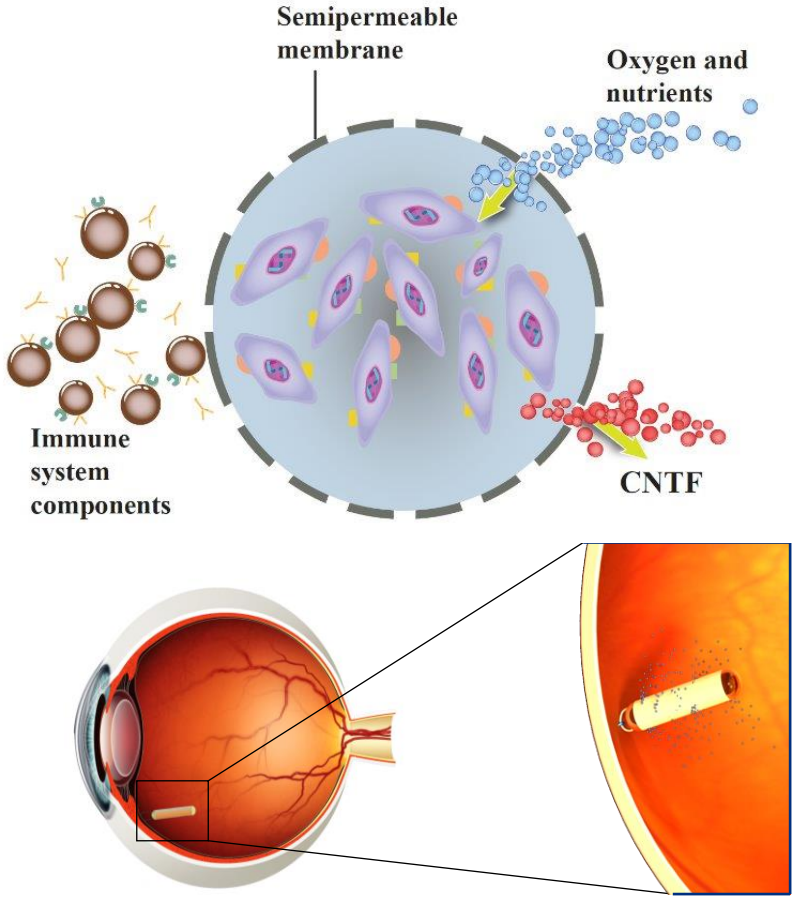
Cell survival pathway activation and macular photoreceptor protection

CNTF, ciliary neurotrophic factor; EZ, ellipsoid zone; MacTel, macular telangiectasia type 2.

1. Charbel Issa P, et al. *Prog Retin Eye Res*. 2013;34:49-77. **2.** Hereen TFC, et al. *Ophthalmology*. 2020;127:1539-1548. **3.** Kedarisetti KC, et al. *Clin Ophthalmol*. 2022;16:3297-3309. **4.** Heeren TFC, et al. *Retina*. 2018;38(suppl 1):S20-S26. **5.** Bringmann A, et al. *Prog Retin Eye Res*. 2006;25:397-424. **6.** Rhee KD, et al. *Proc Natl Acad Sci U S A*. 2013;110:E4520-E4529.

Encapsulated Cell Therapy (ECT): Intravitreal Sustained CNTF Delivery System

- ECT enables continual release of CNTF to the retina over long periods of time, without triggering an immune response[1-3]
- Revakinagene taroretcel-lwey (formerly known as NT-501) is a first-in-class ECT[1,4,5]
  - Houses allogeneic RPE cells with an expression vector for CNTF release (NTC-201-6A cells),[1,5,6] which:
    - Release consistent levels of CNTF for long-term durations[1]
    - Remain alive and productive for many years[3]
    - Obtain oxygen and nutrients from vitreous[3]
    - Are sequestered from the immune system[3]
- **Revakinagene taroretcel-lwey was approved by the FDA for the treatment of adults with MacTel on March 5, 2025**



CNTF, ciliary neurotrophic factor; FDA, US Food and Drug Administration; MacTel, macular telangiectasia type 2; RPE, retinal pigment epithelium.

1. Kauper K, et al. *Invest Ophthalmol Vis Sci*. 2025;66:3. **2.** Wen R, et al. *Prog Retin Eye Res*. 2012;31:136-151. **3.** Tao W. *Expert Opin Biol Ther*. 2006;6:717-726. **4.** Chew EY, et al. *NEJM Evid*. 2025;4:EVIDoa2400481. **5.** Encelto [package insert]. Cumberland, RI: Neurotech Pharmaceuticals; 2025. **6.** Plakkot S, et al. Poster presented at: Association for Research in Vision and Ophthalmology; April 23–27, 2023; New Orleans, LA.

Revakinagene Taroretcel-lwey Has Been Studied Across 3 Randomized, Sham-Controlled Clinical Trials

	NTMT-02[a]	NTMT-03A[b]	NTMT-03B[c]
	67 participants 99 eyes[d] All-treated population	115 participants 115 eyes All-treated population	113 participants 113 eyes All-treated population
Key inclusion criteria	Aged >21 to <80 years; diagnosis of MacTel • **EZ break between 0.16 mm² and 4.00 mm²** • **BCVA of ≥64** ETDRS letters	• **EZ break between 0.16 mm² and 2.00 mm²** • **BCVA of ≥54** ETDRS letters	
Randomization	Treatment: 6 participants[d] Sham: 19 participants Treatment + sham: 32 participants	Treatment: 58 participants Sham: 57 participants	Treatment: 59 participants Sham: 54 participants
Primary endpoint	**Change from baseline** in EZ area loss at Month 24	**Rate of change from baseline** in EZ area loss through Month 24	
Secondary endpoints	Reading speed, aggregate retinal sensitivity (microperimetry), BCVA (safety)		

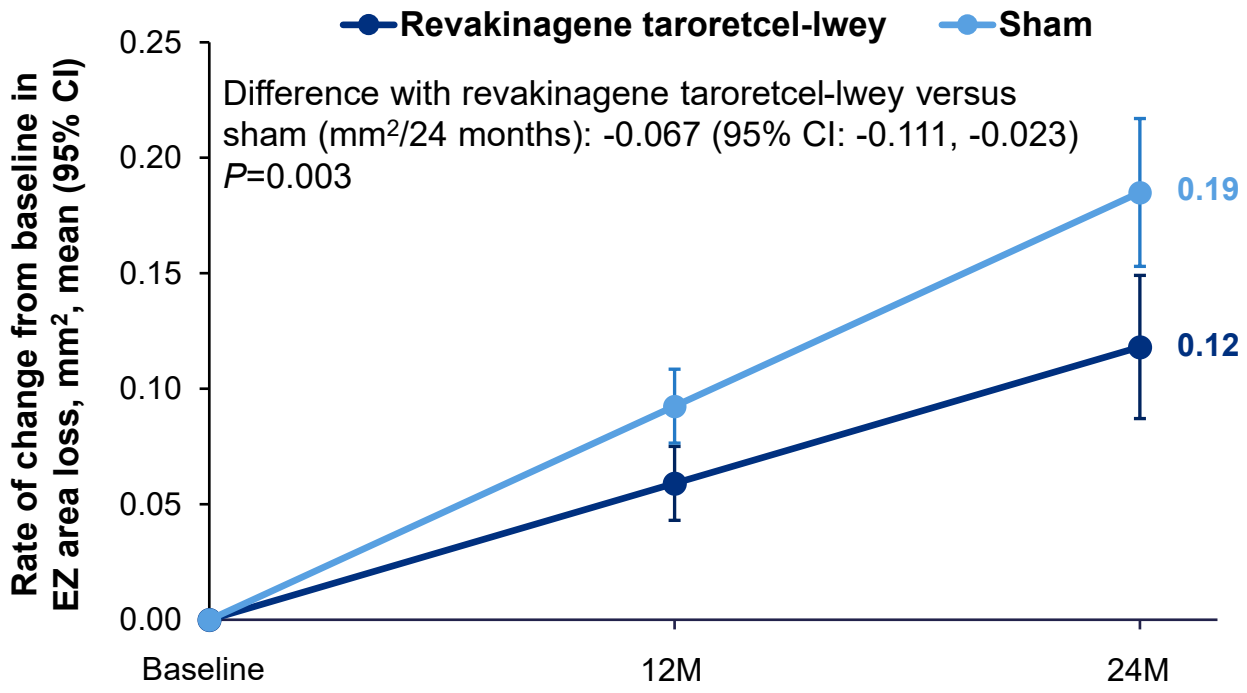
Each study lasted 24 months

BCVA, best corrected visual acuity; ETDRS, Early Treatment Diabetic Retinopathy Study; EZ, ellipsoid zone; MacTel, macular telangiectasia type 2.
[a]NCT01949324. [b]NCT03316300. [c]NCT03319849. [d]Participants with one eligible eye (35 participants) received revakinagene taroretcel-lwey (16 eyes) or sham (19 eyes). In participants with two eligible eyes (32 participants), one eye received revakinagene taroretcel-lwey (32 eyes) and one eye received sham procedure (32 eyes). If both eyes were eligible, the right eye was randomized 1:1 to sham or revakinagene taroretcel-lwey and the left eye received other surgery.

Revakinagene Taroretcel-lwey Demonstrated Greater Preservation of EZ Area Over 2 Years Compared With Sham in All-Treated Participants[a]

- A **7.7%** reduction in photoreceptor loss with revakinagene taroretcel-lwey compared with sham in **Phase 2**
- A **54.6%** reduction in photoreceptor loss with revakinagene taroretcel-lwey compared with sham in **Phase 3, Study A**
- A **30.4%** reduction in photoreceptor loss with revakinagene taroretcel-lwey compared with sham in **Phase 3, Study B**

Rate of Change in EZ Area Loss[b], mm²



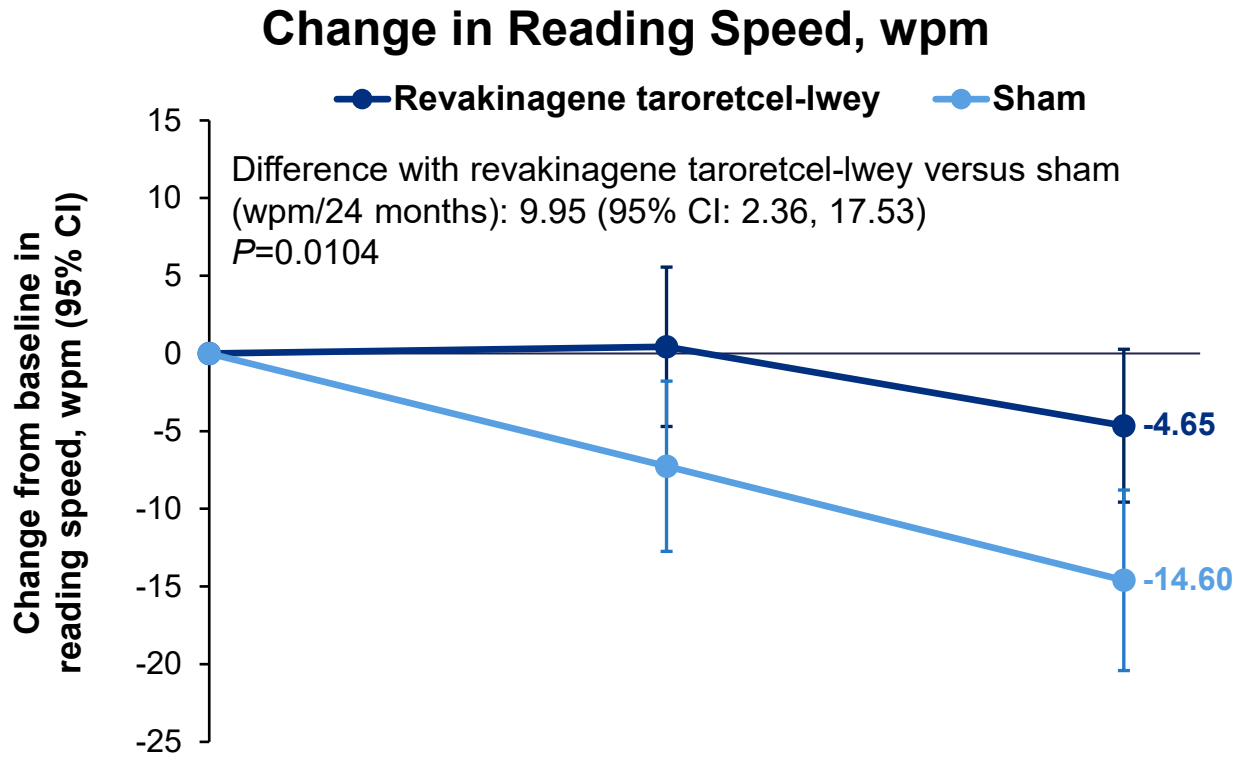
	Baseline	12M	24M
Revakinagene taroretcel-lwey , n	165	153	158
Sham, n	162	157	156

36.2% reduction in photoreceptor loss

CI, confidence interval; EZ, ellipsoid zone; M, month.
[a]Per the NTMT-02 study design, participants with two eligible study eyes received revakinagene taroretcel-lwey in one eye and sham in the other eye. These 32 participants are included in both groups for the pooled analysis, by study eye. [b]Rate of EZ change, difference, and CIs from a repeated measures model. The outcome variable is EZ area assessed longitudinally at baseline, Months 12, 16 (Phase 3 only), 18 (Phase 2 only), 20 (Phase 3 only), and 24. At baseline, EZ area is calculated as the mean area across two independent readers. The model includes treatment group, time (continuous), treatment × time interaction, and participant-specific random intercepts. The difference between treatment groups in rate of EZ change is estimated at Month 12 and Month 24 based on the treatment × time interaction term.

Revakinagene Taroretcel-lwey Preserved Reading Speed Over 2 Years Compared With Sham in All-Treated Participants[a]

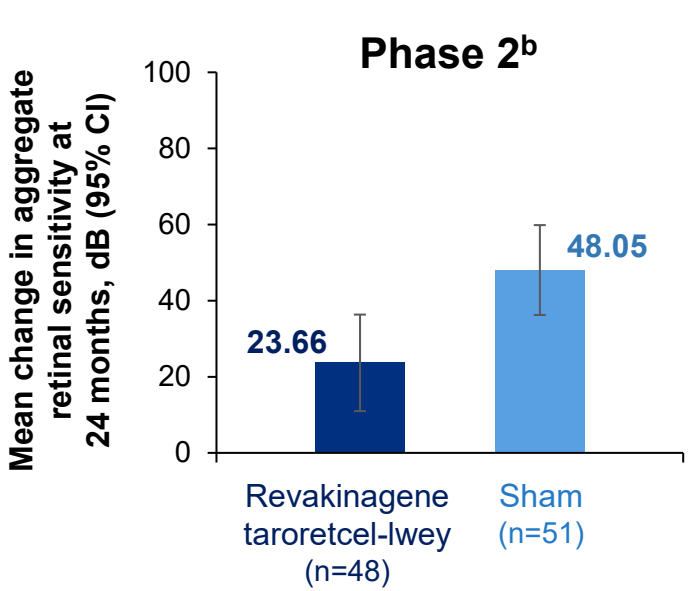
- An **86.1%** reduction in reading speed loss with revakinagene taroretcel-lwey compared with sham in **Phase 2**
- A **49.3%** reduction in reading speed loss with revakinagene taroretcel-lwey compared with sham in **Phase 3, Study A**
- A **71.0%** reduction in reading speed loss with revakinagene taroretcel-lwey compared with sham in **Phase 3, Study B**



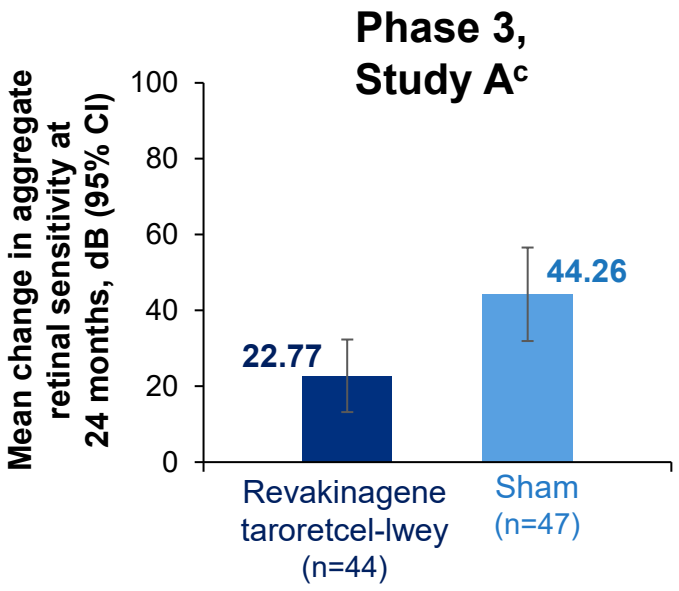
68.2% reduction in reading speed loss

CI, confidence interval; M, month; wpm, words per minute.
[a]Per the NTMT-02 study design, participants with two eligible study eyes received revakinagene taroretcel-lwey in one eye and sham in the other eye. These 32 participants are included in both groups for the pooled analysis, by study eye.

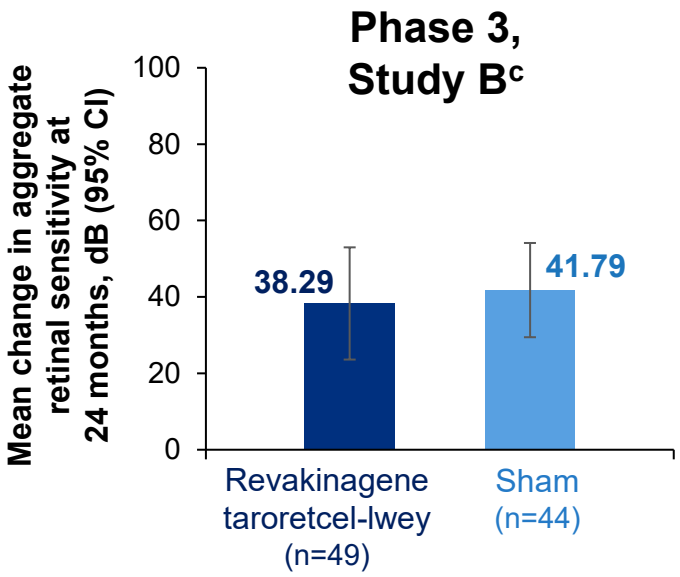
Revakinagene Taroretcel-lwey Preserved Aggregate Retinal Sensitivity (Microperimetry) Over 2 Years Compared With Sham[a]



A **50.7%** reduction in aggregate retinal sensitivity loss with revakinagene taroretcel-lwey compared with sham in NTMT-02



A **48.6%** reduction in aggregate retinal sensitivity loss with revakinagene taroretcel-lwey compared with sham in NTMT-03-A



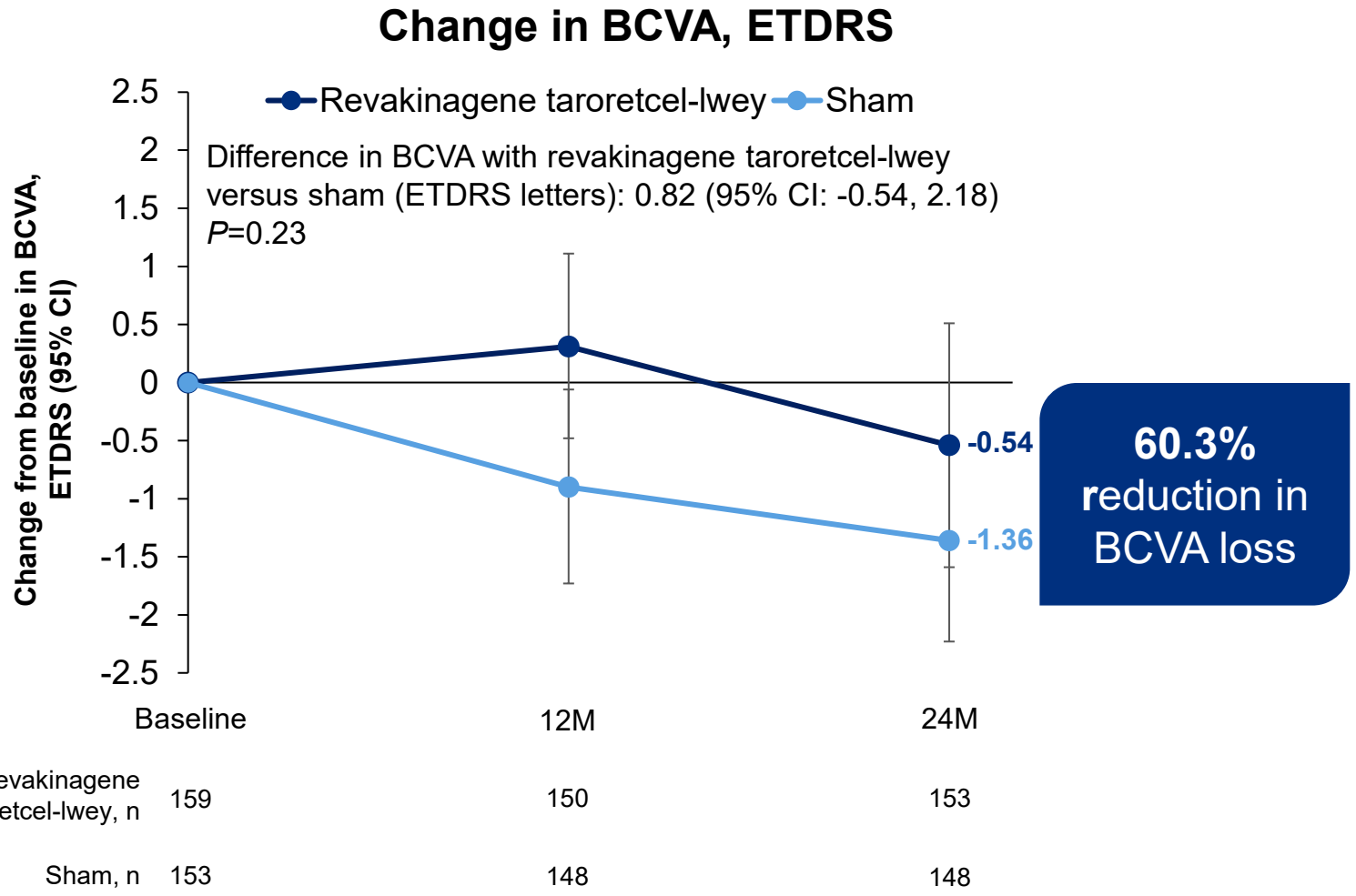
An **8.4%** reduction in aggregate retinal sensitivity loss with revakinagene taroretcel-lwey compared with sham in NTMT-03-B

36.6% reduction in aggregate retinal sensitivity loss across the three studies[d]

CI, confidence interval; dB, decibel; MAIA, Macular Integrity Assessment.
[a]Retinal sensitivity was measured via MAIA microperimetry. [b]In the Phase 2 study, retinal sensitivity is reported for the per-protocol population, which included all treated participants who had no major protocol infractions (defined prior to unmasking of the study). Per the NTMT-02 study design, participants with two eligible study eyes received revakinagene taroretcel-lwey in one eye and sham in the other eye. These 32 participants are included in both groups for the pooled analysis by study eye. [c]In the Phase 3 studies, the retinal sensitivity per-protocol population is reported, including all treated participants who had a baseline and Month 24 microperimetry collected according to study protocol. [d]Results per study in the respective per-protocol populations were weighted by the proportion of treated eyes with non-missing data in each study and combined descriptively.

BCVA Remained Stable for Both Treatment Arms Over 2 Years[a]

Mean change in BCVA from baseline to Month 24 (95% CI), ETDRS		
Study	**Revakinagene Taroretcel-lwey**	**Sham**
Phase 2	-1.93 (-3.80, -0.05)	-2.00 (-3.35, -0.65)
Phase 3, Study A	0.19 (-1.81, 2.20)	-0.57 (-2.26, 1.11)
Phase 3, Study B	-0.29 (-1.87, 1.30)	-1.71 (-3.11, -0.30)



60.3% reduction in BCVA loss

BCVA, best corrected visual acuity; CI, confidence interval; ETDRS, Early Treatment Diabetic Retinopathy Study; M, month.

[a]Per the NTMT-02 study design, participants with two eligible study eyes received revakinagene taroretcel-lwey in one eye and sham in the other eye. These 32 participants are included in both groups by study eye.

Conclusions

- Revakinagene taroretcel-lwey conferred both **anatomic and visual function benefits** across three randomized, sham-controlled studies
- Relative to sham, revakinagene taroretcel-lwey demonstrated a:
  - Preservation of anatomy
    - 36.2% reduction in photoreceptor loss
  - Preservation of function
    - 68.2% reduction in reading speed loss
    - 36.6% reduction in retinal sensitivity loss[a]

[a]Results per study in the respective per-protocol populations were weighted by the proportion of treated eyes with non-missing data in each study and combined descriptively.

Acknowledgments

- Lowy Medical Research Institute
- MacTel Project investigators and their research teams (in the Natural History Registry Study and the Phase 1, 2, and 3 clinical trials)
- Study participants with MacTel
- These trials were funded by Neurotech Pharmaceuticals, Inc
- These data were presented at American Academy of Ophthalmology Retina Subspecialty Day 2024; Chicago, IL; October 18–19, 2024
- Writing and editorial assistance was provided Elizabeth McSpiritt, MD, MPH, and Kristin Carlin, BS Pharm, RPh, of Peloton Advantage, LLC, an OPEN Health company, and was funded by Neurotech