Phase 3 DRAGON Trial in Stargardt Disease

Topline Results

December 1, 2025, 8:00 a.m. ET

Nasdaq: BLTE

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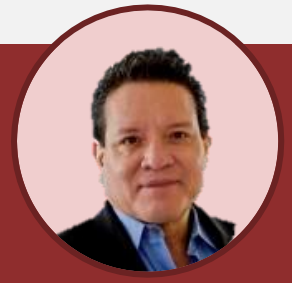
Today's Speakers

Belite Management

Tom Lin, MMED, PhD, MBA
Chairman & Chief Executive Officer

Hendrik Scholl, MD, MA
Chief Medical Officer



Nathan Mata, PhD
Chief Scientific Officer

Key Opinion Leaders

Prof Michel Michaelides, BSc MB BS MD(Res) FRCOphth FACS

- Consultant Ophthalmologist at Moorfields Eye Hospital in the Departments of Medical Retina, Inherited Eye Disease and Paediatric Ophthalmology
- Professor of Ophthalmology at the UCL Institute of Ophthalmology

Prof Leopold Schmetterer, PhD

- Head of Ocular Imaging and Scientific Director at Singapore Eye Research Institute
- Professor at the Nanyang Technological University
- DSMB Chair of the DRAGON study

Prof Ruifang (Helen) Sui, MD, PhD

- Professor, Director of the Department of Ophthalmology at Peking Union Medical College Hospital
- Vice President of the Chinese Medical Doctor Association Ophthalmology Committee & Counsellor of the Chinese Ophthalmology Genetics Alliance

Agenda

- Stargardt Disease Overview
- Study Design
- Efficacy Results
- Safety Results
- Summary
- KOL Panel Discussion
- Q&A

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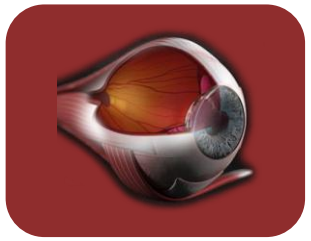
Stargardt Disease Overview

Stargardt Disease Overview



Stargardt disease (STGD1) and/or *ABCA4*-associated retinal dystrophy is a rare and progressive eye disease leading to legal blindness in almost all cases. There is no approved treatment for Stargardt disease

Pathophysiological Changes

- **Autosomal recessive mutations** in the ***ABCA4* gene**, impairing retinoid transport and leading to **toxic bisretinoid buildup** in the **retinal pigment epithelium (RPE)**
- **Bisretinoid accumulation manifests** as yellowish flecks underneath the retina and causes **oxidative stress, toxicity, and eventually RPE cell death**
- RPE damage leads to **secondary degeneration of macular photoreceptors**
- **Progressive macular atrophy** and **retinal degeneration**

Key Symptoms

- **Gradual central vision loss** in both eyes, often **starting in childhood or early adulthood**
- Blurry or **distorted** central **vision**
- **Central blind** spots (scotomas)
- **Photophobia** and **delayed dark adaptation**
- **Impaired color vision**
- **Difficulty** with **detailed tasks** like reading or recognizing faces
- **Peripheral vision preserved** in the majority of cases

Impact on Activities of Daily Living

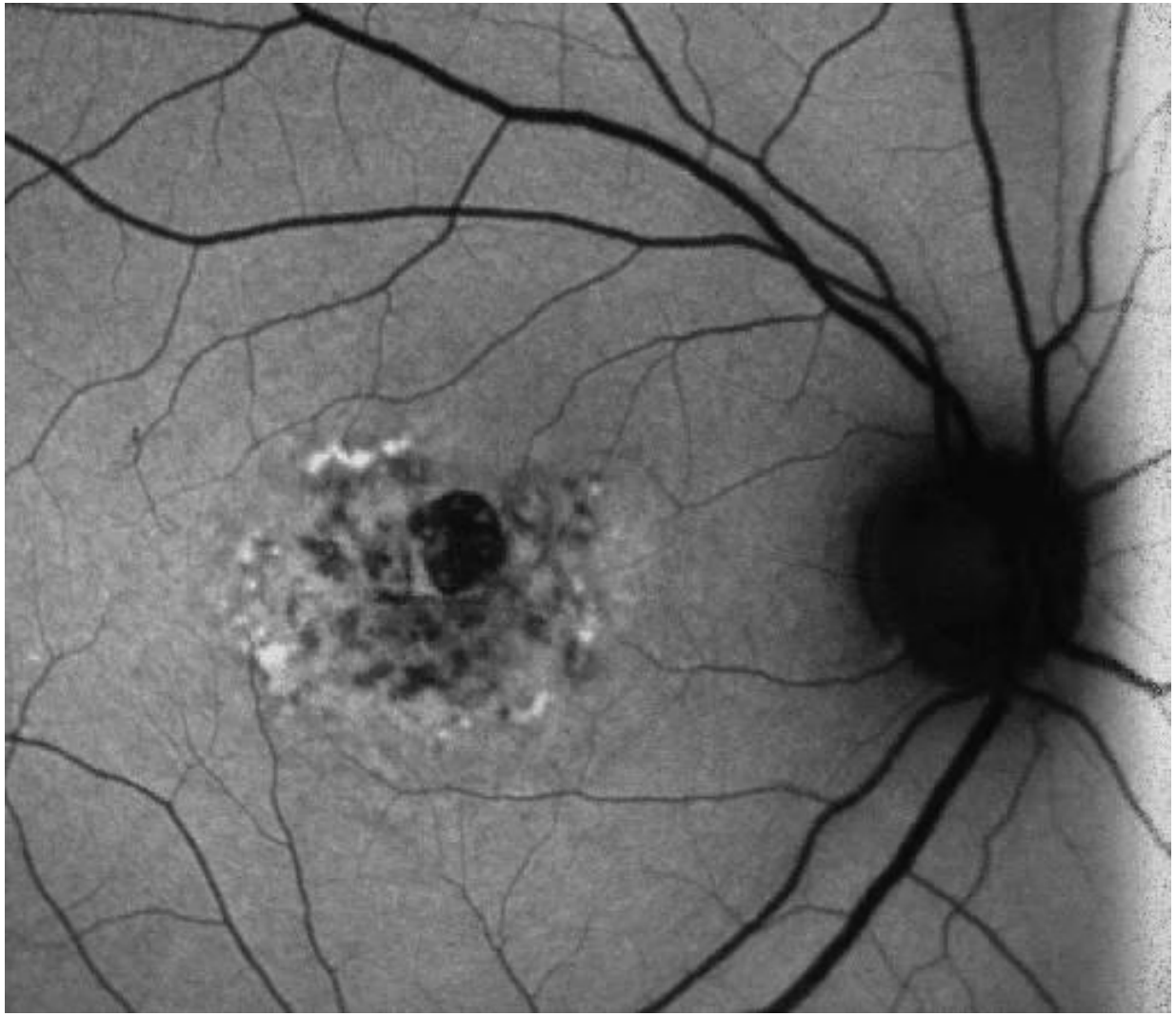
- **Challenges** with **reading**, using **screens**, and **recognizing faces**, harming education and work
- Typically, **inability to drive**, reducing mobility and independence
- **Restrictions in sports, leisure, and simple tasks** like avoiding trips
- **Social struggles**, including interactions, relationships, and events
- Emotional effects like **frustration, worry, anger, and depression**
- Career changes or **job loss**, affecting finances and productivity

Estimated Patient Population Sizes of Stargardt Disease

		Europe	Australia	China	Japan	United States
GnomAD Ethnicity		European	European	East Asian	East Asian	Mixed
Population (N)		742,000,000	26,640,000	1,411,000,000	124,500,000	334,900,000
STGD1 Patients (N)	*High Estimate*	**115,345**	**4,141**	**122,135**	**10,777**	**59,235**
	Low Estimate	**95,517**	**3,429**	**94,940**	**8,377**	**46,767**

Mata NL, Weng S, Michaelides M, Charbel Issa P, Quinodoz M, Rivolta C, Scholl HPN. Bisretinoids as a Source of Early Photoreceptor Pathology in Stargardt Disease. Ophthalmic Res. 2025 Nov 7:1-28. doi: 10.1159/000549368.

Standard of Care Fundus Autofluorescence Imaging in Stargardt Disease



Atrophy Progression over 4 Years

- 37-year-old male patient
- Compound heterozygous for mutations in *ABCA4*
  - Visual acuity (baseline): 20/40
  - Visual acuity (at 4Y): 20/80

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Study Design

Study Design

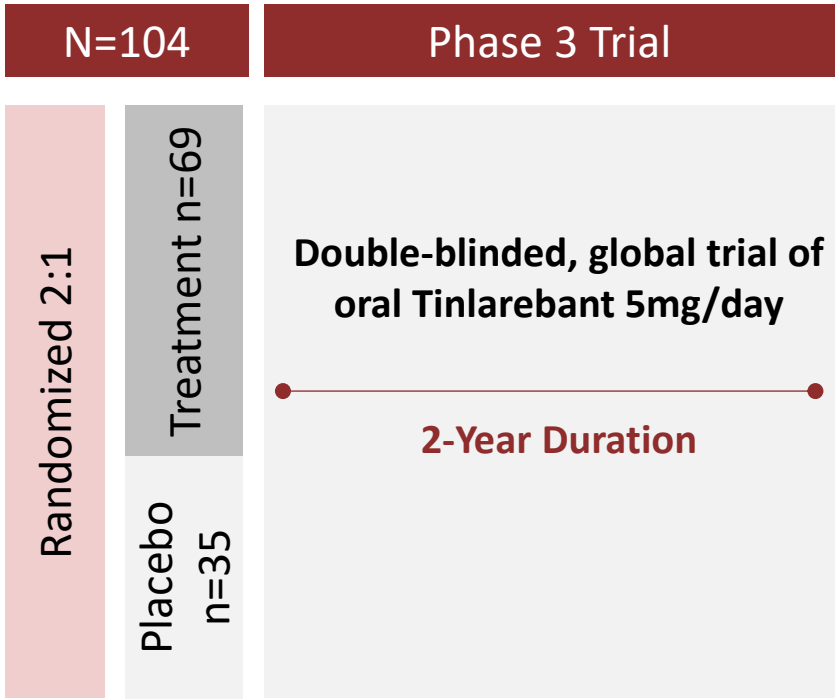
DRAGON Clinical Trial Design in Stargardt Disease

Reduction in atrophic lesion growth rate as measured by fundus autofluorescence imaging is the FDA's accepted primary endpoint in Stargardt disease and geographic atrophy secondary to age-related macular degeneration

DRAGON Design

Key Inclusion Criteria

- Clinical diagnosis of Stargardt disease
- 12-20 years old
- ≥1 mutation identified in the *ABCA4* gene
- Atrophic lesion size (DDAF) within 3 disc areas (7.62 mm^2)
- BCVA of 20/200 or better



Primary Measures

- *Slowing of atrophic lesion growth (DDAF)*
- *Safety and tolerability*

Secondary Measures

- *DAF*
- *BCVA*
- *SD-OCT*
- *Microperimetry*

DDAF = Definitely Decreased Autofluorescence; DAF = Decreased Autofluorescence; BCVA = Best-Corrected Visual Acuity; SD-OCT = Spectral-Domain Optical Coherence Tomography

Study Design

DRAGON Baseline DDAF Lesion Size

Baseline DDAF (mm^2) – Study Eye

	Tinlarebant (5mg)	Placebo
N	63	33
Mean	2.251	2.418
SD	2.0871	2.2050
Median	1.546	1.822
Min – Max	0.07 – 7.02	0.06 – 7.29

Baseline DDAF (mm^2) – Fellow Eye

	Tinlarebant (5mg)	Placebo
N	57	29
Mean	2.511	3.020
SD	2.0263	2.3029
Median	1.946	2.287
Min - Max	0.07 – 7.55	0.25 – 7.54

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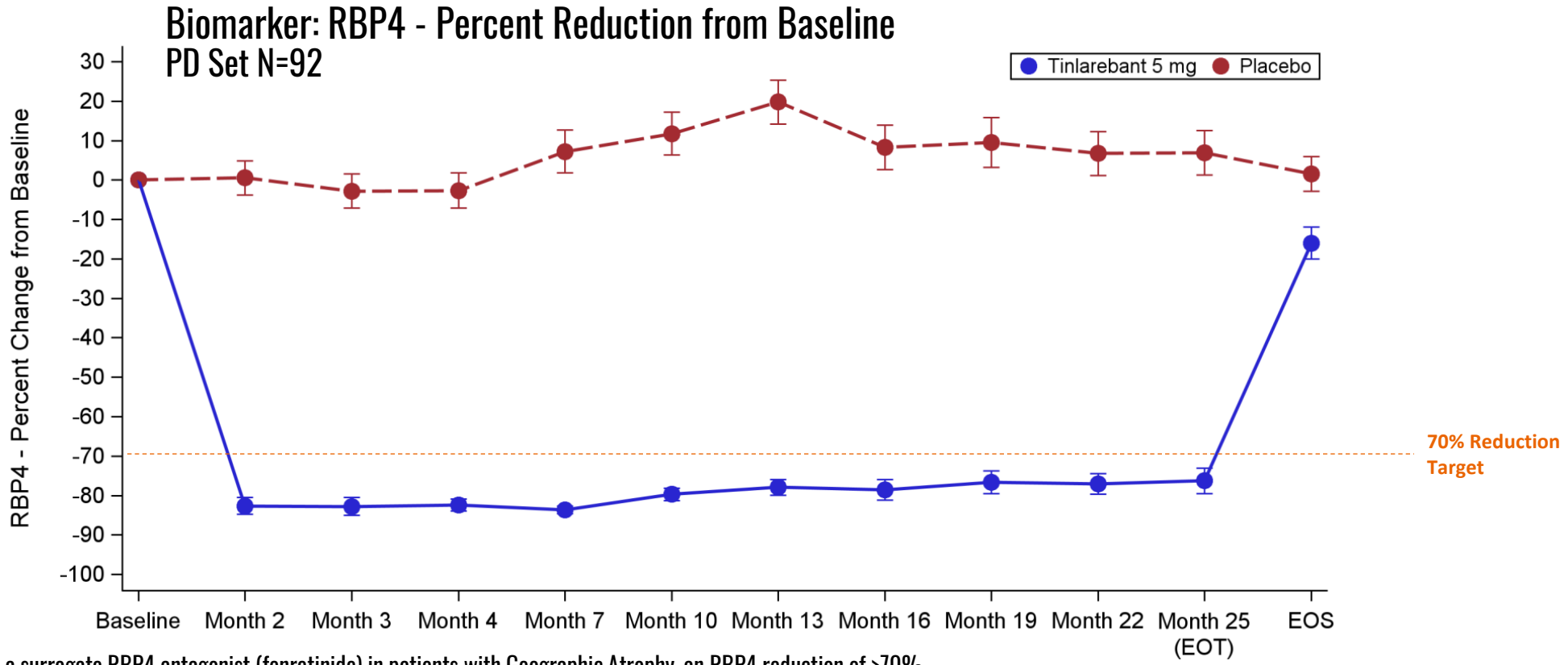
Efficacy Results

Efficacy Results

Tinlarebant Treatment Led to 80% Reduction in RPB4, Well Above Goal of 70%*

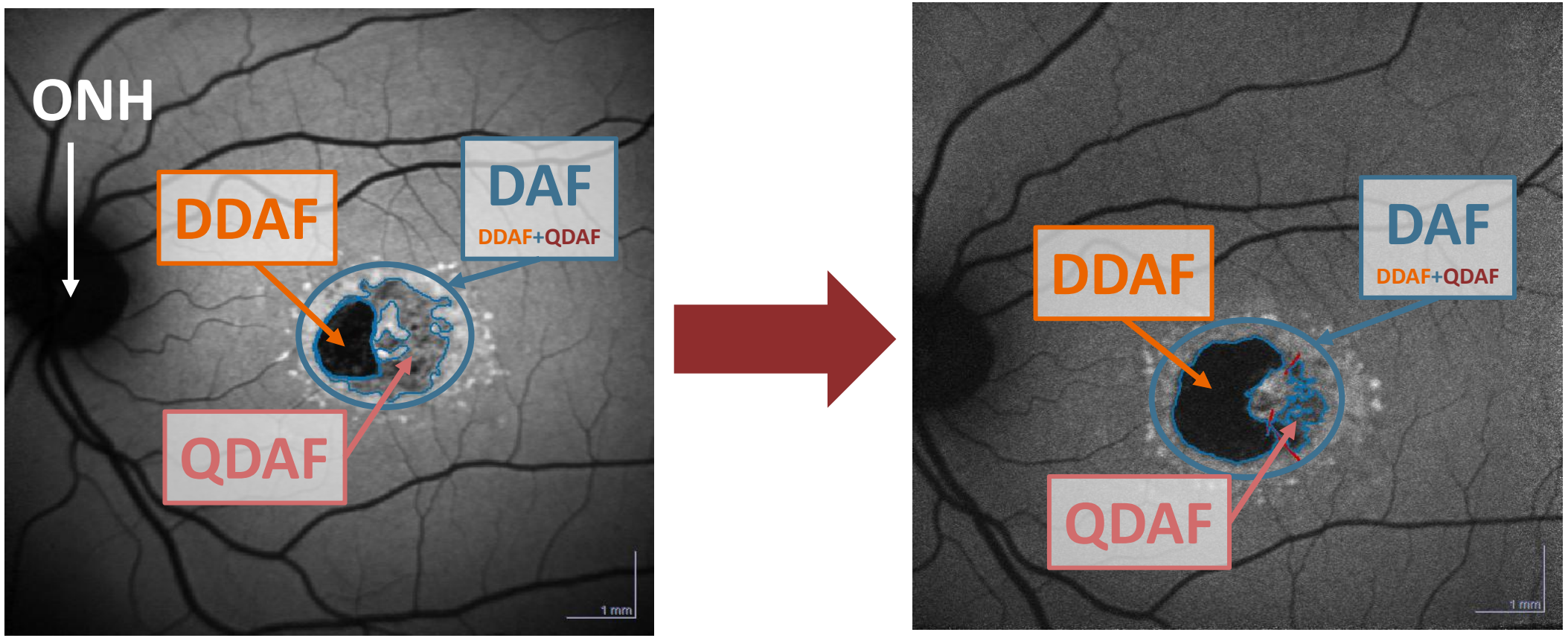


* In a prior study of a surrogate RBP4 antagonist (fenretinide) in patients with Geographic Atrophy, an RBP4 reduction of ≥70% was associated with a statistically significant slowing of lesion growth [Mata et al., Retina. 2013; 33(3): 498-507.]

Daily dosing of 5 mg/day Tinlarebant led to a sustained 80% reduction of RPB4 and RPB4 levels returned to 84 % of the baseline value at the End of Study (EOS)

DDAF Represents Well-demarcated Areas of Complete RPE Loss & Grows Predictably, Making it an Approvable Primary Endpoint

- **DDAF** (definitely decreased autofluorescence): level of darkness close to 100% (at least 90%) in reference to the ONH
- **QDAF** (questionably decreased autofluorescence): between 50% and 90% darkness
- **DAF** (decreased autofluorescence): the sum of DDAF and QDAF



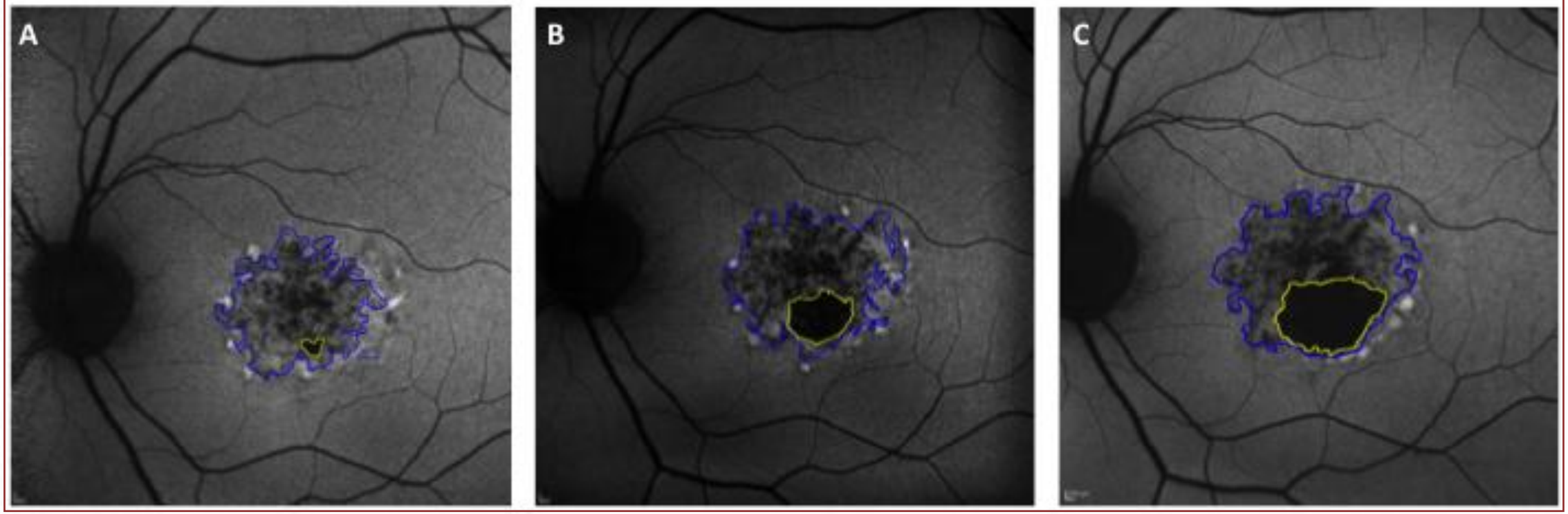
Strauss RW, Ho A, Muñoz B, Cideciyan AV, Sahel JA, Sunness JS, Birch DG, Bernstein PS, Michaelides M, Traboulsi EI, Zrenner E, Sadda S, Ervin AM, West S, Scholl HPN; Progression of Stargardt Disease Study Group (2016) The Natural History of the Progression of Atrophy Secondary to Stargardt Disease (ProgStar) Studies: Design and Baseline Characteristics: ProgStar Report No. 1. Ophthalmology. 123: 817-28.

DDAF Progression Rate in Stargardt

Natural History of the Progression of Atrophy Secondary to Stargardt Disease (ProgStar) study

Overall DDAF growth rate in the ProgStar cohort over 24 months: 0.74 mm^2/year

(confidence interval: 0.64 - 0.85 mm^2/year)



Strauss RW, Ho A, Jha A, Fujinami K, Michaelides M, Cideciyan AV, Audo I, Birch DG, Sadda S, Ip M, West S, Schönbach EM, Kong X, Scholl HPN; ProgStar Study Group (2023) The progression of Stargardt Disease as determined by fundus autofluorescence over a 24-month period (ProgStar Report No. 17). Am J Ophthalmol. 250: 157-170.

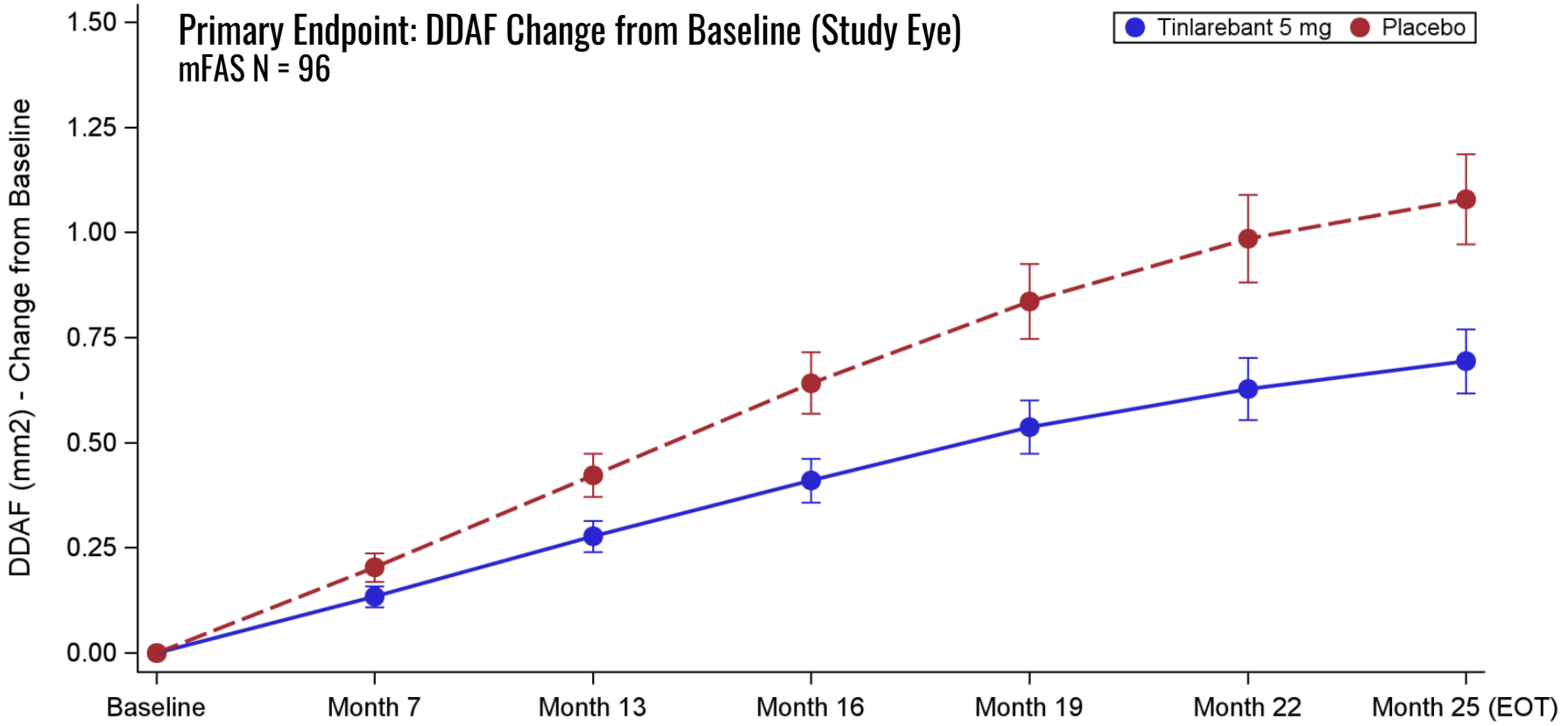
Efficacy Results

Primary Endpoint: DDAF in the Study Eye (Change from Baseline)

- Annualized rate of lesion growth in the aggregate area of atrophy (DDAF) from baseline as assessed by fundus autofluorescence imaging at Month 25.
- Data is shown for the modified full analysis set (mFAS) which consists of all subjects who were randomly assigned to receive study drug and have received at least one dose of study medication. In addition, the mFAS subjects must have a defined DDAF lesion meeting the eligibility criteria at baseline and have at least one post baseline assessment.
- Data analysis used a Mixed Model for Repeated Measures (MMRM) measuring change from baseline in DDAF in the study eye and including terms for treatment, visit, treatment*visit interaction, baseline focality of lesions, and baseline DDAF lesion size.
- The Statistical Analysis Plan (SAP) specified an unstructured covariance matrix for the MMRM. The CRO also performed a post-hoc analysis using a first-order autoregressive covariance matrix to account for the longitudinal nature of the data while maintaining model stability in a relatively small sample such as in the DRAGON trial.

Efficacy Results

Primary Endpoint Showed a Statistically Significant & Clinically Meaningful Outcome



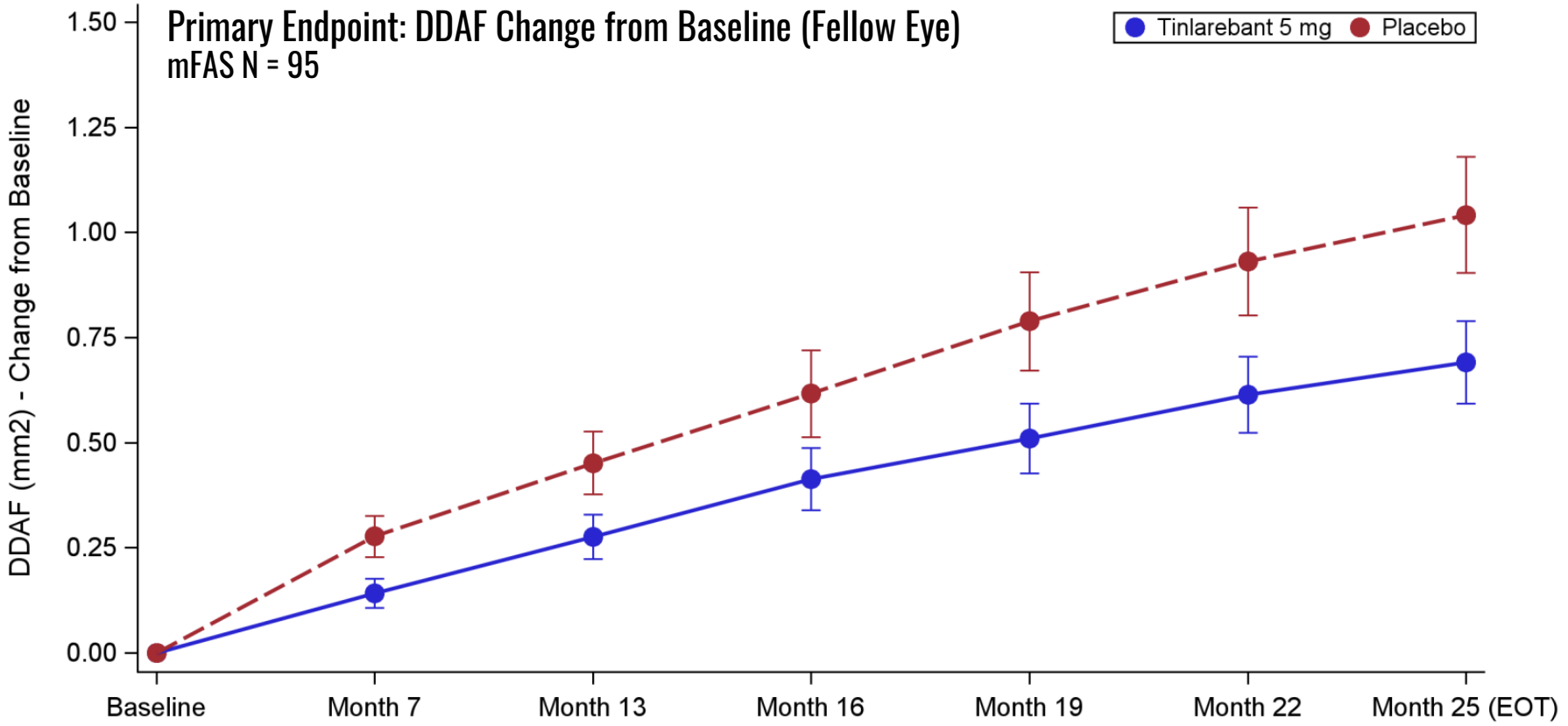
Applying an unstructured covariance matrix, the **treatment effect size was 35.7%** compared to placebo and yielded a **p-value of P = 0.0033**

With a first-order autoregressive covariance matrix, the **treatment effect size remained consistent (35.4%)** with **P <0.0001**

DDAF lesion growth was **slowed to 0.38 mm²/year vs. 0.59 mm²/year for placebo and 0.74 mm²/year observed in ProgStar**

Efficacy Results

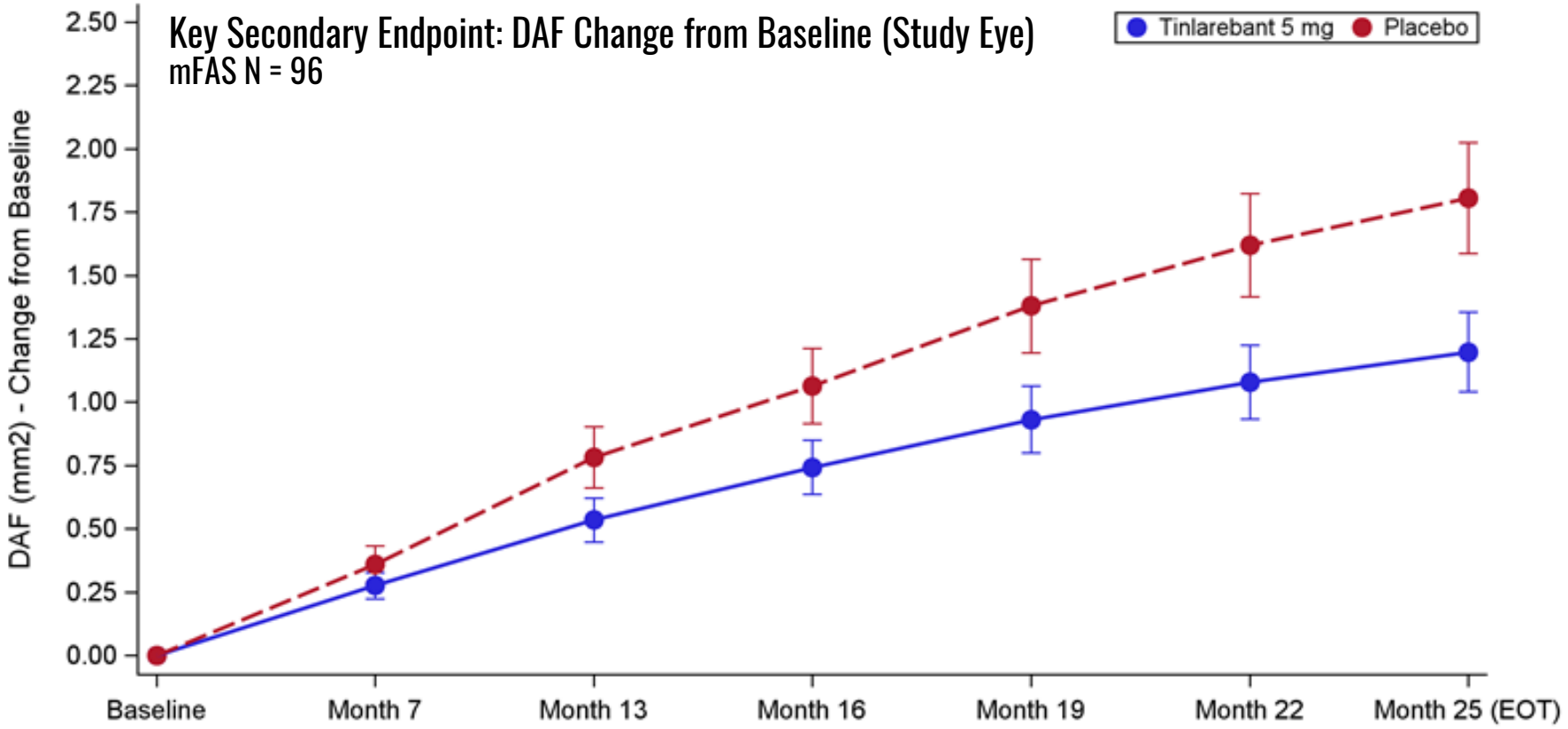
A Statistically Significant Treatment Effect Was Also Observed in the Fellow Eye for the Primary Endpoint



Tinlarebant slowed DDAF lesion growth in the fellow eye by 33.6% compared to placebo (P = 0.041)

Efficacy Results

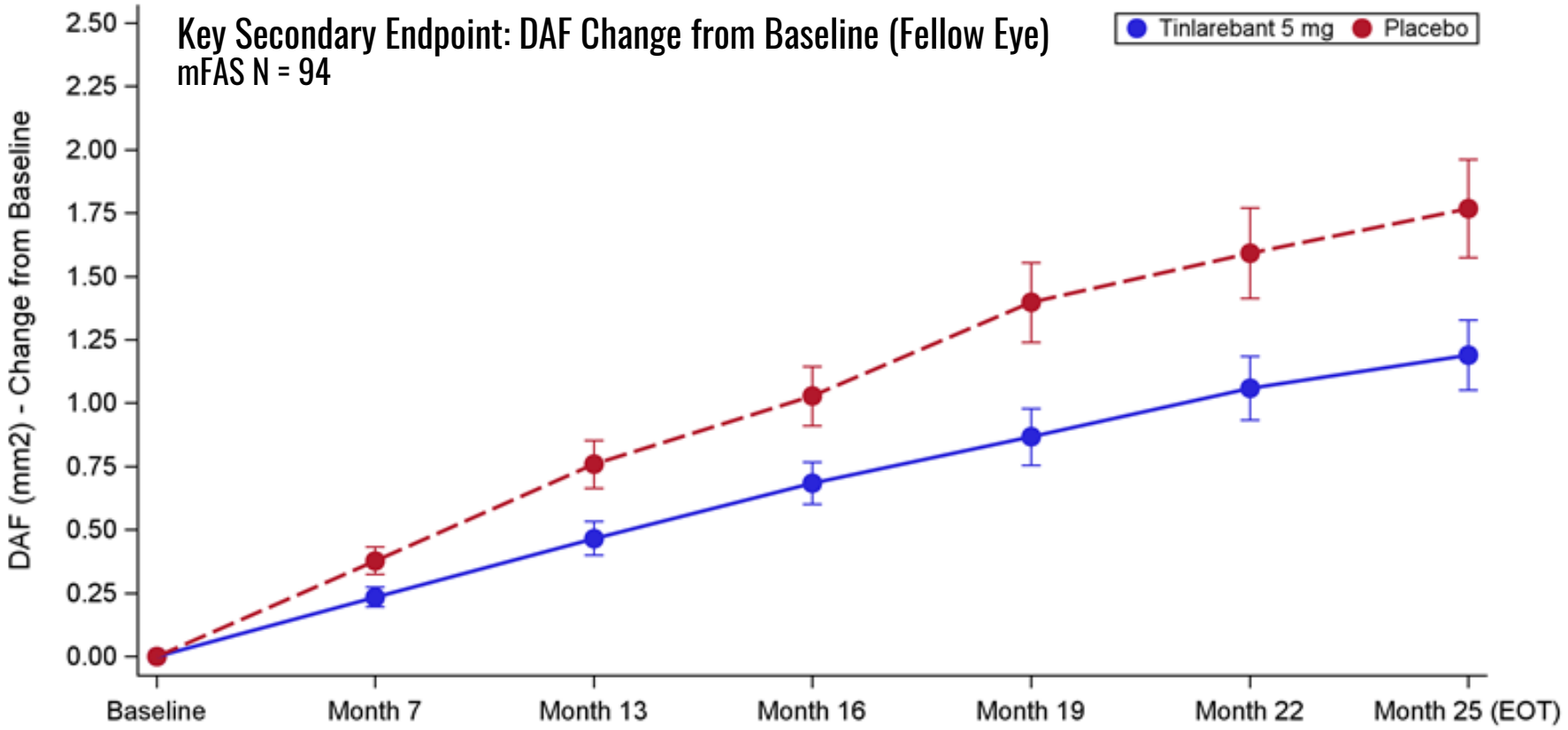
Tinlarebant Slowed DAF Lesion Growth, the Key Secondary Endpoint, in the Study Eye by 33.7%



Tinlarebant slowed DAF lesion growth by 33.7% compared to placebo (P = 0.027)

Efficacy Results

Tinlarebant Slowed DAF Lesion Growth, the Key Secondary Endpoint, also in the Fellow Eye by 32.7%



Tinlarebant slowed DAF lesion growth in the fellow eye by 32.7% compared to placebo (P = 0.017)

Efficacy Results

As Expected, BCVA in Study Eye Did Not Show Any Significant Change

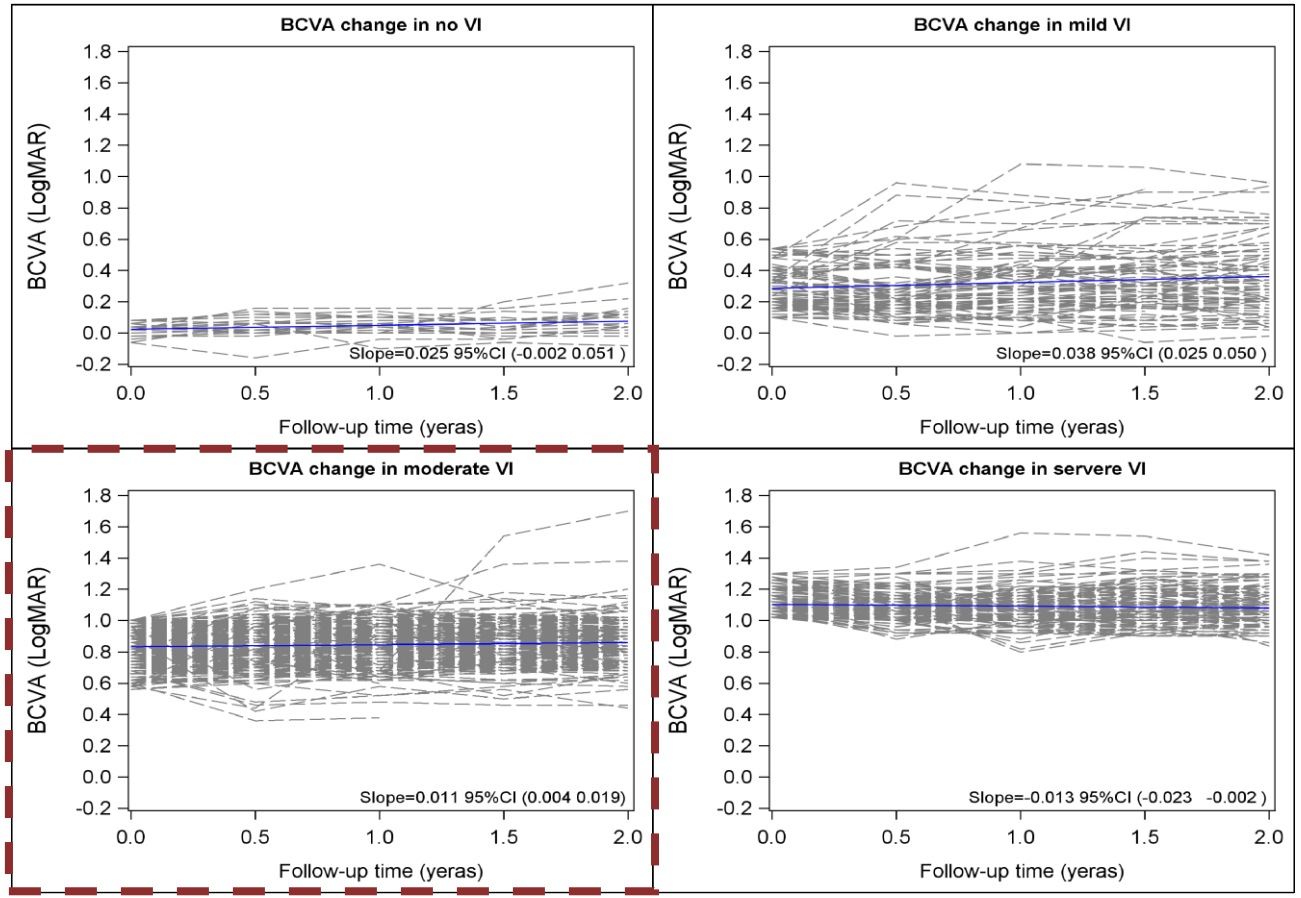
	Tinlarebant	Placebo
BCVA at Baseline	39.9	39.4
BCVA at EOS	39.7	40.0

- The overall change of visual acuity was minimal over the period of 24 months in both treatment groups
- Test–retest variability for ETDRS change scores in Stargardt disease are known to yield a repeatability coefficient ≈ 8 letters (1)
- Such minor changes in average visual acuity over two years are in line with the natural history of Stargardt disease and were observed in the ProgStar Study

(1) Parker MA, Choi D, Erker LR, Pennesi ME, Yang P, Chegarnov EN, Steinkamp PN, Schlechter CL, Dhaenens CM, Mohand-Said S, Audo I, Sahel J, Weleber RG, Wilson DJ. Test-Retest Variability of Functional and Structural Parameters in Patients with Stargardt Disease Participating in the SAR422459 Gene Therapy Trial. Transl Vis Sci Technol. 2016 Oct 1;5(5):10.

ProgStar: Visual Acuity Change over 24 Months

Prospective Cohort (N=434)



- **Overall rate of BCVA loss was 0.55 letters/year over two years**
- **BCVA of eyes with baseline BCVA between 20/70 and 20/200 declined at a rate of 0.6 letters/year**

Kong X, Fujinami K, Strauss RW, Munoz B, West SK, Cideciyan AV, Michaelides M, Sahel JA, Ahmed M, Ervin AM, Schönbach EM, Cheetham JK, Scholl HPN; ProgStar Study Group (2018) Visual Acuity Change over 24 Months and its Association With Foveal Phenotype and Genotype in Individuals With Stargardt Disease: ProgStar Report Number 10. JAMA Ophthalmol. 136: 920-928.

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Safety Results

Safety Results

Tinlarebant Demonstrated a Well Tolerated Safety Profile

Safety Set N = 104

Subjects Who Experienced at Least One Non-Ocular Treatment-Emergent Adverse Events (TEAE), N / (%)

Category	Tinlarebant 5mg (N=69)	Placebo (N=35)
TEAE	59 (85.5%)	27 (77.1%)
Severe TEAE	2 (2.9%)	1 (2.9%)
Serious TEAE	2 (2.9%)	4 (11.4%)
Study Drug-Related TEAE	14 (20.3%)	4 (11.4%)
Study Drug-Related Serious TEAE	0 (0.0%)	0 (0.0%)
TEAE Leading to Study Drug Discontinuation	0 (0.0%)	0 (0.0%)
TEAE Leading to Study Discontinuation	0 (0.0%)	0 (0.0%)
TEAE Leading to Death	0 (0.0%)	0 (0.0%)

- Total of 6 serious adverse events (SAEs) reported in the study – all events were non-ocular, with 4 assessed as unrelated and 2 assessed as unlikely related to the study treatment
- Most reported Non-Ocular adverse events (AEs): Nasopharyngitis (all cases were assessed as unrelated/unlikely related to treatment), Headache, and Acne – most events were mild and resolved during the study period

Safety Results

The Majority of Ocular Adverse Events was Mild

Safety Set N = 104

Subjects Who Experience at Least One Ocular TEAE, N / (%)

Category	Tinlarebant 5mg (N=69)	Placebo (N=35)
TEAE	53 (76.8%)	8 (22.9%)
Severe TEAE	2 (2.9%)	0 (0.0%)
Serious TEAE	0 (0.0%)	0 (0.0%)
Study Drug-Related TEAE	49 (71.0%)	8 (22.9%)
Study Drug-Related Serious TEAE	0 (0.0%)	0 (0.0%)
TEAE Leading to Study Drug Discontinuation	4 (5.8%)	0 (0.0%)
TEAE Leading to Study Discontinuation	2 (2.9%)	0 (0.0%)
TEAE Leading to Death	0 (0.0%)	0 (0.0%)

- Most reported Ocular AEs: Xanthopsia, Delayed dark adaptation, and Night Vision Impairment
- The majority of the events were mild, and most resolved while on study
- There were no serious ocular TEAEs – 4 TEAEs lead to study drug discontinuation and 2 TEAEs lead to study discontinuation

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Summary

Summary

Summary of Results of the DRAGON Trial

- **The DRAGON trial met its primary endpoint:** A highly statistically significant slowing in DDAF lesion growth was observed in subjects treated with 5 mg/day oral Tinlarebant as compared to placebo
- **The treatment effect was 36%** and must be considered **clinically meaningful**
- The observed treatment effect was **supported by the fellow eye data** and the **key secondary endpoint: a reduction of DAF area growth**
- The **change in best-corrected visual acuity was minimal** in both the treatment and the placebo group – and is **in-line with natural history data**
- The biomarker of tinlarebant treatment, **RBP4 reduction, showed a sustained 80% reduction with very little variability**
- **Tinlarebant (5 mg p.o., daily) was well tolerated** in adolescent STGD1 patients

Conclusions

Tinlarebant has the Potential to be the First-Ever Approved Treatment for Stargardt Disease

- First-ever oral therapy in a retinal degenerative disease to demonstrate a **clinically meaningful slowdown of neurodegeneration**
- **36% reduction in DDAF lesion growth rate**, representing a robust and reproducible treatment effect in Stargardt disease
- **Excellent safety and tolerability profile** across two years of treatment
- **Addresses the root pathogenic mechanism** (bisretinoid accumulation), offering a rational, disease-modifying approach where no approved therapies currently exist
- **Broad applicability across disease stages**, from early *ABCA4*-mediated changes to more advanced atrophy
- **Personal clinical impact**: After >20 years caring for these patients, this represents a true game changer – a therapy I would confidently offer to all my Stargardt patients*

* Quoted from Dr. Hendrik Scholl, Chief Medical Officer of Belite Bio, based on his personal experience as an ophthalmologist treating STGD1 patients.

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KOL Panel Discussion

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Q&A

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