



# Early Intervention with an Oral Treatment for Macular Degeneration

Mission for Vision

Nasdaq: BLTE

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# Belite Bio Executive Team



## Belite Management Team



**Tom Lin, MMED, PhD, MBA**  
(Chairman, CEO)

- 16+ years of executive management roles in biotech
- Over 10 new drug developments in multiple therapeutic areas including ophthalmology
- University of Sydney, University of Melbourne, Harvard Medical School, Columbia University, London Business School, Hong Kong University



**Hendrik Scholl, MD, MA**  
(CMO)

- 25+ years of expertise in treating retinal diseases, including Stargardt disease and AMD
- Coordinating principal investigator of the largest natural history study of Stargardt disease (ProgStar Study)
- Participated in over 10 clinical studies both in Stargardt disease and AMD, over 280 publications in peer-reviewed journals
- University Eye Hospital Tübingen, University Eye Hospital Bonn, Wilmer Eye Institute at Johns Hopkins, University Eye Hospital Basel, Medical University of Vienna



**Nathan Mata, PhD**  
(CSO)

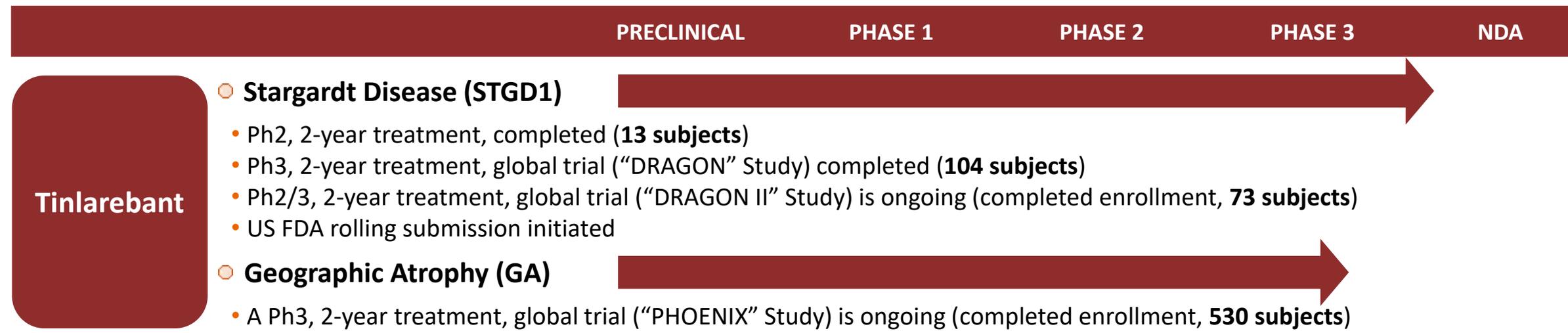
- 15+ years of ophthalmic drug development experience across numerous indications, including two NDAs (Durezol and Zirgan)
- Led clinical development efforts for the first RBP antagonist in advanced dry AMD and the first visual cycle modulator in dry AMD and STGD1
- Introduced the industry's first STGD1 ABCA4 knockout mice model
- University of Texas



**Hao-Yuan Chuang, CFA, MBA, FRM**  
(CFO)

- 16+ years of capital market experience; closed more than US\$32 billion of transactions
- Wanda, Suning, CITIC Securities
- Columbia University, London Business School, Hong Kong University

# Belite Bio Pipeline Overview



- **Tinarebant** is a **novel, once daily oral tablet** designed to bind to serum **retinol binding protein 4 (RBP4)** as a means to specifically reduce retinol delivery to the eye. This approach is intended to **slow or halt the formation of the toxic retinol-derived by-products** that are generated in the visual cycle and are **implicated in progression of STGD1 and GA**.
- Belite Bio believes that **intervention directed at a root cause of retinal pathology**, namely bisretinoid-mediated toxicity but not complement activation, will be the best approach to potentially slow disease progression in STGD1 & GA.
- **Unmet Market Opportunity:**
  - No FDA approved treatments for STGD1
  - No FDA approved orally administered treatments for GA
- **Breakthrough Therapy, Fast Track, and Rare Pediatric Disease Designation** in US and **Orphan Drug** designation in US / EU / JP, **Pioneer Drug** designation in JP, for STGD1
- **14 active patent families**; composition of matter patent until at least **2040** without patent term extension



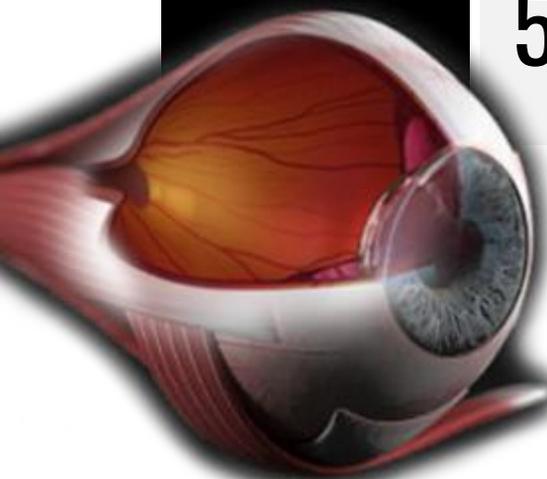
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# Tinlarebant Overview



# Tinlarebant

- DISCOVERY
- PRE-CLINICAL
- PHASE I
- PHASE II
- **PHASE III**
- MARKET



# Market Opportunity

## STARGARDT

**1 in 8,800** <sup>(1,2)</sup>  
 The most common inherited macular dystrophy

Patient population with Stargardt Disease:

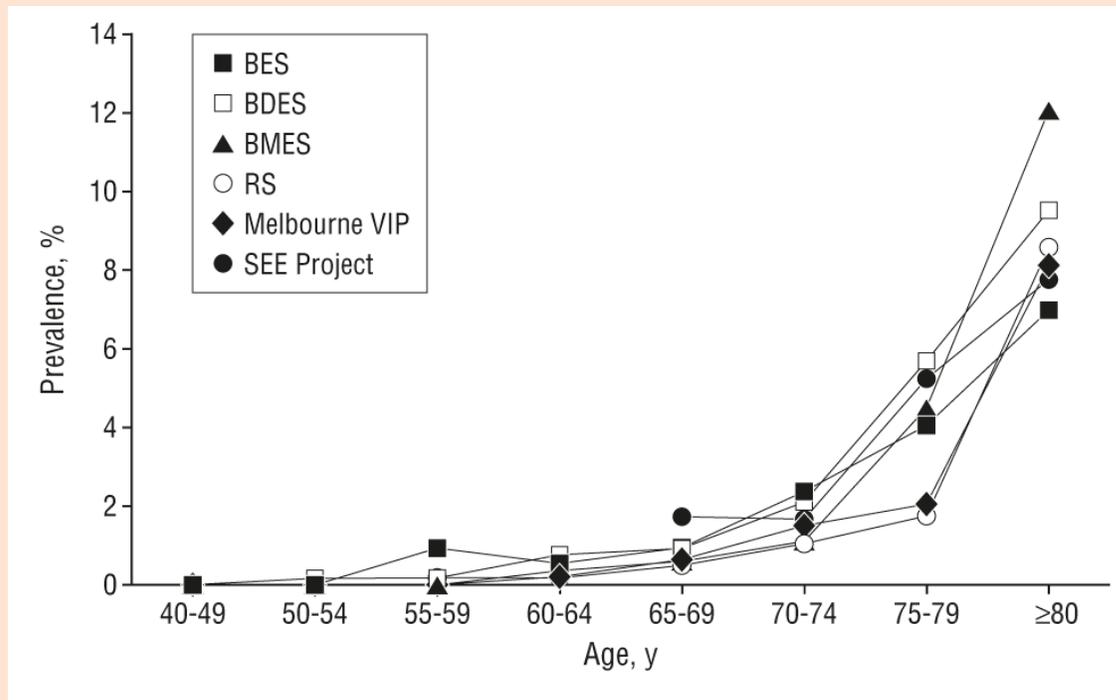
**53k** <sup>(2)</sup>  
 US

**109k** <sup>(2)</sup>  
 China

## Columbia University + NIH Blueprint

“a promising first-in-class oral medication intended to slow or halt the progression of dry AMD”

## GEOGRAPHIC ATROPHY



**An estimated 973,000 people in the United States<sup>(3)</sup> and approximately 5 million globally<sup>(4)</sup> have GA in at least one eye.**

References: (1) Hanany M, Rivolta C, Sharon D (2020) Proc Natl Acad Sci U S A. 117(5):2710-2716. (2) Mata NL et al. (2025) Ophthalmic Res. 68(1):555-572. (3) Wong WL et al. (2014) Lancet Glob Health. 2(2):e106-16. (4) Friedman DS et al. (2004) Arch Ophthalmol. 122(4):564-72.

# Normal Processing of Vitamin A in the Visual Cycle

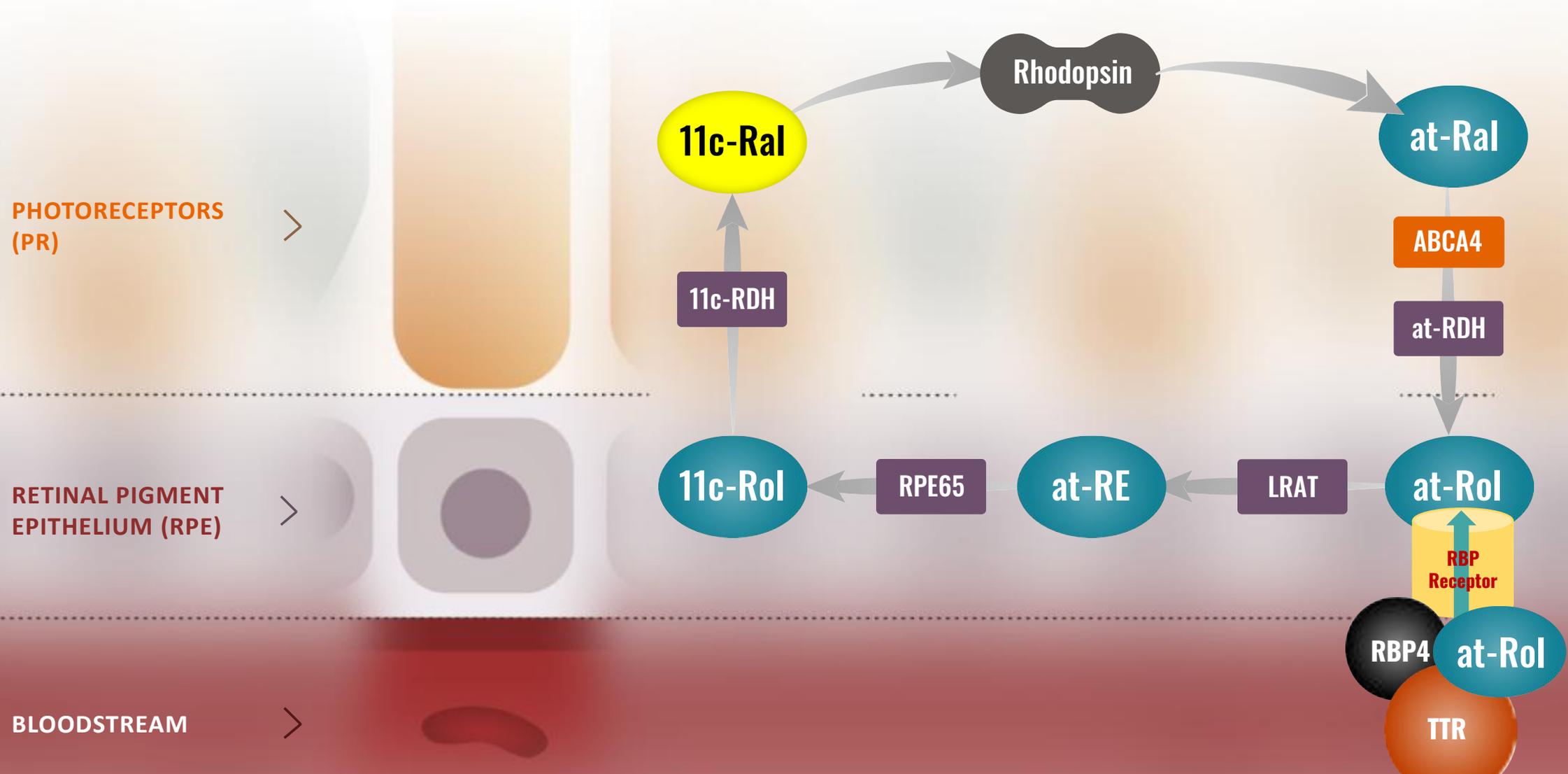
⬇️ Tinlarebant Induced Down-Regulation

■ Enzymes

■ Visual Pigment

● Retinoids

● Visual Chromophore



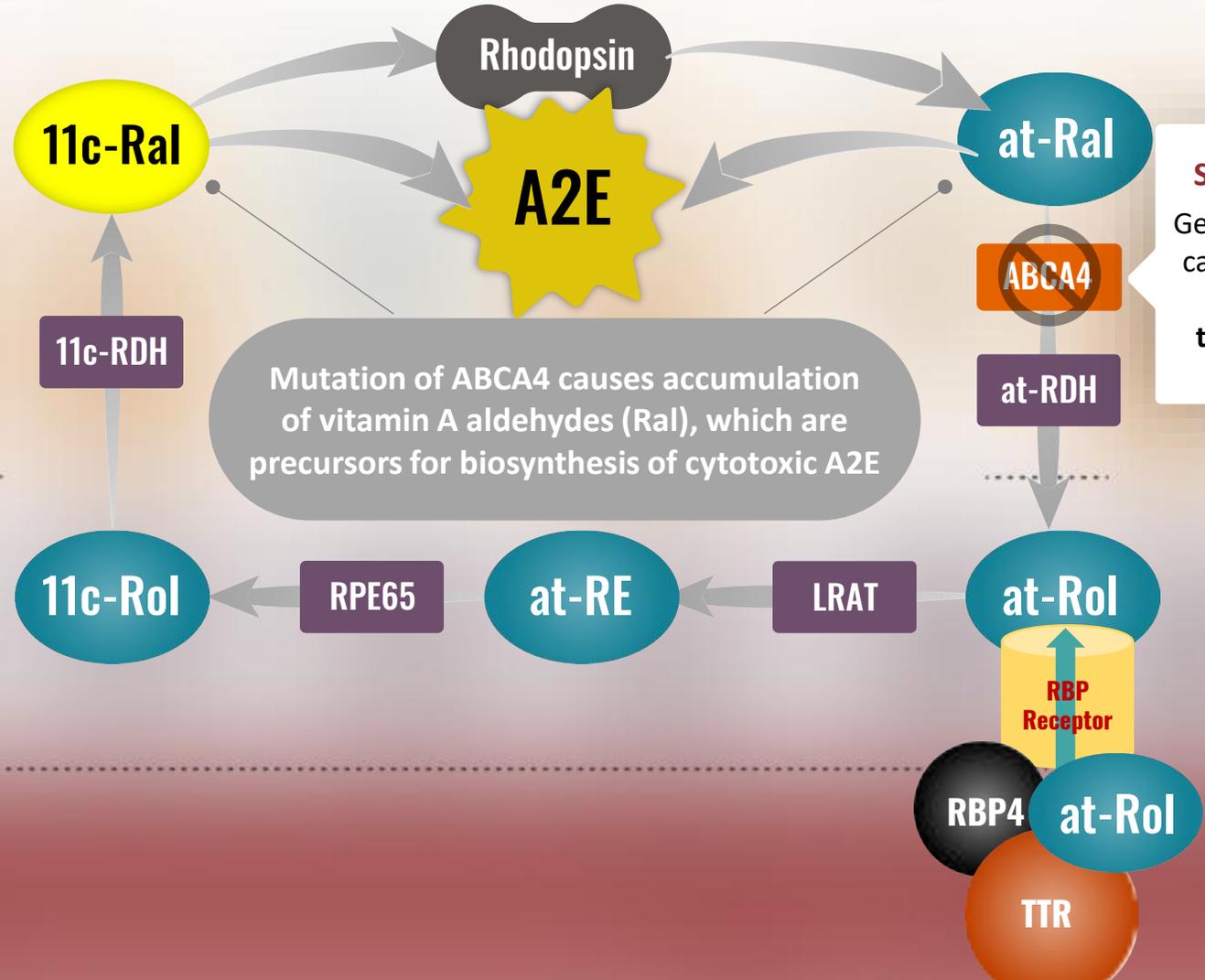
# Formation of Toxic Vitamin A Byproducts in Stargardt Disease 1 (STGD1)

- Tinlarebant Induced Down-Regulation
- Enzymes
- Visual Pigment
- Retinoids
- Visual Chromophore

PHOTORECEPTORS (PR)

RETINAL PIGMENT EPITHELIUM (RPE)

BLOODSTREAM



**STARGARDT 1**  
Gene mutation causes loss of ABCA4 transporter function

# Mechanism of Tinlarebant Action

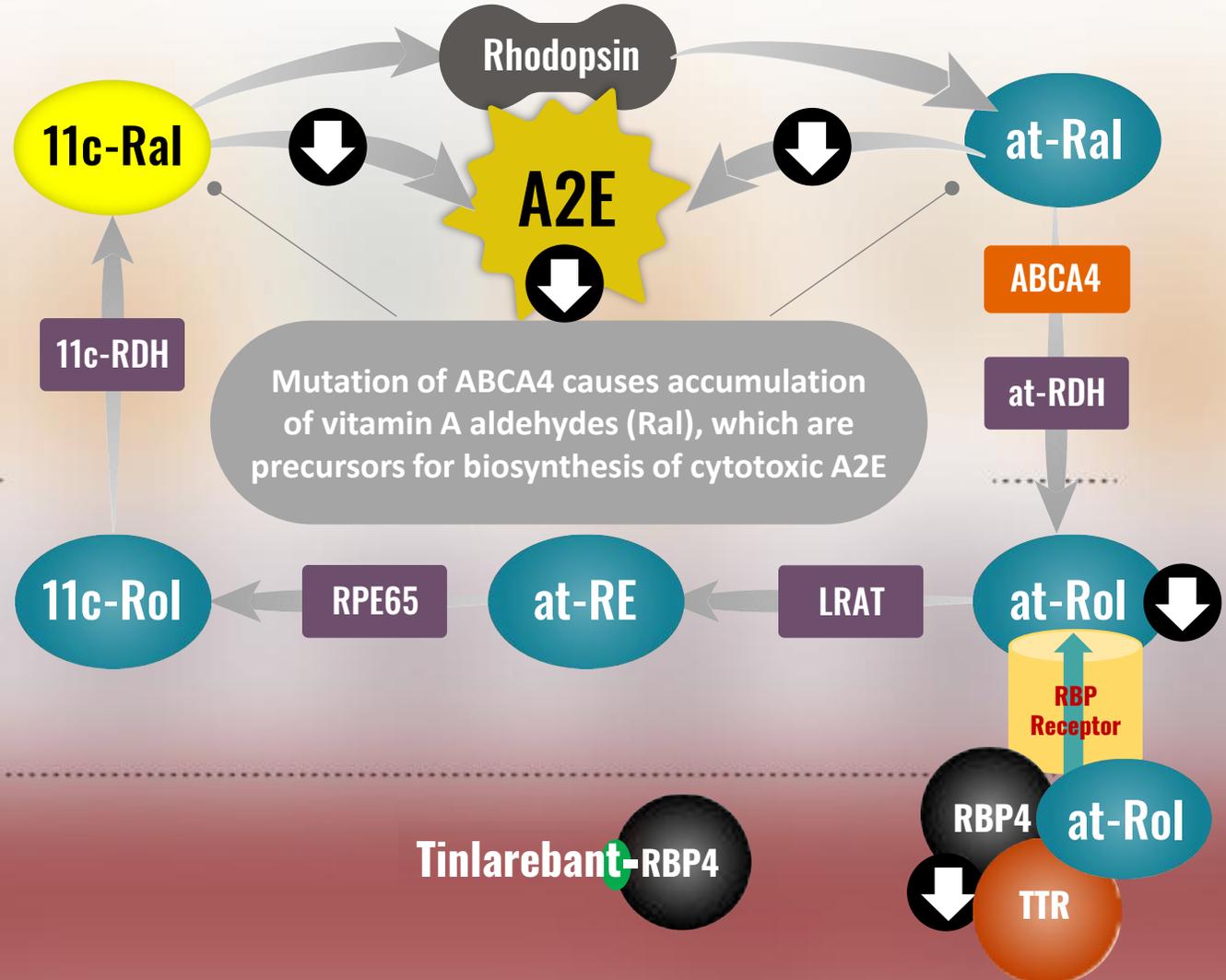
Bisretinoids are derived from vitamin A (retinol). Therefore, reducing the delivery of retinol to the eye is expected to reduce bisretinoid levels in the eye leading to preservation of the retina

- Tinlarebant Induced Down-Regulation
- Enzymes
- Visual Pigment
- Retinoids
- Visual Chromophore

PHOTORECEPTORS (PR)

RETINAL PIGMENT EPITHELIUM (RPE)

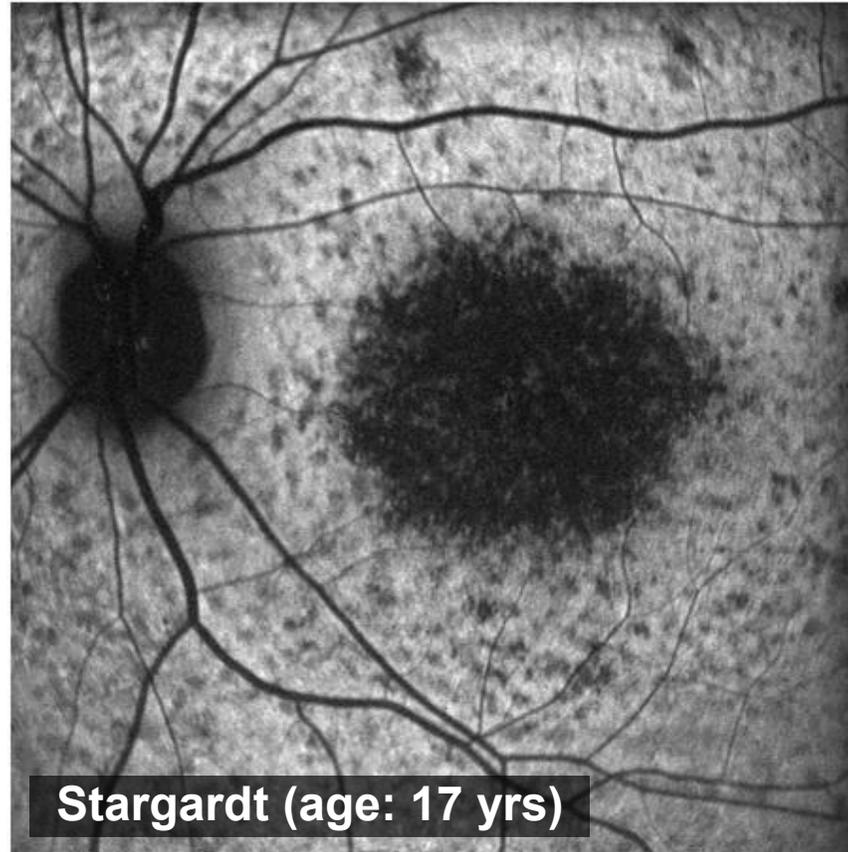
BLOODSTREAM



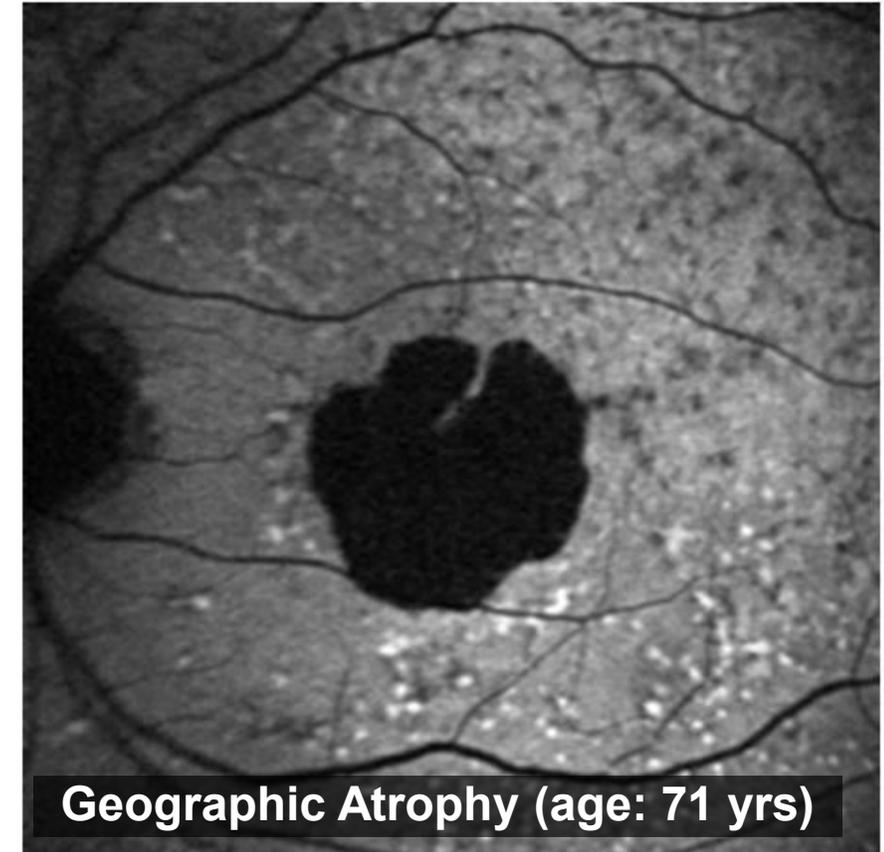
# Similar Pathology in STGD1 & GA



- **STGD1 and GA show a very similar phenotype**, where loss of RPE and photoreceptors in the center of the retina, the macula, leads to loss of vision
- Fundus autofluorescence imaging, visualizing bisretinoid content in the RPE, shows **loss of signal in degenerated areas** and **increase in signal peripheral to lesions**
- **Slowing or halting the spread of retinal lesions is the intended effect of Tinlarebant treatment**



**Stargardt (age: 17 yrs)**



**Geographic Atrophy (age: 71 yrs)**

Reference: Scholl HPN, Fleckenstein M, Charbel Issa P, Keilhauer C, Holz FG, Weber BHF (2007). An update on the genetics of age-related macular degeneration. *Mol Vis* 13: 196-205.



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# DRAGON Clinical Trial

# DRAGON & DRAGON II Clinical Trial Design in STGD1

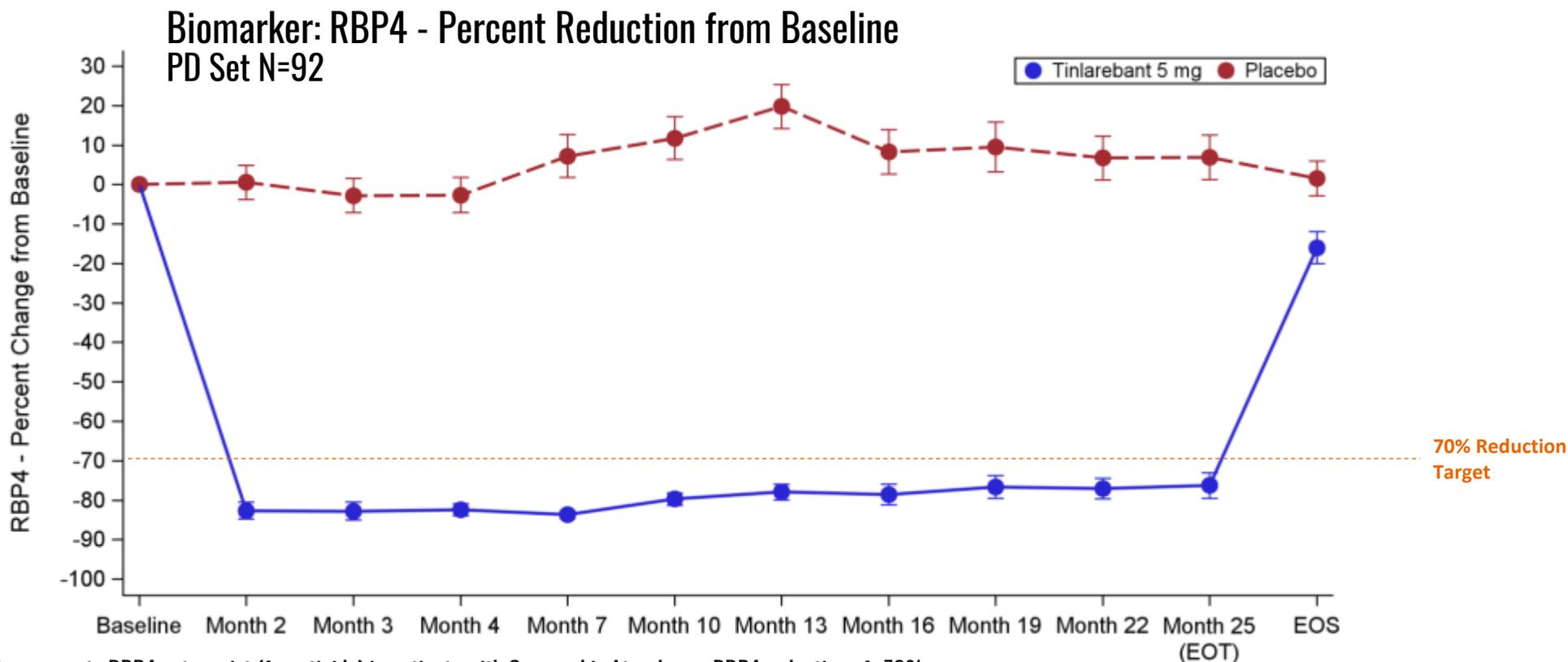


Reduction in lesion growth rate (DDAF) as measured by retinal imaging is the FDA accepted primary endpoint in STGD1 and GA

	STGD1 “DRAGON” Phase 3 <sup>(1)</sup>	STGD1 “DRAGON II” Phase 1b/2/3
<b>Enrollment</b>	104 subjects (have DDAF)	60 subjects (have DDAF)
<b>Sites</b>	Global	Japan, US, UK
<b>Randomization</b>	2:1 ratio (Tinarebant : Placebo)	1:1 ratio (Tinarebant : Placebo)
<b>Masking</b>	Double Blind	
<b>Treatment duration</b>	2 years	
<b>Primary measures</b>	Efficacy as measured through DDAF lesion growth rate, safety & tolerability	
<b>Other measures</b>	QDAF, BCVA, SD-OCT, microperimetry	
<b>Interim analysis</b>	Yes	
<b>Key inclusion criteria</b>	12-20 years old, diagnosed STGD1 with at least 1 mutation identified in the ABCA4 gene, atrophic lesion size within 3 disc areas (7.62 mm <sup>2</sup> ), a BCVA of 20/200 or better	

<sup>(1)</sup> FDA may require another clinical trial depending on the data from the ongoing Phase 3 study.

# Tinlarebant Treatment Led to 80% Reduction in RBP4, Well Above Goal of 70%\*



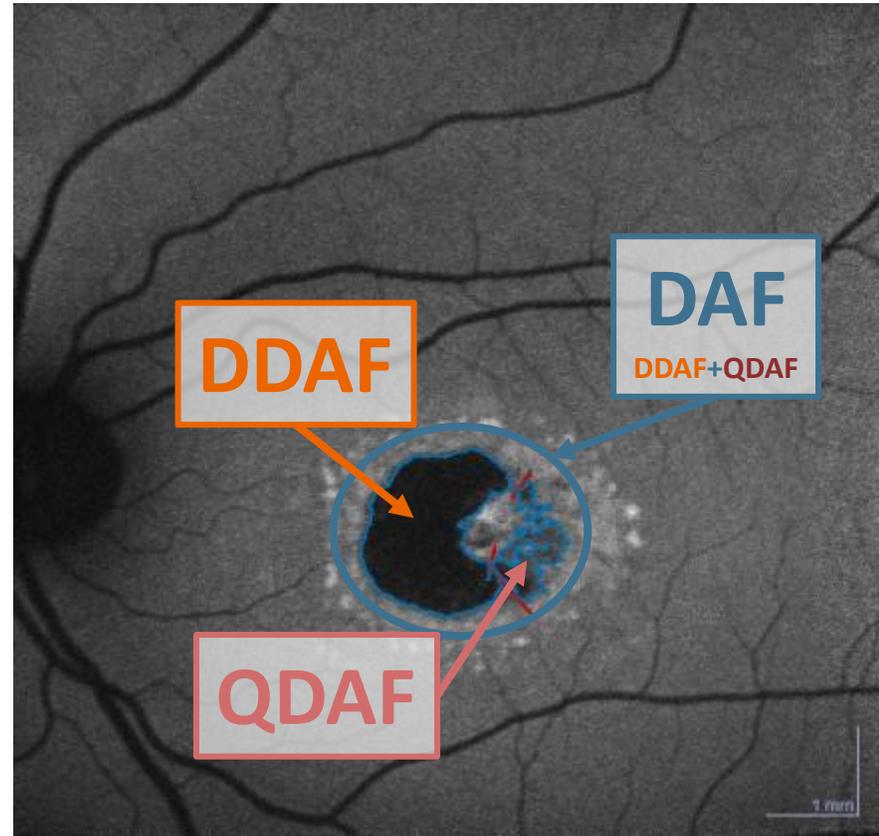
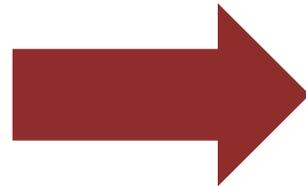
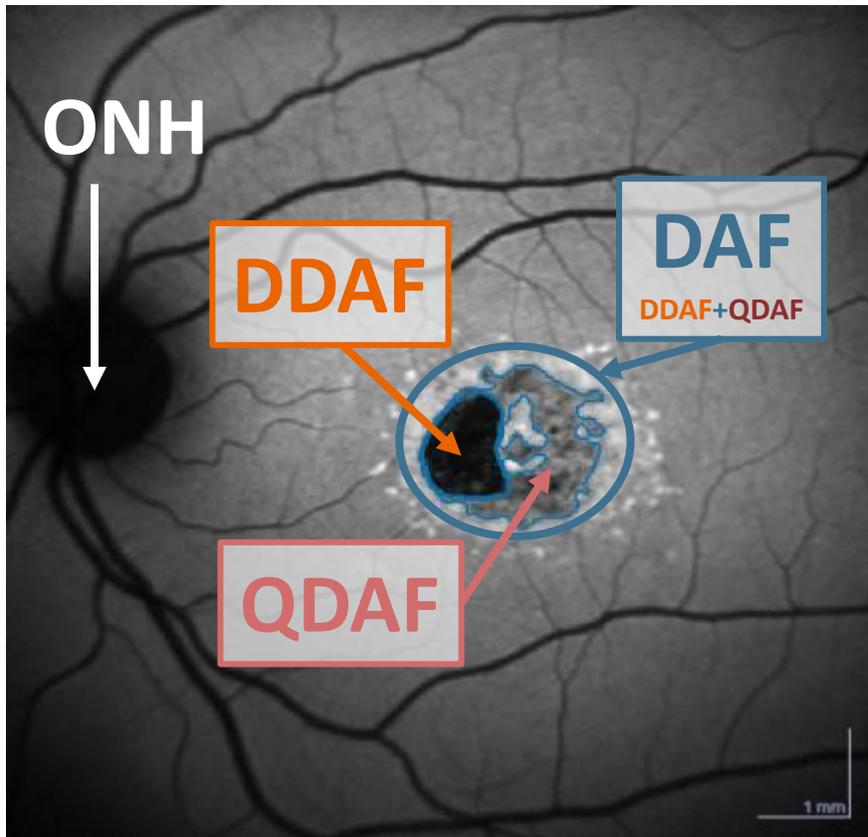
\* In a prior study of a surrogate RBP4 antagonist (fenretinide) in patients with Geographic Atrophy, an RBP4 reduction of  $\geq 70\%$  was associated with a statistically significant slowing of lesion growth [Mata et al., Retina. 2013; 33(3): 498-507.]

Daily dosing of 5 mg/day Tinlarebant led to a sustained 80% reduction of RBP4 and RBP4 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



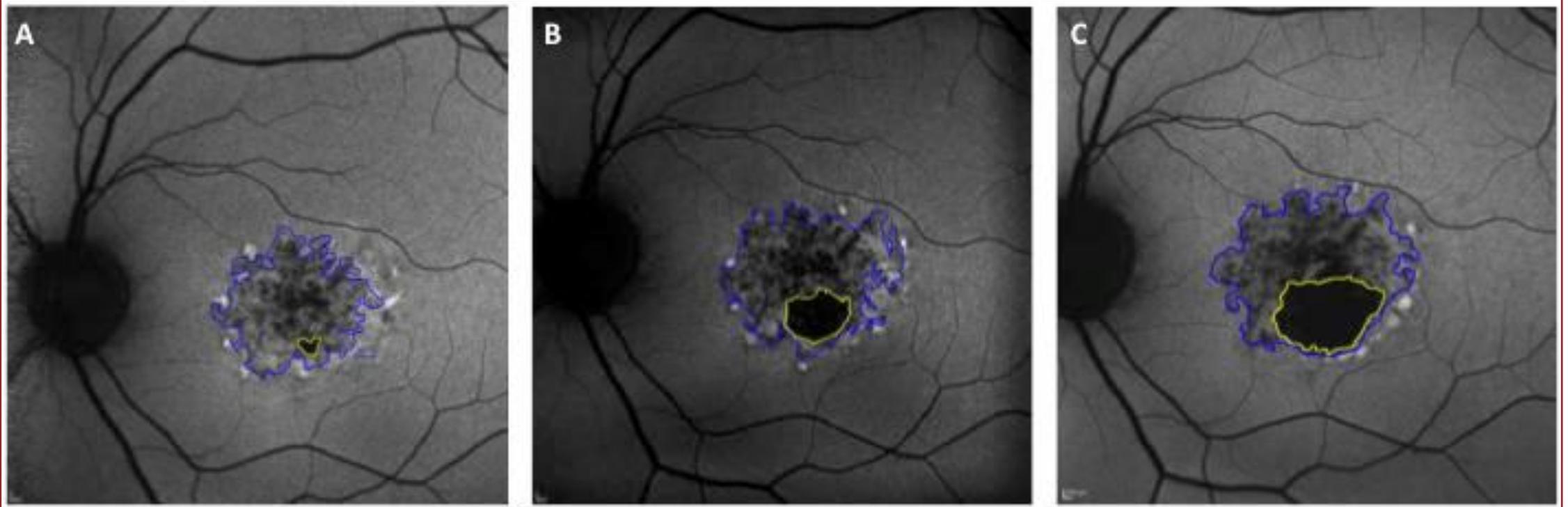
- **DDAF** (definitely decreased autofluorescence): level of darkness close to 100% (at least 90%) in reference to the ONH
- **QDAF** (questionably decreased autofluorescence): between 50% and 90% darkness
- **DAF** (decreased autofluorescence): the sum of DDAF and QDAF



# 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<sup>2</sup>/year**  
*(confidence interval: 0.64 - 0.85 mm<sup>2</sup>/year)*

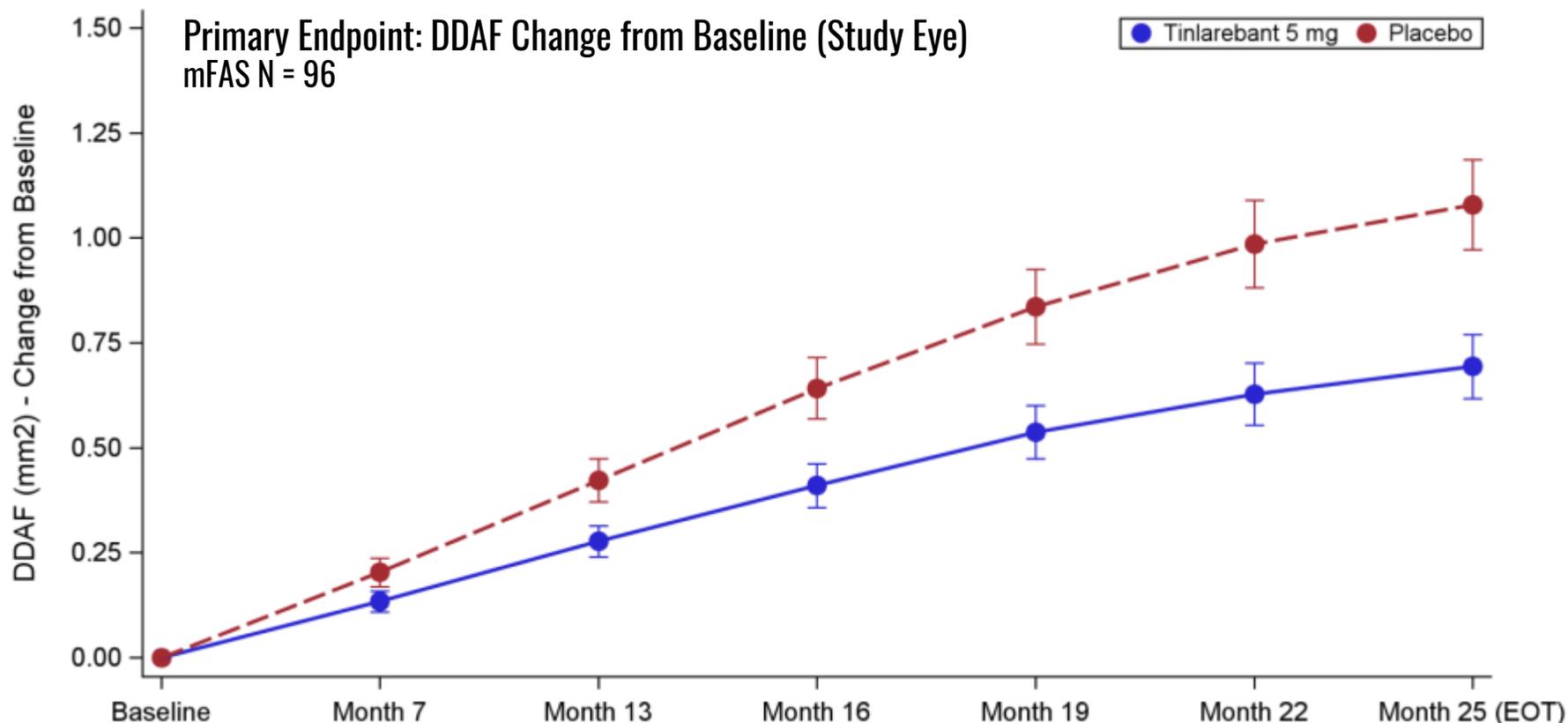


# Primary Endpoint: DDAF in the Study Eye (Change from Baseline)



- 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.

# Primary Endpoint Showed a Statistically Significant & Clinically Meaningful Outcome

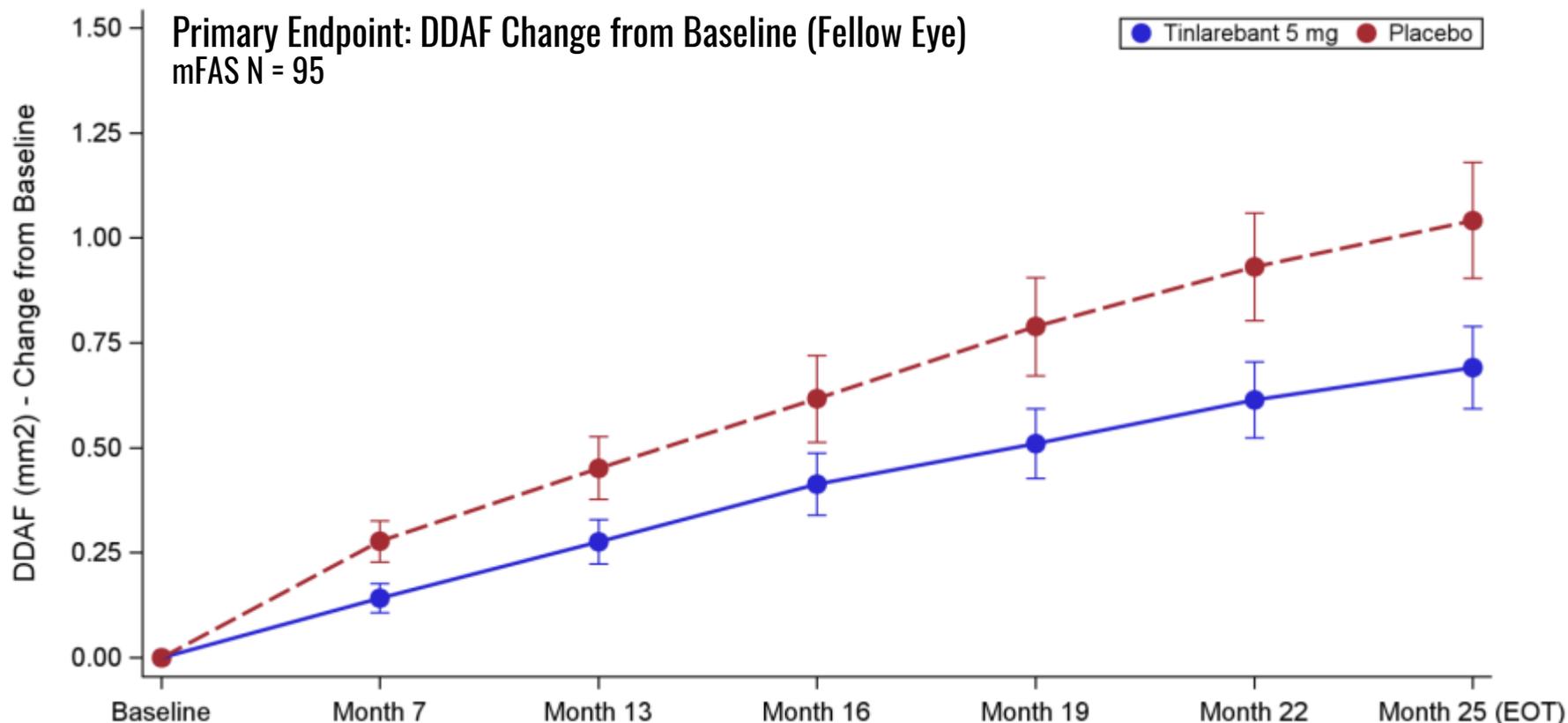


Applying an unstructured covariance matrix, the **treatment effect size was 35.7%** compared to placebo and yielded a **p-value of P = 0.0033**

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

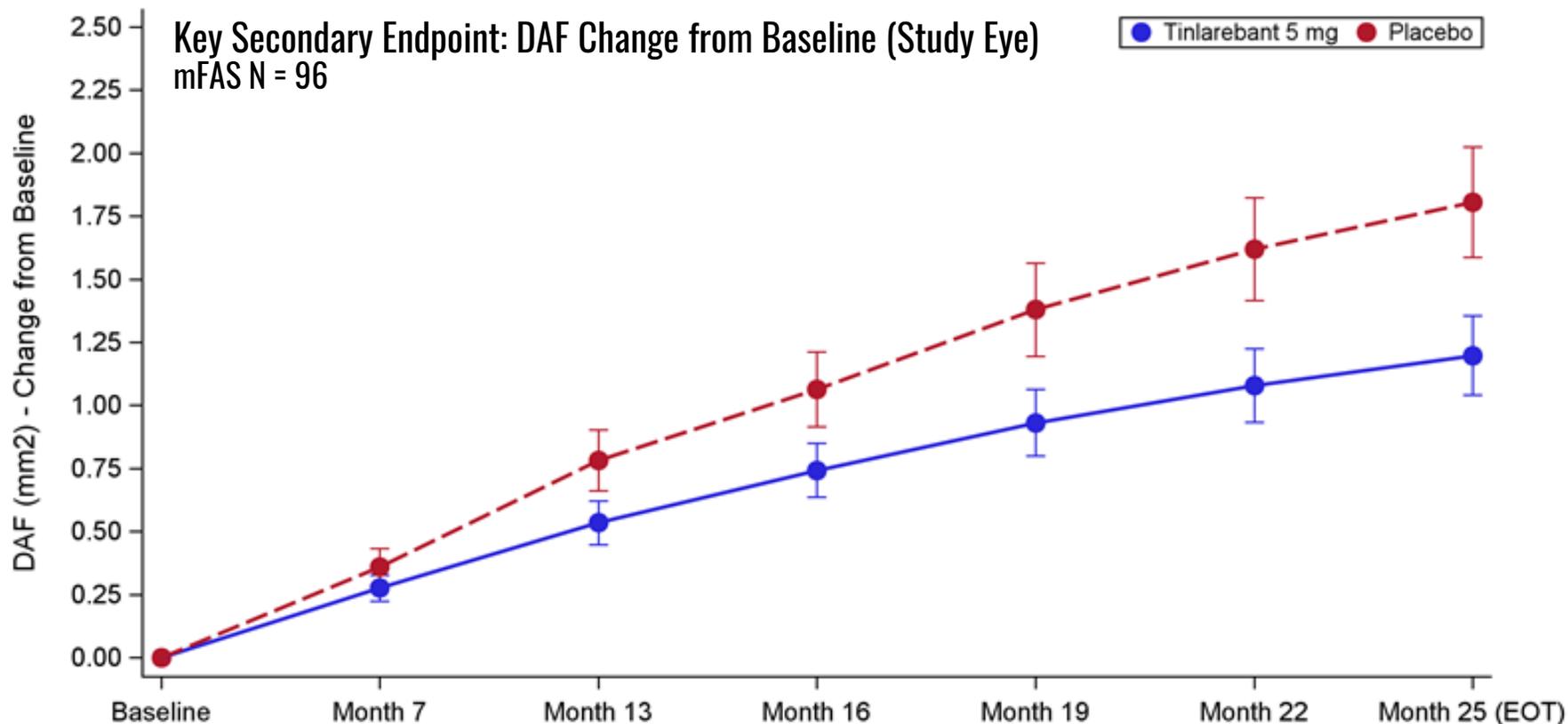
DDAF lesion growth was **slowed to 0.38 mm<sup>2</sup>/year vs. 0.59 mm<sup>2</sup>/year for placebo and 0.74 mm<sup>2</sup>/year observed in ProgStar**

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



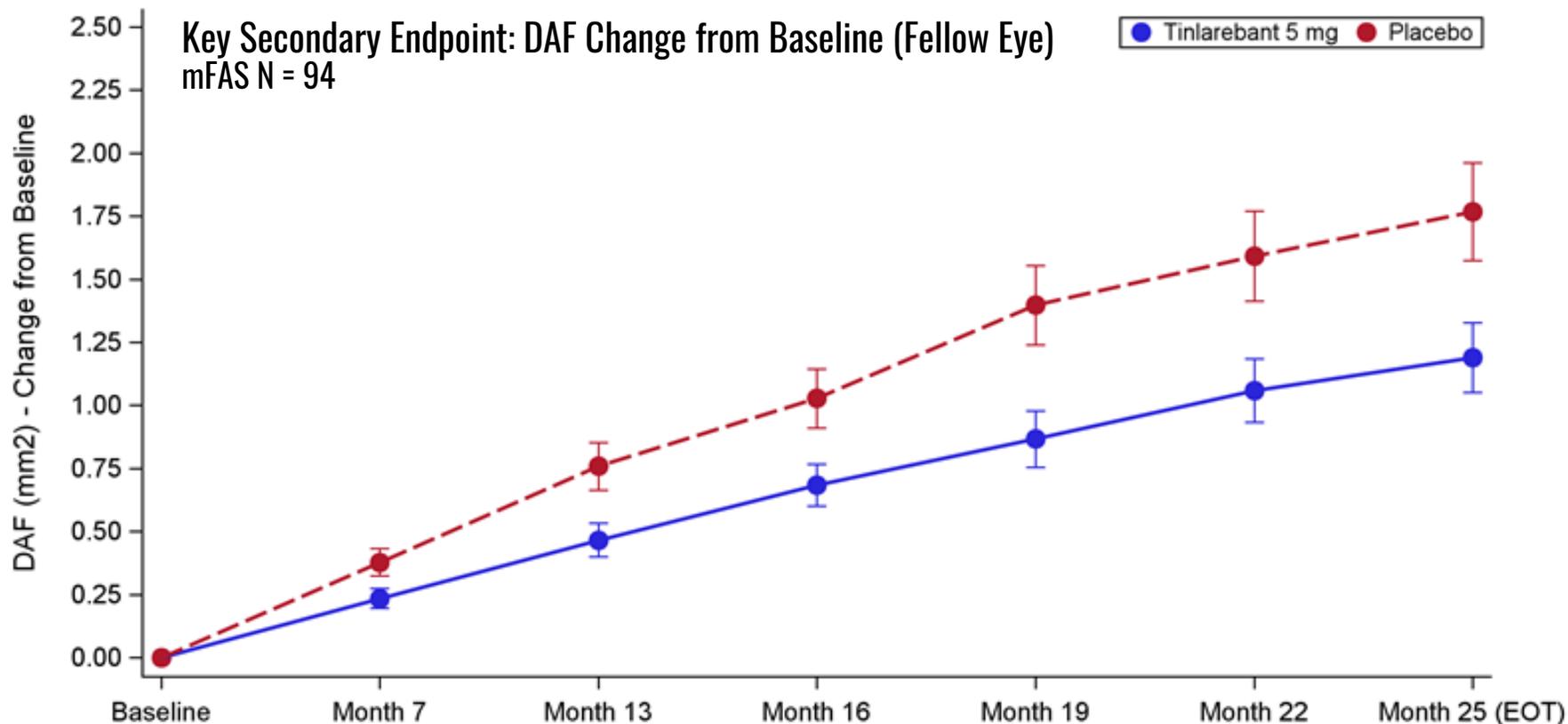
**Tinlarebant slowed DDAF lesion growth in the fellow eye by 33.6% compared to placebo (P = 0.041)**

# 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)**

# 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)**

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



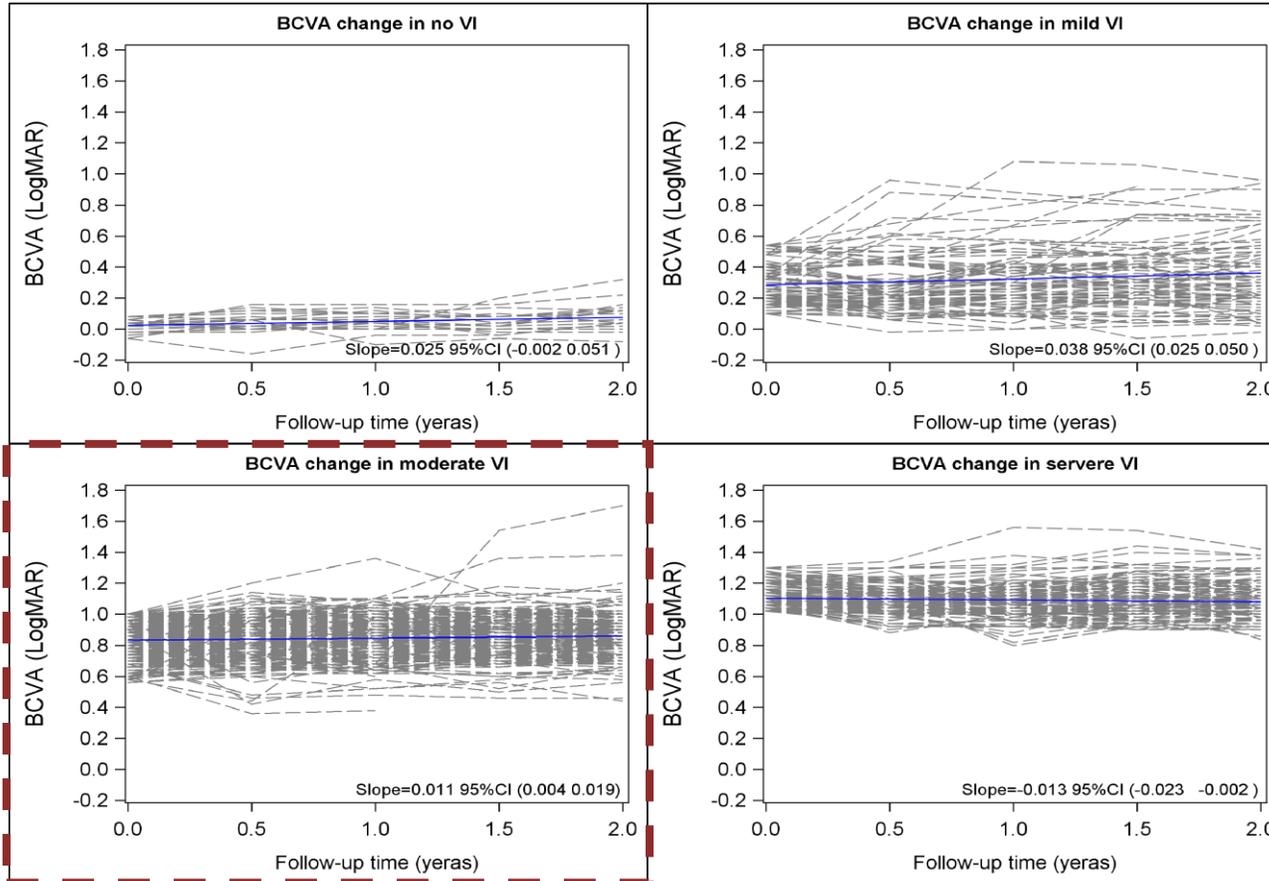
	Tinlarebant	Placebo
BCVA at Baseline	39.9	39.4
BCVA at EOS	39.7	40.0

- 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  $\approx$  8 letters <sup>(1)</sup>
- 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**



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# 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

# The Majority of Ocular Adverse Events were 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

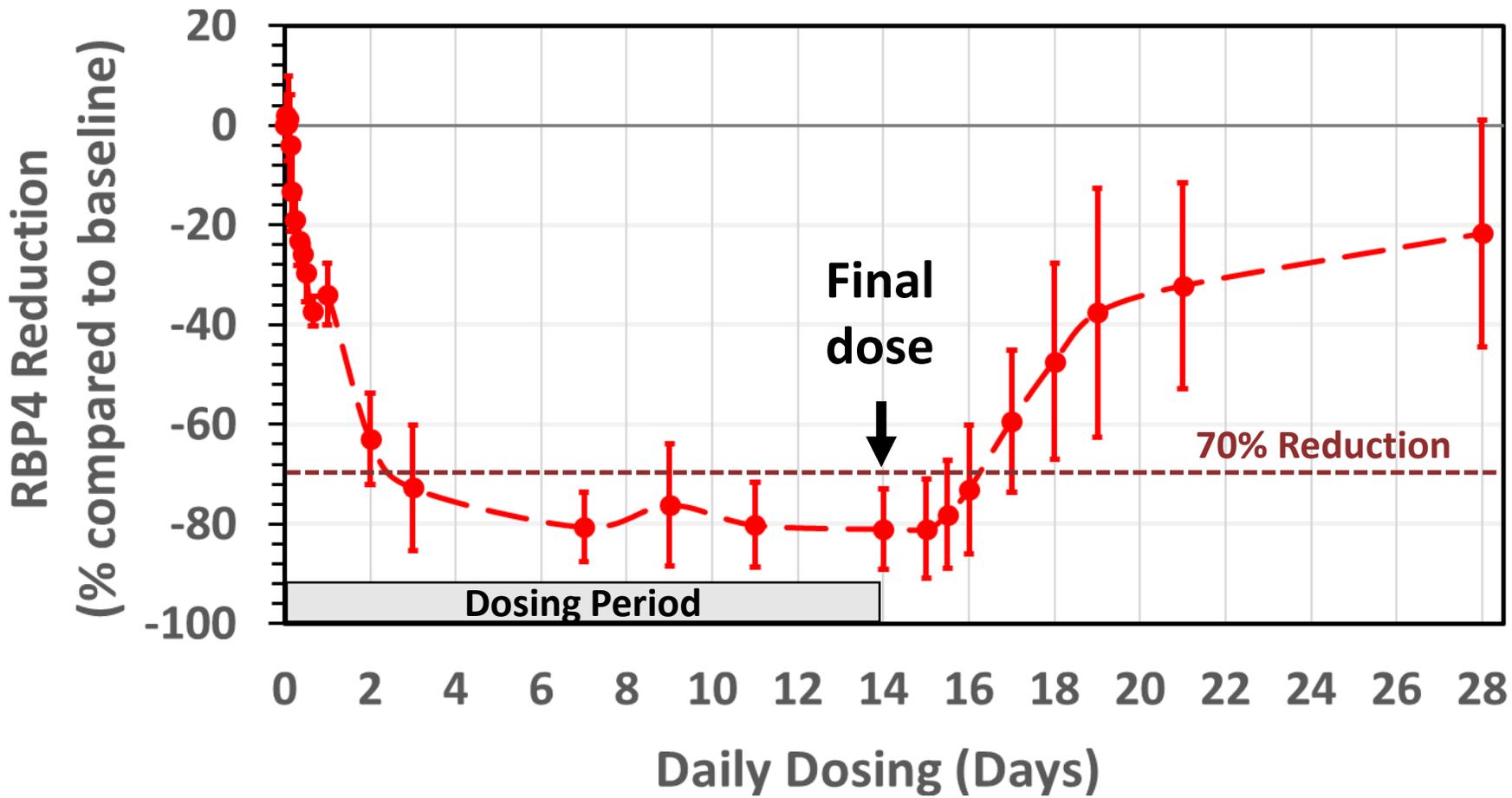


# Phase 3 PHOENIX Trial in Geographic Atrophy

# Tinlarebant: $\geq 70\%$ Reduction of RBP4



Phase 1, 5mg Daily Dosing in Healthy Adults: Mean Percent Reduction of RBP4 (excludes placebo)



# Clinical Trial Design Overview in GA



- **Established Efficacy Endpoint** – Reduction in atrophic lesion growth rate as measured by retinal imaging is the FDA accepted primary endpoint
- **Early Intervention** – Targeting patients with small lesion size to potentially slow disease progress at an early stage
- **Oral Once a Day Treatment** – well suited for long-term treatment for chronic diseases
- **Broad Potential** – Primary focus on GA; potential to treat earlier stages (e.g., intermediate AMD)

	GA Phase 3 “PHOENIX” <sup>(1)</sup>
<b>Enrollment</b>	530 subjects
<b>Sites</b>	Global
<b>Masking</b>	Double Blind
<b>Placebo</b>	2:1 ratio (Tinarebant : Placebo)
<b>Treatment duration</b>	2 years
<b>Primary measures</b>	Slowing of atrophic lesion growth, safety & tolerability
<b>Other measures</b>	BCVA, SD-OCT, microperimetry
<b>Interim analysis</b>	Yes

<sup>(1)</sup> Additional Phase 3 study expected to be required prior to NDA filing



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# Summary

# Summary of Results of the DRAGON Trial



- **The DRAGON trial met its primary endpoint:** A statistically significant slowing in DDAF lesion growth was observed in subjects treated with 5 mg/day oral Tinalarebant 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 tinalarebant treatment, **RBP4 reduction, showed a sustained 80% reduction with very little variability**
- **Tinalarebant (5 mg p.o., daily) was well tolerated** in adolescent STGD1 patients

# 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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# Thank You

For more info please visit: [www.belitebio.com](http://www.belitebio.com)