Results from the Phase 3 DRAGON Study of Tinlarebant for Adolescent and Adult Stargardt Disease

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Tinlarebant is an investigational molecule and is not approved by the FDA or any other regulatory body. No conclusions regarding its safety or efficacy should be made from this information.

Financial Disclosure

- Consultant, Belite Bio
- Principal Investigator, DRAGON Study
- Equity Holder, Belite Bio

Stargardt Disease (STGD1)

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 STGD1.**

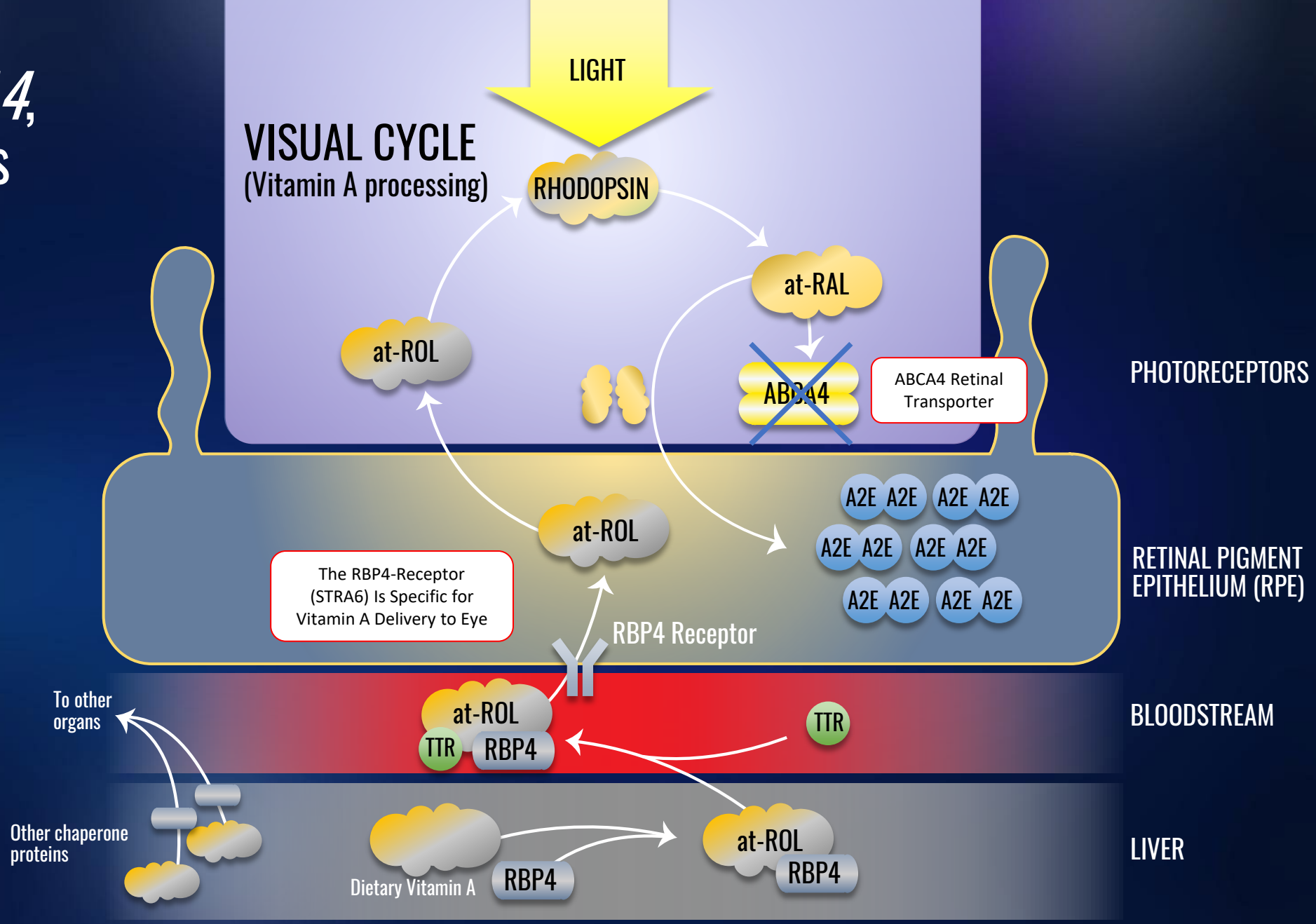


- **Most common** macular dystrophy
- Described by Karl Stargardt in 1909
- **Ocular exam signs:** areas of retinal atrophy centrally and subretinal yellowish flecks
- Manifestation **age range**: 1 – 84+ years

Stargardt K (1909) Über familiäre, progressive Degeneration in der Maculagegend des Auges (On familial, progressive degeneration in the macular region of the eye). *Zeitschrift für Augenheilkunde.* 22: 289–306.
Lambertus S et al. (2015) Early-onset Stargardt disease: phenotypic and genotypic characteristics. *Ophthalmology.* 122: 335-44.Lambertus
S et al. (2016) Progression of Late-Onset Stargardt Disease. *Invest Ophthalmol Vis Sci.* 57: 5186-5191.
Cremers FPM et al. *Prog Retin Eye Res*. 2020;79:100861. 2. Dayma K et al. . *Int J Mol Sci*. 2025;26(14):7006. 3. Roborel de Climens A et al.*Ophthalmic Genet*. 2021;42(2):150-160. 4. Kong X et al. *Ophthalmology*. 2016;123(9):1887-1897.

Mutation of *ABCA4*, in STGD1, Disrupts the Visual Cycle

- Mutation of the *ABCA4* gene causes a loss of transporter function, driving bisretinoid formation due to increasing all-*trans*-retinal[1]
- Over time, this causes accumulation of cytotoxic bisretinoid A2E, which drives degeneration of the retinal pigment epithelium and photoreceptors[1]
- This ultimately causes central vision loss that patients with STGD1 experience[1]

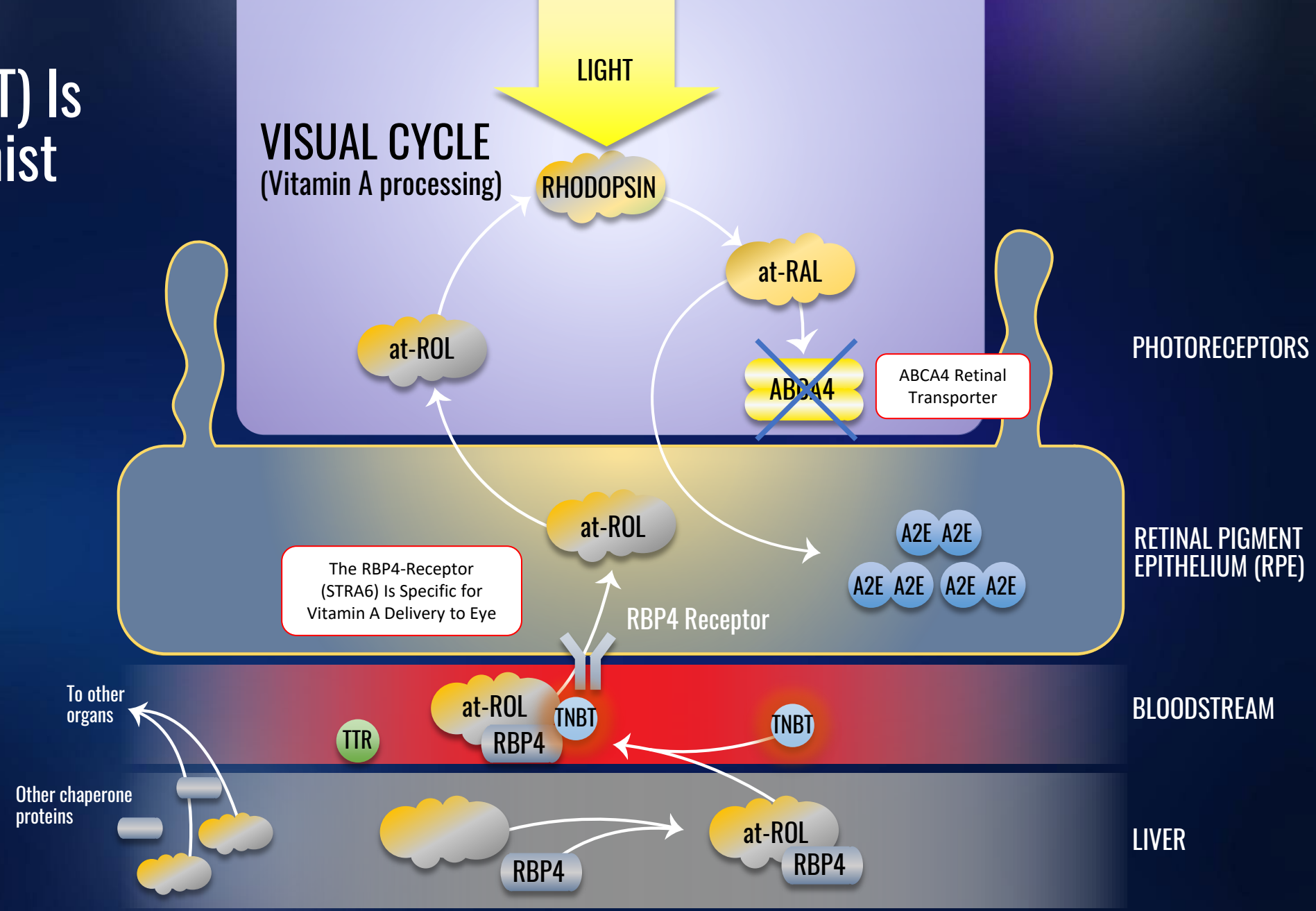


A2E, N-retinylidene-N-retinylethanolamine; *ABCA4*, ATP-binding cassette transporter subfamily A member 4; at-RAL, all-trans-retinaldehyde; at-ROL, all-trans-retinol (vitamin A); RBP4, retinol binding protein 4; STGD1, Stargardt disease type 1; TTR, transthyretin.

1. Cremers FPM, et al. *Prog Retin Eye Res*. 2020;79:100861.

Tinlarebant (TNBT) Is an RBP4 Antagonist

- The RBP4 receptor is the primary pathway for retinol to enter the RPE
- Tinlarebant is an RBP4 antagonist that disrupts the binding of RBP4 to its receptor, thereby inhibiting the transport of retinol into the eye[1]
- Tinlarebant reduces retinol entry in the eye, slowing the buildup of toxic A2E byproducts produced in the visual cycle[1]



A2E, N-retinylidene-N-retinylethanolamine; *ABCA4*, ATP-binding cassette transporter subfamily A member 4; at-RAL, all-trans-retinaldehyde; at-ROL, all-trans-retinol (vitamin A); RBP4, retinol binding protein 4; RPE, retinal pigment epithelium; TNBT, tinlarebant; TTR, transthyretin; STRA6, Stimulated by Retinoic Acid 6.

1. Polasek TM, et al. *Clin Pharmacol Drug Dev*. 2026;15(1):e70009.

The Multicenter Phase 3 DRAGON Trial Was Conducted in Adolescent and Adult Patients With STGD1[1]

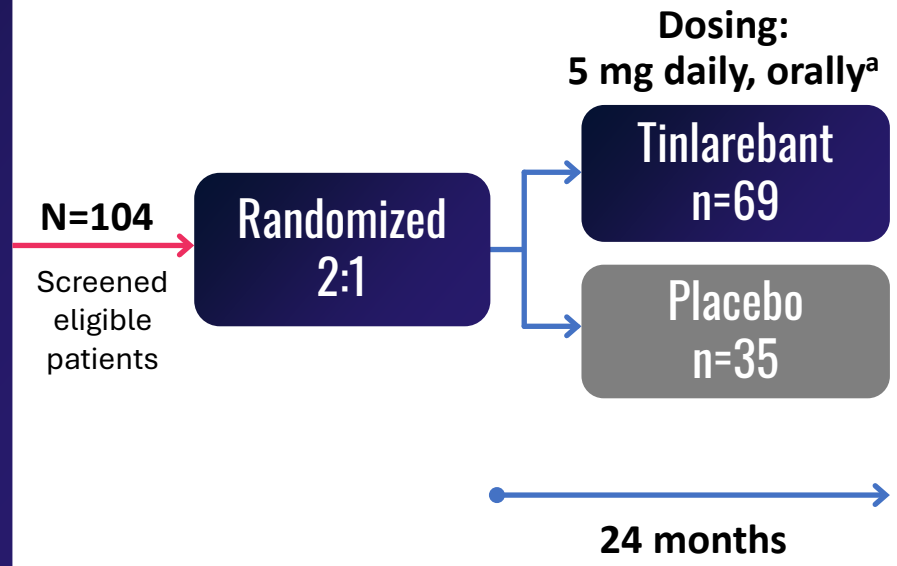
Randomized, Double-Masked, Placebo-Controlled Trial[2]

Key inclusion criteria:

- Age: 12-20 years
- Diagnosed STGD1 with ≥1 mutation in ABCA4 gene
- BCVA of 20/200 or better
- Atrophic lesion size within 3 disc areas (7.62 mm^2)

Key exclusion criteria:

- Other ocular diseases
- Prior gene therapy
- Vitamin A deficiency



Dosing: 5 mg daily, orally

Primary endpoint: Efficacy as measured through DDAF lesion growth rate

Secondary endpoints: DAF (calculated as the sum of DDAF and QDAF), BCVA, reduction in RBP4; safety and tolerability

Exploratory endpoints: Change in QDAF lesion size, retinal thickness by SD-OCT, retinal sensitivity, qAF level

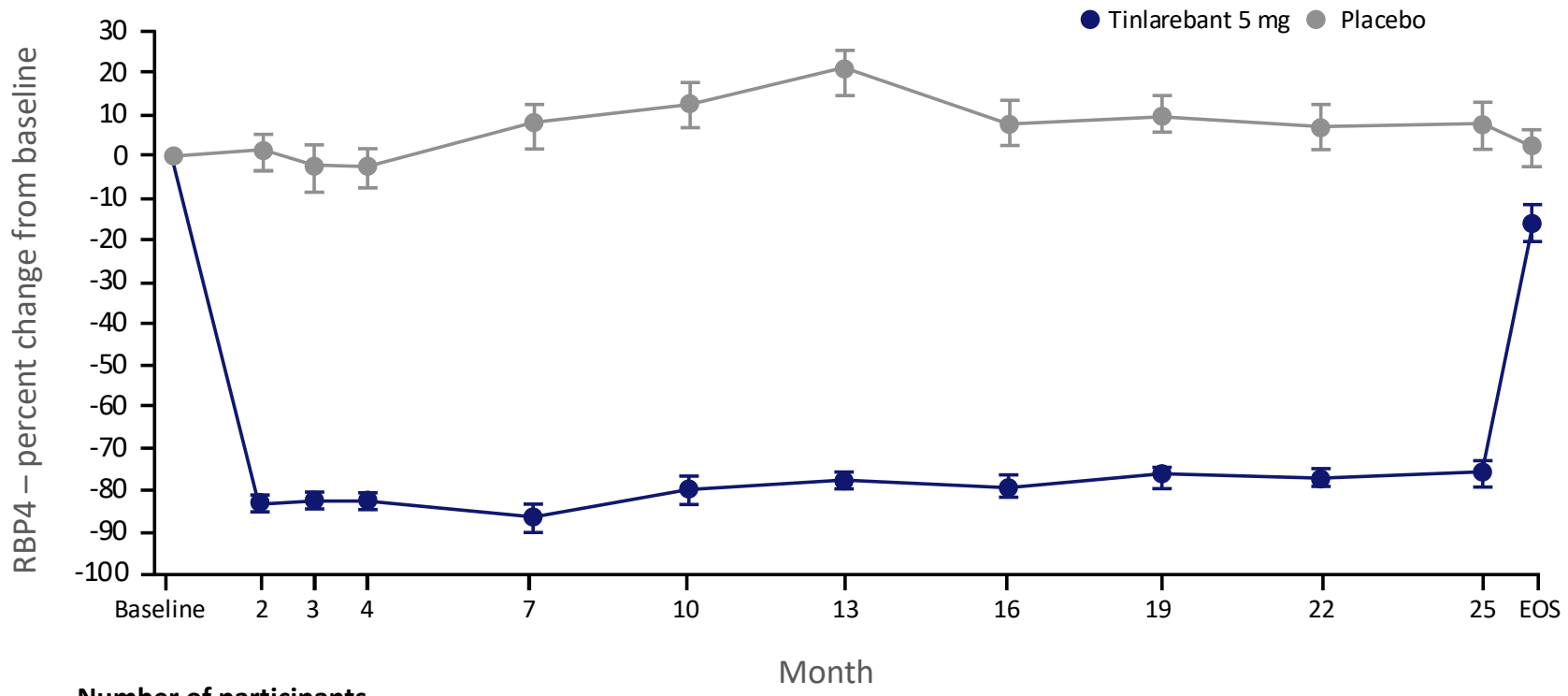
ABCA4, ATP-binding cassette transporter subfamily A member 4; BCVA, best-corrected visual acuity; DAF, decreased autofluorescence; DDAF, definitely decreased autofluorescence; qAF, quantitative autofluorescence; QDAF, questionably decreased autofluorescence; RBP4, retinol binding protein 4; SD-OCT, spectral domain optical coherence tomography; STGD1, Stargardt disease type 1.

[a]Tinlarebant is still investigational and not yet commercially approved (as of August 2026). All dosing information comes from clinical trial protocols, not prescribing guidelines.[2]

1. Phase 3, randomized, placebo-controlled study of tinlarebant to explore safety and efficacy in adolescent Stargardt disease (DRAGON). ClinicalTrials.gov identifier: NCT05244304. Updated October 31, 2025. Accessed June 26, 2026. https://clinicaltrials.gov/study/NCT05244304

In the Phase 3 DRAGON Study, Tinlarebant Caused an 80% Reduction of RBP4[1]

RBP4 – Percent Reduction From Baseline



Number of participants

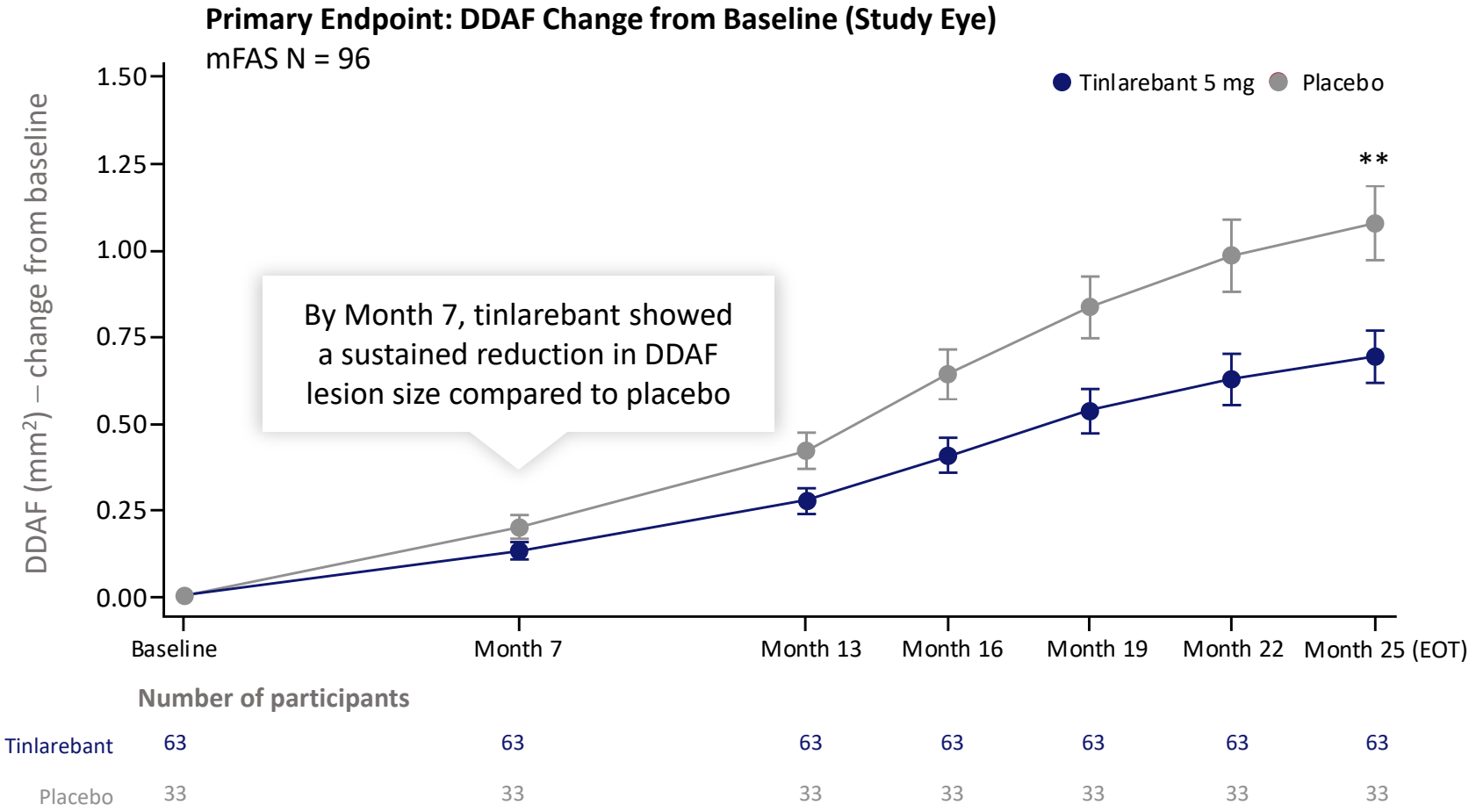
	Baseline	2	3	4	7	10	13	16	19	22	25	EOS
Tinlarebant	59	54	59	59	59	57	59	58	58	57	48	56
Placebo	33	33	33	32	33	31	31	31	31	31	27	28

- Tinlarebant caused a reduction of RBP4 by Month 2, with a sustained effect throughout the study
- The effects of tinlarebant are reversible, as RBP4 levels recovered near baseline following cessation of administration[1]
- The data support the MOA of tinlarebant as a potent antagonist of RBP4

Dataset includes all subjects who received the study drug without interruption for 2 or more weeks.
Graphs show mean ± SEM.
EOS, end of study; MOA, mechanism of action; RBP4, retinol binding protein 4; SEM, standard error of mean.

1. Data on file. Belite Bio, Inc. 2026.

The DRAGON Trial Met Its Primary Endpoint[1]



[a]Eligibility criteria and study power calculation were based on the study eye.[1]
[b]The mFAS consisted of all participants who were randomly assigned to receive study drug and received at least 1 dose of study drug. In addition, the mFAS participants were to have defined DDAF lesion(s) meeting the eligibility criteria at baseline and at least 1 postbaseline assessment.[1]
***P* =0.0033
Dataset represents the least squares mean change from baseline by visit. Missing data (eg, study discontinuation) were imputed.
Graphs show mean ± SEM.
DDAF, definitely decreased autofluorescence; EOT, end of trial; mFAS, modified full analysis set; SEM, standard error of mean.
1. Data on file. Belite Bio, Inc. 2026. **2.** Strauss RW, et al. *Am J Ophthalmol*. 2023;250:157-170.



- By Month 25, tinlarebant demonstrated a statistically significant 35.7% annualized reduction in DDAF lesion growth compared to placebo (***P*=0.0033)[1]
- In patients treated with tinlarebant, DDAF lesion growth was slowed to 0.38 mm^2/year vs 0.59 mm^2/year for placebo[1]
- Baseline values from the ProgStar study show an average DDAF lesion growth of 0.74 mm^2/year[2]

DRAGON Study Results With BCVA Are Consistent With the Inadequacy of This Endpoint for Measuring Progression of STGD1[1,2]

- Despite progressive retinal degeneration, BCVA decline in clinical trials is non-linear and fluctuates significantly across patient groups[2-4]
- In the current study, the overall change of visual acuity was minimal over the 24 months in both treatment groups[1]
- Results are in line with the natural history of STGD1 that was observed in the ProgStar study, where overall rate of BCVA loss was 0.55 letters/year over 2 years[2]

	Tinlarebant (Total letters)	Placebo (Total letters)
BCVA at baseline	39.9	39.4
BCVA at EOS	39.7	40.0

For short-term (≤2 years) clinical trials, BCVA is not a sensitive enough endpoint to reliably detect treatment changes in STGD1[2,4]

BCVA, best-corrected visual acuity; EOS, end of study; STGD1, Stargardt disease type 1.

1. Data on file. Belite Bio, Inc. 2026. **2.** Kong X, et al. *JAMA Ophthalmol.* 2018;136(8):920-928. **3.** Cremers FPM, et al. *Prog Retin Eye Res*. 2020;79:100861 **4.** Verbakel SK, et al. *Invest Ophthalmol Visual Sci*. 2017;58(8):2014.

Non-ocular Adverse Events in the DRAGON Trial

Study Participants Who Experienced at Least 1 Non-ocular TEAE, n (%)[a]		
Category	Tinlarebant 5 mg (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%)

- **6 serious TEAEs** were reported in the study – all events were **non-ocular**:
  - **Tinlarebant:** sialoadenitis, pulmonary embolism
  - **Placebo:** otitis externa, urinary retention, pneumothorax spontaneous, inguinal hernia

- Most common non-ocular AEs (>5% of total participants) in tinlarebant 5 mg vs placebo groups, respectively: **nasopharyngitis** (34.8% and 34.3%), **headache** (23.2% and 8.6%), **acne** (17.4% and 2.9%), **influenza** (14.5% and 0%), **abdominal pain upper** (11.6% and 2.9%), **upper respiratory tract infection** (7.2% and 17.1%), **cough** (7.2% and 5.7%), **pyrexia** (5.8% and 8.6%), **diarrhea** (4.3% and 8.6%), and **urine analysis abnormal** (8.7% and 0%)

[a]A TEAE is defined as any event that is not present before exposure to study drug or any event already present that worsens in either intensity or frequency after exposure to study drug and starts within 28 days after the last dose of study drug (3 months for sites in France and Germany).
AE, adverse event, TEAE, treatment-emergent adverse event.
1. Data on file. Belite Bio, Inc. 2026.

The Majority of Ocular Adverse Events Were Mild (Grade 1)[1]

- Most common **ocular AEs** (>5% of total participants) in tinlarebant 5 mg vs placebo groups, respectively:
  - **Delayed dark adaptation** (33.3% vs 11.4%)
  - **Xanthopsia** (37.7% vs 8.6%)
  - **Night visual impairment** (23.2% vs 0%)
  - **Chromatopsia** (10.1% vs 5.7%)
- These were generally mild, and most resolved during the study
- There were 2 severe and no serious TEAEs[b]
- 4 TEAEs led to study drug discontinuation (delayed dark adaptation [1], xanthopsia [1], and night vision impairment [2])
  - 2 of which led to study discontinuation

Study Participants Who Experienced at Least 1 Ocular TEAE, n (%)[a]		
Category	Tinlarebant 5 mg (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%)

[a]TEAEs with the relationship to the study drug of "possibly related," "probably related," and "definitely related" will be considered as study drug-related TEAEs. A TEAE with missing relationship will be considered a study drug-related TEAE. Ocular TEAEs that happen in both eyes will be counted once for each eye.[1]
[b]Mild refers to Grade 1, moderate refers to Grade 2, and severe refers to Grade 3.
AE, adverse event, TEAE, treatment-emergent adverse event.
1. Data on file. Belite Bio, Inc. 2026.

Summary

- The DRAGON trial met its primary endpoint with a treatment effect of ~36%
  - A statistically significant slowing in DDAF lesion growth was observed in subjects treated with 5 mg/day oral tinlarebant as compared to placebo
- 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) had a side effect profile consistent with its mechanism of action in adolescent and adult STGD1 patients