

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

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Financial Disclosure:

- Consultant of Belite Bio
- Principal Investigator of the DRAGON study



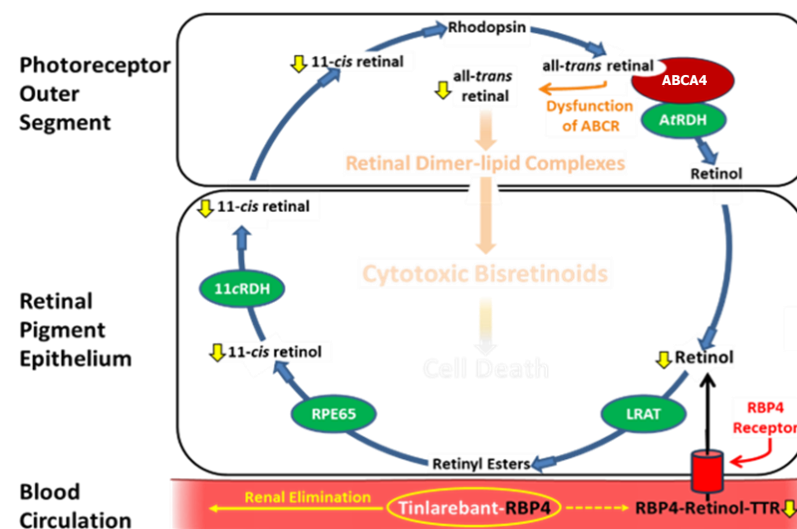
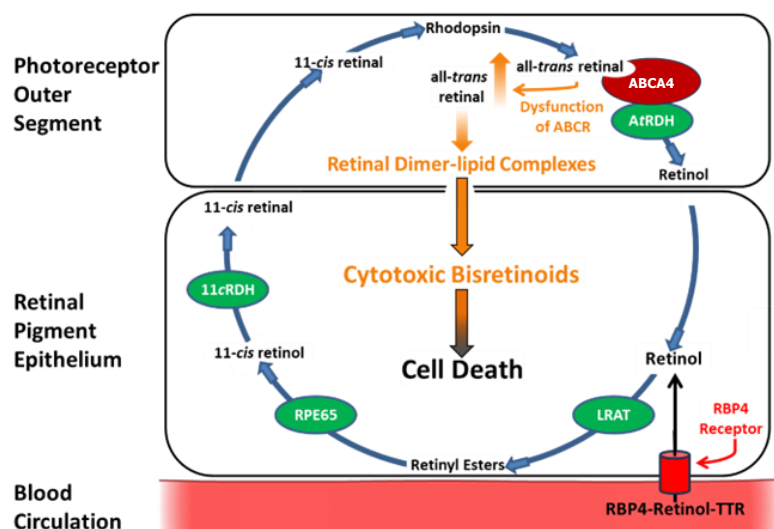
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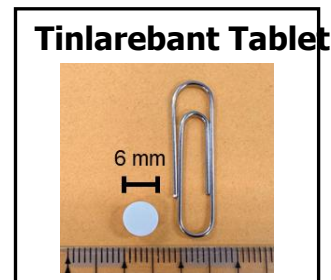
Introduction to STGD1 and Tinlarebant



- Stargardt disease (STGD1) harbor pathogenic mutations in the *ABCA4* gene, resulting in loss of function of the ABCA4 protein in photoreceptor cells, interfering with normal vitamin A processing in the visual cycle which results in the aberrant accumulation of cytotoxic byproducts of vitamin A (*bisretinoids*) and progressive retinal cell death [1]



- Tinlarebant is a novel, **once-a-day oral tablet** designed to antagonize serum retinol binding protein 4 (RBP4) as a means to specifically reduce retinol delivery to the eye and **slow or halt the accumulation of bisretinoids**
- Belite Bio believes that **early intervention directed at emerging retinal pathology**, which is not mediated by inflammation, would be the best approach to potentially slow disease progression in STGD1 & GA.





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

N=104

Phase 3 Trial

Randomized 2:1

Treatment n=69

Placebo
n=35

Double-blinded, global trial of
oral Tinlarebant 5mg/day

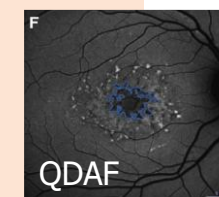
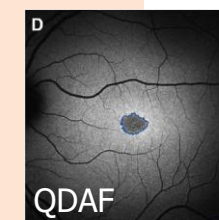
2-Year Duration

Primary Measures

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

Secondary Measures

- DAF
- BCVA
- SD-OCT
- Microperimetry



DRAGON Clinical Trial Demographics and Baseline Characteristics

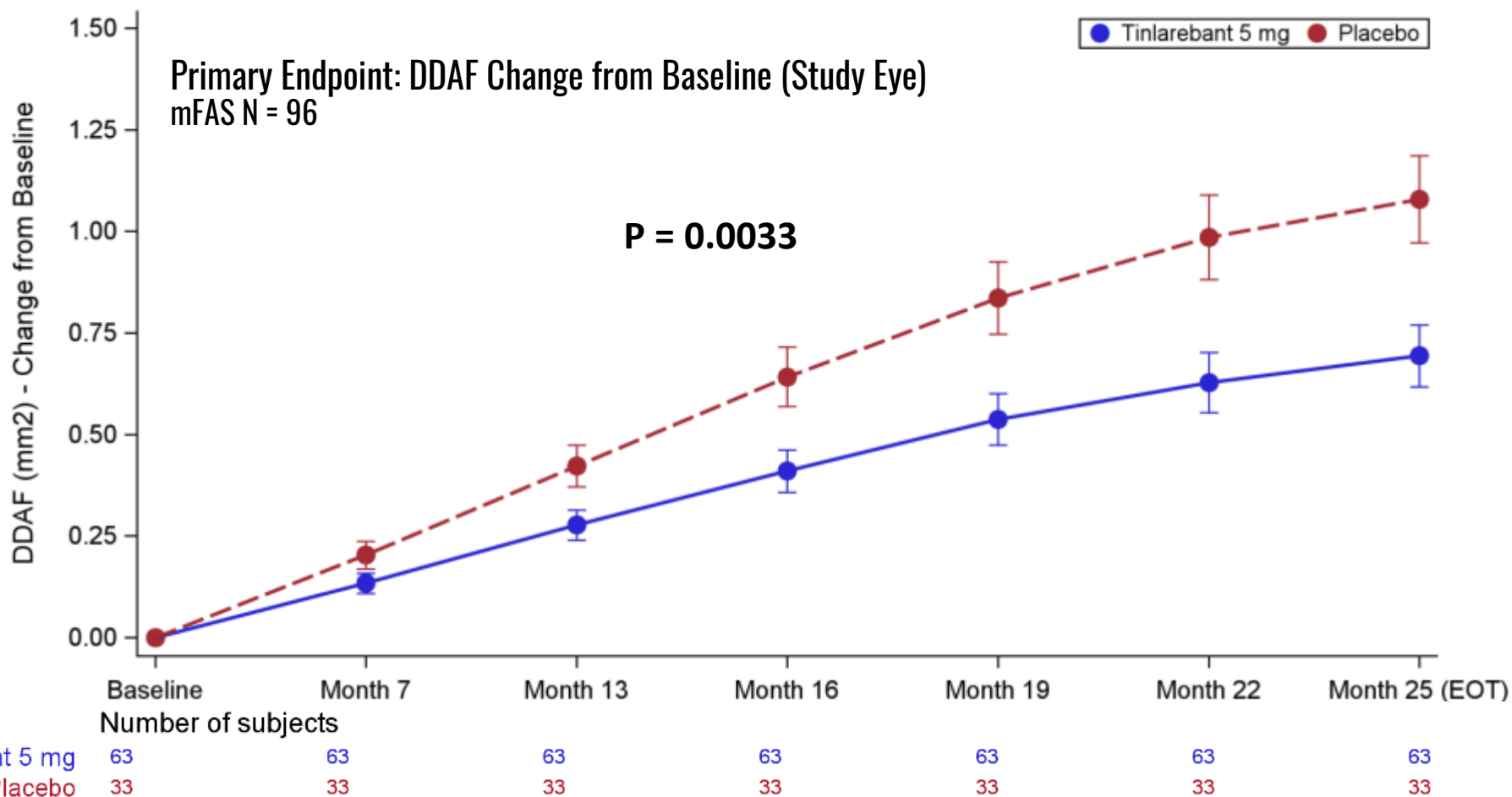


	Mean (SD), Total N=104
Age (Years)	15.4 (2.47)
Baseline Height (cm)	168.12 (10.349)
Baseline Weight (kg)	61.75 (16.891)
Baseline BMI (kg/m2)	21.62 (4.578)

	N (%), Total N=104
Sex	
Male	65 (62.5%)
Female	39 (37.5%)
Race	
White	38 (36.5%)
Asian	58 (55.8%)
Multiple	1 (1.0%)
Other	7 (6.7%)



Primary Endpoint: DDAF Change from Baseline(Study Eye)



mFAS = all subjects randomly assigned to receive study drug and received at least one dose of study medication. In addition, subjects must have had a defined DDAF lesion meeting the eligibility criteria at baseline and have had at least 1 post baseline assessment.

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

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**

BCVA (Study Eye)



	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

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

DRAGON Interim Analysis Conclusions



Belite Bio Announces UK's Medicines and Healthcare Products Regulatory Agency Agrees to Conditional Marketing Authorization Application Based on Interim Analysis Results for the Treatment of Stargardt Disease with Tinarebant

November 02, 2025 09:00 ET | Source: [Belite Bio, Inc](#)

Follow

- Belite Bio has shared the unmasked DRAGON interim data with global regulatory authorities to seek their opinion
- To date, regulatory authorities in China (NMPA) and the United Kingdom (MHRA) have agreed to accept the interim data from DRAGON for full regulatory approval, indicating a statistically significant treatment effect of tinarebant in slowing lesion growth