



# Early Intervention with an Oral Treatment for Macular Degeneration

Mission for Vision

Nasdaq: BLTE

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# At-a-Glance



## “Bringing Hope to Incurable Blindness”



As of 2022/7/21	Belite Bio, Inc
Share Symbol	BLTE
Stock Exchange	NASDAQ
Stock Price	\$ 41.51
Shares Outstanding	24.87 million
Market Cap	\$ 1,032.35 million

# Leadership



## Management



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

- 10 years of executive management role in biotech, including 2 IPO
- Over 10 new drug developments in multiple therapeutic areas, including Ophthalmology, CNS, Cardiovascular, Oncology, Immuno-Oncology, Immunotherapies, Immunosuppressants
- University of Sydney, University of Melbourne, Harvard Medical School, Columbia University, London Business School, HK University



**Nathan Mata, PhD**  
(CSO)

- 15+ years of ophthalmic drug development experience across numerous indications including 2 NDAs for topical therapeutics (Durezol® and Zirgan®)
- Led the clinical development efforts for first RBP antagonist in advanced dry AMD and first visual cycle modulator in advanced dry AMD and STGD
- Introduced the industry's first Stargardt's ABCA4 knockout mice model
- University of Texas



**Jane Chiu, MS**  
(VP, Clinical Operations)

- 25 years clinical operations experience in multiple therapeutical area
- 15+ years as President/Managing Director of multinational CRO, conducting over 100 studies
- 10+ years of clinical operations experience in global pharma (Astellas, Bayer, Pfizer)
- Warwick University



**H.Y. Chuang, CFA, MBA, FRM**  
(CFO)

- 11 years of capital market experience, closed more than US\$32 billion transactions
- Wanda, Suning, CITIC Securities
- Columbia University, London Business School, HK University

# Business Highlights



Oral treatment for  
an unmet market

- **Belite Bio's lead asset LBS-008** is a novel, **orally administered, Retinol Binding Protein 4 ("RBP4") antagonist** intended to slow or halt progression of vision loss in Stargardt disease (STGD1) and dry AMD.
- Currently **no approved treatments** for either STGD1 or dry AMD, significant market opportunity to become **Standard of Care**.
- Clinical development approach **endorsed by US NIH**, specifically **to treat dry AMD**.
- **UK NIHR's** 2018 systematic review of >7,000 publications recommends RBP4 antagonists as a **priority for clinical development to treat both STGD1 and dry AMD**.
- Dry AMD afflict **11 million patients** in the US and **196 million patients** worldwide.
- Without treatment, the continual increase in the size of the elderly population will worsen the impact of this disease.
- STGD1 is an orphan disease affecting approx. 1 in 10,000 children and adults.
- Granted **Fast Track Designation, Rare Pediatric Disease** in US / **Orphan Drug Disease** designation in US and EU for STGD1.
- **Priority Review Voucher (PRV)** eligible, vouchers have sold for \$80M-\$125M.

# Clinical Development Milestones



## Development Milestones

- **Phase 1 trial** in 111 healthy adults in US SAD and Australia SAD & MAD completed.
- A **2-year open label Phase 2 trial** (6 months of interim safety data and preliminary efficacy data available) in 13 adolescent STGD1 patients is ongoing. 12 months of treatment interim data expected in October 2022.
- A **2-year double-blind Phase 3 (the “DRAGON”) trial** has commenced in the US, UK, Germany, Belgium, Switzerland, Hong Kong, Taiwan, and Australia. Several patients have been enrolled. Expect to apply for enrollment in more jurisdictions and enroll at least 60 adolescent STGD1 patients globally.
- A **2-year Phase 2/3 trial in dry AMD** planned in Q4, 2022.

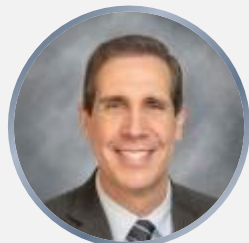
# Board of Directors



## Board



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



**John M. Longo, PhD**  
(Independent Director)

- Prof. of Rutgers Business School
- Chief Investment officer of Beacon Trust



**Gary C. Biddle, PhD, CPA**  
(Independent Director)

- Prof. of University of Melbourne
- INED of Kingdee Software, Shui On Land Limited, Real Pet Food Company.



**Ita Lu**  
(Independent Director)

- Managing partner of Taiwan Capital



**H.Y. Chuang, CFA, MBA, FRM**  
(CFO)



**Yvonne Chen**  
(Affiliated Director)

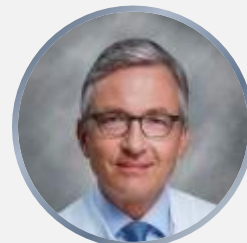
- COO of Lin Bio, our ultimate controlling shareholder



**Serena Chen**  
(Affiliated Director)

- Associate finance director of Lin Bio, our ultimate controlling shareholder

## Clinical Advisory Board



**Dr. Frank Holz**

- Chairman of Ophthalmology, University of Bonn



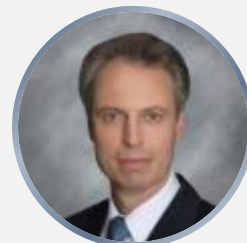
**Dr. Michel Michaelides**

- Ophthalmologist at Moorfields Eye Hospital
- Prof. of Ophthalmology, Univ. College London



**Dr. Quan Nguyen**

- Prof. of Ophthalmology, Stanford University



**Dr. Hendrik P.N. Scholl**

- Prof. and Chairman of the Dept. of Ophthalmology, Univ. of Basel
- Co-Director of the Institute of Molecular and Clinical Ophthalmology in Basel



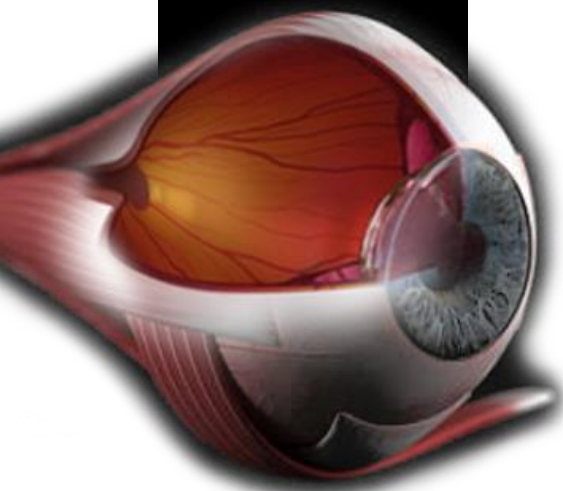
**Dr. Robyn Guymer**

- Prof. of Ophthalmology, University of Melbourne
- Deputy Director of the Centre for Eye Research Australia



LBS  
008

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




# BRING LIGHT TO INCURABLE BLINDESS

For Dry Age-Related Macular Degeneration & Stargardt Disease

## KEY OPPORTUNITY

# Zero Approved Treatments

**FDA** Fasttrack, RPD, ODD  COLUMBIA UNIVERSITY  
+ EU ODD designations for STGD1

## NIH Blueprint

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

Reference:  
<https://www.ninds.nih.gov/About-NINDS/Impact/Translational-Research-Success-Stories>

## Dry AMD MARKET

# 11M

dry AMD patients in the US (90% AMD are dry AMD)

# \$255B

estimated global direct healthcare cost of dry AMD

Reference: Globaldata, Lancet, Orphanet, STEM CELLS Translational Medicine

## STGD1 MARKET

# 1 in 10,000

inherited juvenile onset macular degeneration

# 30,000

STGD1 patients in the US



# Overview of Stargardt Disease & Dry AMD



## Stargardt Disease (STGD1)

- The **most common inherited retinal dystrophy** (blurring or loss of central vision) in both adults and children
- Caused by a **dysfunctional retina-specific gene (ABCA4)** which causes massive accumulation of toxic vitamin A byproducts ('bisretinoids') in the retina leading to retinal cell death and progressive loss of central vision
- Fluorescent properties of bisretinoids and the development of **retinal imaging** help ophthalmologists identify and monitor disease progression

## Dry AMD

- Shares a **similar pathophysiology with STGD1** and is a leading cause of central vision loss in people over 50



Macular degeneration  
causes blurred or loss  
of central vision

**A cytotoxic compound known as A2E is the most abundant bisretinoid identified in the retinas from patients with STGD1 and Dry AMD; A2E has been shown to kill retinal tissue.**

Reference:

[www.rarediseases.info.nih.gov/diseases/181/stargardt-disease](http://www.rarediseases.info.nih.gov/diseases/181/stargardt-disease)

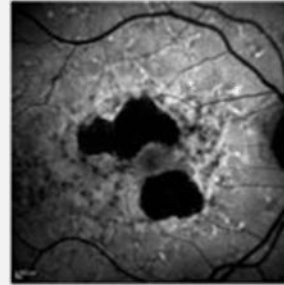
[www.ncbi.nlm.nih.gov/pmc/articles/PMC2848442/](http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2848442/)

# Similar Pathophysiology in STGD1 & Dry AMD

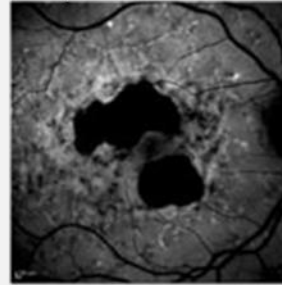


- **STGD1 and dry AMD share a similar pathophysiology** characterized by excessive accumulation of cytotoxic bisretinoids, retinal cell death, and loss of vision
- **Vision loss occurs slowly**, despite peripheral expansion of ‘dead retina’, until the disease reaches the center of the eye (the macula)
- **Slowing the spread of ‘dead retina’ is the intended effect of LBS-008 treatment**

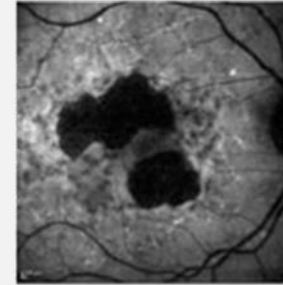
## STGD1: LATE-ONSET (61-YEAR OLD FEMALE)



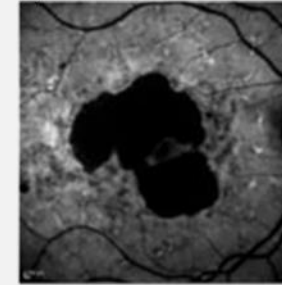
Baseline:  
0.1 LogMAR



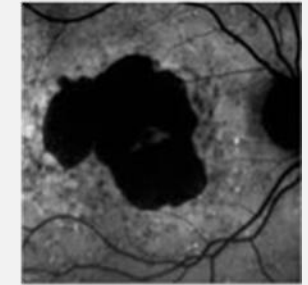
+12 Months:  
0.1 LogMAR



+24 Months:  
0.0 LogMAR



+36 Months:  
0.1 LogMAR

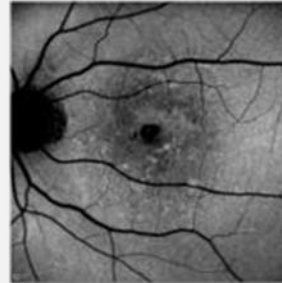


+57 Months:  
0.5 LogMAR

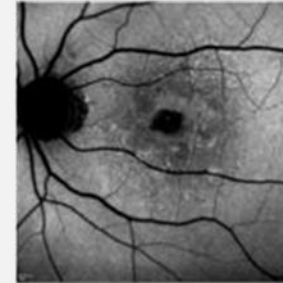
## Dry AMD: ADVANCED (73-YEAR OLD FEMALE)



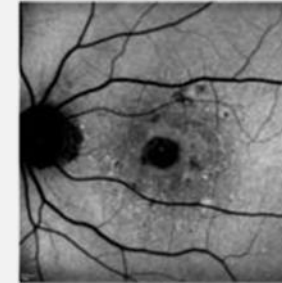
BL:  
0.2 LogMAR



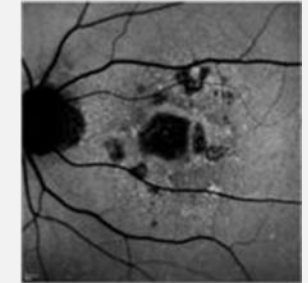
+12 Mo:  
0.2 LogMAR



+ 24 Mo:  
0.3 LogMAR



+ 36 Mo:  
0.4 LogMAR



+55 Mo:  
0.6 LogMAR

# Clear Clinical Development Pathway

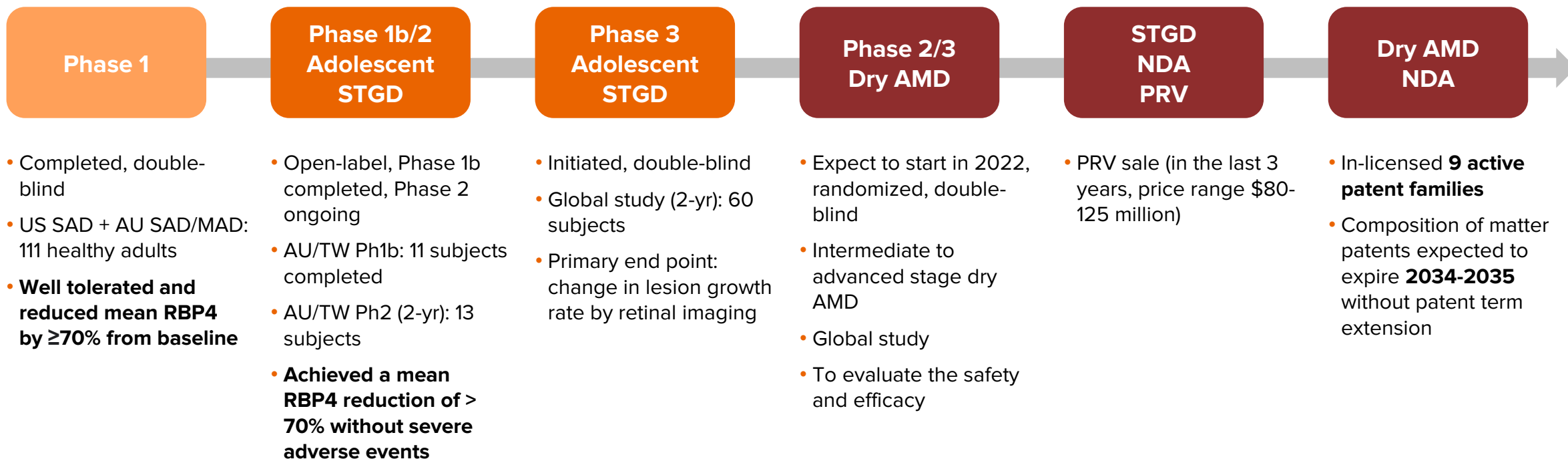


Reduction in Lesion Growth Rate as Measured by Retinal Imaging is an FDA Accepted Primary Endpoint in STGD1 and dry AMD

## completed

## ongoing

## planned



# Clinical Trial Design for STGD1



	STGD1 phase 2	STGD1 phase 3 ("Dragon")
<b>Enrollment</b>	13 participants	60 participants
<b>Sites</b>	Aus & TW	Global
<b>Masking</b>	Open Label	Double Blind
<b>Placebo</b>	N/A	2:1 ratio (LBS-008 : Placebo)
<b>Duration</b>	2 years	2 years
<b>Primary measures</b>	Safety & Tolerability, optimal dose	Safety & Tolerability, Efficacy (Lesion size growth, DDAF)
<b>Other measures</b>	Lesion size (DDAF), QDAF, BCVA, SD-OCT, microperimetry	QDAF, BCVA, SD-OCT, microperimetry



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# Clinical Data

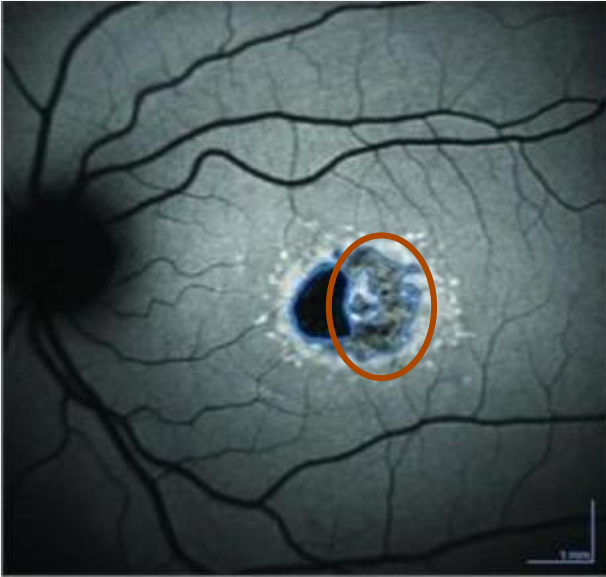
# Interim Phase 2 Results: Summary of Related Adverse Events



Adverse Events	Severity	Relationship to Drug	Frequency (#patients)	% Recovered	% On-going
<b>Xanthopsia</b>	Mild	Definitely Related	6/13	3/6 (50%)	3/6 (50%)
<b>Delayed Dark Adaptation</b>	Mild	Definitely Related	8/13	1/8 (12.5%)	7/8 (87.5%)
<b>Night Vision Impairment</b>	Mild	Definitely Related	1/13	0/1	1/1 (100%)
<b>Increasing error score on FM100</b>	Mild	Probably Related	1/13	0/1	1/1 (100%)

- All instances of DDA and Xanthopsia were **mild** and **transient**
- Subjects shown to have DDA based on laboratory measure were mostly **asymptomatic**
- One subject recorded to have an increasing error score on FM100 test (tests color vision and color perception) showed only a **mild** impact
- **No severe AEs or SAEs** reported and no AEs requiring discontinuation of treatment
- **No clinically significant** findings in relation to vital signs, physical exams or electrocardiograms

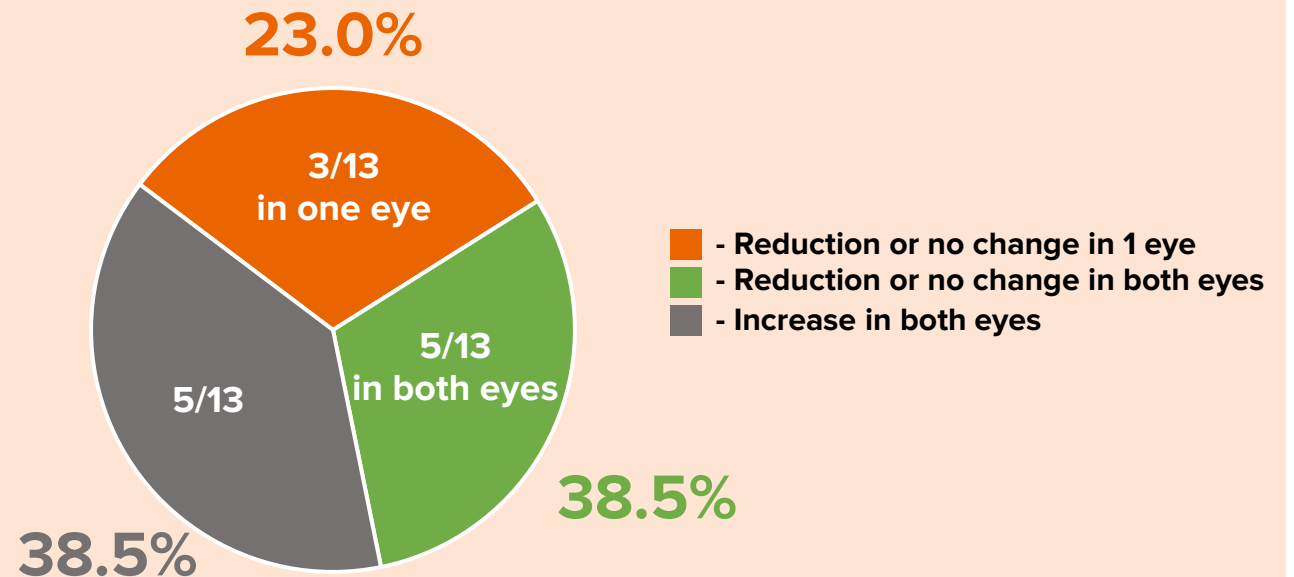
# Interim Phase 2 Data: Change in QDAF in Adolescent STGD1 Subjects



Areas of QDAF progressively evolve into 'dead retina'.

**8 of 13 STGD1 patients showed a reduction or no change in QDAF**

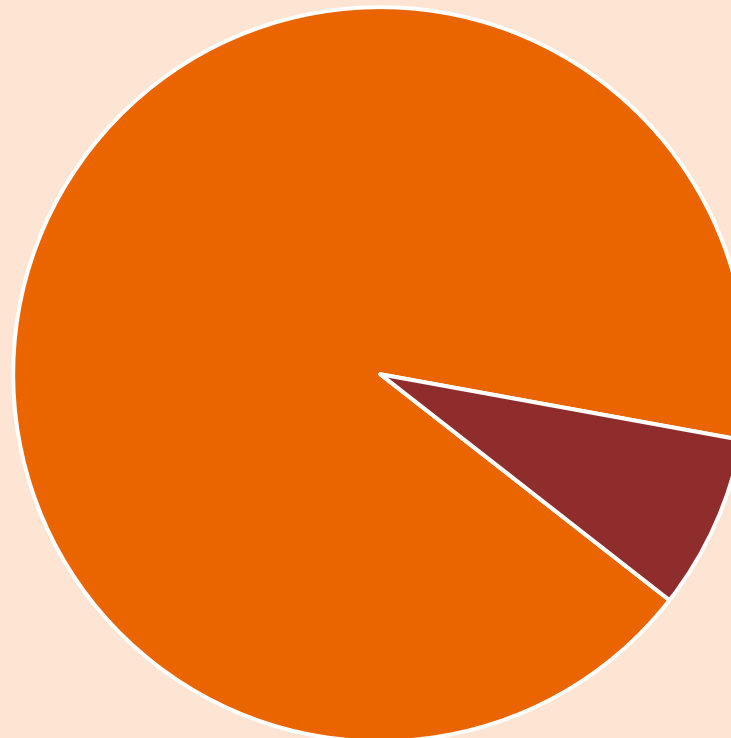
## Distribution of Change in QDAF



# Interim Phase 2 Data: Change in DDAF in Adolescent STGD1 Subjects



DDAF, or lesion (“dead retina”) in STGD1 patient as measured by retinal imaging. This is the area where retinal cells and vision are lost.



**92.3%**

12 of 13 subjects showed no lesion growth

**7.7%**

1 of 13 subjects had lesion growth of 0.3 mm<sup>2</sup> in both eyes

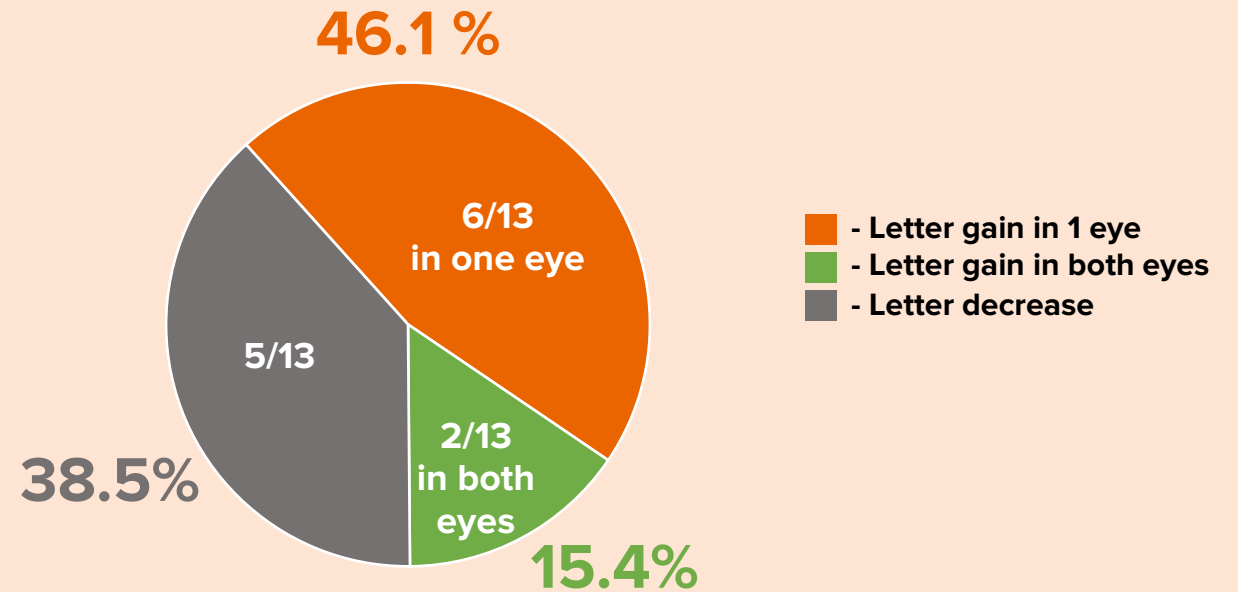


# Interim Phase 2 Results: Change of Vision in Adolescent STGD1 Subjects



Best-Corrected Visual Acuity (BCVA) Test Provides letter score for each eye

## Change in BCVA BCVA gain in 8 of 13 Subjects (61.5%)



# Investment Highlights



- **Early Intervention** with an **novel Oral Treatment** to slow or halt disease progression in both **dry AMD** and **STGD1**.
- Granted **Fast Track Designation, Rare Pediatric Disease** in US / **Orphan Drug Disease** designation in US and EU for STGD1.
- **Clear Clinical Pathway:**
  - LBS-008 continues to be well-tolerated.
  - Ongoing 2-year Phase 2 trial (6 months of reported interim safety data and preliminary efficacy data) in STGD1.
  - A global Phase 3 trial has been initiated in adolescent STGD1 patients.
- **Unmet Market:**
  - **No approved treatments for STGD1 and dry AMD.**
  - STGD1: the most common juvenile macular degeneration (1 in 10,000).
  - Dry AMD: afflict 11 million patients in the US and 196 million patients worldwide.
  - Without treatment, the continual increase in the size of the elderly population will worsen the impact of this disease.



# Thank You

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