



Lin BioScience

Bringing Hope to Incurable Diseases

2025/09

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Novel Treatment for Unmet Medical Need

Lin BioScience, Inc. (TW TPEX: 6696) is a drug development company established in 2016 focusing on advancing novel therapies and first-in-class treatments for unmet medical needs in various therapeutic areas such as ophthalmology, oncology, and metabolic diseases. The Company's pipeline consists of RBP4 IP portfolio, CDC7 IP portfolio and 4 distinct small molecule drug candidates. LBS-008, targeted to treat Stargardt disease and Geographic Atrophy ("GA"), and LBS-009, targeted to treat NASH, derived from the RBP4 IP portfolio, are developed by Belite Bio, a subsidiary company of Lin BioScience. LBS-007, developed from the CDC7 platform technology and targeted to treat various cancers, and LBS-002, targeted to treat glioblastomas and metastatic brain tumors, are developed by Lin BioScience.

LBS-007 is a non-ATP competitive CDC7 inhibitor for the treatment of a broad array of cancers, especially for refractory/relapsed and late-stage cancers such as AML, ALL, ovarian cancer, pancreatic cancer, etc., which has entered phase 1 in 2022. LBS-007 has been granted orphan drug designation (ODD) in the U.S. for the treatment of AML and ALL. LBS-007 has also obtained Fast Track Designation from the US FDA in 2024 for the treatment of AML.

LBS-008 is the only drug candidate intended to treat GA within the current drug development projects of the NIH Blueprint Program ("BPN"), whose mission is to foster small-molecule neurotherapeutic development. The mechanism of action utilized by LBS-008 has been recognized and recommended as a priority for clinical development in both STGD1 and GA in a systematic review published by the U.K. National Institute for Health Research, or the NIHR, in 2018. LBS-008 phase 3 for Stargardt disease is currently ongoing and has completed its enrollment in 2023/2H, with interim results expected in early 2025. Additionally, a Phase 1b/2/3 trial for Stargardt disease was initiated in 2024 and is currently enrolling. For GA, LBS-008 has initiated its phase 3 trial in 2023 and is currently enrolling. LBS-008 has been granted Fast Track Designation, Rare Pediatric Disease designation and Breakthrough Therapy Designation in the U.S., Orphan Drug Designation in the U.S. Europe, and Japan, and Sakigake Designation in Japan for the treatment of STGD1.



Pipeline

Developed by
Lin BioScience

**Oncology
Programs**

LBS
007

Acute Leukemia (FDA ODD)

Multiple Solid Tumors

LBS
002

Glioblastoma /
Brain Metastasis

Developed by
Belite Bio™
Subsidiary company of Lin BioScience

**RBP4 IP
Portfolio**

LBS
008

Geographic Atrophy

Stargardt Disease
(juvenile macular degeneration)

LBS
009

Non Alcoholic Fatty Liver Disease
(NASH) / Type 2 Diabetes

Discovery Pre-Clinical Phase 1 Phase 2 Phase 3 MARKET

Obtained FDA ODD (AML, ALL), FDA fast track (AML)

**Sponsored by Taiwan Industrial Development
Bureau's Innovation Platform Program**

Sponsored by NIH BPN

Obtained US/EU/JP ODD; US FTD, RPD, BTD; JP Sakigake

Chairman

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



- **10+ years of executive management role in biotech, incl. 4 IPO (Lin BioScience & Belite)**

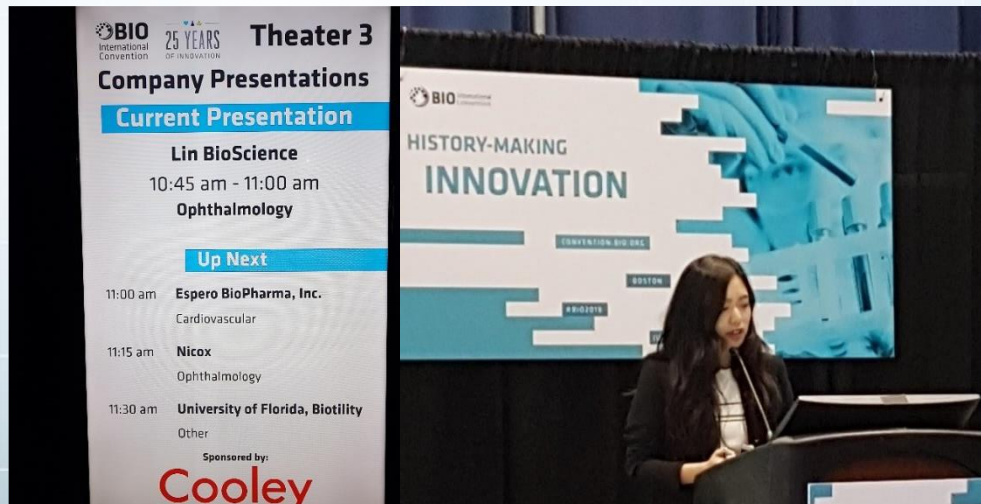
- **Multidisciplinary Specialization Clinical Training & Research in Neuroscience, Cardiovascular & Renal Medicine, Oncology, Immunology & Immunotherapy**

- **PhD in Medicine - University of Sydney**; Specialization: Neurology & Immunology
 - Treatment Strategies for Autoimmune Neuropathies
- **Specialist Certificate in Clinical Neuroscience - University of Melbourne**; Specialization: Neurology
 - Neurological Disorders, Neuroimaging & Diagnostics
 - Clinical Research & Design
- **Master of Medicine - University of Sydney**; Specialization: Multidisciplinary Medicine and Surgery
 - Medicine: Cardiovascular & Renal Medicine, Neonatal Medicine
 - Surgery: Vascular & Endovascular Surgery, Transplant Surgery
- **Cancer Therapeutics & Research Certificate - Harvard Medical School**
- **Master of Business Administration - Columbia University, London Business School, HK University**
- **Extensive Drug development from preclinical to global phase 3 trials**
 - Phase 3 - RBP4 Inhibitor for the Treatment of Atrophic Macular Degeneration & Stargardt Disease
 - Phase 2 - Oubain Antagonist in the Treatment of Essential Hypertension
 - Phase 2 - SERCA2a Inhibitor in the Treatment of Acute Heart Failure
 - Phase 2 - Pan-HER Inhibitor in the Treatment of HER2+ Breast Cancer and Gastric Cancer
 - Phase 3 - Anti-Glycan Active Immunotherapy in the Treatment of Metastatic Breast Cancer
 - Phase 3 - Anti- $\alpha 4$ integrin Antibody in the Treatment of Resistant-Refractory Multiple Sclerosis
 - Phase 2 - mTOR Immunosuppressant in the Treatment of Autoimmune Peripheral Neuropathies
 - Co-invented and applied 64 patents

Management Team

Irene Wang, PhD, MBA (President & CSO)

- PhD in Biochemical Sciences, National Taiwan University, Trained at Scripps Research (TSRI), EMBA from University of California San Diego
- Co-invented and applied 125 patents and published 6 papers
- Extensive Drug development from preclinical to global phase 3 trials and **3 IPOs (including Lin BioScience and Belite Bio)**



“I’ve loved chemistry since I was little. I was dedicated to studying chemistry and scientific research since middle school. And now, I’m working on drug development, doing significant things to improve the lives of human beings.”



Irene Wang, PhD, MBA
President
LIN BIOSCIENCE

Management Team

Serena Chen
CFO



- Certified Public Accountant & master in accounting from National Taipei University.
- Finance manager in a Taiwan biotech company and as assistant manager of audit department in Deloitte Taiwan
- Vast experience in auditing of **listed companies and initial public offering (including Lin BioScience and Belite Bio)**

Tzung-Ju Wu, PhD
Associate Director, R&D



- Ph.D. in Cellular and Molecular Pharmacology from Rutgers University
- 10-years of global Pharma/Biotech R&D experience in Sanofi Genzyme, Taiwan Liposome Company and Insilico Medicine
- Experience in leading R&D teams to conduct innovative research and support drug discovery and development in multi-disease area



**LBS-007
FOR ACUTE LEUKEMIA &
SOLID TUMOR**



CONGRATULATIONS!

LBS-007 Received U.S. FDA Fast Track Designation!!



We are excited to announce that the FDA has granted Fast Track Designation to LBS-007 for the potential treatment of relapsed or refractory acute myeloid leukemia (AML) on **26Nov2024**. The FDA's decision to grant LBS-007 Fast Track Designation for AML underscores the urgent need for a new therapeutic to fill the unmet medical need associated with Leukemia.

LBS
007

THERAPEUTIC OPTIONS FOR CANCER

Unmet Medical Needs for Cancer Treatment - Next Generation Therapies

**Genetic
Therapy**

Immunotherapy

LBS-007

Targeted Therapy

Chemotherapy

LBS-007:

An Innovation to Transform the Cancer Treatment Landscape

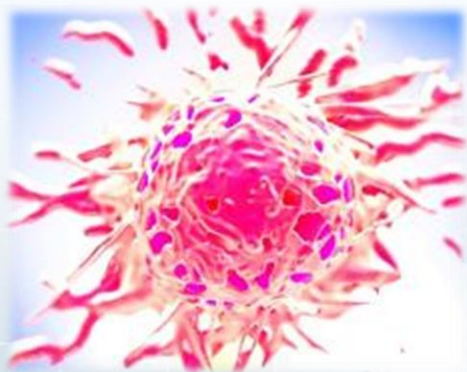
- Cancer treatments are like a pyramid—higher levels have fewer side effects but are more costly and less applicable.
- Chemotherapies form the basis of all cancer treatments. Targeted therapies or immunotherapies are complemented by chemotherapies. Even late-stage cancers and cancers with limited treatment options are often managed by chemotherapies.
- The substantial side effects of chemotherapies, with their technology unchanged over 60 years, remain a significant unmet medical need.

LBS
007

Non-ATP CDC7 Inhibitor

for treatment of Broad Variety of Cancer types

- DISCOVERY
- PRE-CLINICAL
- ☒ PHASE I/II
- PHASE II/III
- MARKET



Novel Anti-Cancer Target Therapy



Orphan Drug Designation

For ALL: #DRU-2017-6250

For AML: #DRU-2024-10100

Investigational New Drug

#120774 became active on 05Oct2024

Fast Track Designation

For AML, granted on 26Nov2024

MARKET

\$5B

Expected 2026 market
size of AML & ALL

\$55B

Expected 2023 market
size of pancreatic, lung,
ovarian cancers

1.7 in 100k⁽¹⁾

4.1 in 100K⁽²⁾

(1) ALL incidence (2016)

(2) AML incidence (2020)

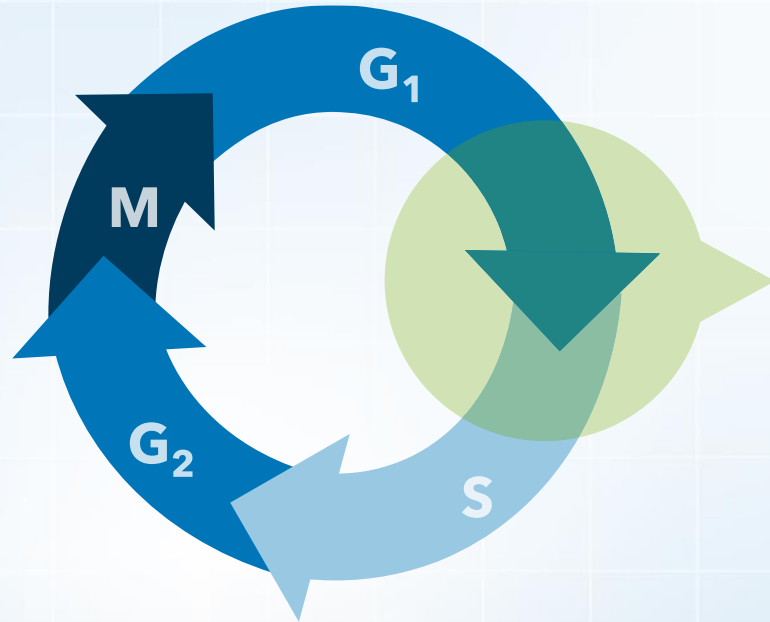
\$6B

Estimated global market

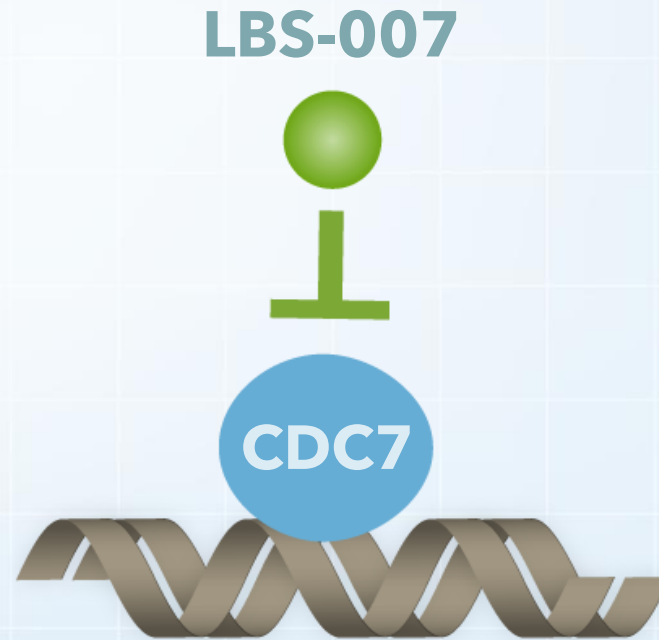
Reference: Globaldata, Marketwatch, NIH National Cancer Institute

LBS
007

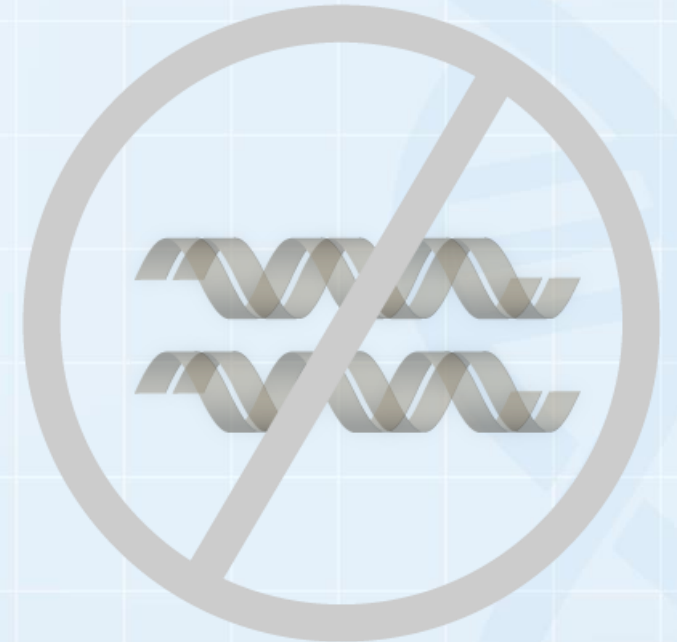
Inhibits CDC7 in Cell Cycle Regulation



- 1 TARGETS**
S Phase Progression



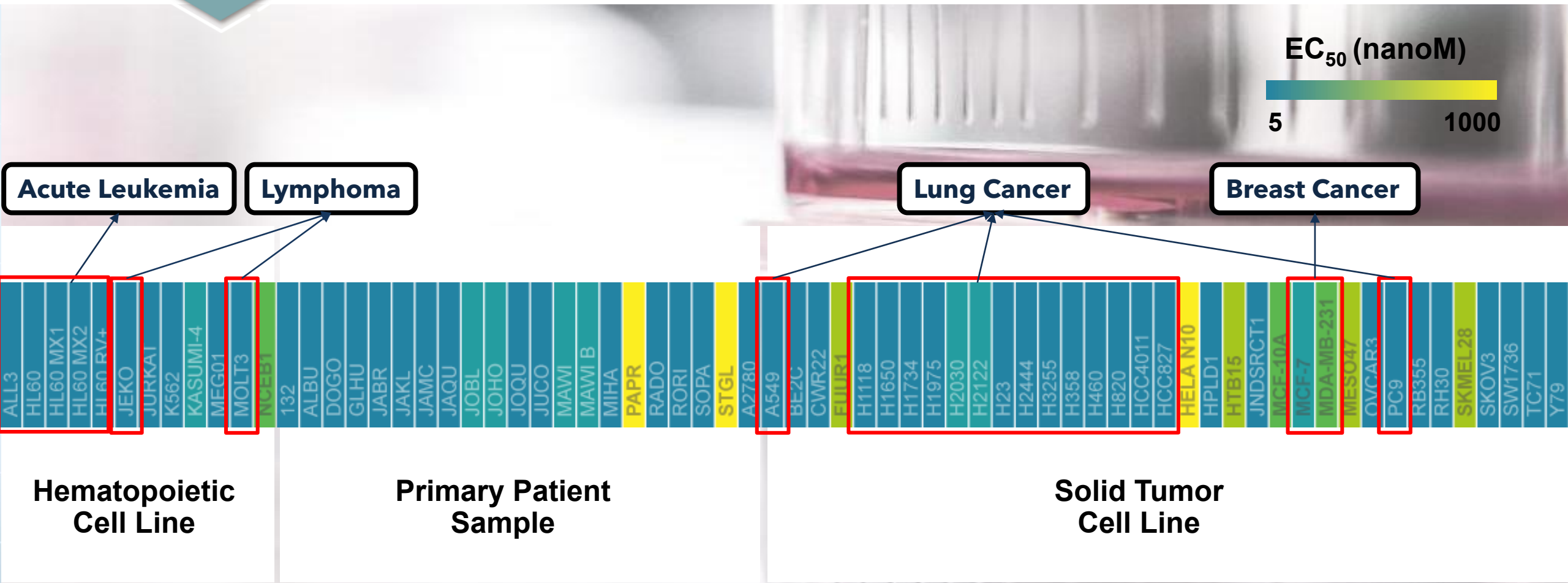
- 2 INHIBITS**
CDC7's role in
DNA Replication



- 3 PREVENTS**
Cell Division



Potently Inhibits Multiple Cancer Cell Lines & Primary Patient Samples of Blood Cancers

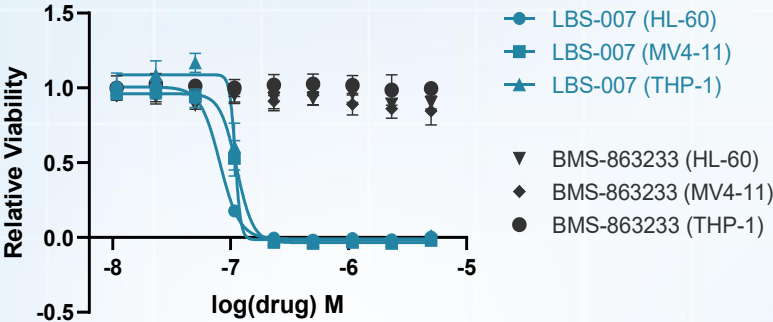




SUPERIOR EFFICACY AT NANOMOLAR POTENCY

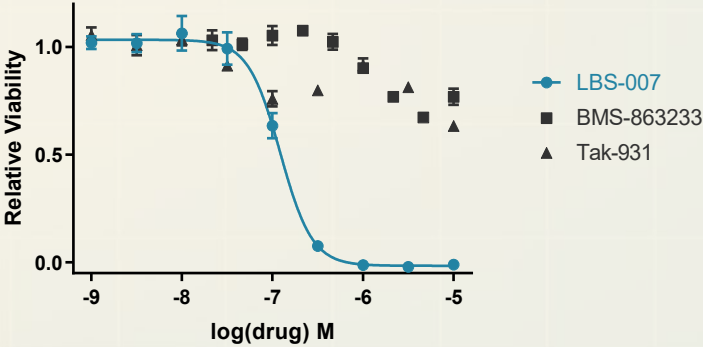
Approx. 150 nanomolar of LBS-007 can achieve therapeutic effect on cancer cells

AML



EC ₅₀ (nM)	HL-60	MV4-11	THP-1
LBS-007	83.8	71.5	108
BMS-863233	> 10,000	5,400	> 10,000

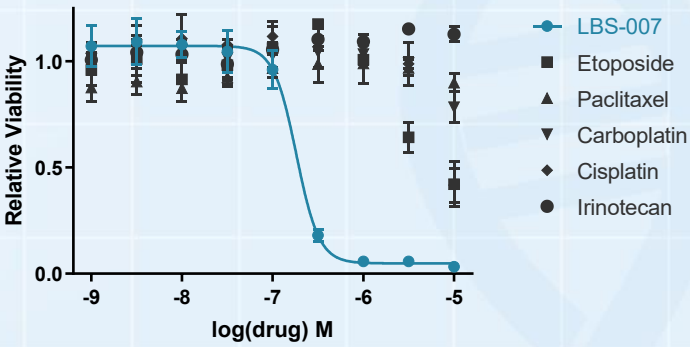
Pancreatic cancer



EC ₅₀ (nM)	Panc-1
LBS-007	127
BMS-863233	1,100
Tak-931	> 10,000

8.6x efficacy

Lung cancer



EC ₅₀ (nM)	H146
LBS-007	183
Cisplatin	2,800
Etoposide	> 10,000
Irinotecan	> 10,000
Paclitaxel	> 10,000
Carboplatin	> 10,000

15x efficacy

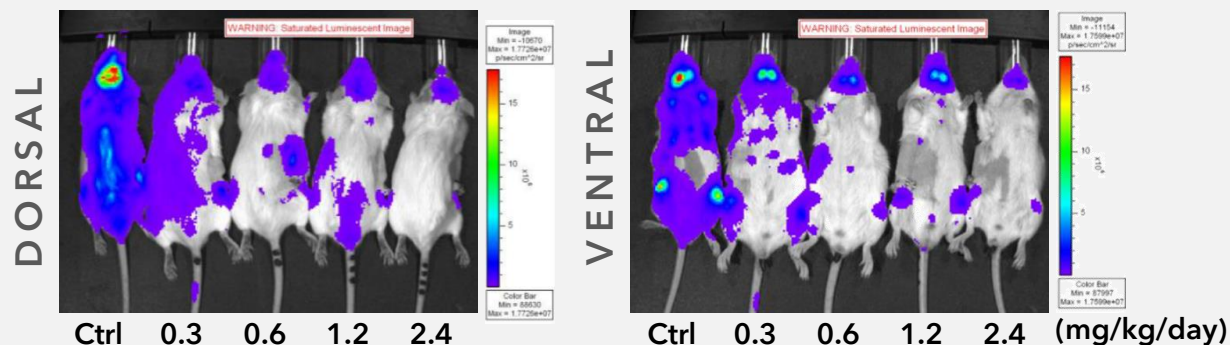
50+
x efficacy

LBS
007

In Vivo Efficacy Demonstrated in Animal Models

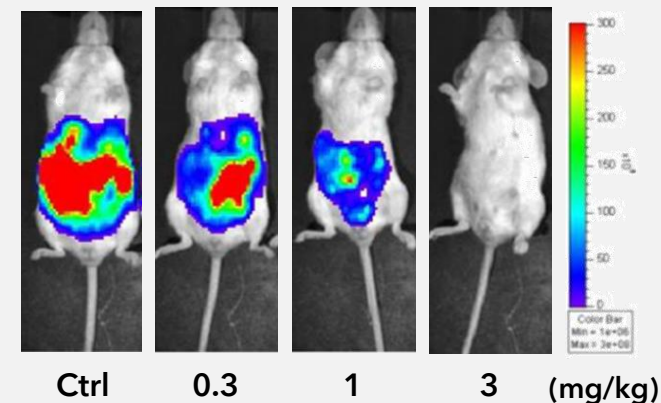
Potent tumor reduction in ALL and solid tumor mouse models

Acute Lymphoblastic Leukemia (ALL)



- ✓ In vivo dose responsive efficacy
- ✓ 95% tumor removal at 2.4 mg/kg/day
- ✓ No significant organ dysfunction or toxicity at therapeutic dose

Ovarian Cancer



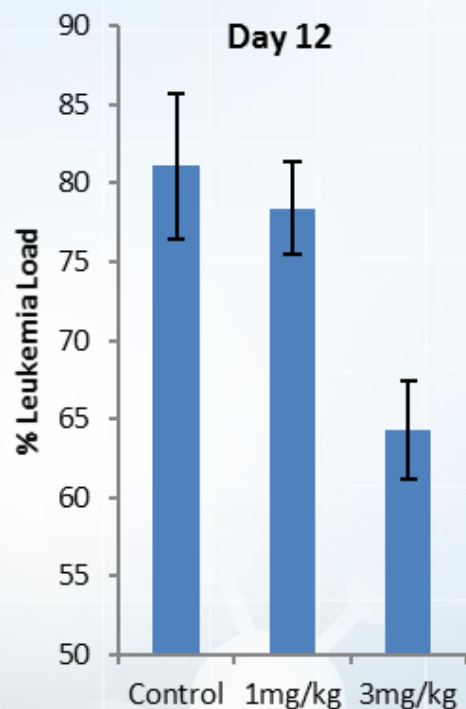
- ✓ In vivo dose responsive efficacy
- ✓ Inhibits ovarian cancer growth in mice
- ✓ Significant improvement in long-term survival

LBS
007

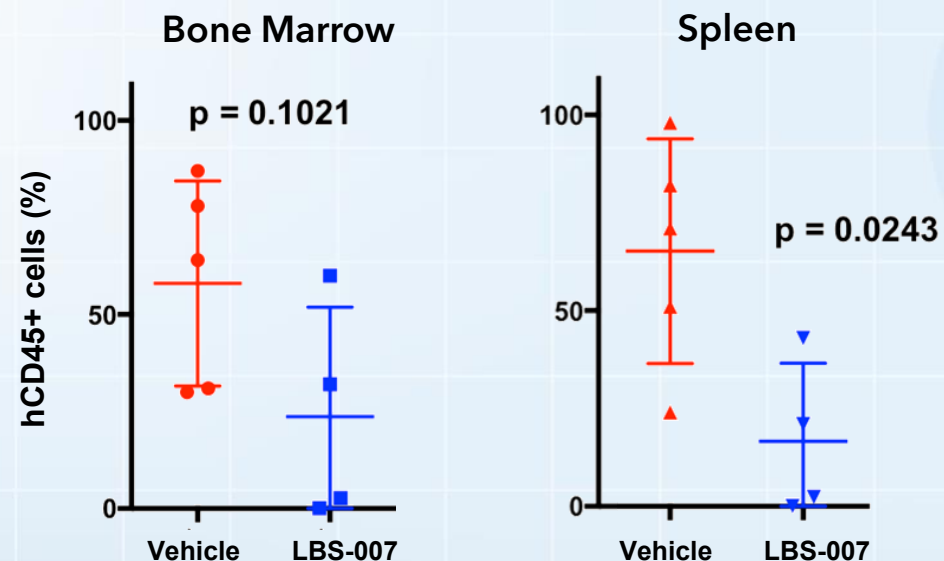
In Vivo Efficacy Demonstrated in Animal Models

Potent tumor reduction in AML mouse models

Aggressive AML mouse model (MLL-AF9)



AML patient-derived xenograft in mice



- ✓ **Disease burden reduced** in aggressive AML mouse model at 3 mg/kg
- ✓ Inhibits human AML growth in mice



Clinical Development Summary



	LBS-007-CT01
Phase	1/2 (Phase 2 dose expansion after determining optimal dose in Phase 1)
Enrollment	Estimated to enroll 90 patients
Sites	Australia, Taiwan, US, China
Masking	Open Label
Treatment duration	7 consecutive days for one 28-day cycle
Primary measures	Safety, tolerability, optimal dose, and PK profile of LBS-007
Other measures	Efficacy of LBS-007
Key Inclusion Criteria	Aged ≥ 18 , with confirmed relapsed or resistant AML or ALL, ineligible for standard therapies with an ECOG of 0 to 2.



MAJOR MILESTONES

Opening a New Era in Cancer Treatment

- ✓ **2023/02/10** - We announced that our new drug, LBS-007, has been approved by the Central Adelaide Local Health Network Human Research Ethics Committee (CALHN HREC) to conduct Phase I/II clinical trials for acute leukemia (including AML and ALL) in Australia. The company aims to address unmet medical needs in the cancer treatment market.
- ✓ **2023/08/11** - We announced that our new drug, LBS-007, has been approved by Taiwan's Food and Drug Administration (TFDA) to conduct Phase I/II clinical trials for acute leukemia (including AML and ALL) in Taiwan.
- ✓ **2024/10/05** - We announced that our new drug, LBS-007, for the treatment of acute leukemia has passed the US FDA IND review for human clinical trials. Efforts to initiate Phase I/II clinical trials in the US are underway.
- ✓ **2024/11/26** - We received Fast Track Designation from the U.S. FDA for our new drug, LBS-007, aimed at treating AML.
- ✓ **2025/07/22** - First U.S. clinical Site Initiation Visit (SIV) completed.
- ✓ **2025/09/29** - We announced that our new drug, LBS-007, has been approved by China's National Medical Products Administration (NMPA) to conduct Phase I/II clinical trials for acute leukemia (including AML and ALL) in China.
- ✓ Following the completion of Phase I clinical trials, the safety and efficacy of LBS-007 will be confirmed. We plan to simultaneously initiate clinical trials targeting other hard-to-treat solid tumors, including pancreatic cancer, small-cell lung cancer, and ovarian cancer.



LBS-008 FOR GEOGRAPHIC ATROPHY & STARGARDT DISEASE



Belite Bio Pipeline Overview



PRECLINICAL

PHASE 1

PHASE 2

PHASE 3

NDA

Tinlarebant

○ Stargardt Disease (STGD1)

- Ph2 24-month final data continues to **show slowing of lesion growth**
- Ph3, 2-year treatment, global trial ("DRAGON" Study) is ongoing (**104 subjects, age 12-20, topline data expected Q4 2025**)
- Ph2/3, 2-year treatment, global trial ("DRAGON II" Study) is ongoing (**60 subjects, age 12-20**)

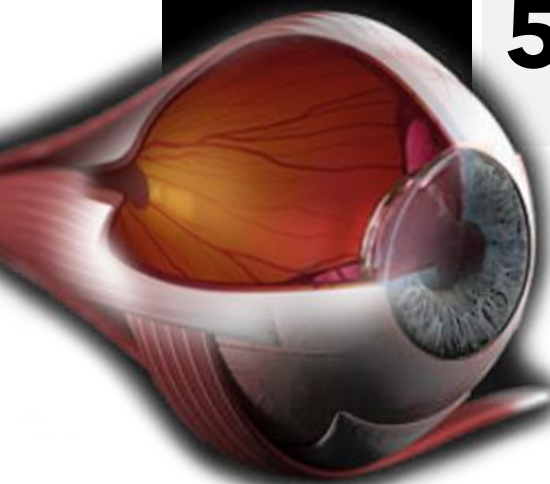
○ Geographic Atrophy (GA)

- A Ph3, 2-year treatment, global trial ("PHOENIX" Study) is ongoing (completed enrollment, **529 subjects**)

- **Tinlarebant** 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 **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.
- **Unmet Market Opportunity:**
 - No FDA approved treatments for STGD1
 - No FDA approved orally administered treatments for GA
- **Breakthrough Therapy Designation, Fast Track Designation & Rare Pediatric Disease** 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

Tinlarebant (LBS-008)

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



STARGARDT

1 in 8,800⁽³⁾

The most common
inherited retinal dystrophy

Patient population with
Stargardt Disease:

53k⁽³⁾
US

109k⁽³⁾
China

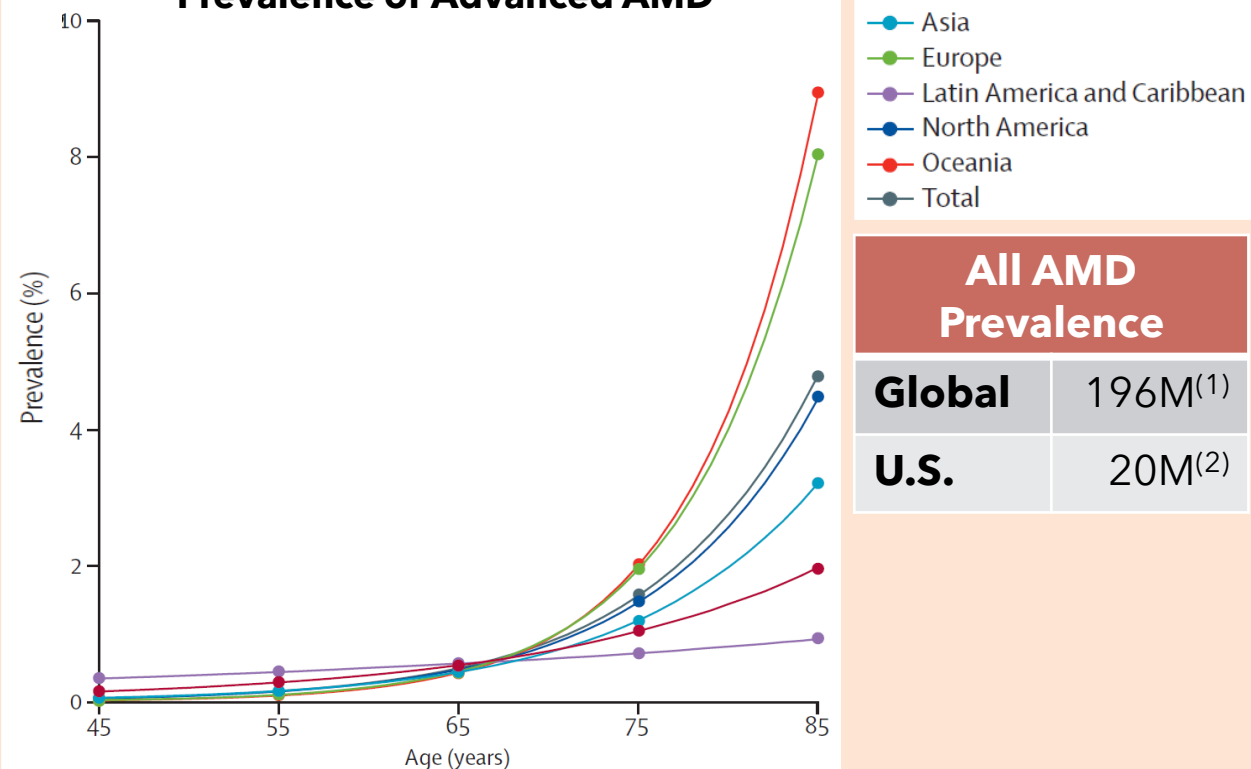
Columbia University + NIH Blueprint

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

Market Opportunity

Advanced AMD

Prevalence of Advanced AMD



• **AMD patient population is expected to grow from 196M in 2020 to 288M in 2040⁽¹⁾**

Reference: (1) Wan LingWong et al. Global prevalence of AMD and disease burden projection for 2020 and 2040. 2014; (2) Prevalence Estimates Vision and Eye Health Surveillance System Vision Health Initiative (VHI) CDC, 2022. (3) HananyM, RivoltaC, Sharon D (2020) Worldwide carrier frequency and genetic prevalence of autosomal recessive inherited retinal diseases. Proc Natl Acad Sci U S A. 117: 2710-2716/Cornelis SS, RunhartEH, Bauwens M, Corradi Z, De BaereE, RoosingS, Haer-WigmanL, DhaenensCM, Vulto-van SilfhoutAT, CremersFPM (2022) Personalized genetic counseling for Stargardt disease: Offspring risk estimates based on variant severity. Am J Hum Genet. 109: 498-507./Mata N, Quinodoz M, Rivolta C, Scholl HPN (2025) in preparation.



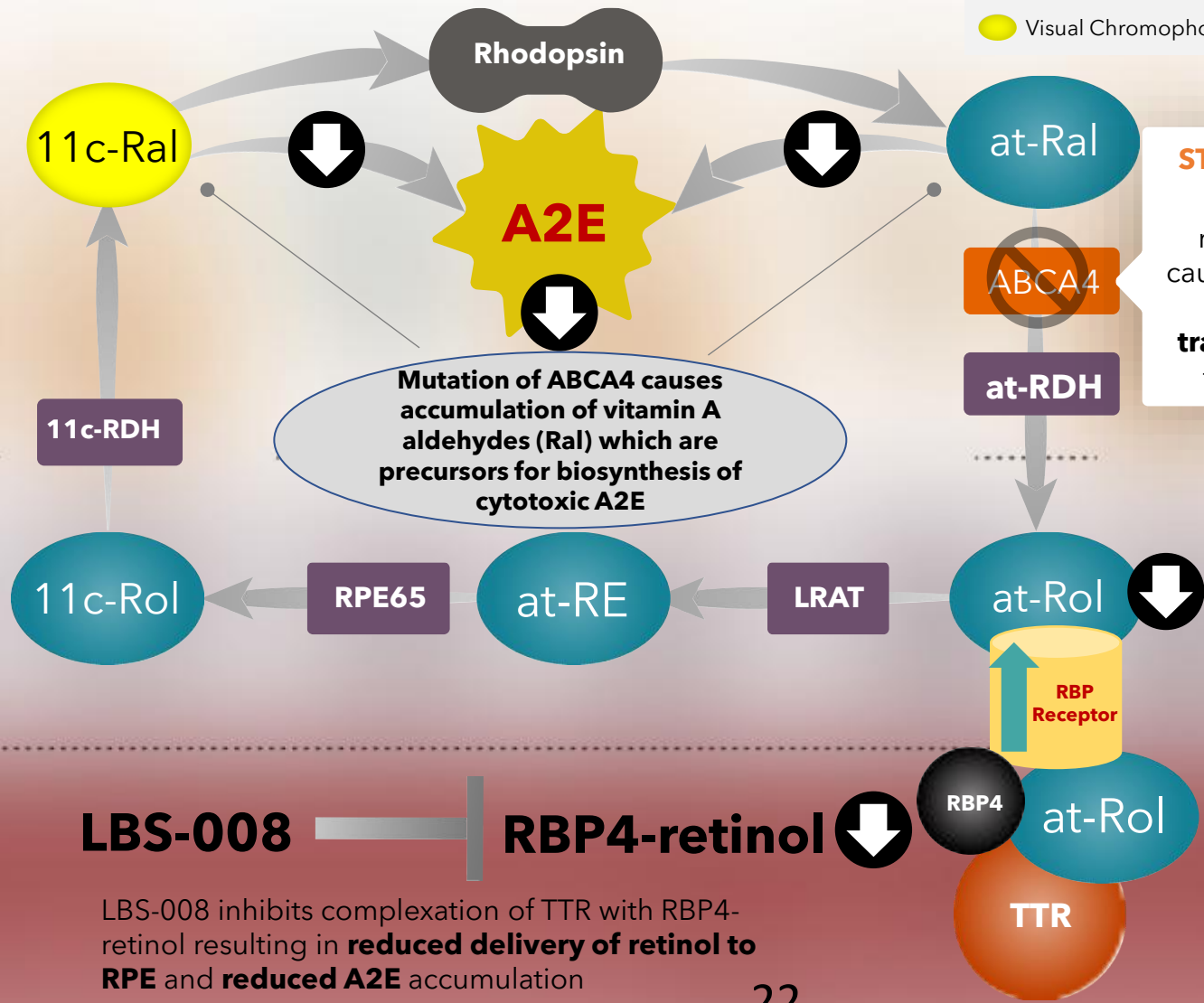
LBS-008 Mechanism of Action

PHOTORECEPTORS (PR)

RETINAL PIGMENT EPITHELIUM (RPE)

BLOODSTREAM

- ⬇ LBS-008 Induced Down-Regulation
- Enzymes
- Visual Pigment
- Retinoids
- Visual Chromophore



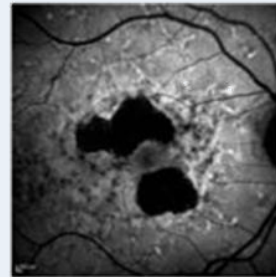
LBS-008 — RBP4-retinol

LBS-008 inhibits complexation of TTR with RBP4-retinol resulting in **reduced delivery of retinol to RPE** and **reduced A2E** accumulation

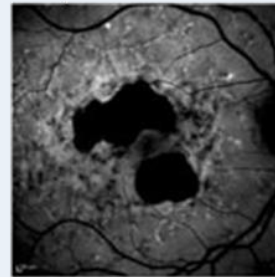
Similar Pathophysiology in STGD1 & GA

- **STGD1 and GA 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 or halting the spread of 'dead retina'** is the intended **effect of Tinalarebant treatment**

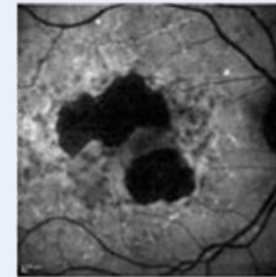
STGD1: 61-year-old female:



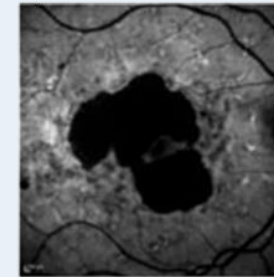
Baseline:
0.1 LogMAR



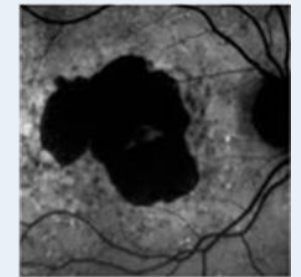
+12 Months:
0.1 LogMAR



+24 Months:
0.0 LogMAR

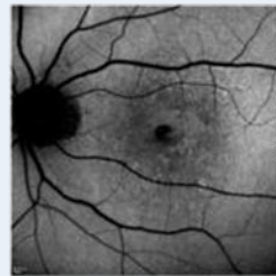


+36 Months:
0.1 LogMAR

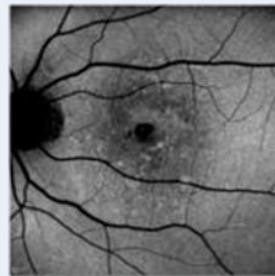


+57 Months:
0.5 LogMAR

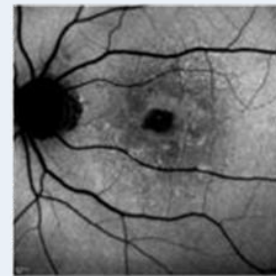
GA: 73-year-old female:



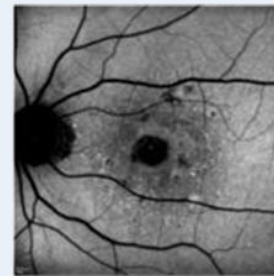
BL:
0.2 LogMAR



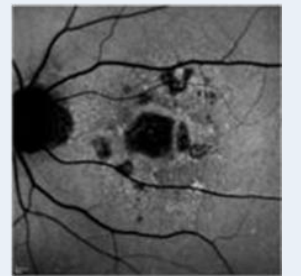
+12 Mo:
0.2 LogMAR



+ 24 Mo:
0.3 LogMAR



+ 36 Mo:
0.4 LogMAR



+55 Mo:
0.6 LogMAR



PHASE 2 STGD1 TRIAL

LBS
008

CLINICAL TRIAL DESIGN OVERVIEW IN STGD1 PHASE 2

Reduction in Lesion Growth Rate (DDAF) as Measured by Retinal Imaging is an FDA Accepted Primary Endpoint in STGD1 and GA

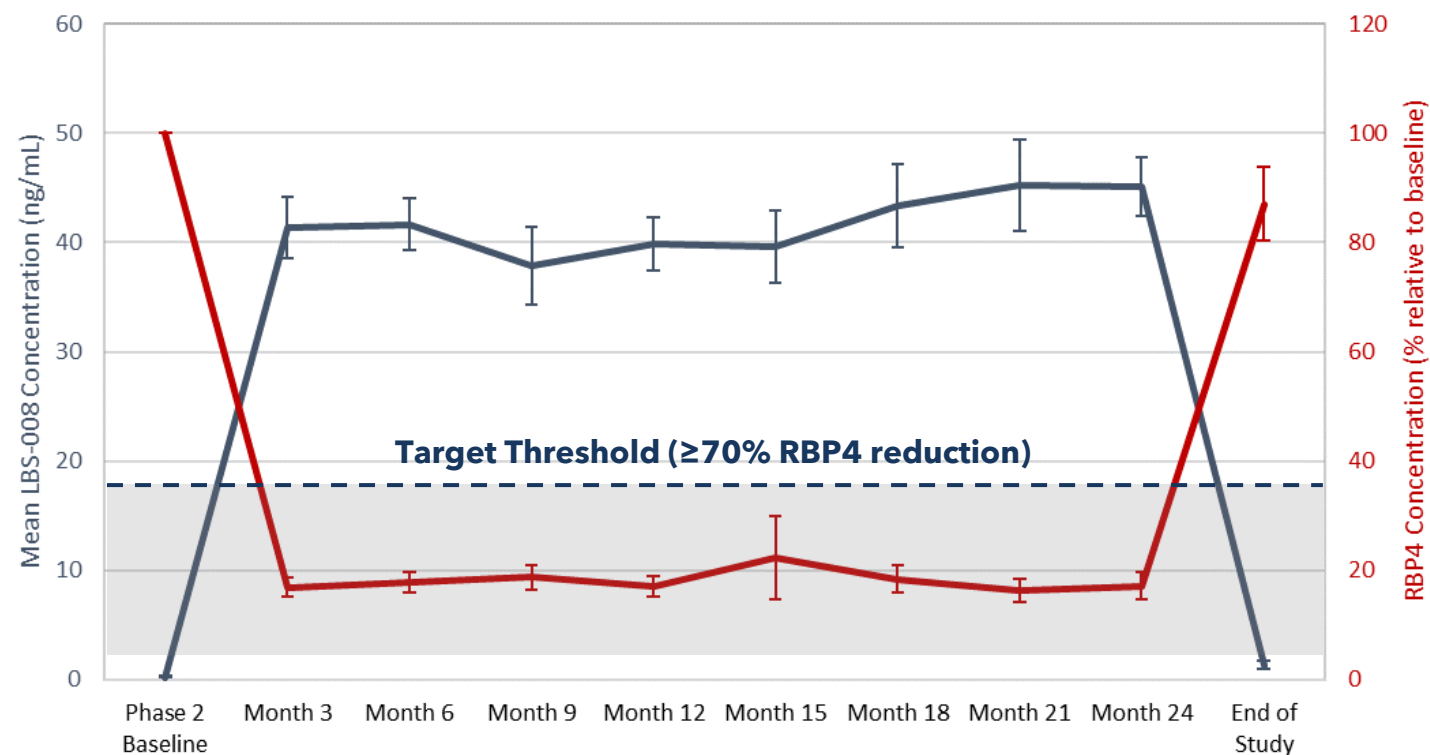
STGD1 Phase 2 "LBS-008-CT02" (Preliminary 24-Month Interim Data Available)	
Enrollment	13 subjects* (QDAF, no DDAF)**
Sites	Australia & Taiwan
Masking	Open Label
Placebo	N/A
Treatment duration	2 years
Primary measures	Safety & tolerability, optimal dose
Other measures	DDAF, QDAF, BCVA, SD-OCT, microperimetry
Interim analysis	Yes
Key inclusion criteria	12-18 years old, diagnosed STGD1 with at least one mutation identified in the ABCA4 gene

*LBS-008-CT02 initially enrolled 13 subjects in Australia and Taiwan. One subject in Australia was lost to follow up, therefore 12 subjects with complete 24-month data were evaluated.

**DDAF = definitely decreased autofluorescence; QDAF = questionably decreased autofluorescence.

LBS
008

PH2 24-MONTH: REDUCTION OF PLASMA RBP4 LEVELS



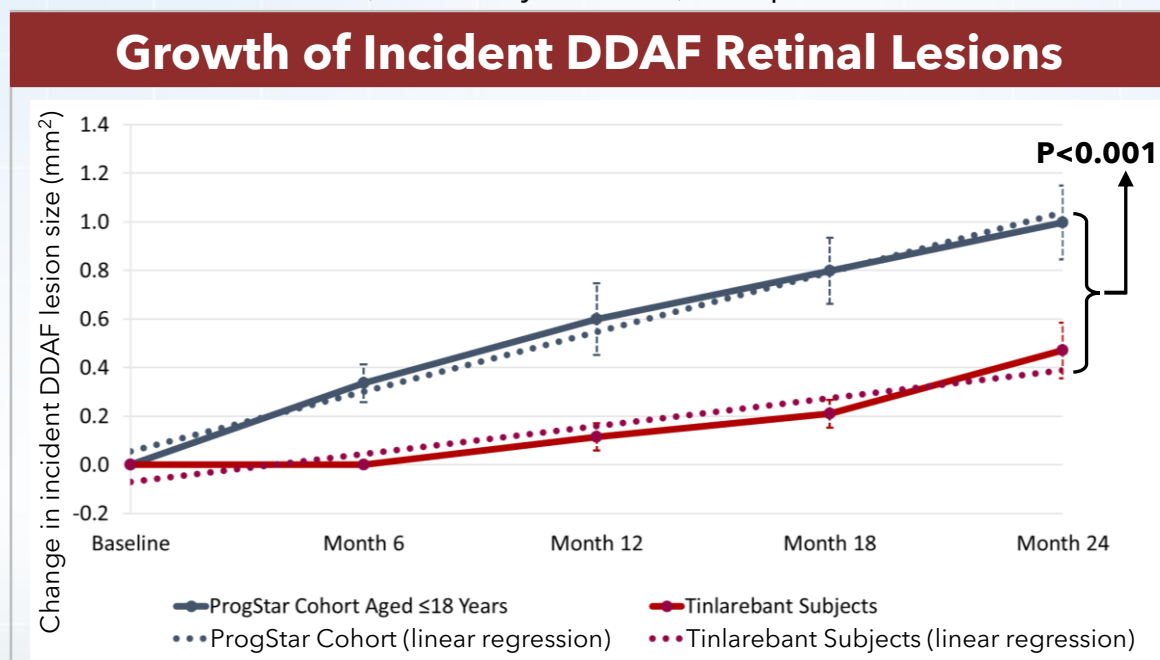
- The 5 mg daily dose was effective to reduce RBP4 level by a mean of approximately 80% relative to baseline
- RBP4 levels returned to 87% of the baseline value at End of Study (28-days following drug cessation)
- Recovery of RBP4 concentration correlated well with the decreased tinlarebant exposure

Note: Preliminary data and is subject to data verification and clean-up

LBS
008

PH2 24-MONTH: SUSTAINED LOWER LESION GROWTH COMPARED TO PROGSTAR

- A comparison of incident DDAF lesion growth among ProgStar subjects with similar baseline characteristics as subjects in LBS-008-CT02 (i.e., ≤ 18 years old) was performed



	LBS-008-CT02	ProgStar Cohort ^{1,2}
Patient Pool	N=12	N=51* (aged ≤ 18 years)
Mean change in incident DDAF lesion size at Month 24	0.51 $\pm$ 0.4 mm ²	1.00 $\pm$ 1.3 mm ²

Note:

* Only 50 patients from ProgStar Cohort (aged ≤ 18) were included in the analysis due to one subject having ungradable screening FAF data

1. Strauss RW, Ho A, Muñoz B, et al. ProgStar Report No. 1. Ophthalmology. 2016;123(4):817-28.

2. Strauss RW, Muñoz B, Ho A, et al. ProgStar Report No. 9. JAMA Ophthalmol. 2017; 135(11):1232-1241.

- No development of DDAF** in 5 of 12 subjects at the 24-month timepoint
- A comparison of the 24-month DDAF lesion growth between Tinalarebant-treated subjects and ProgStar participants possessing similar baseline characteristics (aged ≤ 18 years) showed a sustained lower DDAF lesion growth in Tinalarebant-treated subjects over the 24-month treatment period ($p < 0.001$)

Note: Preliminary data and is subject to data verification and clean-up



DRAGON & DRAGON II STGD1 TRIALS



DRAGON & DRAGON II CLINICAL TRIAL DESIGN IN STGD1

Reduction in Lesion Growth Rate (DDAF) as Measured by Retinal Imaging is an FDA Accepted Primary Endpoint in STGD1 and GA

	STGD1 “DRAGON” Phase 3 ⁽¹⁾	STGD1 “DRAGON II” Phase 1b/2/3
Enrollment	104 subjects (have DDAF)	60 subjects (have DDAF)
Sites	Global	Japan, US, UK
Randomization	2:1 ratio (Tinarebant : Placebo)	1:1 ratio (Tinarebant : Placebo)
Masking	Double Blind	
Treatment duration	2 years	
Primary measures	Efficacy as measured through DDAF lesion growth rate, safety & tolerability	
Other measures	QDAF, BCVA, SD-OCT, microperimetry	
Interim analysis	Yes	
Key inclusion criteria	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 ²), a BCVA of 20/200 or better	

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



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%)



DRAGON INTERIM ANALYSIS CONCLUSIONS

- **No modification of the study is required**
 - **Continue the study without sample size increase**
 - **Tinlarebant (5 mg p.o., daily) continues to be safe and well tolerated in adolescent STGD1 patients**
 - **At the time of the Interim Analysis, the overall withdrawal rate is 9.6% (10 of 104 Subjects); the withdrawal rate due to ocular adverse events is 3.8 % (4 of 104 Subjects)**
 - **Visual acuity was stabilized in the majority of subjects, with mean change from baseline of less than three letter scores under both standard and low luminance, throughout the two-year study**
-
- **Additional DSMB comments:**
 - **It is recommended to submit the data for further regulatory review for drug approval**



DRAGON INTERIM SAFETY DATA

TREATMENT-EMERGENT ADVERSE EVENTS

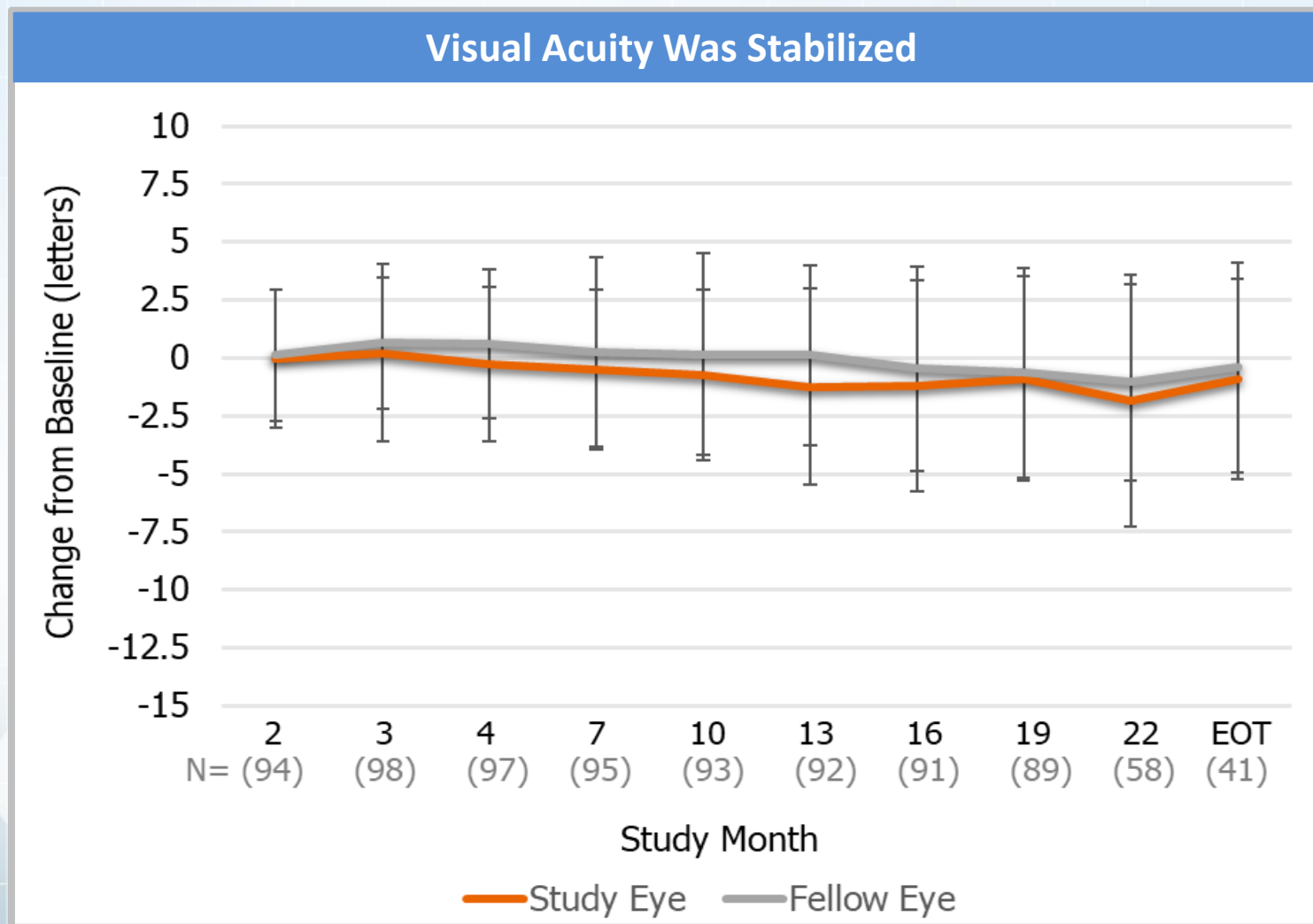
Adverse Events	Severity	Frequency N=104 (# and % of patients)
Xanthopsia	Mild	28 (26.9%)
Delayed Dark Adaptation	Mild	27 (26.0%)
Night Vision Impairment	Mild	15 (14.4%)
Headache	Mild	8 (7.7%)

- Tinalrebant (5 mg p.o., daily) continues to be **safe** and **well tolerated** in adolescent STGD1 patients
- Xanthopsia and Delayed Dark Adaptation are the most common drug related ophthalmic AEs
- Majority of Xanthopsia, Delayed Dark Adaptation and Night Vision Impairment were **mild**; some resolved while on treatment
- Headache is the most common treatment-related non-ocular AE
- No severe or serious treatment-related AEs reported
- No clinically significant findings in relation to vital signs, physical exams, cardiac health, or organ functions

Note: Data Extraction Date as of 11Feb2025; for ocular AE, only frequency > 10% is listed and for non-ocular AE, only frequency > 7% is listed

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DRAGON INTERIM VISUAL ACUITY DATA CHANGE FROM BASELINE (ETDRS LETTER SCORE, MEAN)



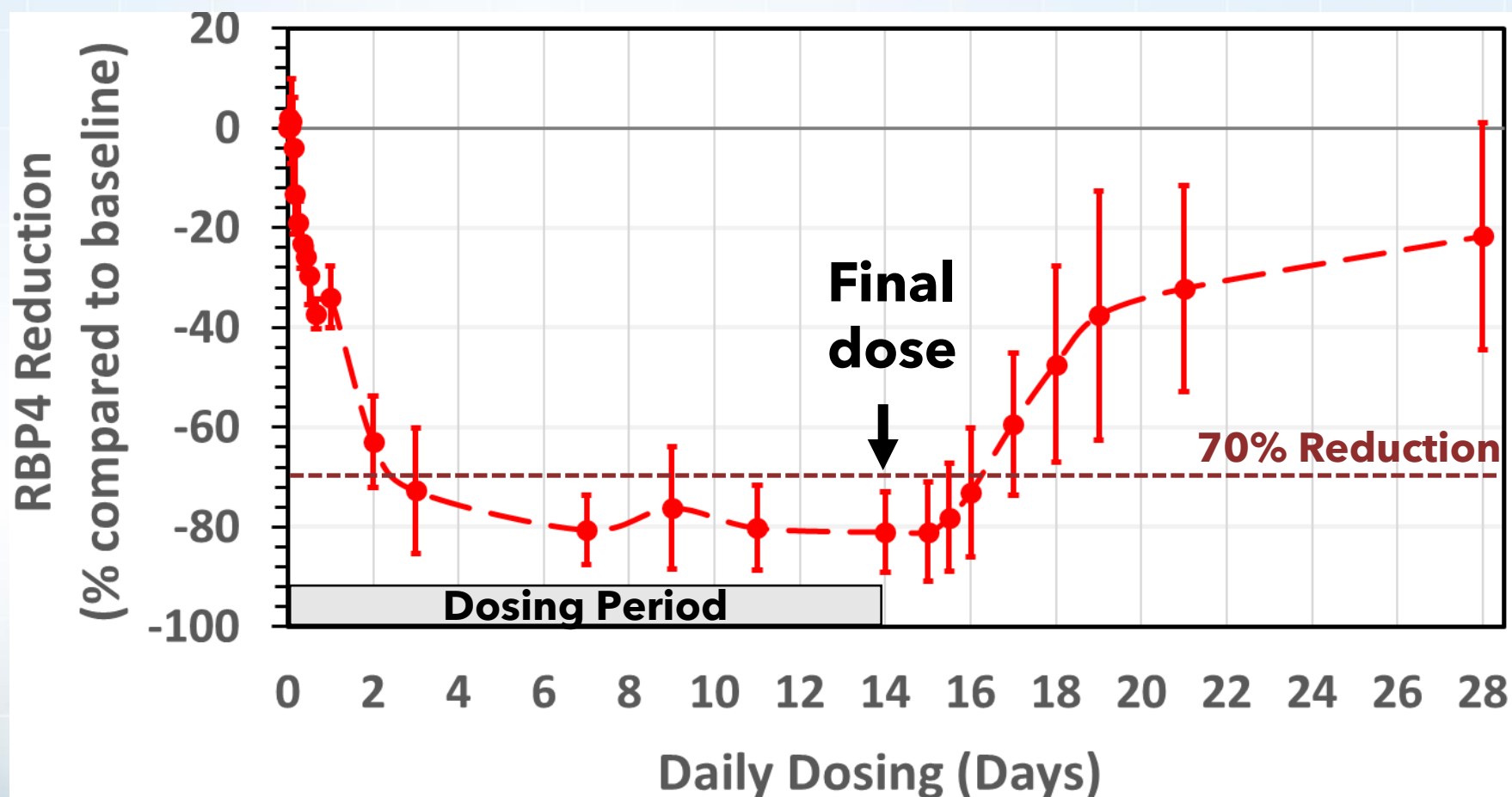


PHASE 3 PHOENIX TRIAL IN GEOGRAPHIC ATROPHY

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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 for STGD1 and GA
- **Early Intervention** – Targeting patients with small lesion size to potentially slow or halt 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” ⁽¹⁾
Enrollment	529 subjects
Sites	Global
Masking	Double Blind
Placebo	2:1 ratio (Tinarebant : Placebo)
Treatment duration	2 years
Primary measures	Slowing of atrophic lesion growth, safety & tolerability
Other measures	BCVA, SD-OCT, microperimetry
Interim analysis	Yes

⁽¹⁾ Additional Phase 3 study expected to be required prior to NDA filing



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