Belite BIO

Q2 2024 Financial Results Conference Call

August 12, 2024, 4:30 p.m. ET
Nasdaq: BLTE

For more info please visit: www.belitebio.com

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This presentation contains forward-looking statements, including statements regarding the potential implications of clinical data for patients, and Belite Bio's advancement of, and anticipated preclinical activities, clinical development, regulatory milestones, and commercialization of its product candidates. Actual results may differ materially from those indicated in the forward-looking statements as a result of various important factors, including but not limited to Belite Bio's ability to demonstrate the safety and efficacy of its drug candidates; the clinical results for its drug candidates, which may not support further development or regulatory approval; the timing to complete relevant clinical trials and/or to receive the interim/final data of such clinical trials; the content and timing of decisions made by the relevant regulatory authorities regarding regulatory approval of Belite Bio's drug candidates; Belite Bio's ability to achieve commercial success for its drug candidates, if approved; Belite Bio's ability to obtain and maintain protection of intellectual property for its technology and drugs; Belite Bio's reliance on third parties to conduct drug development, manufacturing and other services; Belite Bio's limited operating history; Belite Bio's ability to obtain additional funding for operations and to complete the development and commercialization of its drug candidates; Belite Bio's ability to enter into additional collaboration agreements beyond its existing strategic partnerships or collaborations, as well as those risks more fully discussed in the "Risk Factors" section in Belite Bio's filings with the U.S. Securities and Exchange Commission. All forward-looking statements are based on information currently available to Belite Bio, and Belite Bio undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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Belite Participants

Belite Management Team

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

- 10+ 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, HK University

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)

- 13+ 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

Tinlarebant
(LBS-008)

2Q 2024 Highlights

- DRAGON II: completed enrollment in Japan for Phase 1b, enrollment ongoing in U.S. and UK
- Obtained Pioneer Drug (Sakigake) Designation in Japan for the treatment of STGD1
- PHOENIX: approximately 200 subjects have been enrolled
- Raised $25 million in gross proceeds in a registered direct offering

Belite Bio Pipeline Overview

	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	NDA

Tinlarebant (LBS-008)

- **Stargardt Disease (STGD1)**
  - Ph2, 24-month final data continued to **show slowing of lesion growth**
  - Ph3, 2-year treatment, global trial ("DRAGON" Study) is ongoing (completed enrollment, **104 subjects, age 12-20,** interim analysis expected in 4Q 2024)
  - Ph 1b & 2/3, 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 **(~430 subjects, ~200 enrolled)**

- **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
- **Fast Track Designation & Rare Pediatric Disease** in US and **Orphan Drug** designation in US / EU / JP, **Pioneer Drug** (Sakigake) 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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Phase 2 STGD1 Trial

Tinlarebant
(LBS-008)

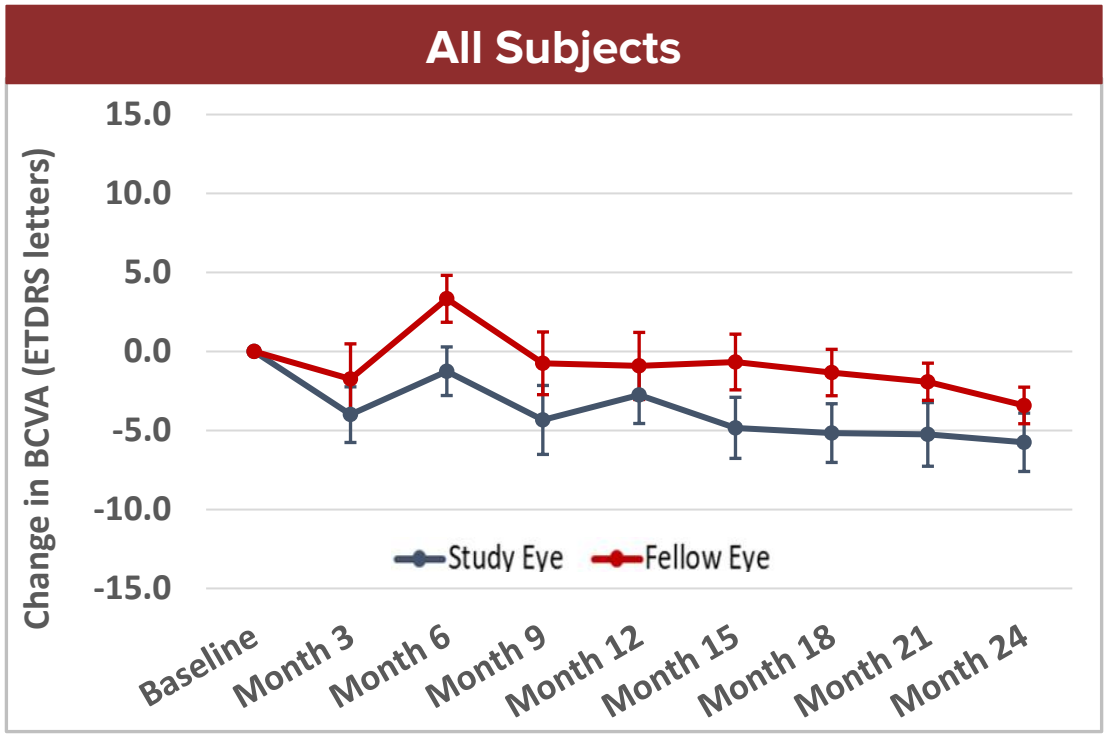
Review of Study and Results to Date

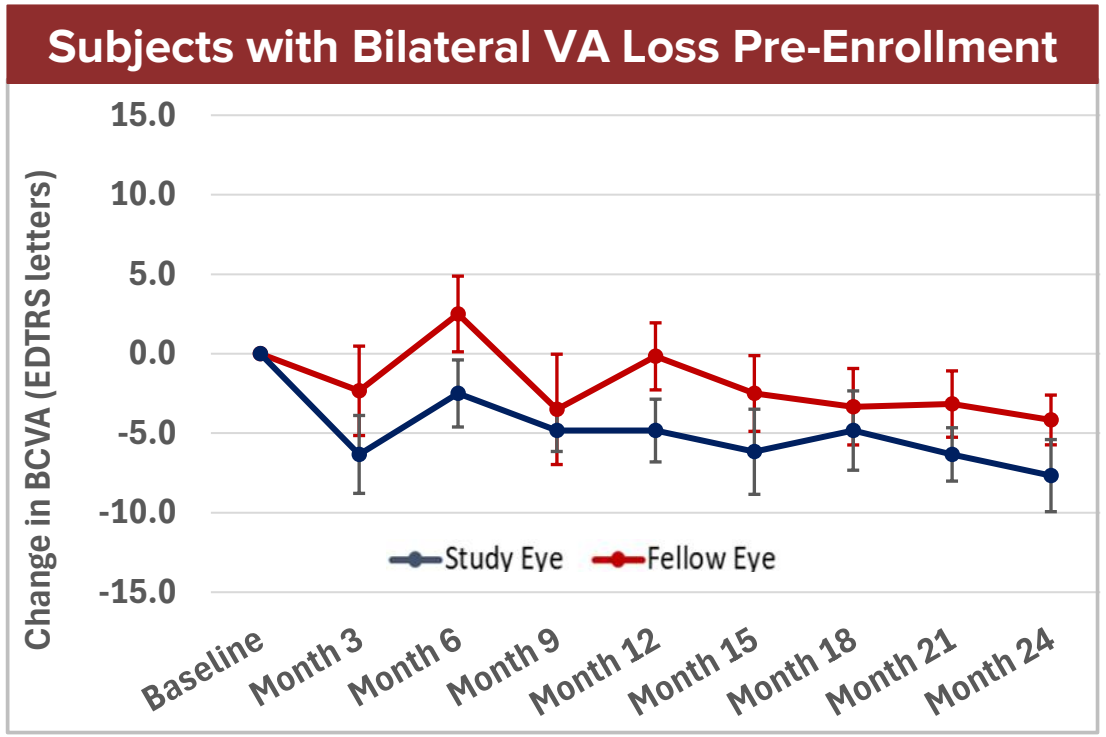
- ABC4 mutations in the study cohort
  - Severe biallelic ABCA4 mutations were found in 11 of 13 subjects
  - In vitro testing of the moderate alleles in subjects 3 and 5 showed pathogenicity
  - Variants with CADD score >20 predicted to be among the 1% most deleterious
  - Subjects 1, 3, 4, 12, and 13 did not develop atrophic lesions during the Phase 2 study despite harboring severe or likely severe alleles
  - Subjects 9 & 10 (brothers) and 12 & 13 (brother/sister) all of whom harbor identical mutations
- Visual acuity outcomes prior to study enrollment (subgroup of 6 subjects)
  - Mean bilateral BCVA loss of ~10 letters/year prior to enrollment
  - Natural history would predict clinically significant visual loss in subjects over two years of study
  - Loss of visual acuity in these subjects suggests that foveal-involved non-atrophic (QDAF) lesions can compromise visual acuity
  - Sibling subjects with identical mutations show very different BCVA loss

Tinlarebant (LBS-008)

Visual Acuity Outcomes During the Phase 2 Study

- Visual acuity was stabilized during the study with a mean loss of 5 letters in the cohort over 24 months of treatment (2.5 letters lost/year, left panel)[1]
- For the 6 subjects with prior BCVA loss:
  - **Pre-enrollment, mean BCVA** loss pre-enrollment was **~10 letters/year**
  - **Following 24 months of treatment**, mean BCVA loss of was 3.8 letters **(1.9 letters/year**, right panel)[1]



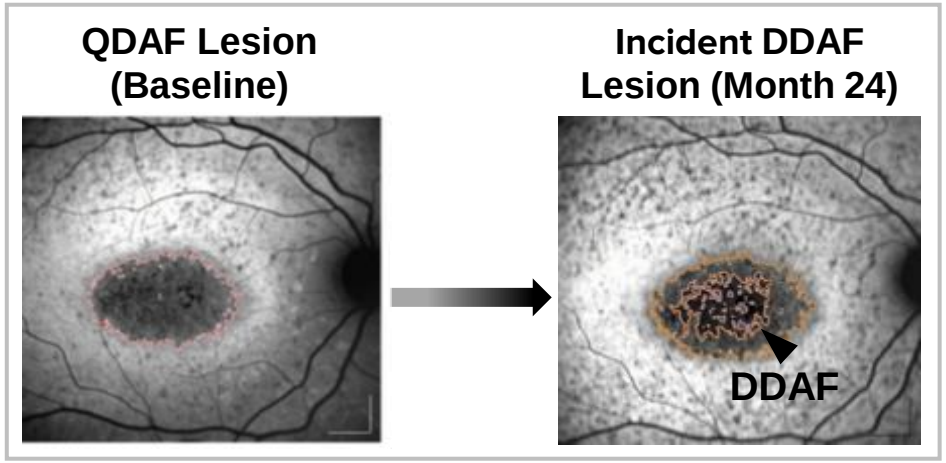


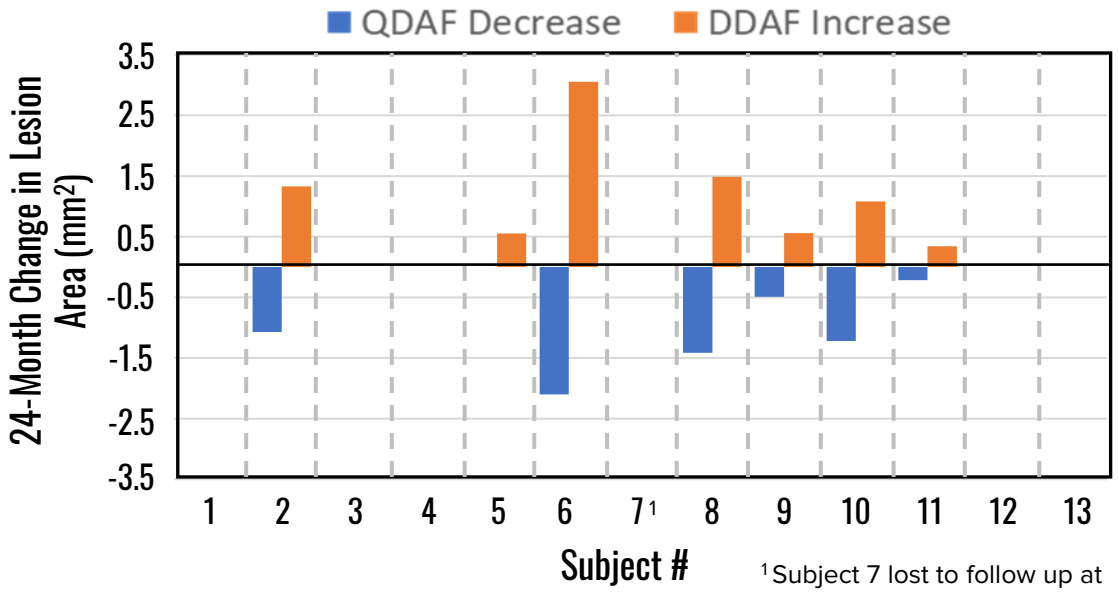
[1] Mean BCVA values at each timepoint were used to calculate the mean BCVA change over 24 months

Tinlarebant
(LBS-008)

24-Month Change in QDAF and DDAF Lesion Size

- Transition to, and growth of, incident DDAF was examined
- In 5 of 12 subjects (42%),
  - No change in QDAF lesion size
  - No incident DDAF lesion growth observed
- In 7 of 12 subjects (58%)
  - Incident DDAF lesions occurred within the QDAF lesion
  - No expansion of QDAF lesion size
  - Suggests DDAF expansion may stop at QDAF boundary
- Only one subject (#5) showed a new DDAF lesion (0.5 mm^2), which was outside of the QDAF lesion area that was identified at baseline
- Sibling subjects with identical *ABCA4* mutations and similar disease duration (9, 10, 13) showed different disease progression based on QDAF/DDAF lesion growth





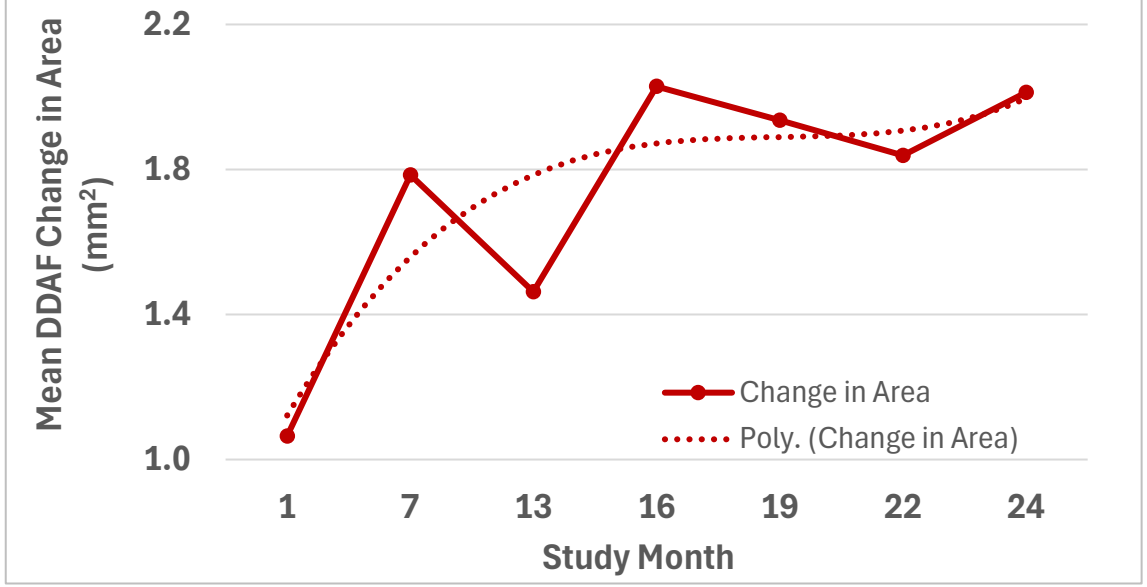
[1] Subject 7 lost to follow up at Month 12

Tinlarebant (LBS-008)

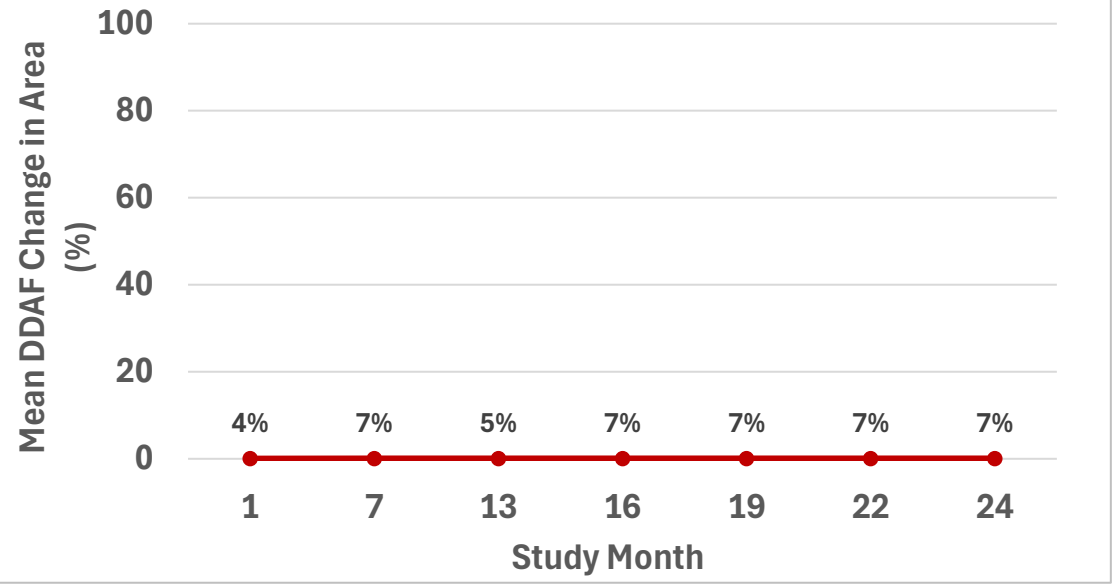
Growth of Atrophic Macular Lesions Stabilized at Months 16-24

- Analysis of retinal images using new grading methodology showed atrophic lesions within the macula (6 mm outer ring) in 12 eyes of 8 subjects at baseline
- In these subjects, growth of atrophic macular lesions was stabilized from Months 16 – 24
  - Left Panel: mean change of macular lesion growth and a 3rd order polynomial function of the lesion data
  - Right Panel: extent of macular lesion involvement shown as a percentage; growth is halted at 7% from Months 16 – 24

Mean Change in Area of Macular Involvement



Mean Change in Percent of Macular Involvement



Tinlarebant
(LBS-008)

Ph2 24-month: safe and well-tolerated in adolescent STGD1 patients

Adverse Events	Severity	Frequency (# and % of patients)
Xanthopsia/Chromatopsia	Mild	10/13 (76.9%)
Delayed Dark Adaptation	Mild	9/13 (69.2%)
Night Vision Impairment	Mild	1/13 (7.7%)
Increasing error score in FM100	Mild	1/13 (7.7%)
Intermittent headaches	Mild	2/13 (15.4%)

- Tinlarebant (5 mg p.o., daily) continues to be **safe** and **well-tolerated** in adolescent STGD1 patients
- Tinlarebant treatment produced a mean **80% reduction of RBP4** (from baseline) throughout the study
- Delayed Dark Adaptation and Xanthopsia/Chromatopsia are the most common drug related ophthalmic AEs
- All instances of Delayed Dark Adaptation, Night Vision Impairment, and Xanthopsia/Chromatopsia were **mild** and **transient**
- No severe and moderate drug related AEs reported and no AEs requiring discontinuation of treatment
- No clinically significant findings in relation to vital signs, physical exams, cardiac health, or organ functions

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DRAGON & DRAGON II
STGD1 Trials

Tinlarebant (LBS-008)

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[1]	STGD1 "DRAGON II" Phase 1b/2/3
Enrollment	104 subjects (have DDAF)	60 subjects (have DDAF)
Sites	Global	Japan, US, UK
Masking	Double Blind	
Placebo	2:1 ratio (Tinlarebant : Placebo)	1:1 ratio (Tinlarebant : Placebo)
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.

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Phase 3 PHOENIX Trial in Geographic Atrophy

Tinlarebant (LBS-008)

Clinical Trial Design Overview in GA

- **Established Efficacy Endpoint** – Reduction in lesion growth rate (DDAF) 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 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	Approximately 430 subjects targeted (Enrolling)
Sites	Global
Masking	Double Blind
Placebo	2:1 ratio (Tinlarebant : 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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Q2 2024 Financial Results

For more info please visit: www.belitebio.com

2024 Second-Quarter Financial Results

	For the Three Months ended June 30 (In thousand USD)	
	2024	**2023**
Total operating expenses	10,471	6,871
- R&D	9,078	5,516
- G&A	1,393	1,355
Net loss	(9,494)	(6,812)

- Cash, time deposits and U.S treasury bills: $112.3 million

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Q&A to begin shortly

For more info please visit: www.belitebio.com

Appendix

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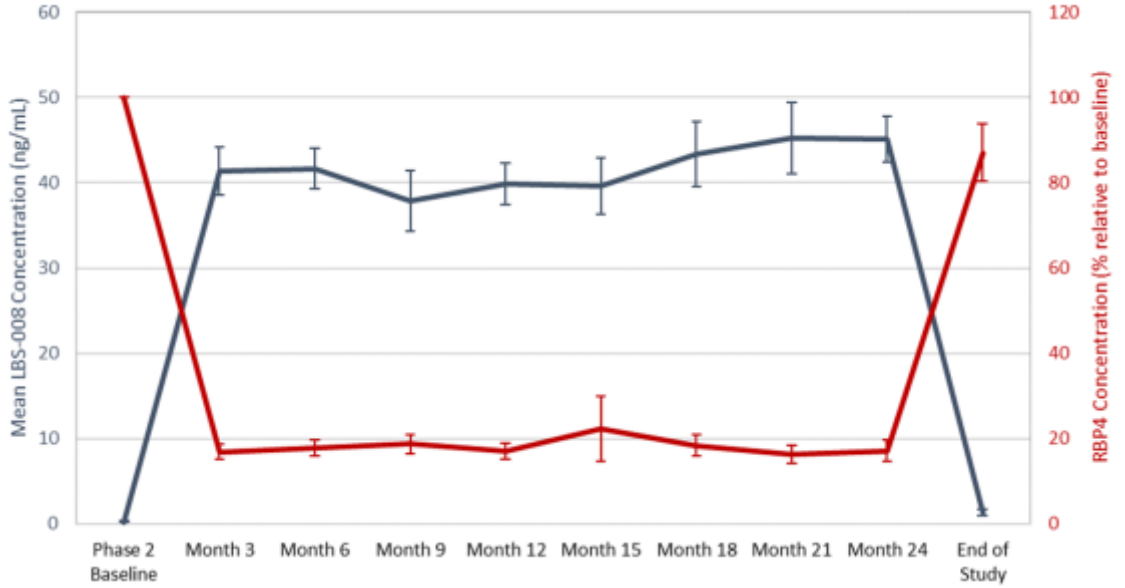
Tinlarebant (LBS-008)

Phase 2 Clinical Trial Design and PK/PD Profile

Clinical Trial Design Overview

	STGD1 Phase 2 "LBS-008-CT02"
Enrollment	13 subjects[1] (QDAF, no DDAF)[2]
Sites	Australia & Taiwan
Masking	Open Label
Dose	5 mg/day
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

Pharmacokinetic and Pharmacodynamic Profile



- The **5 mg** daily dose was effective to reduce RBP4 level by a mean of approximately **80%** relative to baseline
- RPB4 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

[1]The study enrolled a total of 13 subjects. One subject was lost to follow up at Month 12. Therefore, the efficacy analyses included just the 12 subjects who completed 24 months of treatment.
[2]All enrolled subjects showed only questionably decreased autofluorescence (QDAF) at Baseline and all lesions were foveal-involved; no definitely decreased autofluorescence (DDAF) lesions were identified.

Tinlarebant (LBS-008)

ABCA4 Mutations in the Phase 2 Study Cohort

- Severe biallelic ABCA4 mutations were found in 11 of 13 subjects (subjects 3 and 5 harbored one moderate allele each)
- *In vitro* testing of the moderate alleles in subjects 3 and 5 showed pathogenicity
- Variants with CADD score >20 predicted to be among the 1% most deleterious

(1) Combined Annotation–Dependent Depletion (CADD) https://cadd.gs.washington.edu/

N/A: Not available

Subject	cDNA change	Protein change	CADD v1.6[1]	Assessment	Function tested in vitro	ClinVar
1	c.1922G>C	p.Cys641Ser	25.7	likely severe		not provided
1	c.2905A>G	p.Lys969Glu	28.9	likely severe		Pathogenic
2	c.768G>T	p.(Leu257Valfs*17)	23.1	severe	yes	Pathogenic/Likely pathogenic
2	c.[1532G>A;6006-1G>C]	p.[Arg511His;?]	15.3; 34	severe		not provided
3	c.4539+2028C>T	p.[=,Arg1514Leufs*36]	1.79	moderate	yes	Pathogenic/Likely pathogenic
3	c.2894A>G	p.Asn965Ser	24.8	severe	yes	Pathogenic
4	c.2576del	p.Gln859Argfs*41	N/A	severe		Pathogenic
4	c.[2588G>C;5603A>T]	p.[Gly863Ala;Asn1868Ile]	31; 23.3	moderate/severe	yes; yes	Likely pathogenic
5	c.3260A>G	p.Glu1087Gly	29.5	likely severe		Pathogenic
5	c.5714+5G>A	p.[=,Glu1863Leufs*33]	19.1	moderate	yes	Pathogenic/Likely pathogenic
6	c.5461-10T>C	p.[Thr1821Aspfs*6,Thr1821Valfs*13]	16.8	severe	yes	Pathogenic
6	c.6079C>T	p.Leu2027Phe	27.6	severe	yes	Pathogenic/Likely pathogenic
7	c.634C>T	p.Arg212Cys	26.3	severe	yes	Pathogenic/Likely pathogenic
7	c.2894A>G	p.Asn965Ser	24.8	severe	yes	Pathogenic
8	c.161G>A	p.Cys54Tyr	33	severe	yes	Pathogenic/Likely pathogenic
8	c.4139C>T	p.Pro1380Leu	25.3	moderate/severe	yes	Pathogenic/Likely pathogenic
9	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
9	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance
10	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
10	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance
11	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
11	c.5761G>A	p.Val1921Met	25.5	likely severe		Pathogenic/Likely pathogenic
12	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
12	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance
13	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
13	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance

Tinlarebant (LBS-008)

ABCA4 Mutations in the Phase 2 Study Cohort (cont.)

- Subjects 1, 3, 4, 12, and 13
  - Did not develop atrophic lesions during the Phase 2 study
  - Despite harboring severe or likely severe alleles

Subject	cDNA change	Protein change	CADD v1.6[1]	Assessment	Function tested in vitro	ClinVar
1	c.1922G>C	p.Cys641Ser	25.7	likely severe		not provided
1	c.2905A>G	p.Lys969Glu	28.9	likely severe		Pathogenic
2	c.768G>T	p.(Leu257Valfs*17)	23.1	severe	yes	Pathogenic/Likely pathogenic
2	c.[1532G>A;6006-1G>C]	p.[Arg511His;?]	15.3; 34	severe		not provided
3	c.4539+2028C>T	p.[=,Arg1514Leufs*36]	1.79	moderate	yes	Pathogenic/Likely pathogenic
3	c.2894A>G	p.Asn965Ser	24.8	severe	yes	Pathogenic
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4	c.[2588G>C;5603A>T]	p.[Gly863Ala;Asn1868Ile]	31; 23.3	moderate/severe	yes; yes	Likely pathogenic
5	c.3260A>G	p.Glu1087Gly	29.5	likely severe		Pathogenic
5	c.5714+5G>A	p.[=,Glu1863Leufs*33]	19.1	moderate	yes	Pathogenic/Likely pathogenic
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(1) Combined Annotation–Dependent Depletion (CADD)
https://cadd.gs.washington.edu/

N/A: Not available

Tinlarebant (LBS-008)

ABCA4 Mutations in the Phase 2 Study Cohort (cont.)

- Subjects 9 & 10 (brothers)
- Subjects 12 & 13 (brother/sister)
- These 4 subjects harbor identical mutations
- BCVA and lesion data show a different course of disease

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11	c.5761G>A	p.Val1921Met	25.5	likely severe		Pathogenic/Likely pathogenic
12	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
12	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance
13	c.1804C>T	p.Arg602Trp	24.7	severe	yes	Pathogenic/Likely pathogenic
13	c.5645T>C	p.Met1882Thr	24.8	likely severe		Uncertain significance

(1) Combined Annotation–Dependent Depletion (CADD)
https://cadd.gs.washington.edu/

N/A: Not available

Tinlarebant (LBS-008)

Visual Acuity Outcomes Prior to Study Enrollment

- Subgroup of 6 subjects
  - Mean bilateral BCVA loss of ~10 letters/year prior to enrollment
- The natural history would predict clinically significant visual loss in these subjects over 2 years of study
- Loss of visual acuity in these subjects suggests that foveal-involved non-atrophic (QDAF) lesions can compromise visual acuity
- Sibling subjects with identical mutations show very different BCVA loss

Subject	Race	Gender	Age at First Exam	Age at Enrollment	Disease Duration (yrs)	BCVA at Diagnosis Exam (Right / Left)	BCVA at Enrollment (Right/Left)	Annual Letter Loss (Right/Left)
1	Caucasian	Female	13	15	2	36/37	34/36	-
2	Caucasian	Female	11	12	1	33/39	40/42	-
3	Asian/ Caucasian	Female	13	13	<1	49/44	49/48	-
4	Caucasian	Female	14	15	1	39/44	45/45	-
5*	**Caucasian**	**Female**	**12**	**13**	**1**	**70/61**	**45/55**	**25/6**
6*	**Caucasian**	**Female**	**13**	**18**	**5**	**70/65**	**35/36**	**7/6**
7	Caucasian	Male	15	15	<1	59/75	69/40	-
8*	**Caucasian**	**Male**	**8**	**13**	**5**	**57/57**	**31/31**	**5/5**
9	Asian	Male	12	13	1	50/35	45/35	-
10*	**Asian**	**Male**	**11**	**12**	**1**	**50/59**	**45/35**	**5/24**
11*	**Asian**	**Female**	**13**	**14**	**1**	**59/59**	**50/50**	**9/9**
12	Asian	Female	10	18	8	35/44	35/35	-
13*	**Asian**	**Male**	**10**	**12**	**2**	**59/59**	**35/35**	**12/12**

*Subjects showing annual bilateral BCVA loss prior to Phase 2 enrollment; BCVA loss for subjects with <1 year disease not considered

Tinlarebant
(LBS-008)

Novel Lesion Size Grading Method Improves Measurement of Atrophic Macular Lesions

Region Finder

- Relies heavily on the grader's ability to visually determine the types and boundaries of areas with different levels of disc-derived autofluorescence (DDAF, DAF, QDAF)
- There is intra- and inter-grader variability
  - Due to subjective assessment of lesion grayscale compared to the optic disc
- General assessment without specific focus on key regions
  - Potentially leading to less precise measurements

Novel Lesion Size Grading Method

- Utilizes mathematical classification of lesions based on the proximity of their gray values to that of the optic disc, reducing subjective bias
- Focuses on specific regions (macular area within central 6mm diameter and smaller zones of 1mm and 3mm ETDRS), enhancing the accuracy of localization and quantification
- Independent of initial lesion size, employing a pixel-based method that consistently evaluates the percentage of a region affected at specified DAF thresholds

DAF @ >90 %