

GENE COMPANY - EXHIBIT 1087

Gene Company PTY LTD, v. The Trustees of Columbia University in the City of New York IPR2024-00313

AAO 2024

AAO 2024 Virtual PLAYING

https://aao2024virtual.org/session/245925

Home

Sessions

Happening now

Speakers

Social Wall

FAQs

Tech Support

Sessions > SYM51-New Re...

LIVE

Download this video

TEASE comprises four studies of giledeuritol^a in Stargardt disease

Phase 2 Tolerability and Effects of ALK-001^a on Stargardt Disease

	TEASE-1 NCT02402660 ¹	TEASE-2 NCT02402660 ¹	TEASE-3 NCT02402660 ¹	TEASE-4 NCT04239625 ²
Design	Placebo-controlled, double-masked, randomized, 24-month	Placebo-controlled, double-masked, randomized, 24-month	Open-label, 24-month	Open-label, 24-month extension study
Retinal phenotype*	Well-delineated RPE atrophy	Decreased retinal sensitivity without well-delineated RPE atrophy	Early signs of maculopathy	Retinal phenotype varies

Upon completion, eligible to enroll in TEASE-4

^a Giledeuritol = ALK-001 = C20-03-Vitamin A;
¹ Retinal phenotype as determined at screening;
RPE, retinal pigment epithelium.
More information available about TEASE-1, -2, and -3 at: <https://clinicaltrials.gov/study/NCT02402660>. Accessed March 20, 2024.
More information available about TEASE-4 at: <https://clinicaltrials.gov/study/NCT04239625>. Accessed March 20, 2024.

11

36:52 AAO 2024

Enter Theatre Mode

This Session

Event

Live Chat

like to welcome you to this session of the virtual meeting!

Luis Cordoves Dorta 01:57 PM

The results fo the Mactel treatment trial are somehow disappointing, particularly the reading speed and atrophic area, where confidence intervals intersect substantially. Any comment about that?

Luis Cordoves Dorta 01:57 PM

But new treatments approaches are the basis for future therapies.

Meredith Jones 02:02 PM

@Luis Cordoves Dorta
you can use the Mobile Meeting Guide to interact with this session (<https://aao.apprisor.org/apsSession.cfm?id=SYM51>). This chat is not being monitored by the panel.

Luis Cordoves Dorta 02:05 PM

Thanks.

02

Enter message 250

Type @ to mention users, sessions

SYM51-New Retinal Therapies: Recent Outcomes From Clinical Trials

Today 20 Oct. 2024 - 01:45 PM-03:00 PM

TEASE-1

Study is a randomized, double-masked, placebo-controlled (partial placebo crossover to gildeuretinol) clinical trial

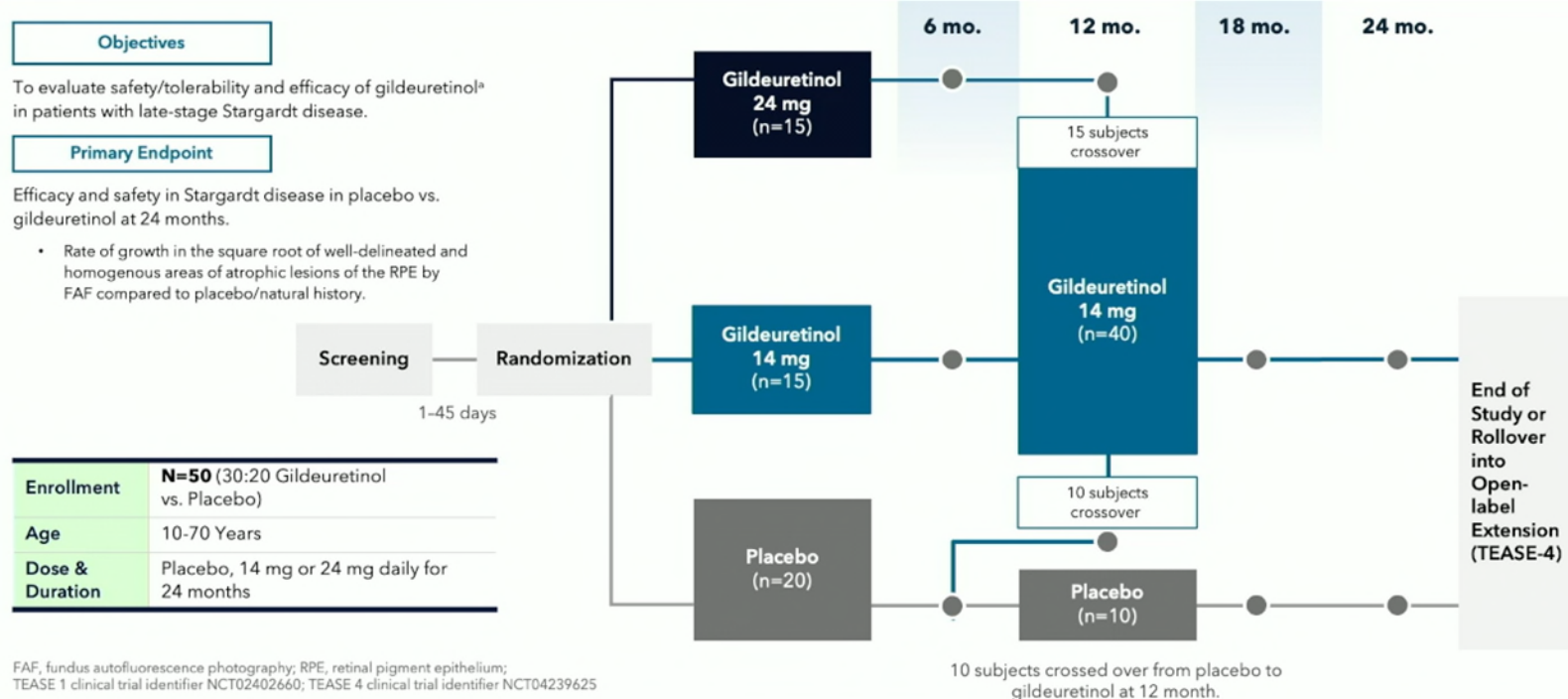
Objectives

To evaluate safety/tolerability and efficacy of gildeuretinol^a in patients with late-stage Stargardt disease.

Primary Endpoint

Efficacy and safety in Stargardt disease in placebo vs. gildeuretinol at 24 months.

- Rate of growth in the square root of well-delineated and homogenous areas of atrophic lesions of the RPE by FAF compared to placebo/natural history.

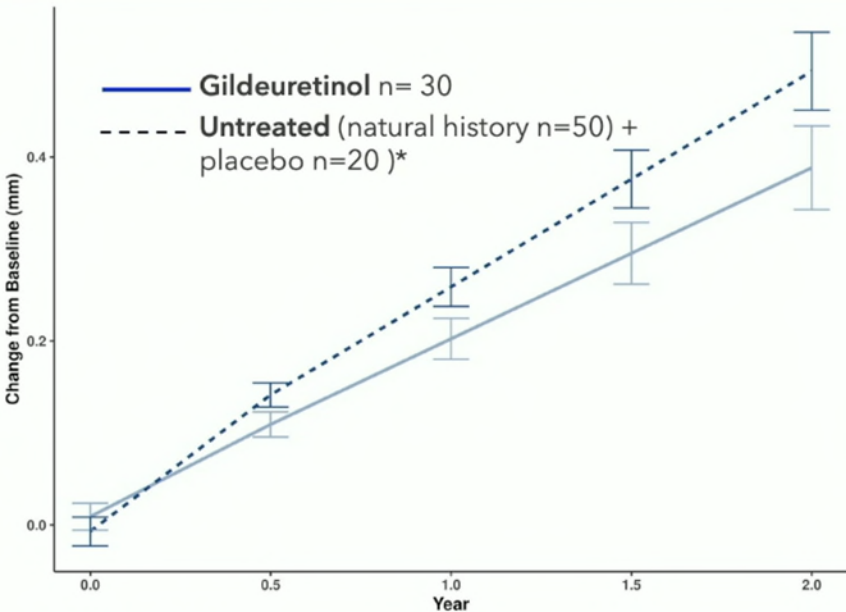


Enrollment	N=50 (30:20 Gildeuretinol vs. Placebo)
Age	10-70 Years
Dose & Duration	Placebo, 14 mg or 24 mg daily for 24 months

FAF, fundus autofluorescence photography; RPE, retinal pigment epithelium;
TEASE 1 clinical trial identifier NCT02402660; TEASE 4 clinical trial identifier NCT04239625



Primary endpoint: gildeuretinol slows the growth rate of atrophic lesions by 21% Statistically-significant with or without natural history cases (TEASE-1 data only)



21% slower growth rate ($p<0.001$)
28% on untransformed areas ($p<0.001$)

Growth Rate	Gildeuretinol	Untreated	Difference (95% CI)
Linear Estimate (mm/year)	0.19	0.24	0.05 (0.03 to 0.07)
Area Estimate (mm ² /year)	0.90	1.25	0.35 (0.26 to 0.44)

Results from a linear mixed effect model includes all eyes, all timepoints, and pools randomized patients with 59 natural history cases. Comparison is made between 6-24 months

Change from Baseline (mm)

Results from a linear mixed effect model accounting for baseline imbalances, and incorporating all measurements, at all time points, from all graders.

*10 patients in placebo crossed over to treatment at month 12



TEASE-2

Study is a randomized, double-masked, placebo-controlled clinical trial

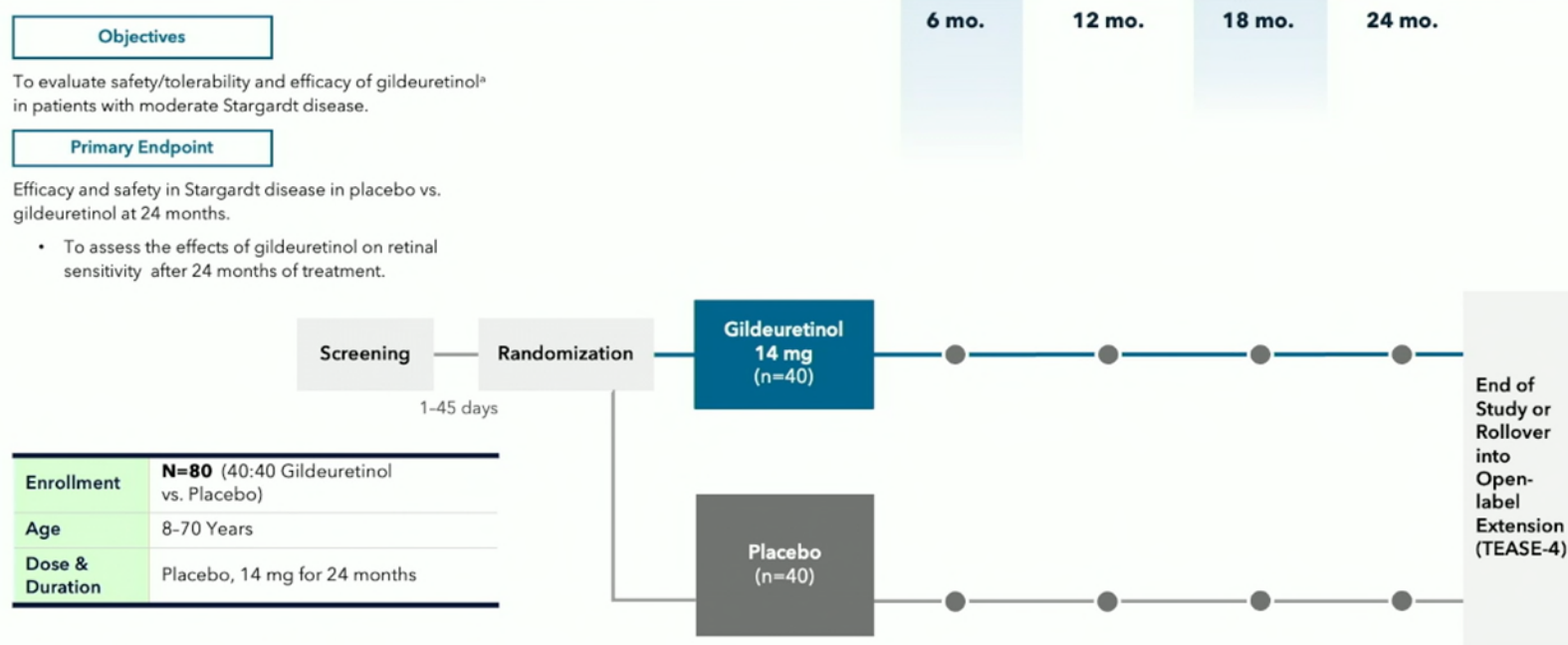
Objectives

To evaluate safety/tolerability and efficacy of gildeuretinol^a in patients with moderate Stargardt disease.

Primary Endpoint

Efficacy and safety in Stargardt disease in placebo vs. gildeuretinol at 24 months.

- To assess the effects of gildeuretinol on retinal sensitivity after 24 months of treatment.



a. Gildeuretinol = ALK-001 = C20-D3-Vitamin A;
TEASE 2 clinical trial identifier NCT02402660; TEASE 4 clinical trial identifier NCT04239625



TEASE-3

Study is an open-label clinical trial

Objectives

To evaluate safety/tolerability and efficacy of gildeuretinol^a in patients with early Stargardt disease.

Primary Endpoint

Progression of Stargardt disease from baseline to 24 months, as well as in comparison to age matched historical imaging of siblings.

- Evaluate the effects of gildeuretinol in the progression of the signs and symptoms.

Screening

1 - 45 days

Randomization

Open Label
14 mg
(n= up to 10,
6 enrolled)

6 mo.

12 mo.

18 mo.

24 mo.

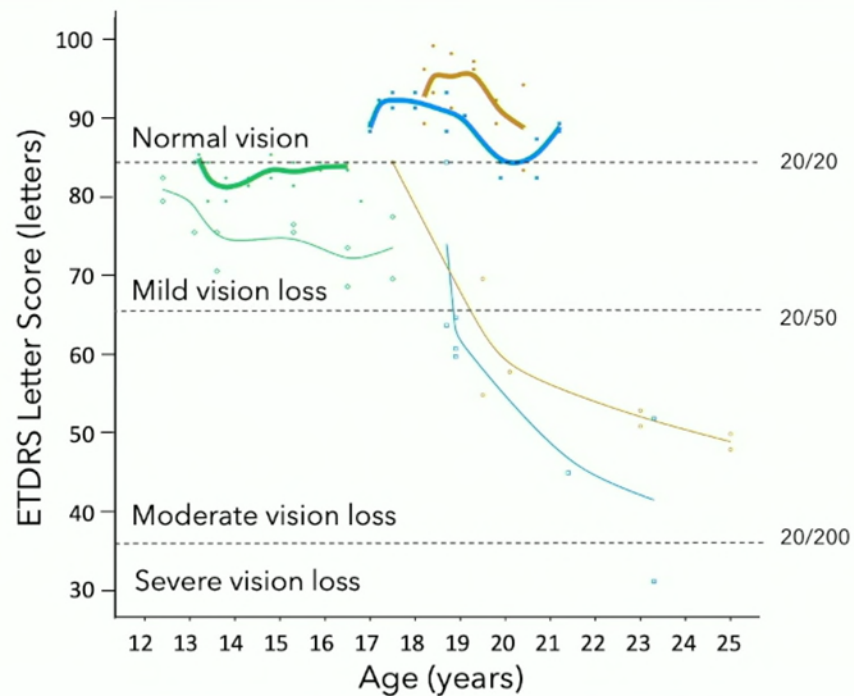
End of
Study or
Rollover
into
Open-
label
Extension
(TEASE-4)

Sample Size	N up to 10 (6 enrolled)
Age	8 - 70 years, subject with early signs of maculopathy as identified by abnormal central FAF or OCT, expected to progress
Dose & Duration	14 mg gildeuretinol daily for 24 months

a. Gildeuretinol = ALK-001 = C20-D3-Vitamin A;
FAF, fundus autofluorescence photography; OCT, optical coherence tomography;
TEASE 3 clinical trial identifier NCT02402660; TEASE 4 clinical trial identifier NCT04239625



Gildeuretinol treatment was associated with relatively stable visual acuity



- Standardized ETDRS BCVA for treated patients
- Snellen acuity for age-matched untreated siblings

— P1, Patient 1
— Sibling to P1
— P2, Patient 2
— Sibling to P2
— P3, Patient 3
— Sibling to P3



TEASE-4

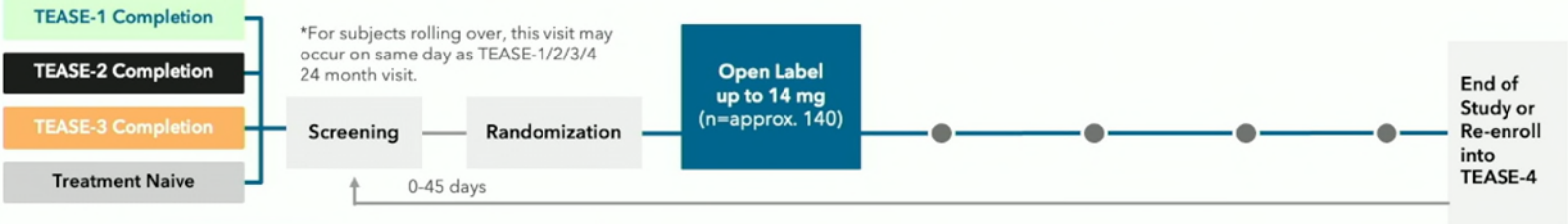
Study is an open-label clinical trial

Objectives

To evaluate long-term safety, tolerability, and pharmacokinetics of gildeuretinol^a.

Primary Endpoint

Evaluate the long-term safety effects of gildeuretinol, the lowest dose capable of maintaining 80% deuterated vitamin A in plasma (a percentage expected to show efficacy).



Sample Size	N=approx. 140
Age	8-70 years, patients who completed TEASE I, II, and III, or new patients with at least one eye with BCVA > 40 letters (VA 20/160)
Dose & Duration	14 mg for 3 months followed by doses up to 14 mg for a total of 24 months

a. Gildeuretinol = ALK-001 = C20-D3-Vitamin A;
BCVA, best corrected visual acuity;
TEASE 4 clinical trial identifier NCT04239625



Conclusions

- In TEASE-1, giledeuretinol safely and significantly slows the growth rate of atrophic lesions in Stargardt disease
- First treatment to show efficacy in Stargardt in a randomized clinical trial
- In TEASE 3, giledeuretinol was well tolerated and associated with relatively stable visual acuity compared to age matched sibling untreated control group whose vision declined
- Topline results for TEASE-2 are expected in 2025
- Giledeuretinol for Stargardt disease has been granted orphan drug status and breakthrough therapy designation

25



10