SCENIC

X-LINKED RETINITIS PIGMENTOSA (XLRP) SCENIC CLINICAL DATA SUMMARY

agtc

Q4 2021

OVERVIEW

XLRP, an inherited retinal disease, affects approximately 20,000 people, mostly boys and men, in the United States and Europe. Caused by *RPGR* gene mutations and loss of the *RPGR* protein, the disease progresses to degeneration of rod and cone photoreceptor cells. Most patients with XLRP are legally blind by age 45, and there are no therapies available.

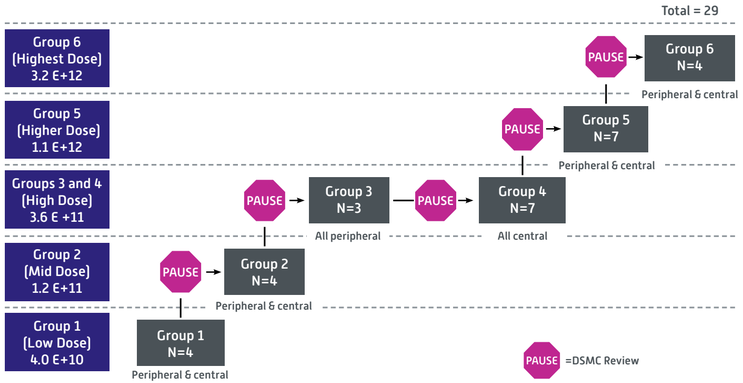
The AGTC mission is to restore visual function in patients with XLRP and other rare inherited conditions. Our candidate therapy for XLRP uses a proprietary viral vector technology to deliver a functional copy of the *RPGR* gene to rod and cone photoreceptor cells. Here are Q4 interim results from our evolving clinical development program.

INTERIM SUMMARY: 12-MONTH DATA

XLRP Phase 1/2 Trial Design and Dosing Schedule

HORIZON

Dose Level



DSMC=Data Safety Monitoring Committee.

Phase 1/2 clinical trial: 12-month summary (N=29)

In Groups 2 & 4, measurable improvements were observed in visual sensitivity for 2 of the evaluable 9 centrally dosed patients, while a third patient identified as a responder at Month 6 fell just below the cutoff (patients are defined as responders when at least 5 loci within the central 36 loci of the perimetry grid increase by at least 7 decibels [dBs]). **8 of 9 patients treated centrally also had stable or improving visual acuity, a result not reported in competitors' XLRP trials.**

In Groups 5 & 6, data were reported from 11 patients; 7 patients in Group 5 and 4 patients in Group 6. One patient in Group 5 and 2 patients in Group 6 would not meet the inclusion criteria for the Skyline and Vista trials, resulting in a total of 8 patients who were included in the responder analysis. Four of these 8 patients **(50%)** were considered responders, and these 4 met the criteria of at least a 7-dB improvement in at least 5 loci. One additional patient did not meet these criteria, but at 12 months had a statistically significant improvement in retinal sensitivity in the treated eye compared with the untreated eye.

Phase 1/2 safety data

Data from all 29 patients across 6 dose groups continue to demonstrate a favorable safety profile with no dose-limiting inflammatory responses observed. This safety profile, which has shown no clinically significant inflammation not manageable with steroids, continues to be observed out to 24 months.

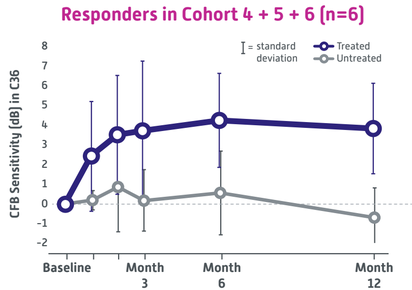
A SIGNIFICANT INCREASE IN VISUAL SENSITIVITY AT MONTH 12, WITH SUPPORTIVE EVIDENCE OF BIOLOGICAL RESPONSE

Microperimetry results for the 6 responders across Groups 4-6 show a significant increase in mean visual sensitivity across the central 36 loci for treated compared with untreated eyes. Best Corrected Visual Acuity (BCVA) data from all 20 centrally dosed patients continue to provide supportive evidence of biological response to AGTC's XLRP candidate at 12 months.

Microperimetry – 6 Responders at Month 12

Groups 4-6

Increased mean sensitivity relative to baseline across the central 36 loci

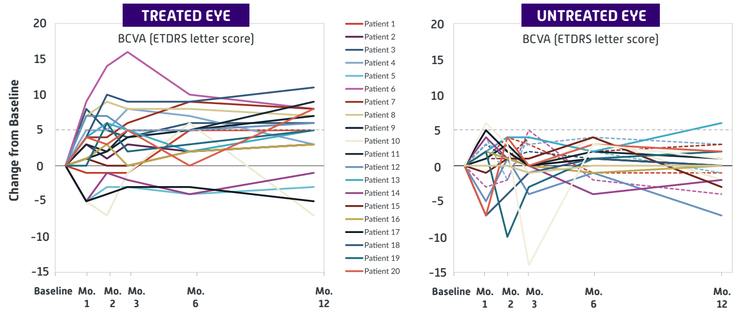


Responder identified as patient with ≥7-dB improvement in sensitivity at ≥ 5 loci in central 36 loci of perimetry grid at Month 12

BCVA – Individual Patient Data at Month 12

All Groups, N=20 Centrally Dosed

Supportive evidence of statistically significant improved visual acuity across all centrally dosed patients



ETDRS=Early Treatment Diabetic Retinopathy Study.

SCENIC

Encouraging Clinical Results From an Interim Data Analysis in XLRP

- 50% response rate in patients who met Phase 2/3 inclusion criteria
- BCVA shows statistically significant supportive evidence of biological response at Month 12
- 69% of patients had either complete recovery or improvement in EZ line at 6 months.

Clinical Milestones

- Presented 12-month trial results from the ongoing Phase 1/2 clinical trial at the American Academy of Ophthalmology Annual Meeting in November 2021
- Provide Skyline trial results from the 3-month masked interim analysis in 2022
- Provide 12-month results from Skyline Trial in Q1 of 2023
- Provide interim results from the VISTA trial in the first half of 2023

To stay up to date AND learn more about the upcoming Vista trial, visit Vistatrialhcp.com

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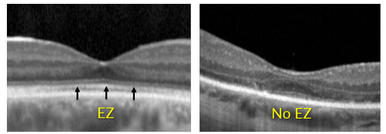
STRUCTURE/FUNCTION CORRELATION SUPPORTS TREATMENT EFFECT

The ellipsoid zone (EZ), a defined region within the photoreceptor layer of the retina (shown below), degenerates over time in patients with XLRP and is eventually lost. **Intact retinal anatomy at baseline predicts potential for improvement. Of the 20 patients evaluated, 9 of the 13 (69%) patients with intact baseline retinal anatomy, had either complete recovery or improvement in EZ line at 6 months.** The additional 7 patients had end-stage retinal anatomy (no EZ) and showed no structural or functional improvements after treatment.

A significant association between improvements in visual sensitivity measured by Macular Integrity Assessment (MAIA) and improvements in retina health was found. Eight of the 18 patients in groups 4-6 had improvements in either MAIA or EZ. Four of the six patients with MAIA improvement also had EZ improvement. There was a statistically significant association between MAIA improvement and EZ improvement (P = .0212)

Optical coherence tomography (OCT) scan of the central retina



What We Offer to People With Inherited Retinal Disorders (IRD)

Clinical research isn't possible without patient volunteers. AGTC is committed to easing the burden of enrollment through several patient support initiatives. In 2020, AGTC partnered with Serva Health, a nurse-staffed patient engagement center, to offer white-glove assistance navigating the prescreening and screening process. We also partnered with 2020 Onsite to bring assessments directly to patients' homes through a mobile vision clinic. Lastly, our advocacy team created the AGTC Patient Advisory Council (PAC), made up of a group of advocates either living with or caring for someone with an IRD. In partnership we have created patient-centric protocols and study materials for all age groups.

High Quality Manufacturing Platform

AGTC has developed a proprietary manufacturing platform that produces a high percentage of full capsids as well as residuals that are below level of detection. The result is a high-quality product that supports late-stage developments of our XLRP and achromatopsia programs, as well as supporting the continued development of our preclinical programs. We are constructing a Good Manufacturing Practices (GMP) manufacturing facility and Quality Control (QC) lab to support our programs including commercial launch of late-stage products.

Advanced Platform Generating Potential Best-In-Class Pipeline

XLRP: Lead Phase 2/3 ophthalmology candidate	Manufacturing	Integrated gene therapy platform
• Potential best-in-class late-stage asset • Improvements in visual sensitivity and acuity sustained for at least 12 months • No product-related serious adverse events across 100-fold dose range • Key clinical readouts expected in 2022	• Unmatched productivity, scalability, and quality • 10-fold reduction in process residuals and 10-fold increase in productivity • At commercial scale: 40 L ≥2000 ophthalmic doses	• Differentiated with demonstrated capabilities • Broad preclinical data • Robust use of disease-specific animal modeling • Novel, patent-protected vector components

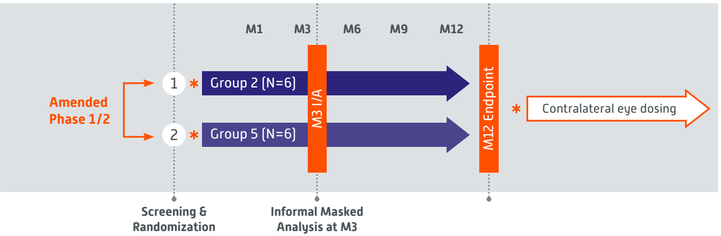
CLINICAL DEVELOPMENT PROGRAM: NEXT STAGE

AGTC has expanded the clinical development program for its XLRP candidate with the SCENIC trials, Skyline and Vista. To refer a patient, go to **[www.vistatrialhcp.com]** and click on the "refer a patient" button, or **call 1-855-VIEWVISTA (855-843-9847)** to speak directly with a nurse.

Skyline Clinical Trial

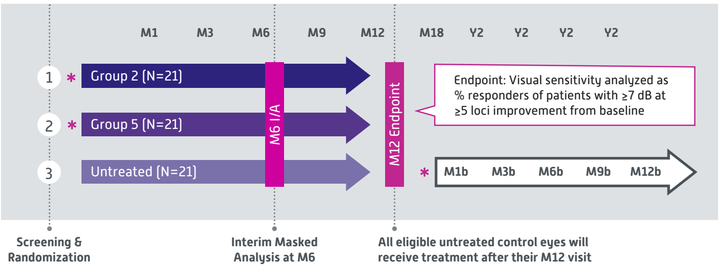
Phase 1/2 expansion. First path to verify correlation of visual sensitivity changes to mobility maze outcomes and to maintain patient and site engagement

SKYLINE



Vista Phase 2/3 Trial Design

VISTA



At the time of M6 analysis we expect to have 3 groups of data (24-month data from the Phase 1/2 trial, 12-month data from the Skyline trial, and 6-month data from the Vista trial) to review with the FDA to seek potential trial acceleration, including early dosing of second eye.

*Subretinal treatment.