VISTA | agtc

X-linked retinitis pigmentosa

A vision of the future

Talk to your doctor and see if participating in the VISTA clinical trial is right for you or your child.

About the VISTA clinical trial

The VISTA clinical trial is for males between the ages of 13 and 50 who have X-linked retinitis pigmentosa (XLRP).

XLRP is an inherited rare genetic eye disease in which light-sensitive cells in the retina (the thin layer of tissue that lines the inner surface of the back of the eye) are damaged or do not function correctly. XLRP is caused by mutation or damage to one of the genes responsible for the function of retina cells, leading to a loss of vision over time.

All study-related travel expenses will be covered for both participants and a study partner/parent or caregiver as applicable. Please work with your study site to answer any travel-related concerns or questions.

The VISTA clinical trial is studying an investigational novel gene therapy

- The VISTA clinical trial is studying an investigational (not yet approved for sale by governmental authorities like the FDA in the US or EMA in Europe) gene therapy called AGTC-501, designed to replace the mutated RPGR gene that causes XLRP and correct the underlying deficiency
- In this process, a person's eye is injected with a healthy version of the gene, which is intended to replace the damaged or mutated gene
- The purpose of the VISTA clinical trial is to assess how well 2 different doses of AGTC-501 might work to improve vision and other symptoms of XLRP when compared to study participants who have not received AGTC-501
- Participants in the treatment group will undergo a procedure to receive AGTC-501 into one eye. Participants in the control group will not receive AGTC-501 initially, but will have the option to receive AGTC-501 after 1 year, if they meet eligibility requirements
- The safety and tolerability of these 2 doses will also be assessed in this study
- If you or your child are eligible and choose to participate in the VISTA clinical trial, and if you are assigned to receive AGTC-501, you and your study doctor will not know which dose you or your child will receive

Control groups are very important in clinical research. To learn more about why they are included in this study, see page 10.

VISTA clinical trial eligibility

To be eligible for the phase 2/3 VISTA clinical trial, you or your child must:

	Be male with a diagnosis of X-linked retinitis pigmentosa confirmed by a qualified healthcare professional
	Have a mutation in the RPGR gene confirmed by genetic testing
	Be between the ages of 13 and 50 years
	In at least 1 eye, have a best visual acuity score of the following:
	• No better than 75 letters and no worse than 35 letters on an eye chart for adults, OR
	• No better than 20/32 and no worse than 20/200 on an eye chart for participants 17 years and under

Genetic testing is available

If you or your child have been diagnosed with XLRP and have had the RPGR gene mutation confirmed through genetic testing, you may be eligible for the VISTA clinical trial.

If you are unsure whether your or your child's vision loss is due to XLRP and genetic testing has not been conducted, you may call (855) 843-9847 or visit VISTAtrial.com to learn more about free genetic testing options.

How the VISTA clinical trial works

Qualification for the VISTA clinical trial involves confirmation of XLRP with an RPGR mutation by genetic testing, as well as medical history review. If you or your child decide to participate in the VISTA clinical trial, you may be asked to participate in a pre-screening visit prior to screening for the clinical trial. You will then attend 3 screening visits at a study site. If confirmed eligible, you will be randomly assigned to 1 of 2 treatment groups or the untreated control group.

If you are assigned to 1 of the 2 treatment groups:

1. You will have eye surgery to receive 1 of 2 possible doses of AGTC-501. Neither you nor the study doctor will know which dose you receive
2. After the surgery, you will have at least 9 follow-up visits during the first year to do additional testing
3. After the first year, there will be additional visits at months 18 and 24 following the surgery
4. There will be yearly visits for the next 3 years (for a total of 5 years following the surgery for long-term follow-up after dosing)

If you are assigned to the control group:

1. You will have up to 10 visits at the study site and 4 virtual visits within the first year
2. After the first year, you may choose to end participation in the clinical trial or have eye surgery and receive AGTC-501, if eligible
3. If you are eligible and choose to have eye surgery to receive AGTC-501 after the first year, you will have at least 9 follow-up visits during your second year and additional visits at months 18 and 24 after the surgery
4. There will be yearly visits for the next 3 years (for a total of 6 years following the surgery for long-term follow-up after dosing)

Considerations

Why participate?

While there is no guarantee that this clinical trial will help improve your XLRP symptoms, what researchers learn may lead to better medications and treatments for patients with XLRP in the future.

Why is a control group needed?

Control groups are very important in clinical research. For this study it will allow researchers to compare results from a group of participants who received AGTC-501 to results from those who did not. This comparison will help researchers show whether AGTC-501 had any effect.

After the first year, participants in the control group may choose to end participation or receive AGTC-501, if eligible.

If you qualify and enroll, you will receive:

- Study-related medical care, including the procedure, medicines, and the investigational medication, at no cost
- Close monitoring by doctors who specialize in inherited retinal disease
- All study-related travel covered by the sponsor at no cost to you or your caregiver

What to expect

	Trial participation lasts from 1 to 6 years ● Participation for the treatment groups lasts for approximately 5 years ● Participants in the control group will participate for approximately 1 year before being eligible for treatment. If you are eligible and choose to receive the study drug after 1 year, you may participate in the study for up to an additional 5 years
	You may have to travel for the study visits depending on your study site (all travel for study-related assessments will be covered by the sponsor at no cost to you)
	It is important to consider the time associated with study participation

To learn more about the VISTA clinical trial, visit VISTAtrial.com or call 1-855-VIEWVISTA (855-843-9847).

About the study sponsor

AGTC, the study sponsor, is a biotechnology company that uses a gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases.

AGTC's initial focus is in the field of ophthalmology, where it has active clinical trials in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM CNGB3 & ACHM CNGA3).

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