VISTA | beacon therapeutics

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 12 and 50 who have X-linked retinitis pigmentosa (XLRP) caused by a mutation (or defect) in the *RPGR* gene. 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) do not function correctly.

XLRP can be caused by a mutation in a gene called Retinitis Pigmentosa GTPase Regulator *(RPGR)*. A change in *RPGR* can result in loss of vision due to degeneration (breakdown) of special cells in the retina called photoreceptors, which are needed for vision.

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) new gene therapy called AGTC-501. The study drug (AGTC-501) is designed to provide a healthy version of the damaged or mutated *RPGR* gene to allow the cells in the retina to make a healthy RPGR protein.

- Throughout this booklet, "you" refers to study participants, whether the participant is you or your child.
- The purpose of Vista is to assess how well 2 different doses of AGTC-501 might work to preserve and/or improve vision and other symptoms of XLRP when compared to study participants who have not received AGTC-501.
- The safety and tolerability of these 2 doses will also be assessed in this study.

How the Vista clinical trial is designed

- Participants will be assigned to one of three groups. Group 1 and Group 2 will receive one of 2 doses of the study drug. Group 3 will be the untreated control group.
- Eligible participants in Groups 1 and 2 will undergo a procedure to receive an injection of AGTC-501 in one eye. The study doctor and study participants will not know which dose is given.
- Participants in the control group will not receive AGTC-501 initially, but they will have the option to receive AGTC-501 after at least 1 year if they meet eligibility requirements.

Control groups are very important in clinical research. To learn more about why they are included in this study, see page 7.

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

To be considered for the Vista clinical trial, you must:

	Have 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 male between 12 and 50 years of age.
	In at least 1 eye, have a best corrected visual acuity score (BCVA) of no better than 78 letters and no worse than 34 letters on an eye chart.

In order for you to qualify and be considered for the Vista clinical trial, the above criteria needs to be confirmed along with a medical history review. You may be asked to provide documentation or permission to review your medical records.

Genetic testing is available

If you are unsure whether your 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

If you meet eligibility criteria and decide to participate in the Vista clinical trial, you will then attend 3 screening visits at a study site. If you qualify and wish to participate, you will be randomly assigned to 1 of 2 treatment groups or the control group.

If you are assigned to treatment groups 1 or 2:

- 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.
- After the surgery, you will have at least 15 study visits over the first two years of the study for your doctor to do additional testing.
- After your Month 24 visit, follow-up visits will occur at Years 3, 4, and 5 (for a total of 5 years following the surgery) for long term follow up after dosing.

If you are assigned to the control group:

- You will not receive the study drug in at least the first year of participation.
- You will have up to 10 visits at the study site within the first 2 years.
- After all participants in all 3 study groups complete their Month 12 visit and all data has been captured, you will have the option to receive AGTC-501 if you meet eligibility requirements.
- If at that time, you are eligible and choose to receive the study drug, you will continue participation with the follow-up visit schedule described for treatment groups 1 and 2.

Considerations

What to expect

	Trial participation lasts from 2 to 6 years depending on which group you are assigned to.
	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.

Why am I being asked to pre-screen?

You may be asked to complete a pre-screening visit prior to screening for the clinical trial. This is an abbreviated visit that allows the clinical trial site to better understand your eligibility before completing the full screening requirements.

Why participate?

While there is no guarantee that this clinical trial will improve your XLRP symptoms, what researchers learn may lead to better 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 the study drug to results from those who did not. This comparison will help researchers show whether the study drug had any effect.

As a reminder, participants in the control group will not receive AGTC-501 initially, but they will have the option to receive AGTC-501 after at least 1 year if they meet eligibility requirements.

If you qualify and enroll, you will receive:

- Study-related medical care, including the procedure, medicines, and the study drug, 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.

To learn more about the Vista clinical trial, visit VistaTrial.com or call (855) 843-9847

beacon therapeutics

About the study sponsor

Beacon Therapeutics, the clinical trial sponsor, is an ophthalmic gene therapy company founded to restore and improve the vision of patients with both prevalent and rare retinal diseases that result in blindness.

Beacon Therapeutics' lead development candidate is AGTC-501, a late-stage gene therapy program for the treatment of XLRP.

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