Talk to your doctor and see if participating in the SKYLINE clinical trial is right for you or your child.
ABOUT THE SKYLINE CLINICAL TRIAL

The SKYLINE clinical trial is enrolling males between the ages of 8 and 50 who have X-linked retinitis pigmentosa (XLRP). XLRP is a rare genetic eye disease in which light-sensitive cells in the retina are damaged or do not function correctly. Retinitis pigmentosa is caused by mutation or damage to one of the genes responsible for forming the retina cells, leading to loss of vision over time.

THE SKYLINE CLINICAL TRIAL IS INVESTIGATING A NOVEL GENE THERAPY

- The SKYLINE clinical trial is studying an investigational (not yet FDA-approved) gene therapy called AGTC-501 designed to replace the mutated RPGR gene that causes XLRP.
- In this process, a person is injected with a healthy gene, which is intended to replace the damaged or mutated gene.
- Patients who enroll in the SKYLINE clinical trial will undergo a procedure to receive the investigational study drug into one eye.

WHAT THE SKYLINE CLINICAL TRIAL IS STUDYING

- The purpose of the SKYLINE clinical trial is to assess how well 2 different doses of AGTC-501 might work to improve vision and other symptoms of XLRP.
- The safety and tolerability of these 2 doses will also be assessed in this study.
- If you are eligible and you choose to participate in the SKYLINE clinical trial, you and your study doctor will not know which dose you or your child will receive.

All study-related travel expenses will be covered for both participants and a study partner/parent or caregiver as applicable. You may be eligible for extended stay or alternative travel accommodation as needed. Please work with your study site to answer any travel-related concerns or questions.
HOW THE SKYLINE CLINICAL TRIAL WORKS

Qualification for the SKYLINE clinical trial involves confirmation of XLRP with an *RPGR* mutation by genetic testing, as well as medical history review. If you decide to participate in the SKYLINE clinical trial, you will attend 3 screening visits at a study site, and if confirmed eligible, you will undergo a procedure to receive AGTC-501 in one eye.

Neither you nor the study doctor will know which dose of AGTC-501 you are assigned to receive.

After the surgery, you will have at least 9 follow-up visits over the first year to do additional testing. After the first year, there will be additional visits at months 18 and 24. There will be yearly visits for the next 3 years (5 years total).

SKYLINE CLINICAL TRIAL ELIGIBILITY

To be eligible for the phase 2 SKYLINE 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 8 and 50 years at the time of screening
- In both eyes, have a best visual acuity no better than 20/32 and no worse than 20/200 on an eye chart

GENETIC TESTING IS AVAILABLE

If you or your child has been diagnosed with XLRP and have had the *RPGR* gene mutation confirmed through genetic testing, you may be eligible for the SKYLINE 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, please call 855-GOSCENIC ([855] 467-2364) or visit scenictrials.com to learn more about genetic testing options.
CONSIDERATIONS

WHAT TO EXPECT

• Study participation is approximately 5 years
• There are up to 18 study visits in total, with up to 13 of those being in the first year of enrollment
• You may have to travel for the study visits depending on your study site (all travel for study-related assessments will be provided at no cost to you)
• It is important to consider the time associated with study participation

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.

If you qualify and enroll you will receive:

• All 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 travel for study-related assessments provided at no cost to you or your caregiver (if applicable)

TO LEARN MORE ABOUT THE SKYLINE CLINICAL TRIAL, visit scenictrials.com or call 855-GOSCENIC / (855) 467-2364.
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).