

Clinical studies to evaluate gene therapy in ACHM and XLRP

AGTC, a biotechnology company, is conducting Phase 1/2 clinical studies at several sites throughout the U.S. to evaluate investigational gene therapies in people with ACHM and XLRP. AGTC is also conducting a Natural History Study (NHS) in people with XLRP.

ACHM Phase 1/2 Clinical Studies

To be considered, individuals must:

- Have a retinal condition consistent with a diagnosis of ACHM and a documented mutation in the CNGA3 or CNGB3 gene
- Be at least 6 years of age
- Be able to complete tests of visual and retinal function
- Have a visual acuity of 20/80 or worse
- Have acceptable laboratory parameters

Please visit bit.ly/ACHM-trial-CNGA3 or bit.ly/ACHM-trial-CNGB3 to read more about this study at clinicaltrials.gov

XLRP Phase 1/2 Clinical Study

To be considered, individuals must:

- Have a retinal condition consistent with a diagnosis of XLRP and a documented molecular diagnosis of a mutation within the RPGR gene
- Be male and at least 6 years of age
- Have best-corrected visual acuity of 20/50 or worse in the study eye for Groups 1-3; and best-corrected visual acuity of 20/32 or worse in the study eye for Group 4
- Be able to complete tests of visual and retinal function
- Not have any pre-existing eye conditions that would interfere with the interpretation of study endpoints
- Not have previously received an AAV gene therapy product
- Have acceptable laboratory parameters

Please visit bit.ly/XLRP-trial to read more about this study at clinicaltrials.gov

XLRP Natural History Study

The objective of the NHS study is to gather information about the XLRP condition, and monitor certain eye tests over time to understand better the progression of the disease on your eyes and the impact of the condition on your quality of life. No experimental therapies will be administered to individuals in the NHS. Individuals with qualifying mutations in the RPGR gene will be evaluated every 6 months for 3 years using a variety of non-invasive visual function tests to more fully characterize their clinical condition.

Please visit bit.ly/XLRPnaturalhistorystudy to read more about this study at clinicaltrials.gov

AGTC is developing innovative treatments that offer hope for individuals with unmet medical needs.



Gene Therapy Clinical Studies for
Achromatopsia (ACHM) and
X-Linked Retinitis Pigmentosa (XLRP)



Please visit www.agtc.com for more information about IRDs and updates on our current and planned clinical studies.

Please contact advocacy@agtc.com for more information.

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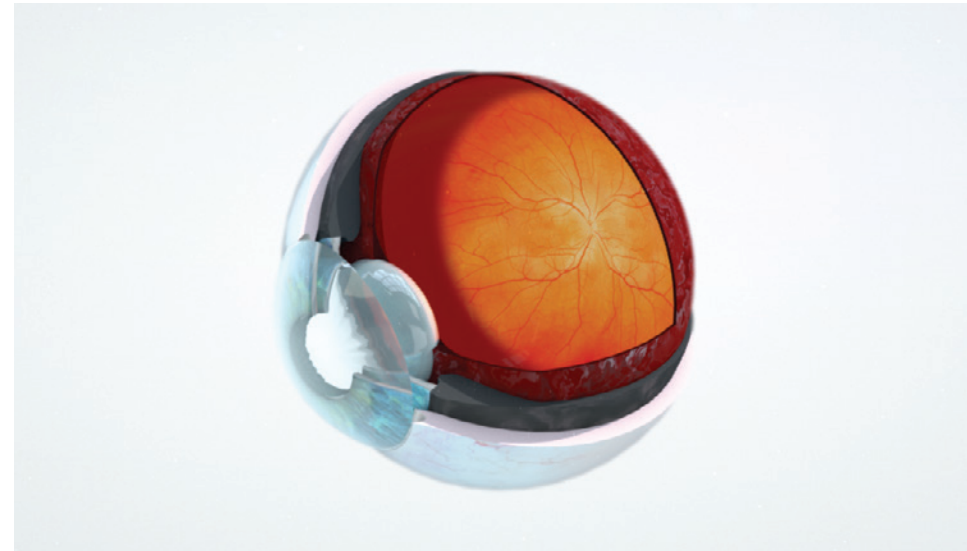


What is an inherited retinal disorder (IRD)?

An inherited retinal disorder, sometimes called an IRD, is a condition that is passed down through family members. IRDs affect the retina, a layer of cells that lines the back of the eye and detects light. Many IRDs are rare conditions, meaning they affect small groups of people. For years there were no medical treatments for IRDs. Today, doctors are conducting clinical research studies to see if new treatment options may help people with IRDs see better.

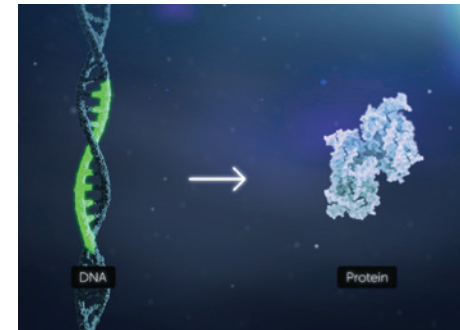
How do I know if I have an IRD?

The body is made up of trillions of cells and each cell requires proteins to work properly. Genes within our cells hold the code for how to make proteins and researchers have identified several of the genes that can cause IRDs. A genetic test can help your doctor determine if you have a non-working gene that is causing vision loss or may cause vision loss in the future. Your doctor may order a blood or saliva test to find out if you have an IRD. Genetic testing may be provided for individuals with a family history of IRDs who have not received a confirmation of specific genetic changes.

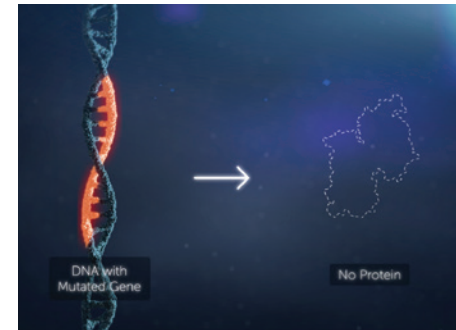


What does it mean to have a non-working gene?

Genetic information is carried in your DNA. Your DNA has a code that gives your cells instructions to make proteins. These proteins are needed for almost everything your body does. If a part of your DNA code is changed, the protein may be made incorrectly or not at all. Without the protein, the cell cannot do its job. If the cell cannot do its job, there can be problems like vision loss. As part of the genetic testing process, you may hear your doctor or genetic counselor mention phenotypes or genotypes. A phenotype is a symptom caused by a change in the DNA code. A genotype refers to the specific change in the DNA code.



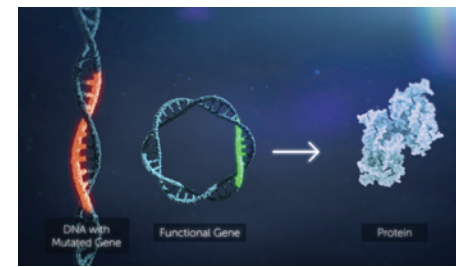
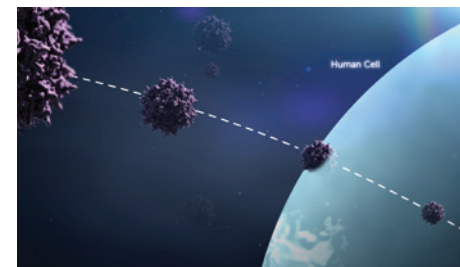
Working gene produces protein



Non-working gene does not produce protein

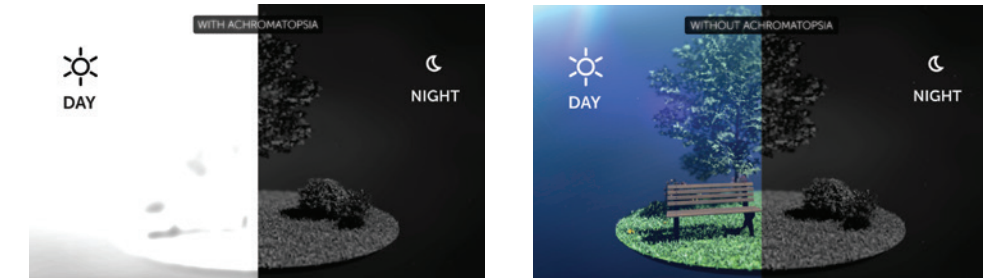
What is gene therapy?

Gene therapy is a medical treatment that delivers a working copy of a gene to a cell in a person's body. The working copy allows the body to produce proteins that help the cell work effectively.



What is achromatopsia (ACHM)?

ACHM is a rare IRD that makes it harder for cells in the retina to detect light signals. People with ACHM often have poor vision, are sensitive to light and are unable to see different colors.



What is X-linked retinitis pigmentosa (XLRP)?

XLRP is a rare IRD characterized by progressive degeneration of the retina, which can start with difficulty seeing at night and eventually lead to blindness in adult men.

What is a clinical research study?

Clinical research studies, also called clinical trials, are conducted by doctors and researchers to understand the potential benefits and risks of new, unapproved medical treatments. Phase 1 and 2 studies are early studies intended to obtain initial data about the safety of the new investigational therapy and its potential effectiveness. If Phase 1 and 2 studies produce positive data, Phase 3 studies may be conducted. Phase 3 studies are larger studies intended to obtain more extensive data about the safety and effectiveness of the treatment.

A Natural History Study (NHS) is a longterm study to gather information about the progression of a condition and the impact of the condition on a patient's quality of life. Individuals may undergo routine testing of the progression of their condition but no investigational therapies are administered.