

For more information about the VISTA clinical trial, including how your patients may participate, please contact Serva Health at **(855) 843-9847**, or visit **www.VISTAtrialHCP.com** and click on "Refer a Patient."

# An investigational gene therapy for males with X-linked retinitis pigmentosa

## **The VISTA Clinical Trial**

The VISTA clinical trial is for male patients ages 13–50 years (inclusive) diagnosed with X-linked retinitis pigmentosa (XLRP), a rare form of RP that causes progressive vision loss. The multicenter study will assess the safety, tolerability, and efficacy of AGTC-501, an investigational gene therapy.<sup>1</sup>

## **Key Eligibility Criteria**

To be eligible for the VISTA clinical trial, an individual must meet the following criteria<sup>1</sup>:

- Be male with a diagnosis of XLRP 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 at the time of screening
- Have best corrected visual acuity no better than 75 letters and no worse than 35 letters on an ETDRS chart (or Snellen equivalent, no better than 20/32 and no worse than 20/200) in at least one eye

## About XLRP, a Rare Form of RP

Retinitis pigmentosa (RP) describes a group of rare genetic eye diseases that damage light-sensitive cells in the retina, leading to loss of sight over time. In about 10% of RP cases, the nonworking gene is passed down from the mother to her male children, resulting in a form of RP known as X-linked RP (XLRP). XLRP causes gradual vision loss in boys and young men. The disease begins with night blindness and is followed by a slow narrowing of the peripheral field of vision. The decline in visual acuity results in legal blindness by the time the individual reaches their 40s.<sup>2</sup>

References: 1. Protocol AGTC-RPGR-002. Version 2.0. December 2021. 2. AGTC website. X-linked retinitis pigmentosa. https://agtc.com/programs/x-linked-retinitis-pigmentosa Accessed December 1, 2020. 3. AGTC Investors Web page. https://ir.agtc.com/. Accessed December 1, 2020.





© agtc. All rights reserved

V2 | 25JAN2022 | AGTC-RPGR-002

# **About AGTC-501**

Gene therapy is the process of injecting a person with a healthy gene to replace one that is damaged or mutated.

The VISTA clinical trial for XLRP involves an experimental procedure with an investigational study drug, known as AGTC-501 (designated as rAAV2tYFGRK1-RPGR), which is designed to replace the mutated RPGR gene that causes XLRP.<sup>2</sup>

Data from all 29 patients across 6 dose groups of the initial phase 1/2 XLRP clinical trial continued to indicate a favorable safety profile with no secondary inflammation observed. Furthermore, a combined analysis of visual sensitivity data\* from all centrally dosed patients showed robust and durable signs of improvement.<sup>†</sup>

#### **About AGTC**

AGTC, the study sponsor, is a clinical-stage biotechnology company that uses a proprietary 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 XLRP and achromatopsia (ACHM CNGB3 & ACHM CNGA3).<sup>3</sup>

# **Genetic Testing is Available**

If your patient has been diagnosed with XLRP and has had the RPGR gene mutation confirmed through genetic testing, they may be eligible for the VISTA trial. If you are unsure whether your patient's vision loss is due to XLRP and they have not received genetic testing, please contact Serva Health at (855) 843–9847, or visit www.VISTAtrialHCP.com to learn more about genetic testing options.<sup>2</sup>

#### **Learn More or Refer a Patient**

For more information about our trials, including how your patients may participate, please contact Serva Health at **(855) 843–9847**, or visit **www.VISTAtrialHCP.com** and click on "Refer a Patient."

'Improvement in visual sensitivity is based on multiple measures including change from baseline in visual sensitivity of at least 7 decibels in at least 5 loci or a statistically meaningful improvement in sensitivity improvement profile between the treated and untreated eye.

'Visual sensitivity, visual acuity, and safety data were reported for 12-month time points for Groups 2 and 4, and 6-month time points for Groups 5 and 6.