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

The VISTA clinical trial is for male patients ages 12–50 years (inclusive) diagnosed with X-linked retinitis pigmentosa (XLRP), a rare form of retinitis pigmentosa (RP) that causes progressive vision loss. This multicenter study will evaluate the efficacy of 2 doses of AGTC-501, an investigational gene therapy, compared to a control group.[1]

About XLRP, a Rare Form of RP

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 pathogenic genetic variant is passed down from the mother, resulting in a form of RP known as XLRP. XLRP causes gradual vision loss primarily in boys and young men.[2] The disease begins with night blindness and is followed by a gradual narrowing of the peripheral field of vision. The decline in visual acuity results in legal blindness in approximately 50% of patients by the time the individual reaches their 50s.[3]

About AGTC-501

Beacon Therapeutics is currently developing AGTC-501 for treatment of XLRP caused by mutations in the retinitis pigmentosa GTPase regulator (RPGR) gene. AGTC-501, an AAV vector-based gene therapy delivered subretinally, is designed to provide full-length functioning RPGR protein to target the genetic root cause of XLRP. Gene therapy is the process of modifying a patient's cells by adding a functional copy of the genetic factors contributing to the retinal disease. The new functional copy allows a patient's own body to produce proteins to treat or prevent the progression of the disease. A single treatment may provide long lasting benefits to patients.[2]

For more information, please visit BeaconTx.com.

References:

1. Protocol AGTC-RPGR-002. Version 4.0. February 2024.
2. Beacon Tx website. X-linked retinitis pigmentosa. https://www.beacontx.com/aav-gene-therapy/. Accessed March 19, 2024
3. Di Iorio, V et al., Spectrum of Disease Severity in Patients with X-linked Retinitis Pigmentosa Due to RPGR Mutations. IOVS, December 2020, Vol 61, 36.

Key Eligibility Criteria

To be eligible for the VISTA clinical trial, an individual must meet the following criteria[1]:

- 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 12 and 50 years at the time of screening
- Have best corrected visual acuity no better than 20/32 and no worse than 20/200 in one eye

Genetic Testing is Available

If your patient has been diagnosed with XLRP and has had the *RPGR* genetic 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 **VISTAtrialHCP.com** to learn more about genetic testing options.

About Beacon Therapeutics

Beacon Therapeutics, the clinical trial sponsor, is an ophthalmic gene therapy company founded to restore and improve the vision of patients with a range of 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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