Janssen's Commitment to X-linked Retinitis Pigmentosa (XLRP)

At Janssen, we're translating our understanding of the underlying biology of retinal diseases to develop needed therapies that restore and preserve vision.

Our clinical-stage retinal portfolio includes a gene therapy candidate, AAV-RPGR, under investigation for the treatment of XLRP. This asset, being jointly developed with MeiraGTx Holdings plc., is designed to counteract the loss of retinal cells resulting from mutation in the RPGR gene.

5.5M
people worldwide are affected by inherited retinal diseases.[4]

What is XLRP?

Retinitis pigmentosa is a group of inherited retinal diseases (IRDs) that cause serious vision impairment and often blindness.

XLRP is a severe form of retinitis pigmentosa that results in early onset, rapidly progressive retinal degeneration, and loss of eyesight.[1]

What causes XLRP?

XLRP is most commonly caused by changes in the retinitis pigmentosa GTPase regulator (RPGR) gene, which codes for a protein that plays a vital role in the development and health of the light-sensitive cells (photoreceptors) that make up the retina.[1]

Absence of this functional protein results in both rods and cones functioning poorly, leading to retina degeneration and legal blindness.[1]

XLRP typically progresses from childhood, causing progressive "tunnel vision" ultimately resulting in legal blindness by age 40.[2]

Who is affected?

- Retinitis pigmentosa impacts **1 in 3,500** people globally.[2]
- **15-20%** of all retinitis pigmentosa cases are X-linked.[2]
- Due to its inheritance pattern, XLRP **predominantly affects men.**[2]

How is XLRP diagnosed?

- Evaluating personal and family medical history.[3]
- Imaging techniques and genetic testing.[3]

How is XLRP treated?

- There are **currently no approved treatments** for XLRP.[2]
- Clinical trials are underway to determine the therapeutic potential of gene therapies.

If you or a loved one have questions about your eye health, please speak with your doctor about your concerns and symptoms.

References
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4. Hanany, M., Rivolta, C. and Sharon, D., 2020. Worldwide carrier frequency and genetic prevalence of autosomal recessive inherited retinal diseases. Proceedings of the National Academy of Sciences, 117(5), pp.2710-2716.

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