RP2 and RPGR Vectors For Treating X-linked Retinitis Pigmentosa

Summary (1024-character limit)
The National Eye Institute (NEI) seek research co-development or licensees for advancing AAV8/9-based therapies for X-linked forms of retinitis pigmentosa (XLRP) caused by mutations in RPGR (retinitis pigmentosa GTPase regulator) or RP2 (retinitis pigmentosa 2) gene.

NIH Reference Number
E-050-2015

Product Type
- Therapeutics

Keywords
- pigmentosa, retina, gene therapy, hereditary ocular disease, Wu

Collaboration Opportunity
This invention is available for licensing and co-development.

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Description of Technology

X-linked forms of retinitis pigmentosa (XLRP) are relatively severe blinding disorders, resulting from progressive photoreceptor dysfunction primarily caused by mutations in RPGR or RP2 gene.

This technology is poised to advance RPGR or RP2 gene therapy to clinical stage using AAV8 or AAV9 vector carrying human full-length RPGR or RP2-coding sequence. The investigators have performed a wide dose range study over 18-months and found it to preserve rod and/or cone function as evidenced by ERG and/or OCT, optomotor tests. Morphologically, the treatment preserved rod and cone viability, and corrected mistrafficking of cone opsin and/or rhodopsin. The therapeutic effect was also achieved in advanced disease stage. The broad treatment window and long-lasting therapeutic effects make the RPGR and RP2 gene therapy attractive for clinical development.

This technology is available for licensing, or the NEI research team will entertain potential collaborations that will advance this technology through the remaining preclinical stage toward IND development under

a CRADA or a license combined with a CRADA project.

This patented treatment of albinism has not been approved by the U.S. Food and Drug Administration (FDA), and currently there is no active clinical trial to assess this albinism therapy benefit. However, if you are interested in participating in a future or upcoming NIH clinical trial on albinism, please contact NIH clinical coordinator, Ms. Ellaine Galindez-Balut (ellaine.galindez-balut@nih.gov), for helpful information. Thank you.

Potential Commercial Applications

- Use in gene therapy to prevent or cure XLRP
- Preserving cone and/or rod function, restoring ERG and protein in the retina, increasing photoreceptor numbers, decrease in retinal detachments
- Improving quality of life, visual acuity, ability to drive and independent living

Competitive Advantages

- Pre-clinical dose efficacy study done
- RPGR and RP2 available in both AAV-8 and AAV-9 vectors.
- Preclinical data to show that treatment at advanced age also shows remarkable preservation of retinal structure and function.

Inventor(s)

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Development Stage

- Pre-clinical (in vivo)

Publications

Li L, et al. Ablation of the X-linked retinitis pigmentosa 2 (Rp2) gene in mice results in opsin mislocalization and photoreceptor degeneration [PMID: 23745007]

Zhang H, et al. Mistrafficking of prenylated proteins causes retinitis pigmentosa 2. [PMID: 25422369]

Mookherjee S, et al. Long-term rescue of cone photoreceptor degeneration in retinitis pigmentosa 2 (RP2)-knockout mice by gene replacement therapy [PMID: 26358772]

Wu Z, et al. A long-term efficacy study of gene replacement therapy for RPGR-associated retinal degeneration [PMID: 25877300]

Patent Status

- **U.S. Patent Filed:** U.S. Patent Application Number USPTO 62/131,661 , Filed 11 Mar 2015

Related Technologies

- E-164-2014
- E-162-2016

Therapeutic Area

- Eye and Ear, Nose & Throat