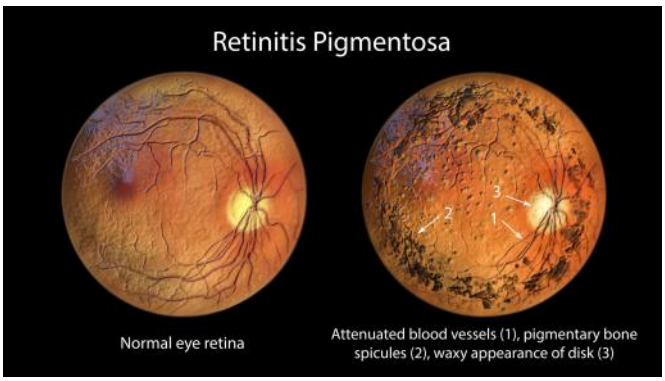
PROJECT NUMBER

15619, 15879

Gene therapy for retinitis pigmentosa due to mutations in the rhodopsin gene

1 in 3,000-7,000 people suffers from retinitis pigmentosa (RP), with 25-30% of RP cases being autosomal dominant RP (adRP). Of these, 25% are due to mutations in the rhodopsin gene (RHO). The clinical phenotypes of rhodopsin-mediated adRP (RHO-adRP) are diverse and range from mild night blindness to severe visual impairment. There are currently no universally effective treatments or cures for RHO-adRP.

Applications: Gene therapy, therapeutics, ophthalmology



Features	Benefits
AAV-based gene therapy for retinitis pigmentosa caused by mutations in the rhodopsin gene	The developmental product offers a potential long-lasting therapy for all RHO-adRP sufferers
Delivers artificial mirtrons (intron-derived microRNAs) to suppress the expression of the endogenous mutated rhodopsin	Pre-clinical development ongoing at Oxford via an MRC DPFS-funded project, the principal objective of which is to deliver a clinical candidate

Features	Benefits
Also delivers a functional version of the rhodopsin gene to replace the endogenous mutated form	Oxford has a track record in the development of gene therapies for inherited retinal diseases and possesses extensive pre-clinical development, regulatory and clinical trials experience
Patent applications pending in the US, Europe and Japan	

Patented and Available for

- Co-development
- Licensing

To find out more, contact enquiries@innovation.ox.ac.uk and quote the project number.