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New gene therapy could bring relief for eye disease patients

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A new gene therapy for a common cause of blindness could spell the end of invasive monthly injections into the eye, according to researchers at the Lions Eye Institute (LEI) in Perth, Western Australia.

The treatment focuses on wet Aged-Related Macular Degeneration (AMD), the most common cause of blindness in the developed world. Wet AMD leads to rapid vision loss and costs up to $6 billion worldwide each year.

Principal clinical investigator Professor Ian Constable and the LEI clinical team have recruited 40 patients to the trial. Professor Constable said the gene therapy was proving well tolerated and promising in human trials currently under way.

Early results on safety and efficacy from the first eight patients in the trial were reported to the Association for Research in Vision and Ophthalmology (ARVO) annual conference in Florida earlier this month by principle scientific investigator Winthrop Professor Elizabeth Rakoczy.

"To date, the safety profile is excellent - we have found no serious adverse effects in the eye - and so far we have promising data on how it works," Professor Constable said.

Wet AMD occurs when there is an overproduction of the protein vascular endothelial growth factor (VEGF) in the retina. VEGF helps

support oxygen supply to tissue when circulation is inadequate. When too much VEGF is produced it can cause disease, including blood vessel disease in the eye.

Current treatment for wet AMD is intensive and intrusive - involving monthly injections of anti-VEGF drugs that limit production of the protein.

"The gene therapy involves a single injection of a modified and harmless version of a virus containing a specific gene that stimulates supply of a protein which then blocks over-production of VEGF," Professor Constable said.

The rights to the technology have been licensed by US company Avalanche Biotechnologies. Professor Constable said there were very few examples in Australia of a research group taking an idea from the laboratory bench to commercialisation.

"This is research of international significance and a huge academic achievement for The University of Western Australia, the Centre for Ophthalmology and Visual Science and the Lions Eye Institute," he said.

The science behind the treatment began more than 20 years ago when Professor Constable recruited Professor Rakoczy, a molecular ophthalmologist, to the LEI. Extensive laboratory and pre-clinical research was funded by the National Health and Medical Research Council (NHMRC) and conducted in Perth, Beijing and Singapore.

It was the first research in Australia using gene therapy in ophthalmology or any other medical field and was named by the NHMRC in its 10 of the best national research projects in 2005.

The research started in a mouse model and progressed to Briard dogs,

whose sight was restored within a month of receiving treatment.

Professor Constable said despite the very promising interim results, more testing was required.

"After the Perth trial, multi-centre studies will have to be run in the United States and FDA (US Food and Drug Administration) approval sought but we believe we are on track to test the investigational therapy in more patients, and, if proven safe and effective, make it widely available," he said.

Provided by University of Western Australia