



**FIGHTING BLINDNESS**

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# **RETINA AUSTRALIA**

**Member of Retina International  
Associate Member of AMD Alliance International**

## **PRESS RELEASE**

### **PROGRESS WITH TREATMENT OF GENETIC BLINDNESS**

In a world's first, teams of ophthalmic surgeons in the UK and the USA have achieved positive results from the introduction of a gene therapy into the retina of the eyes of humans affected with a retinal dystrophy (genetic blindness).

The genetic blindness involved in the trials is Leber's Congenital Amaurosis (LCA), a particularly 'virulent' type which affects persons with this genetic defect either from birth or from a very early age.

Mr Graeme Banks, President of Retina Australia says the progress that has now been announced is exciting, especially given the demonstrable difference that has been made to the vision of the participants in Phase 1 of the trials, and taking into account that only low doses of the gene therapy were used. He said the main initial aim was to assess the efficacy/safety of such therapies.

"Whilst these initial gene therapy human trials commenced in the UK and the USA early in 2007, human gene therapy trials are also being progressed in Australia at the Lions Eye Institute, Perth, with the support of Retina Australia", Mr Banks said. "The very promising results from these first trials will provide the impetus for further work not only with the LCA gene but also with other genes responsible for genetic blindness, as the principles will be the same. "

Mr Banks said that research to find the genes responsible for retinal dystrophies such as LCA, Retinitis Pigmentosa, Macular Degeneration, and Stargardt's Disease, commenced in the early 1980s with the number of variations expected to total around 350 when they are all isolated. However, as specific genes have been found, work has commenced on developing a gene therapy for each one. He explained that the gene therapy has to be specific for each retinal dystrophy!

"One of the major tasks now faced by Retina Australia is the development of a genetic register of persons with retinal dystrophies whereby affected persons

may, voluntarily, provide a blood sample from which their genetic material will be extracted. The aim is to determine their specific errant gene so that as gene therapies become available in the future there will be knowledge as to who will benefit from each specific therapy."

The ultimate aim is to provide the opportunity to all persons in Australia with genetic blindness, and their families, to benefit from gene therapies as and when they come on line.

Graeme Banks OAM  
President

**Full details of the work done by the surgeons in the UK and USA are outlined in the reports accompanying this Press Release.**

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## Background Notes

Excluding the genes responsible for Macular Degeneration, one in every 80 persons world wide is a carrier of a gene causing genetic blindness.

This translates to one in every three thousand persons being directly affected with genetic blindness in some form. Retinitis pigmentosa and macular degeneration are the major types of genetic blindness.

Retina Australia is undertaking a major public awareness exercise aimed at informing persons with retinal dystrophies of the organisation and the services it can offer through its State bodies.

Retinal dystrophies are the major cause of youth blindness and in the working age population are second to diabetes as the cause of vision impairment. Macular degeneration is responsible for most of the vision impairment in persons over the age of 55 – one in three with persons over 75 years.

Graeme Banks