

## **Research at Lions Eye Institute, University of Western Australia**

In Australia, Prof Rakoczy and her team at the Lions Eye institute, Perth, successfully applied gene therapy for LCA using a dog model of the disease. Following gene therapy, the vision impaired dogs showed remarkable improvement in their sight. After this success, the team focused its attention on Age Related Macular Degeneration (AMD).

In general, ocular gene therapy is used to produce a particular protein that is either not present or present, but is non functional in the eye due to a genetic mutation. Unfortunately, the genetic mutation is not always known thus limiting the usefulness of gene therapy. Hence, development of strategies that do not require the knowledge of the mutation is very important.

AMD, which is the major cause of blindness in the developed world, is a complex disease. While genetics may play an important role, the genetic mutations causing AMD remain unknown in most cases. To be able to tap into the long-term benefits of gene therapy for the treatment of AMD, Prof Rakoczy and her team has developed the concept of Secretion Gene Therapy. Here, the same recombinant virus delivery system used for the human LCA trials is utilised to produce a natural soluble protein. This natural soluble protein is able to neutralise the affect of factors responsible for promoting the growth of damaging new and leaky blood vessels seen in the wet form of AMD. Preclinical trials showed remarkable inhibition of these damaging blood vessel growth and leakiness and maintenance of vision. Prof Rakoczy is in the final stages of gaining approval from the TGA for human trials in Australia and she hopes to start clinical trials later this year.

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