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NEWS

Gene therapy receives Champalimaud Vision Award

The Champalimaud Vision Award has been awarded to research teams for the development of a gene therapy...

By [European Pharmaceutical Review](#)

4 September 2018

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The 2018 [Champalimaud Vision Award](#) recognised research teams for the development of [gene therapy](#) for the Leber Congenital Amaurosis, a genetic form of childhood blindness, whose genetic cause was discovered by Dr Michael Redmond.

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conditions.

The work of these researchers built on the previous cloning of the RPE65 gene by Dr Redmond and his elucidation of its critical role in vitamin A metabolism for vision. Dr Redmond also received an award for his work.

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Visual perception starts in the eye with the activation of neural responses triggered by light. George Wald received the Nobel Prize for demonstrating the central role of vitamin A in biochemical events that convert light into neural visual impulses, 50 years ago.

After 25 years, Dr Redmond cloned the RPE65 gene and demonstrated that it is essential for converting dietary vitamin A into the biological form that is active in retinal photo-receptor cells, and it was soon found that RPE65 gene mutations render children functionally blind from birth.

The three research teams that developed this revolutionary gene therapy are: Dr Jean Bennett and Dr Albert Maguire, from the [Scheie Eye Institute, University of Pennsylvania School of Medicine](#) and [Children's Hospital of Philadelphia](#); Dr Robin Ali and Dr James Bainbridge, from the Institute of Ophthalmology of the [University College London](#) and [Moorfields Eye Hospital](#); and Dr Samuel Jacobson and Dr William Hauswirth, from the Scheie Eye Institute, University of Pennsylvania School of Medicine and the [University of Florida College of Medicine](#).

Working synergistically, these scientists engineered solutions for providing a functional replacement of RPE65 using gene augmentation therapy in the eye. This restored vision to treated children and adults, and in turn their success enabled the entire field of gene therapy for human disease.

[Moorfields Eye Hospital, Scheie Eye Institute, University College London, University of Florida College of Medicine, University of Pennsylvania School of Medicine](#)

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