**Choroideremia gene therapy gives long-term benefit to vision**

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Encouraging results from a 3.5-year follow up to the Phase I clinical trial

**Results from a 3.5-year follow-up study show that patients who took part in the world’s first gene therapy clinical trial for choroideremia have had a long-lasting benefit to their eyesight. The clinical trial was led by Professor Robert MacLaren at the University of Oxford and was made possible due to research by Professor Miguel Seabra at Imperial College London supported via the**[**Tommy Salisbury Choroideremia Fund at Fight for Sight**](http://www.fightforsight.org.uk/get-involved/tommy-salisbury-choroideremia-fund/)**.**

[Choroideremia](http://www.fightforsight.org.uk/about-the-eye/a-z-eye-conditions/choroideremia/) is a rare inherited cause of severe sight loss that affects the male population. Symptoms start in childhood with trouble seeing at night and eventually lead to complete blindness by around the age of 40.

Initial results from the early-stage (Phase I) clinical trial were [published in The Lancet](http://www.thelancet.com/journals/lancet/article/PIIS0140-6736(13)62117-0/abstract) in 2014. They showed that two of six patients treated with gene therapy for choroideremia had significant improvements in their vision, in the treated eye.

The follow-up report, which is [published in The New England Journal of Medicine](http://www.nejm.org/doi/full/10.1056/NEJMc1509501), shows that the benefits have lasted for 3.5 years since treatment, even though vision has become worse in the untreated eye during this time. Two other patients in the study haven’t had any more sight loss in their treated eye, while the youngest participant showed no change in either eye. One participant has had a steady decline in vision.

Dolores Conroy, Director of Research at Fight for Sight said: “Maintaining these positive results is very encouraging as similar mechanisms for other conditions have not shown improvement lasting over time.”

Professor MacLaren said: “This clinical trial marked a major step in developing gene therapy treatments for retinal diseases.”