**Year Six Report**

**RP Fighting Blindness Centre for the Development of Gene Therapy for Inherited Retinal Dystrophies, also known as the ‘Gene Team’.**

**Principal Investigators**

Professor Robin Ali, Professor James Bainbridge and Professor Michel Michaelides

**Research Institution**

UCL Institute of Ophthalmology

**Progress**

**Continuation of three gene therapy clinical trials:**

* LCA2 (RPE65 deficiency) - 15 subjects enrolled in this trial, which will be completed in 2018.
* Achromatopsia (CNGB3 deficiency) - 12 subjects enrolled in this trial, which will be completed at the end of 2018.
* XRP3 (RPGR deficiency) - a clinical grade vector has been manufactured and the trial started in May 2017 with eight enrolled subjects.

**Preclinical development:**

* Achromatopsia (CNGA3 deficiency) - vector manufacture has started and a clinical trial is planned for 2019.
* LCA4 (AIPL1 deficiency) - a clinical grade vector has been manufactured and will be used in experimental treatment once suitable patients under the age of three years have been identified.
* LCA13 (RDH12 deficiency) - the team are working on optimising the vector for this therapy in preparation for future clinical trials.

**Other research:**

The team are continuing to extend their pipeline of potential gene therapies, including research into LCA5, RP2, Stargardt disease, Usher syndrome, Bardet Biedl syndrome and Batten disease.