Gene Team update 2019

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The Gene Team is led by Professor Robin Ali, James Bainbridge and Michel Michaelides at the UCL Institute of Ophthalmology and Moorfields Eye Hospital. We know that gene therapy is a viable treatment route for some inherited retinal dystrophies, illustrated by the recent recommendation by NICE of Luxturna, a treatment for a specific type of Leber congenital amaurosis. This has given the whole community a sense of optimism about future treatments in the NHS.

So far, the Gene Team has:

- Completed an early stage clinical trial of RPE65 gene therapy to treat LCA2, one form of Leber congenital amaurosis, and gone on to undertake a further trial using an improved gene delivery system (vector). They are now considering a phase 3 trial.

- Developed a gene therapy for LCA4, another type of Leber congenital amaurosis and, following preclinical safety testing, used this to treat a very young patient who will be monitored over the coming months.

- Commenced a clinical trial of CNGB3 gene therapy to treat one form of achromatopsia – this has enrolled 11 adults and nine children.

- Developed a gene therapy vector for X-linked RP3, one of the most common forms of RP, and commenced a clinical trial involving 10 adults and one child. Completed preclinical development of a gene therapy for LCA13 caused by RDH12 deficiency.

The researchers are continuing with the early development of gene therapies for various retinal conditions and related syndromes.

A huge thank you to everyone who is supporting this important work.

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