Doctors test gene therapy to treat blindness

By **Ben Hirschler**
LONDON | Tue May 1, 2007 11:11am EDT

(Reuters) - A team of British doctors has carried out the world's first eye operations using gene therapy to try to cure a serious sight disorder, officials said on Tuesday.

The group from Moorfields Eye Hospital and University College London (UCL) has operated on a small number of young adults with Leber's congenital amaurosis, a type of inherited childhood blindness caused by a single abnormal gene.

The condition prevents the retina from detecting light properly, resulting in progressive deterioration and severely impaired eyesight. There is no effective treatment.

The new experimental procedure involves inserting normal copies of the faulty RPE65 gene into cells of the retina -- the light-sensitive layer of cells at the back of the eye -- using a harmless virus or vector.

The British doctors are working alongside Seattle, Washington-based biotech firm Targeted Genetics Corp., which made the vector being used in the Phase I/II trial.

It will be several months before the success of the procedure can be properly assessed but medics said there had been no complications so far.

The move into human testing follows 15 years of laboratory and animal experimentation, including tests on dogs whose vision was restored to the extent they could navigate a maze with ease.

"Testing it for the first time in patients is very important and exciting and represents a huge step towards establishing gene therapy for the treatment of many different eye conditions," Robin Ali, professor of human molecular genetics at UCL, said in a statement.

The clinical trial was given 1 million pounds ($2 million) of funding by Britain's Department of Health, which said the pioneering research underlined the country's leading position in gene therapy in Europe.

The idea of using gene therapy to fix diseases caused by genetic faults has long appealed to scientists, although getting the idea to work in practice has proved tricky.

Some gene therapy approaches have helped patients. But one 18-year-old volunteer died in a gene therapy experiment in 1999 and two French boys cured of a rare immune disease later developed leukemia.

Over 70 percent of gene therapy trials to date have been for cancer, where the process is complicated by the need to reach multiple sites in the body.

The eye, by contrast, is relatively straightforward, said Andrew George of London's Imperial College.

"The eye is good for gene therapy because it is a simple organ and it is easy to see what is going on. There is hope that once gene therapy is developed in the eye, scientists could move on to more complex organs," he said.

http://www.reuters.com/article/2007/05/01/us-blindness-genes-idUSL016653620070501?pageNumber=1