

## **Encouraging trial results for cystic fibrosis gene therapy treatment**

Imperial Innovations is pleased to note the announcement this morning that a gene therapy treatment for cystic fibrosis developed by the UK Cystic Fibrosis Gene Therapy Consortium has produced encouraging results in a major UK trial.

The placebo-controlled trial, which involved 116 patients aged 12 and above, was run at the Royal Brompton Hospital in London and the Western General Hospital in Edinburgh. Patients received monthly doses of either the gene therapy, named pGM169/GL67A, or a placebo for one year. The trial reached its primary endpoint and showed that patients who received the therapy had a significant but modest benefit in lung function compared with those who received a placebo.

The trial is the first to show that repeated doses of gene therapy can have a meaningful effect on the disease, and change the lung function of patients. However, the team say more research is needed to improve the effectiveness before the therapy will be suitable for clinical use.

Imperial Innovations holds a patent portfolio and Orphan Drug Designations in Europe and US for the pGM169/GL67A product and is actively seeking commercial partners to further develop the therapy following the announcement of these positive trial results.

The current trial was launched in 2012 and funded by a partnership between the National Institute for Health Research (NIHR) and the Medical Research Council (MRC). The findings are published today in *The Lancet Respiratory Medicine*.

The study was carried out by the UK Cystic Fibrosis Gene Therapy Consortium, a group of scientists and clinical teams from Imperial College London, the Universities of Oxford and Edinburgh, Royal Brompton & Harefield NHS Foundation Trust and NHS Lothian who came together in 2001 to develop a gene therapy, supported by the Cystic Fibrosis Trust.

Imperial Innovations has supported the CFGTC since 2006, providing advice on intellectual property and the contractual relationships underpinning CFGTC research. In 2010, Imperial Innovations formally became the lead technology transfer office acting on behalf of the CFGTC Universities in managing the intellectual property and commercialisation strategy for the gene therapy programme. Following this appointment, Innovations has assisted the CFGTC with patent filings, strategic development, has appointed a board of commercial and regulatory advisers, and has applied for and received orphan drug designation for the CFGTC's clinical stage non-viral gene therapy product, pGM169/GL67A.

**Professor Eric Alton**, Co-ordinator of the CFGTC and consultant physician at Royal Brompton Hospital, said:

***"Patients who received the gene therapy showed modest but significant benefit in tests of lung function compared with the placebo group. The effect was inconsistent, with some patients responding better than others. There were no safety concerns.***

***"These results are encouraging, as they lay the groundwork for further trials which we hope could improve the effect. We hope to carry out follow-up studies looking at higher, more frequent doses, combinations with other treatments, and better methods for delivering the DNA into cells."***

**Tony Hickson**, Managing Director of Technology Transfer at Imperial Innovations, said:

***"The work of the Cystic Fibrosis Gene Therapy Consortium over the past decade has advanced treatment options for cystic fibrosis, and Imperial Innovations has been proud to support their work."***

***"We are delighted to be continuing our support as the CFGTC looks to take their treatment through to the next stage of clinical trials and explores partnership opportunities to enable this to happen"***

