



## JOURNALIST FACTSHEET

### GENE THERAPY

#### **What is gene therapy?**

Gene therapy is a way of treating or curing a disease by adding a copy of a healthy gene to do the job of a faulty one.

For gene therapy to be effective, the healthy gene has to be transported to the cells where it is needed and has to tell the body to turn it on when it gets there.

The carrier for transporting a healthy gene is called a vector or gene transfer agent (GTA). The body is told how to turn the gene on by using a promoter.

A gene therapy product is made up of the healthy gene, the gene transfer agent and the promoter.

#### **Gene therapy in Cystic Fibrosis**

Cystic Fibrosis (CF) is caused by a single faulty gene that controls the movement of salt and water through the cells in the body. In people with Cystic Fibrosis, this gene doesn't work or only partly works meaning that the internal organs such as the lungs, liver and digestive system become clogged with thick sticky mucus which attracts infection. In turn, the body tries to fight off infection, which causes inflammation and damage to the organs.

In 1989, scientists discovered the faulty CF gene. Since then, much time and money has been spent working to create gene therapy for people with Cystic Fibrosis. If scientists can find a way of adding a healthy copy of the gene to the lungs, this will allow normal movement of salt, thus breaking the cycle of infection, inflammation and damage.

90% of people with Cystic Fibrosis die of chronic lung damage. Effective gene therapy would stop the lungs becoming damaged by correcting the fault in the lungs.

Gene therapy to the lungs is difficult as they are designed to keep things out. In people with CF, the thick sticky mucus in the lungs creates another barrier to overcome.

### **What is the Cystic Fibrosis Trust funding?**

In the early 2000's, the Cystic Fibrosis Trust brought together the UK's leading CF gene therapy teams into a single working group – the UK CF Gene Therapy Consortium. There are now over 80 scientists and clinicians in Edinburgh, London and Oxford dedicated to gene therapy for Cystic Fibrosis. It is the largest group studying CF gene therapy in the world.

**The UK CF Gene Therapy Consortium has developed a product for CF gene therapy. Detailed trials to ensure it is safe are now being carried out.**

*The CF Trust invests over £4 million each year in gene therapy research.*

### **Clinical Trials**

200 people with CF in Edinburgh and London have already been recruited to take part in a multi-dose clinical trial and will be monitored for a year to measure vital functions. This is called the Run-in.

In February 2009, a pilot trial for CF gene therapy began. 27 young adults with Cystic Fibrosis in London are being given a single dose of the gene therapy product. This will provide important information on the correct level of dose and possible toxic effects.

Once the results of the pilot study have been analysed, a further round of safety tests is necessary. At the same time, up to 100 of the people recruited for the Run-in will be chosen to take part in the multi-dose clinical trial. Doctors and scientists are looking for the most suitable candidates from the original 200 patients to take part.

If all is well in the pilot trial and as long as the safety studies and the product are passed by the regulatory authority, these patients, all over 12, will be given either the gene therapy product or a placebo in a double blind trial for a year.

**This is the first time anywhere in the world that CF gene therapy has been studied in this way.**

It is estimated that the product for the multi-dose clinical trial will cost at least £6½ million. A more precise figure will be known following the pilot trial.

If successful, a major multi-centre trial (potentially including Europe and the USA) would take place. This would be the definitive study for safety and efficiency.

If this trial was successful, a major pharmaceutical company would then develop the treatment into a product for the market and everyday clinical use. This is a best case scenario for CF gene therapy.

## **Wave 2 gene therapy**

The 80 dedicated scientists and clinicians who make up the Consortium have been working on the Wave 1 product, as described above. Although this is their focus, there have also been some interesting developments on a Wave 2 product, which uses a viral carrier, and which looks able to be given repeatedly (although a virus is good at getting to the lungs, they cannot normally be re-used as the body becomes immune). This product development is also being rigorously pursued.

## **ENDS**

### **For all media inquiries please contact:**

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- The Cystic Fibrosis Trust is the UK's only national charity dealing with all aspects of Cystic Fibrosis (CF). It funds research to treat and cure CF and aims to ensure appropriate clinical care and support for people with Cystic Fibrosis.
- Cystic Fibrosis (CF) is one of the UK's most common life-threatening inherited diseases. Cystic Fibrosis is caused by a single defective gene. As a result, the internal organs, especially the lungs and digestive system, become clogged with thick sticky mucus resulting in chronic infections and inflammation in the lungs and difficulty digesting food.
- Each week five babies are born with Cystic Fibrosis and three young people die – 90% from lung damage. Around half of the CF population can expect to live over 38 years, although improvements in treatments mean a baby born today is expected to live even longer.

- Further information can be found on our website [www.cftrust.org.uk](http://www.cftrust.org.uk). Help and advice for those affected by Cystic Fibrosis is available through our Helpline on 0300 373 1000. For further information, media should contact Gemma Foy on 0208 290 7912 or email [gfoy@cftrust.org.uk](mailto:gfoy@cftrust.org.uk)

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*Patron: HRH Princess Alexandra, the Hon. Lady Ogilvy, KG, GCVO President: Duncan Bluck CBE*  
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