

Statement from the Cystic Fibrosis Trust

Over the last decade, the Cystic Fibrosis Trust and our supporters have made a considerable investment in world leading research to determine whether gene therapy to the lungs is a possible treatment for people with Cystic Fibrosis.

In 2008, for the first time ever, a potential gene therapy for Cystic Fibrosis entered preliminary clinical trials and thirty people with Cystic Fibrosis have received a single dose of the treatment to test for safety.

The Cystic Fibrosis Trust has provided more than thirty million pounds to the Gene Therapy Consortium over the last nine years, reflecting considerable tenacity from donors, researchers and clinicians, as well as carers and patients living with Cystic Fibrosis. The potential clinical benefit of gene therapy is yet however still to be proven and there is a long way to go.

Since 2008 the Cystic Fibrosis Trust has, like many other charities, experienced a significant drop in income and the fundraising environment and resources available to the Trust remain extremely tight.

Getting both the science and the funding of this research programme right is not easy and in the current economic climate the Trust has had to reassess both its own costs and its commitments. To make this reassessment and to ensure the available resources are being used as effectively and efficiently as possible, we have asked the UK CF Gene Therapy Consortium to fully review where the research programme has got to so far.

In a more constrained funding environment the Trust's aim is to realise benefits from the investments to date in the shortest possible time frame. The research focus is now to demonstrate specific outcomes, such as testing efficacy in the lung, and preparing the gene therapy programme to be managed by a pharmaceutical partner.

We can all be proud of our work to seek a gene therapy treatment for Cystic Fibrosis. Since the 1990s the Trust has funded much work into a possible gene therapy approach to tackle the basic cause of Cystic Fibrosis in the lungs. We can reassure supporters that we are very keen to support the work of the UK CF Gene Therapy Consortium as best we can in its attempt to conclude whether gene therapy to the lungs of people with Cystic Fibrosis has got useful clinical and therapeutic benefits.

This programme of work has been an enormous commitment for many people, not least people living with Cystic Fibrosis who have been directly involved in the trials to date. We all hope that the investment and effort to date will yield some clear indicators as to whether gene therapy can become a significant component in the future treatment of Cystic Fibrosis.

We are therefore now working with the Consortium on a revised programme and we will put this on our website as soon as it is available. Whatever the outcome of

the reassessment and the revised programme, the Cystic Fibrosis Trust expects the UK Gene Therapy Consortium to remain its single largest research project.

At the same time as reassessing the programme, we are actively looking at how we can continue to fund the future commitments to the Consortium and we will be seeking support from other organisations, including the Government, medical research bodies and others, to enable the work of the UK CF Gene Therapy Consortium to continue to maximise its chances of success.

The Cystic Fibrosis Trust continues to support other research as part of the international fight against Cystic Fibrosis, works to improve standards of clinical care in the UK, and provides direct support to people living with Cystic Fibrosis and their families.

To enable the further investment to happen in the gene therapy research the Trust would of course be delighted to hear of potential new sources of funding for this work. If you feel you can help then please contact Matthew Reed, Chief Executive of the Cystic Fibrosis Trust. Thank you.

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