

CF Trust announces new fundraising drive to pay for crucial gene therapy trial

May 2011

Following our announcement in March that we were reviewing our costs and commitments to the UK CF Gene Therapy Consortium, we are pleased to confirm that a multi-dose clinical trial for CF gene therapy is planned to start in January 2012.

This is a crucial stage in what has been 10 years of development and follows successful tests on safety and toxicity over the last two years.

The plan is for over 100 people with Cystic Fibrosis in Edinburgh and London to be given either the gene therapy product, made up of the healthy gene, the gene transfer agent and the promoter or a placebo, once a month for a year. The UK CF Gene Therapy Consortium will then analyse the results to see if the patients show improvements in their lung function over this time period. If the results are positive the aim is to then find a pharmaceutical partner to take the product into a phase 3 clinical trial from 2015 onwards.

The progress is a great tribute to those in the UK CF Gene Therapy Consortium whose work has been recognised across the world, and for the thousands of families and benefactors whose huge efforts have raised the £30 million the research has cost so far.

The gene therapy programme has cost significantly more than was planned at its outset and we are still considerably short of the full investment required to ensure the multi-dose trial goes ahead. A major push is therefore underway to raise the further £6 million required to ensure the trial can take place.

This is a lot of money and, of course, there are no guarantees of outcomes in this work. But the chance of securing a multi-dose trial is a once-in-a-lifetime opportunity which we cannot miss. Without proceeding with this trial we would not know whether effective gene therapy for CF could become a reality. So, we will be making every effort to raise the money required and looking for the support of all in the CF community and beyond.

The Trust is therefore starting a special campaign to raise the full sums needed to fund the multi-dose clinical trial. Raising the funds required in the current economic climate is a huge challenge, but the Trust is confident that the CF community will do all it can over the coming months to make the trial possible.

Several new fundraising initiatives are about to start and over the coming weeks we will be letting everyone know how they can play their part. In the meantime we are asking everyone in the Cystic Fibrosis community and beyond to consider what they can do to help raise the funds required.

As this is the first such gene therapy trial in the world and has the potential to benefit people with CF across the globe we will be extending the campaign to all our network of friends and donors worldwide.

If you are able to get involved today then please contact us with details of how you might help financially or what other support you might be able to offer.

Thank you for your continued support. We will update this page with further information after Cystic Fibrosis Week.

Thank you.