INVOLVE

NHS National Institute for Health Research

Example 5: A randomised double-blind placebo controlled Phase 2B clinical trial of repeated application of gene therapy in patients with Cystic Fibrosis.[1]

About the research

Lead researcher: Professor Eric Alton, Professor of Gene Therapy and Respiratory Medicine, National Heart & Lung Institute, Imperial College.

Funder: National Institute of Health Research Efficacy and Mechanism Evaluation (EME) Programme**.**

Project aim: To assess the safety and effectiveness of gene therapy for cystic fibrosis (CF).

Type of research: Clinical trial.

Duration: 30 months – started in March 2012.

Who we spoke to

We interviewed the project manager, Tracy Higgins, at the National Heart & Lung Institute, Imperial College. Her comments are in blue below.

About the involvement

How patients influenced the research programme

Thirteen years ago, three groups in the UK – from Edinburgh, Oxford and Imperial College – came together to form The UK CF Gene Therapy Consortium. The aim was to stop duplicating research and to develop a translational programme to get gene therapy into clinical practice. At the beginning the Consortium was funded by The CF Trust.

> "When we set up the Consortium, we also set up a scientific advisory group which included two parent reps – they've been with us ever since. We gave them progress reports on an annual basis. They would ask lots of

[1] A description of some of the terms used in this example such as double blind, clinical trial and placebo can be found in the MRC Clinical Trials Unit glossary www.ctu.mrc.ac.uk/glossary.aspx

questions and fed their views into the design of the whole programme. ” **Tracy**

Patient / carer involvement prior to applying for funding

The researchers have developed a close relationship with the patient organisation over the years and as a result have got involved in giving presentations to parents' meetings all over the country. This provided a route for more informal feedback.

> “The senior academics gave lots and lots of lay presentations. The people there always asked really good questions and helped refine the details of the trial a bit further. ” **Tracy**

Impact of the early involvement

Since the aim of the consortium has always been to get to the stage of a clinical trial, the parent reps have had an influence on every aspect of the **trial design**. For example, they have contributed to the development of a placebo, and commented on the acceptability of the gene therapy product.

> “We've been asking them about the process. Patients have to be nebulised (inhaling a mist into their lungs) in a sealed room. The cubicles are tiny – so how long can they bear to sit there for? Also we know there are side-effects. The researchers were worried about some of the symptoms – would people want that once a month? But the parents were saying 'We don't mind – don't worry about it'. That helps to refine the trial design. Then we could be sure patients and parents would accept what we were asking them to do. ” **Tracy**

The overall impact of the involvement has been to ensure the research team is very **patient-focussed** in all that it does.

> “ We've spent a lot of time talking to patients and parents – but it's difficult to pinpoint exactly what difference it's made. It's made us always question everything we do in relation to what the patient would think. When we sit down in the strategy group overseeing the whole consortium – with every question that comes up – we talk about what the patient would think. There is no dividing line. It's not an add-on. That's what helps you reach your goal of getting products into clinic. ” **Tracy**

Continuation of involvement following funding

Some of the patient / parent representatives who were on the Consortium's original scientific advisory group have now joined the Trial Steering Group. They have commented on the consent forms and patient information sheets. Other people are involved more informally, for example one patient helping with recruitment to the trial using Twitter.

All the people who were involved in the developing the bid had their expenses paid, but were not paid for their time.

Lessons learnt

> “One of biggest challenges is time. Researchers are always working on grant applications up to the last minute and they don’t want to send the draft round until they’ve got the near final version. But if that’s 24hrs before you’re due to submit… You’ve got to engage earlier with patient reps when you’ve got something specific for them to comment on. ” **Tracy**

> “This study has been unique because we have been involved with the patient group for so long and as a group they have become really knowledgeable about the research. On another project, our patient rep has found it more challenging - we realised it’s because we haven’t taken enough time to increase her level of knowledge. With the CF group - their level of knowledge has become that much higher. ” **Tracy**

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References:

Alton EW, Boyd AC, Cheng SH, Cunningham S, Davies JC, Gill DR, Griesenbach U, Higgins T, Hyde SC, Innes JA, Murray GD, Porteous DJ. A randomised, double-blind, placebo-controlled phase IIB clinical trial of repeated application of gene therapy in patients with cystic fibrosis Thorax. 2013 Mar 22 [epub ahead of print]

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