European Cystic Fibrosis Society

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Cystic Fibrosis Research News

Title:

Optimising equity of access: how should we allocate slots to the most competitive trials in Cystic Fibrosis (CF)?

Lay Title:

Making sure that everyone with Cystic Fibrosis (CF) has an equal chance to take part in popular clinical trials: how should this be achieved?

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What was your research question?

We aimed to understand how places on popular clinical trials (where there is a high demand to take part but only very limited places available) should be allocated across the UK. We wanted to find out whether this should be driven by clinical need, patients' engagement/adherence or be random.

Why is this important?

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The opportunity to take part in a clinical trial can allow people with CF early access to CFTR modulator therapies, with high potential for clinical benefit. Therefore, the number of people wishing to participate can substantially exceed the number of slots available. There is currently no nationally agreed approach for how places on popular clinical trials should be allocated.

What did you do?

We developed an online survey, recruiting UK-based people with CF/their families, and those working in CF (e.g. in clinical care teams, charities, and those in the pharmaceutical industry ('pharma')). Recruitment was done through social media, newsletters and personal contacts. To find out the results of the survey, we looked at the frequencies and percentages of each response, and common themes or issues arising for questions where a response was given in a person's own words.

What did you find?

We received 203 responses. Overall, 75% favoured allocating trial places to individual clinical sites based on their patient population size. Currently, few centres have a clear way for allocating clinical trial places at their own site. At face-value, survey respondents believed that all people eligible to take part should have an equal chance of getting a place. Further questioning revealed preference for prioritisation strategies such as treatment adherence (healthcare professionals were less likely to favour this strategy). Overall, the majority of respondents would prefer to allocate trial places locally, but 80% would engage in a system of national allocation (e.g. using the UK CF Registry) if necessary.

What does this mean and reasons for caution?

Fair allocation of places on popular clinical trials does not appear to have a solution which is acceptable to all members of the UK's lay and professional CF community. We consider that this project has demonstrated insufficient support from key members of the CF community for a change to allocating trial places on a national rather than local basis. The situation relating to clinical trials and access to CFTR modulator treatment is often specific to each country. Ours was a UK-based study and therefore whilst we believe that some of the findings will be relevant to other countries, at this stage they are most relevant to the UK-based sites.

What's next?

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Our results reinforce the potential for trial access issues to impact patients' well-being. This highlights the necessity of transparency, tact and empathy in supporting the CF population without whose involvement, future clinical trials would be unable to progress.

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