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Cystic Fibrosis Research News

Title:
How representative are clinical trial cohorts of the general CF population? Implications for trial planning

Lay Title:
People with CF who take part in clinical trials do not represent the general CF population well

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What was your research question?
We wanted to 1) Identify what percentage of people with CF who receive care at our centre would be eligible to take part in a trial and 2) What are the most common reasons that mean patients are not able to take part in trials.

Why is this important?
It is important that people who take part in CF trials are representative of the general population of people with CF. This means that when drugs move from trials to clinic doctors understand how safe and effective the drugs will be for the general CF population. Additionally, understanding what percentage of the population are eligible to take part in trials will help people who run trials to understand how many people with CF are likely to be able to take part. This will help them to set realistic recruitment targets.

What did you do?
We looked at the inclusion and exclusion criteria of all the trials we were running at our centre and pooled them to identify the most common trial entry criteria. An example inclusion criterion was having a predicted FEV_1 between 40 and 90%. Example exclusion criteria were having received a transplant or having abnormal liver function tests. We went through the electronic records of people with CF aged 12+ at our centre and identified if they would be eligible to take part based on the common trial entry criteria.

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What did you find?

We looked at the records of 653 people with CF. Only 198 (31%) of people at our site would be able to take part in a trial based on our entry criteria. The most common exclusion reason was having an FEV_1 over 90% predicted. More than a third of people at our site are unlikely to be able to take part in current trials because of this. The second most common reason was that people were experiencing chest infections or colds at the time. Other reasons included having abnormal liver function tests or having other serious illnesses.

What does this mean and reasons for caution?

The people who take part in clinical trials are not very representative of the general CF population. This may make it harder to understand how safe and effective drugs are when they move from trials to clinic. Only a relatively small percentage of people with CF are able to take part in current trials which may make it hard to recruit enough people to run trials of new medicines successfully.

This trial was not a real trial. Actual trials may have slightly different criteria, so our percentages are only an estimate.

What's next?

We are working with people who design trials to highlight what a small percentage of people with CF are eligible to take part in current trials and to find solutions to allow more people to take part for example looking at ways to allow people with high lung function to take part.

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