Cystic Fibrosis Research News

Title:

Speeding up access to new drugs for Cystic Fibrosis: Considerations for clinical trial design and delivery

Authors:

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What was your research question?

How can we continue to make rapid progress developing increasingly effective and safe drug treatments for people with CF, in this landscape with recently established effective but expensive medicines? How can we be more efficient in our clinical trial design and also optimise clinical trial access for people with CF?

Why is this important?

The development of new effective drug treatments for people who have CF has accelerated over the last decade, particularly the CF transmembrane regulator (CFTR) protein modulator drugs which tackle the root cause of CF for 90% of people with CF. Whilst these drugs have shown dramatic benefits in terms of improved lung function and reduced chest exacerbations, further improvements and additional better new drugs will still be needed - for example, more effective drugs with less side effects or interactions with other drugs. People who have lung scarring will also need better antibiotic, mucus clearing, and anti-inflammatory drugs.

What did you do?

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The ECFS Strategic Planning Task Force gathered views from a broad range of patient organisations, clinical and research teams, the pharmaceutical industry, and regulatory agencies, discussing the challenges and proposing solutions. All parties agreed that more progress would depend on more efficient clinical trials using increasingly sensitive ways to prove the effectiveness of new drugs. Gaining regulatory approval for these new designs and outcome measures would be important. Approaches for people with rare CF mutations and mutations that don't respond to CFTR modulators are a priority. Global co-ordination between CF-specific clinical trial networks will enhance efficiency.

What did you find?

Future clinical trials of CFTR modulator drugs will likely need to have short (around 4 weeks) periods of comparison with placebo, followed by long open label studies to ensure long term safety and effectiveness. Comparisons with existing modulator drugs would need to be unfeasibly large and prohibitively expensive. New antibiotic, mucus clearing, or anti-inflammatory drugs may need to use chest exacerbations, rather than lung function, as the main outcome measure. More sensitive measures of lung function may also be useful. Improved access for people with CF to studies such as through trial tracker websites should be a priority.

What does this mean and reasons for caution?

Although improved treatments for people with CF are developing rapidly, there is still much work to do. People with CF and clinical care teams need to be aware there is still a requirement for more clinical trials and more progress. Even highly effective CFTR modulator drugs are unlikely to significantly reverse lung scarring, so people with CF are still going to require better drugs to help stabilise their lung disease and reduce the chance of chest exacerbations. More effective development of more new drugs from different pharmaceutical companies will also assist to reduce ultimate drug costs.

What's next?

In partnership with other CF patient organisations and trial networks we plan to develop a global strategy proposal to increase the efficiency of CF clinical trial design and delivery, including for people with rare CF mutations, aiming for improved affordable access to highly effective therapies for this serious disease.

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