

Cystic Fibrosis Research News

Title:

Development of Novel Therapeutics for all Individuals with CF (the future goes on)

Lay Title:

Best Therapeutic Approaches to Treat Everyone with CF

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What was your research question?

How can we treat every person with CF?

Why is this important?

This is very important because it is estimated that worldwide there are ~20% who are not eligible for the current CFTR modulator drugs. Moreover, it is also estimated that among the 80% who are eligible, only 14% actually have access to those drugs due to their high cost.

What did you do?

This is a review article from presentations given at the 2022 Basic Science Conference.

What did you find?

These presentations focussed on aspects of what is still missing to treat all individuals with CF. They include: 1) finding more about the mechanisms of disease to develop new drugs, i.e., to identify what is wrong so that we can fix it; and 2) the broad range of different strategies being investigated to temporarily or permanently fix the range of different mutations that can occur in the CF gene of an individual's own cells.

What does this mean and reasons for caution?



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Although very promising, these novel approaches still need more research to be carried out (and funding to support it...).

What's next?

The next steps involve continuing the research to build on the successes so far achieved and continuous funding so that we may achieve the proposed goals with no delays.

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