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Cystic Fibrosis Research News

Title:

Outcomes and endpoints reported in studies of pulmonary exacerbations in people with cystic fibrosis (CF): a systematic review

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What was your research question?

What outcomes and endpoints have been reported in studies involving treatment of pulmonary exacerbations in people with CF, and which of these directly or indirectly capture how people feel, function and/or survive?

Why is this important?

There is no consensus about which outcomes should be evaluated in studies of pulmonary exacerbations in people with cystic fibrosis (CF). Outcomes used for evaluation should be meaningful; that is, they should capture how people feel, function or survive and be acknowledged as important to people with CF. We aimed to summarise the outcomes and corresponding endpoints which have been reported in studies of pulmonary exacerbations, and to identify those which are most likely to be meaningful. Selecting meaningful outcomes for evaluation in studies is likely to improve the value of the research and minimise research waste.

What did you do?

We searched Medline, Embase and the Cochrane database until July 2020 to identify studies written in English evaluating outcomes and endpoints for pulmonary exacerbation studies in people with CF of all ages, including randomised trials, observational studies, reviews and abstracts. Registered trials proposing novel outcomes for evaluation were also included. The number of studies reporting each outcome was reported. We recorded whether each outcome reflected how the individuals feel, function or survive.

What did you find?

144 studies met the inclusion criteria. A wide range of outcomes and corresponding endpoints were reported. Death, quality of life, patient-reported outcomes, lung function, and structural lung changes were identified as outcomes that are most likely to be meaningful.

What does this mean and reasons for caution?

Researchers should be cautious when selecting outcomes in studies for pulmonary exacerbations to ensure that they capture how people feel, function or survive. Also, the outcomes should be acknowledged as being meaningful to people living with CF to ensure the value of the research/study.

What's next?

This review is a first step towards developing a consensus core outcome set; a standardized collection of outcomes that should be evaluation in all studies of pulmonary exacerbations in CF. This is likely to improve the value of the research in this field.

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