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Cystic Fibrosis Research News

Title:

Application of Multiple Event Analysis as an alternative approach to studying pulmonary exacerbations as an outcome measure

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What was your research question?

Pulmonary exacerbations (PEx) are episodes of increased respiratory symptoms that occur in people with cystic fibrosis (CF). Pulmonary exacerbations may require long hospitalizations for treatment and can lead to permanent lung damage. When testing new treatments for CF, PEx are an important outcome measure to help determine if a treatment is helping patients.

When pulmonary exacerbations are used as an outcome measure, there are a few ways to analyze them. The time between the start of a study and the first PEx can be measure (*time to first PEx*). Alternatively, all PEx that occur from the start of a study until the end can be analyzed (for example, *number or rate of PEx over time*). In this research, we used data from a previous clinical study in CF and measured PEx in both ways. We determined whether risk factors for a time to first PEx and rate of PEx over the study were the same.

Why is this important?

Pulmonary exacerbations affect quality of life and mortality in CF and are an important outcome in clinical trials. Understanding the risk factors that can contribute to PEx allows clinicians to improve treatment. Previous studies have mostly focused on risk factors that contribute to the amount of time that goes by before a PEx. However, analyses that account for all the exacerbations in a given time period could allow for the detection of more risk

factors. This approach might reduce the number of patients that need to be studied in order to detect risk factors, which could be very useful in clinical trials.

What did you do?

We analyzed data from the Early Pseudomonas Infection Control (EPIC) Observational Study which enrolled participants between 2004 and 2006 who were 12 years of age or younger and culture negative for Pseudomonas aeruginosa. We performed the analysis using the time to the first PEx, or the several PEx that occurred during the study period, and compared the factors identified by each analysis.

What did you find?

The results showed that analysis of that included all pulmonary exacerbations that occurred identified 2 more factors (gender and flu vaccination in previous year) that were associated with pulmonary exacerbations compared to analysis of time to first PEx. Taking into account the presence of other risk factors, the following were associated with higher occurrence of PExs: female gender, older age at enrolment, household cigarette smoke exposure, increased cough at the most recent encounter, having used antibiotics since the previous encounter, a positive culture for any CF organism at the most recent encounter, and having had a PEx in the last 30 days. Factors associated with lower occurrence of PExs were: weight percentile, flu vaccination and swimming.

What does this mean and reasons for caution?

The analysis of several PEx found that the risk of PEx occurrence is higher (by 17%) in female than males whereas taking flu vaccination decreases the risk by 16%. There may be other factors that we did not consider and that could affect the associations found. For instance, the positive effect of swimming may be reflective of general health or socioeconomic status.

What's next?

Future research should consider, if the timeframe is long, analyzing all available PEx events that occur during the timeframe of the study to maximize the information obtained from studies.

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