



Cystic Fibrosis Research News

Citation:

Goss CH, Rubenfeld GD, Ramsey BW, Aitken ML. Clinical trial participants compared with nonparticipants in cystic fibrosis. *Am J Respir Crit Care Med.* 2006 Jan 1;173(1):98-104.

What was your research question? (50 words maximum)

How do cystic fibrosis (CF) patients who choose to participate in clinical trials differ from those who do not choose to participate in clinical trials? Does participation in clinical trials impact clinical outcome as measured by change in lung function?

Why is this important? (100 words maximum)

Clinical trials are vital to advancing the care of patients with CF. The clinical trial is considered the gold standard for assessing the effectiveness of various therapies and interventions. Important differences may exist between patients who are eligible but not participating compared with those who participate, which limits the generalizability of the trial results.

What did you do? (100 words maximum)

Using data from the Cystic Fibrosis Foundation Registry between 1992 and 1998, we performed a cohort (group) analysis and identified three cohorts. Cohort 1 included all patients who were older than six years in 1992. Cohort 2 included the same patients as Cohort 1 minus those who died or were lost to follow up in the subsequent seven years. Cohort 3 included only patients eligible for participation in a specific trial, which was the phase 3 intermittent administration of an inhaled drug called tobramycin, and were followed in the registry in 1995 and 1996.



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What did you find? (100 words maximum)

Cohort 1 included 13,041 patients. Of those, approximately 4,300 people died or were lost to follow-up. Among the 8,735 patients in Cohort 2, 30.2% enrolled in at least one clinical trial between 1992-1998, with a 7% yearly average participation rate. Compared to non-participants, participants had more advanced disease and higher rates of bacterial infection, and were more likely to be white and have private (non-government) health insurance. There were no gender differences in participation. In the patients eligible for the tobramycin study (Cohort 3), there were no significant differences between participants and non-participants regarding lung function, bacterial infection rates, gender, age, weight, or health insurance type.

What does this mean and reasons for caution? (100 words maximum)

The overall clinical trial participation rate is very high in the CF community. Although clinical trial participants had more advanced disease at baseline, their lung function decline was lower than non-participants. This may be because people who participate in clinical trials spend more time with doctors and are closely monitored.

It is concerning that clinical trials participants are more likely to have private health insurance than non-participants. This may mean that people with different levels of education and income are not well represented in clinical trials. Efforts should be made to ensure adequate representation from different groups.

What's next? (50 words maximum)

No follow-up work was planned. The authors encouraged future investigators to continue to reevaluate how representative their research will be and ensure that



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all patients with CF have an opportunity to participate in clinical research if they are interested.