



# Cystic Fibrosis Research News

## **Citation:**

Zelnick LR, Schildcrout JS, Heagerty PJ. Likelihood-based analysis of outcome-dependent sampling designs with longitudinal data. *Stat Med.* 2018 Jun 15;37(13):2120-2133.

## **What was your research question? (50 words maximum)**

Identifying new biomarkers is important to the CF research community, and it is natural to use already collected data to decide which individuals to include in future studies. We investigated new ways of sampling (termed “outcome dependent sampling designs”) that can provide cost-effective ways to conduct biomarker substudies.

## **Why is this important? (100 words maximum)**

Using already collected information to decide which individuals to include in future studies could potentially reduce the number of individuals needed for each study. In addition, it could allow us to more efficiently detect new biomarkers that are related to CF. Studies that use existing data in this way could save researchers time, resources, and money compared with traditional study designs.

## **What did you do? (100 words maximum)**

We showed how to correctly design and analyze this type of study with math and computer simulation. We then gave an example of how outcome dependent sampling could be used to design a study with CF patient registry data.

## **What did you find? (100 words maximum)**

These special study designs and the accompanying analysis techniques can save money and resources compared to a traditionally designed study. The relative savings depend on how the biomarker and the outcome (such as lung function) are related. The savings also depend on what aspect of that relationship is most interesting to the researcher.

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

Researchers who are planning studies and want to use existing data can save resources and money by choosing carefully who to study. In addition, they need to consider how to analyze their data using these special techniques. Researchers should, however, make sure the mathematical assumptions are reasonable before using these techniques.

## **What’s next? (50 words maximum)**



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We would like to give researchers additional advice on how to design a study like this one. We might also make a computer program that would allow scientists to analyze their results more easily.