



Cystic Fibrosis Research News

Citation:

Ma, Huijuan, Limin Peng, Zhumin Zhang, and HuiChuan J. Lai. "Generalized accelerated recurrence time model for multivariate recurrent event data with missing event type." *Biometrics* 74, no. 3 (2018): 954-965.

What was your research question? (50 words maximum)

We tested statistical models of recurrent biomedical events with missing data. Using a dataset from the Cystic Fibrosis Foundation Patient Registry (CFFPR), we looked at how the recurrence times of nonmucoïd and mucoïd *Pseudomonas aeruginosa* (Pa) infections are influenced by potential risk factors in young children with cystic fibrosis (CF).

Why is this important? (100 words maximum)

Pa is a major pathogen in CF lungs that leads to chronic infections and lung function decline. It has two different types, mucoïd Pa and nonmucoïd Pa. Mucoïd Pa is more likely to be drug resistant while the nonmucoïd type is more virulent. It is of interest to investigate risk factors of the different types of Pa and their effect patterns. While the CFFPR has documented Pa types, there are around 10% of cases with missing or unknown Pa type. These missing data need to be properly handled to provide improved disease management to patients.

What did you do? (100 words maximum)

We developed statistical methods based on strategies of inverse probability weighting and estimating equation projection and applied them to the 2007 CFFPR dataset to properly handle the missing Pa types. The application to the CFFPR dataset was expected to provide statistical findings with enhanced validity compared to other statistical methods used in the past.

What did you find? (100 words maximum)

Our data analyses indicated the benefit of early CF diagnosis on delaying nonmucoïd Pa and mucoïd Pa infections and reducing recurrences. They also suggested that the beneficial effect of newborn screening on onset and recurrence of mucoïd Pa may be stronger than that on the nonmucoïd Pa. The effect of gender was found to be significant on the onset of mucoïd Pa but not on nonmucoïd Pa. Girls with CF were more likely to develop mucoïd Pa infections earlier in life than boys with CF.



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

Early disease diagnosis, particularly by newborn screening, may significantly delay the onset and recurrence of mucoid Pa in CF children.

What's next? (50 words maximum)

It would be interesting to validate the findings with a more recent CFFPR cohort with longer follow-up.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/29427311/>