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Applying a longitudinal two-part joint modeling approach to randomized controlled trial HAQ-DI outcome scores with an excess of zeroes

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HAQ-DI outcomes cores in randomized controlled trials often result into scores that reach the lower limit. For example, when patients receive medication over time their score will shift towards better scores, until reaching the best possible score (0). This results into an excess of zeroes that is caused by censoring. Several so-called two-part statistical models have been developed to deal with an excess of zeroes. These models contain two distributions; resulting into two separate regression coefficients. However, the scores of patient reported outcomes should be seen as one process. Therefore, the purpose of this study was to use the two-part joint mixed modeling approach to analyze longitudinal HAQ-DI RCT data with an excess of zeroes, and to compare the performance of these newer methods with the traditional methods. A linear mixed model and a logarithmic transformation of the linear mixed model $(\ln(x+1))$ were compared with a two-part joint binomial/normal (Tobit) mixed model and a binomial/gamma two-part joint mixed model. In order to compare the models, differences between observed and predicted values were used. Results showed that the predicted values of the linear mixed model and the log-transformed linear mixed model were similar to each other and did not predict the zeroes accurately. Of the two two-part joint models, the Tobit model showed the best predictions in comparison to the observed scores; the binomial/Gamma did not perform very well. Therefore, the use of a Tobit mixed model is advised in HAQ-DI outcome scores with an excess of zeroes.

Biography

Alette S Spriensma is currently working on her PhD at the VU University Medical Center in Amsterdam at the Department of Epidemiology and Biostatistics. Her research topic is on the use of mixed distribution models to analyze (longitudinal) left censored outcome variables.

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