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Random effect Models for Quality of Life Analysis in Oncology

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In Oncology, the Health-related Quality of Life (QoL) has become an essential criterion in clinical trials. However, the longitudinal analysis of this criterion is complex and non-standardized. Indeed, the observations are obtained through self-questionnaires (Patient-Reported Outcomes) and there are both multiple responses, repeated and ordinal ones. From a statistical standpoint, QoL is not directly measurable and is considered as a latent trait which is accessible through responses to items. To evaluate QoL in most cancer clinical trials, the QLQ-C30 questionnaire has been used. Nowadays, the statistical analysis is done on a score from the EORTC recommendations, corresponding to the average of item responses.

Longitudinal competing models are exploited such as a linear mixed model (LMM) classically used for score modelling and generalized linear mixed models (GLMM) employed for ordinal categorical data. The latter model family builds on the Item Response Theory (IRT) and allows considering raw data (item responses). Regarding the longitudinal analysis, the IRT models are proposed as an alternative to LMM and extended to take into account the clinical covariates and data characteristics.

These presented models were compared through the analysis of a dataset from a clinical trial and then a simulation study was performed. The IRT model for polytomous data is quite complex and fastidious to estimate the regression coefficients and to predict the random effects. Finally, a less complex approach of linearization advanced by Schall in 1991 is proposed to estimate these GLMM in order to complete the simulation study.