
Novel Statistical Methods for Patient-Reported Outcome Measures

Organizer and Chair: Lisa Lix (University of Manitoba)

MYRIAM BLANCHIN, University of Nantes

Assessing Change in Longitudinal PROMs Studies: Using IRT Polytomous Models to Detect and Adjust for Response Shift

Assessing change in longitudinal PROMS studies can be a challenge. Patients' standards, values, or conceptualization of what the PROMs are intended to measure can change reflecting patient adaptation to the disease. As a consequence of this "response shift", observed patient's evolutions may thus not properly reflect true perceived health changes. Item Response Theory models relies on an underlying response model relating parameters characterizing the PROMS instrument to a latent parameter interpreted as the true individual value of the concept measured by the PROMs. A change in parameters related to the PROMS instrument might reflect response shift. We will describe how Item Response Theory models could be used to detect response shift and to measure true perceived health changes.

ELEANOR PULLENAYEGUM, University of Toronto

Handling Health Utility Data: the Example of the EQ-5D

Health utility is a patient-reported outcome that is key to economic evaluations. The EQ-5D is a popular instrument that captures health utilities through a short questionnaire. EQ-5D health utilities pose some interesting challenges. Health utilities measured using the EQ-5D have an unusual distribution that is difficult to model parametrically. Moreover, the scoring algorithm that is used to convert questionnaire responses to health utilities does so subject to prediction errors; the resulting EQ-5D health utilities are subject to sources of uncertainty that are mistakenly ignored in practice. This talk will discuss how to address these challenges.

TOLULOPE SAJOBI, University of Calgary

On Discrimination in Multivariate Non-Normal Repeated Measures Data

Multivariate repeated measures data arise in studies in which two or more groups of individuals are repeatedly measured on several outcome variables. Repeated measures discriminant analysis models, which assume structured means and/or covariances, have been developed for predicting group membership in repeated measures data. However, these procedures may be sensitive to departures from the multivariate normality assumption. We propose repeated measures discriminant analysis procedures, which assume parsimonious covariance structures, based on maximum trimmed likelihood estimation methods for predicting group membership in multivariate non-normal repeated measures data. Data from a longitudinal health-related quality of life study are used to demonstrate the implementation of our methods. Repeated measured discriminant analysis models can be used to classify new observations into population groups.