Modelling Patient-Reported Outcomes: A case-study of COPD patients

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The World Health Organization defines health as a complete physical, mental, and social well-being and not merely the absence of disease or infirmity. In this sense, patient-reported outcomes (PRO) are becoming primary outcome measurements in observational and experimental studies, as they capture evidence of patients' status that is difficult to evaluate physically, such as pain, quality of life or, satisfaction with care. PRO are usually obtained using item-based questionnaires, assigning scores to each item response and summing the scores across a group of items to create overall scores, usually called dimensions, which decompose the health aspect they are evaluating.

The binomial distribution is the most common candidate when modeling discrete and bounded outcomes, such as PRO dimensions. However, the fact that questionnaire items are answered by the same individuals sets up a correlation structure in the ordinal responses that constitute the final score, which increases the variability beyond the mean-variance structure of the binomial distribution, a property called overdispersion. In fact, PRO scores tend to have skewed distributions, often showing U, J or J-inverse shapes.

In this talk, we are going to present the main contributions of our research group in the field of PRO modeling, from the proposal of an optimal probability distribution to a joint model for the analysis of longitudinal PRO and survival data. Additionally, we will present the most clinically significant results obtained from applying the developed models to a health-related quality of life study in patients with Chronic Obstructive Pulmonary Disease (COPD).

Keywords: PRO, COPD, beta-binomial distribution, longitudinal data, survival, joint modeling.