Short courses

Joint Models under the Bayesian approach by Dimitris Rizopoulos

Abstract: In follow-up studies, different types of outcomes are typically collected for each subject. These include longitudinally measured responses (e.g., biomarkers), and the time until an event of interest occurs (e.g., death, dropout). Often these outcomes are separately analyzed, but on many occasions, it is of scientific interest to study their association. This type of research question has given rise in the class of joint models for longitudinal and time-to-event data. These models constitute an attractive paradigm for the analysis of follow-up data that is mainly applicable in two settings: First, when the focus is on a survival outcome, and we wish to account for the effect of endogenous time-dependent covariates measured with error. Second, when the focus is on the longitudinal outcome, and we want to correct for non-random dropout.

This full-day course is aimed at applied researchers and graduate students and will provide a comprehensive introduction to this modeling framework. We will explain when these models should be used in practice, which are the key assumptions behind them, and how they can be utilized to extract relevant information from the data. Emphasis is given on applications, and after the end of the course, participants will be able to define appropriate joint models to answer their questions of interest.

Necessary background for the course: This course assumes knowledge of basic statistical concepts, such as standard statistical inference using maximum likelihood and regression models. Also, a basic knowledge of R would be beneficial but is not required. Participants are required to bring their laptop with the battery fully charged. Before the course instructions will be sent for installing the required software.

Target audience: Professional statisticians working in applied environments where hierarchical modeling and survival analysis are key issues; this would include biostatisticians working in the pharmaceutical industry, regulatory agencies, or academic centers.

Time-schedule:

- Introduction & Motivation: Which type of research questions requires joint modeling

- Review of Mixed Models: Definitions, linear mixed model estimation, how to fit in R, Missing Data

- Review of Relative Risk Models: Definitions, Cox model, estimation, time-dependent covariates, extended Cox model

- The Basic Joint Model: Definition of joint models, assumptions, estimation, comparison with time-dependent Cox model, connection with missing data

- Extensions of the Basic Joint Model: Functional form, Multiple longitudinal outcomes, Competing Risks, Multi-State Models, Recurrent Events

- Special topics: Dynamic predictions for the survival and longitudinal outcomes

Learning Objectives: After this course, participants should be able to identify settings in which a joint modeling approach is required. From the course, it will become clear which joint models can be used depending on the actual research questions to be answered, and which model-building strategies are currently available. Further, participants should be able to construct and fit an appropriate joint model, correctly interpret the obtained results, and extract additional useful information (e.g., plots) that can help communicate the results better.

The course will be explanatory rather than mathematically rigorous. Therefore emphasis is given in sufficient detail for participants to obtain a clear view of the different joint modeling approaches and how they should be used in practice. To this end, we first motivate joint modeling using real datasets and then illustrate in detail the virtues and drawbacks of each of the presented joint modeling approaches. For completeness and throughout the course, references are provided to material with more technical information.

Presenter Background: Dimitris Rizopoulos is a Professor in Biostatistics at the Erasmus University Medical Center. He received an M.Sc. in statistics (2003) from the Athens University of Economics and Business, and a Ph.D. in Biostatistics (2008) from the Katholieke Universiteit Leuven. Dr. Rizopoulos wrote his dissertation, as well as a number of methodological and applied articles on various aspects of models for survival and longitudinal data analysis, and he is the author of a recent book on the topic of joint models for longitudinal and time-to-event data. He has also written two freely available packages to fit this type of models in R under maximum likelihood (i.e., package JM) and the Bayesian approach (i.e., package JMbayes). He currently serves as co-Editor for Biostatistics.