Clinical Prediction Models: A Practical Approach to Development, Validation, and Updating

This book aims to provide insight and practical illustrations on how modern statistical concepts and regression methods can be applied in medical prediction problems, including diagnostic and prognostic outcomes. Many advances have been made in statistical approaches towards outcome prediction, but these innovations are insufficiently applied in medical research. Old-fashioned, data hungry methods are often used in data sets of limited size, validation of predictions is not done or only in a simplistic way, and updating of already available models is not considered. A sensible strategy is needed for model development, validation, and updating, such that prediction models can better support medical practice.

Clinical Prediction Models presents a practical checklist with 7 steps that need to be considered for development of a valid prediction model. These include preliminary considerations such as dealing with missing values; coding of predictors; selection of main effects and interactions for a multivariable model; estimation of model parameters with shrinkage methods and incorporation of external data; evaluation of performance and clinical usefulness; internal validation; and presentation format. The steps are illustrated with many small case studies and R computer code, with data sets made available in the public domain [http://www.clinicalpredictionmodels.org/]. The book further focuses on generalizability of prediction models, including patterns of invalidity that may be encountered in new settings, approaches to modifying and extending a model, and comparisons of centers after case-mix adjustment by a prediction model.

The text is primarily intended for epidemiologists and applied biostatisticians. It can be used as a textbook for a graduate course on predictive modeling in diagnosis and prognosis. It is beneficial if readers are familiar with common statistical models in medicine: linear regression, logistic regression, and Cox regression. The book is practical in nature. But it also provides a philosophical perspective on data analysis in medicine that goes beyond predictive modeling. In this era of evidence-based medicine, randomized clinical trials are the basis for assessment of treatment efficacy. Prediction models are key to individualizing diagnostic and treatment decision-making.

Ewout Steyerberg (1967) is Professor of Medical Decision Making, in particular prognostic modeling, at Erasmus MC – University Medical Center Rotterdam, the Netherlands. His work on prediction models was stimulated by various research grants including a fellowship from the Royal Netherlands Academy of Arts and Sciences, and by sabbaticals at Duke University and Harvard University. He has published over 250 peer-reviewed scientific articles in collaboration with many medical researchers, both in methodological and applied medical journals.