Yale SCHOOL OF PUBLIC HEALTH

Biostatistics

The Predictive Individual Effect for Survival Data: A Patient-Oriented Summary Measure for Treatment Benefit

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12:00 Noon Eastern time, Tuesday, November 8, 2022
47 College Street, Room 106 A&B

ABSTRACT

Clinical prediction models are becoming more and more popular over recent years in healthcare. Predictive modeling is a widely used clinical trials application to extract useful information from clinical trial datasets, trends, and associations in large clinical trial datasets with many variables for better decision making ultimately leading to more accurate clinical research results. These models are analytical power houses of increasing value for practicing personalized, patient-centered medicine, providing both patients and their clinicians with individualized information on prognosis or response to therapy. Such learning is useful for possible adaptive changes in trial parameters to optimize clinical trial operation and patient benefit.

The call for patient-focused drug development is loud and clear, as expressed in the 21st Century Cures Act and in recent guidelines and initiatives of regulatory agencies. Among the factors contributing to modernized drug development and improved healthcare, activities are easily interpretable measures of clinical benefit. This is obvious for survival data if the proportional hazards assumption is questionable, for example for immuno-oncology trials. Special care is needed for cancer trials with time-to-event endpoints if the proportional hazards assumption is questionable. In addition to hypothesis testing and estimation of hazard ratios and median survival times, we propose the predictive individual effect as a more tangible measure of clinical benefit. It uses the data from the actual trial to quantify a new patient's potential survival gain, that is, the difference between the patient's predicted survival times under the test and control treatment. The predictive individual effect has a clear and simple interpretation. The usefulness of the new measure is illustrated with four examples from recent Oncology trials.