STATISTICAL
SIGNIFICANCE

Can biomarkers be combined to guide treatment?

[Single-index methods for estimation and evaluation of marker-guided treatment rules based on multivariate marker panels]

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Imagine people walking around with microchips under their skin, containing all the vital information, including the entire genome. Then, if someone got into an accident or developed a rapidly progressing disease, the medical team could simply read that information of their chip and promptly choose the treatment that is best for the particular person. That's how we hope to see the future medicine.

Personalized medicine has recently become an actively discussed topic among medical researchers for both its potentially enormous practical implications and the increasing availability of large amounts of patient data, such as genome-wide scans, routinely collected during clinical trials. The ultimate goal of individualized or guided treatment is to prescribe each patient with a therapeutic option that leads to the largest expected benefit for that individual. As a result, targeted treatment regimens could be applied systematically in medical practice and lead to both better outcomes for individuals and for the overall population of patients.

Using data from large randomized clinical trials, statisticians can identify biomarkers that predict treatment response and develop marker-guided treatment rules that target therapy at the responsive patients. For example, while some cancer patients benefit greatly from aggressive chemotherapies, others might be harmed by the potentially severe side effects. Statistical models that predict which patients are more likely to benefit from a particular therapy based on their individual characteristics, such as blood markers, tumor type, or genetic markers, could be hence used to guide treatment choice on individual level. However, the role of statistics extends further than that. It is crucial that statisticians also critically and honestly evaluate the developed marker-guided decision rules with respect to their impact on the target population of patients. Such evaluations then provide an important basis for policymakers to make informed decisions about changes in clinical practice and help avoiding costly implementation of policies and guidelines that do not lead to a scientifically meaningful improvement in the population outcome of interest.