Validating predictors of therapeutic success: a causal inference approach

ABSTRACT

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In personalized medicine medical decisions, practices, and/or products are tailored to the individual patient. The idea is to provide the right patient with the right drug at the right dose at the right time. However, our current lack of ability to predict an individual patient's treatment success for most diseases and conditions, is a major challenge to achieve the goal of personalized medicine. In the present work, we argue that many of the techniques often used to evaluate predictors of therapeutic success may not be able to answer the relevant scientific questions and we propose a new validation strategy based on causal inference. The methodology is illustrated using data from a clinical trial in opiate/heroin addiction. The user-friendly R library EffectTreat is provided to carry out the necessary calculations.