

Uncertainty quantification of treatment regime in precision medicine by confidence distributions

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Personalized decision rule in precision medicine can be viewed as a discrete parameter, for which theoretical development for statistical inference is lagged behind. In this talk, we propose a new way to quantify the estimation uncertainty in a personalized decision based on recent developments of confidence distribution (CD). Specifically, in a parametric regression model setup, suppose the binary decision for treatment versus control for an individual x_a is determined by a linear decision rule $D_a = \mathbf{1}(x_a\beta > x_a\gamma)$, where β and γ are unknown regression coefficients in models for potential outcomes of treatment and control, respectively. The data-driven decision $\widehat{D}_a = \mathbf{1}(x_a\hat{\beta} > x_a\hat{\gamma})$ relies on the estimates $\hat{\beta}$ and $\hat{\gamma}$, which in turn introduces uncertainty on the decision. In this talk, we propose to find a CD for $\eta_a = x_a\beta - x_a\gamma$ and compute a *confidence measure* of $\{D_a = 1\} = \{\eta_a > 0\}$. This measure has a value between 0 and 1, and provides a frequency-based assessment on how reliable our decision is. For example, if the confidence measure of the decision $\{D_a = 1\}$ is 63%, then we know that, out of 100 patients who are the same as patient x_a , 63 will benefit to have the treatment and 38 will be better off to be in the control group. This new confidence measure is inline with classical assessments of sensitivity and specificity; but different from the classical assessments, this measure can be directly computed from the observed data without the need to know the truth whether $\{D_a = 1\}$ or $\{D_a = 0\}$. Utility of this new measure will be illustrated in an application to design adaptive clinical trials.

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