DEVELOPING INDIVIDUALIZED EFFICACY MEASURES FOR CLINICAL TRIALS

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Clinical trials often use efficacy measures that are summaries of many disease features. These summaries account for patient differences by combining into one measure the many ways that the disease may affect the individual. We hypothesize that power to could be increased if we use a different summary for each patient. This individualized summary, based on the patients baseline values might be more sensitive to treatment effects and would allow smaller clinical trials.

We assume that efficacy is measured by a vector of quantitative patient characteristics. We seek to replace the sum of these characteristics by a weighted sum were the weights are functions of the patients baseline values. Historical data are used to find a linear function which maps each patients vector of patient baseline variables into a vector of weights, where the weighted sum of outcome measures will maximize the effect size. More formally, suppose that bi is the p-vector of baseline values for patient i and f_i is the p- vector of outcomes. Using historical data we find a constant matrix A, such that $b_i'A\ f_i$ has the greatest effect size.

There is a closed form solution to this problem. These methods are applied to data in psychology and neurology and is evaluated using cross validation.