

On use of desirability functions to measure patients' disease improvement in a longitudinal study

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Abstract

Multiple outcomes are frequently used to assess chronic disease progression. We use desirability functions to provide an overall improvement score that incorporates multiple outcome measures, which may have different impact levels on disease progression. In some clinical trials, judgments on disease improvement can be subjective. To minimize the subjectivity and biases, we propose a data-driven approach to estimate the desirability function shapes and weights based on a given gold standard. Our method provides each patient with a meaningful overall improvement score that facilitates comparison and clinical interpretation. We also extend the methodology in a novel way to monitor patients' disease progression when there are multiple time points and illustrate our method using a longitudinal data set from a randomized two-arm clinical trial for scleroderma patients.

Keywords: desirability function, longitudinal data, multiple outcome, regression, scleroderma