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Is the difference in means always a good measure for an effect?

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When comparing a medical treatment with placebo it is usual to apply a two-sample t-test. n1 patients are given treatment and n2 patients are given placebo. The standard assumptions for using a two-sample t-test are assumed. It is also assumed that large response values in the treatment group are desirable. Usually H0: "The distribution means are equal" is tested against H1: "The distribution means are different". The distribution mean difference, which is to be estimated, is used as a measure of how good the medical treatment is compared to placebo. This measure has a deficiency. It is compatible with the situation that n1 and n2 are so large that the test is statistically significant while large distribution standard deviations cause so large overlap of the data in the two groups that it is meaningless to denote the result as clinically significant. We propose the supplemental measure P(X1>X2), where X1 and X2 is respectively the response of a randomly selected patient given the treatment and the response of a randomly selected patient given placebo. This measure can be interpreted as an approximate fraction of patients in the population in question who would respond better to treatment than placebo. A formula for P(X1>X2), which is a function of the unknown distribution means and the unknown distribution standard deviations, is derived. Based on this formula confidence intervals for P(X1>X2), with specified confidence level, are constructed based on parametric as well as non-parametric bootstrap, where the specified confidence level is ascertained by double bootstrap.

Keywords

Two-sample t-test, Measures for an effect, Bootstrap confidence intervals

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