Statistical Inference for Seamless Phase II/III Clinical Trials

Ken-Ning Hsu[1]

[1] PharmaEssentia Corp.

Abstract

Seamless phase II/III trials have been advocated following the US Food and Drug Administration's Critical Path Initiative lunched in 2004. Statistical methods have been proposed to address the treatment selection (phase II portion) and final test (phase III portion) issues. We compare two popular methods of Shun et al. (2008) and Friede et al. (2011) in terms of the familywise error rate and power as a function of interim analysis information time and the correlation between the short-term surrogate endpoint, on which the treatment selection is based, and the long-term primary endpoint, on which the final test is focused.

Keyword: Familywise error rate, exact distribution, treatment selection, combination of p-values, select the winner design