中央研究院統計科學研究所

學 術 演 講

講 題：Statistical Principles for Platform Trials

演 講 人：Prof. Xingping Cui

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Riverside, California, USA

時 間： 2024-06-12(Wed.) 15:50~16:30

地 點：Auditorium, B1F, Institute of Statistical Science；The tea reception will be held at 15:40.

備 註：Online live streaming through Cisco Webex will be available.

Abstract

While within a clinical study there may be multiple doses and endpoints, across different studies each study will result in either an approval or a lack of approval of the drug compound studied. The False Approval Rate (FAR) is the proportion of drug compounds that lack efficacy incorrectly approved by regulators.

While Tukey's (1953) Error Rate Familywise (ERFw) is meant to be applied within a clinical study, Tukey's (1953) Error Rate per Family (ERpF), defined alongside ERFw, is meant to be applied across studies. We show that controlling Error Rate Familywise (ERFw) within a clinical study at 5% in turn controls Error Rate per Family (ERpF) across studies at 5-per-100, regardless of whether the studies are correlated or not. Further, we show that ongoing regulatory practice, the additive multiplicity adjustment method of controlling ERpF, is controlling FAR exactly (not conservatively) at 5-per-100 (even for Platform trials). In contrast, we will show that if a regulatory agency chooses to control the False Discovery Rate (FDR) across studies at 5% instead, then this change in policy from ERpF control to FDR control will result in incorrectly approving drug compounds that lack efficacy at a rate higher than 5-per-100.

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