

Proof of Mechanism and Proof of Concept Clinical Trials

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Abstract: In typical clinical development programs for new treatment of chronic diseases, Phase I trials recruit healthy volunteers. Hence clinical efficacy cannot be observed in Phase I. The first Phase II clinical trial is known as the Proof of Concept (PoC) trial and this is to compare the test drug against a placebo control in patients with the target disease. However, in Phase I, biomarkers based on mechanism of action can be observed. Therefore some of the Phase I clinical trials are considered as the Proof of Mechanism (PoM) trials. There are huge difficulties and challenges in design and analysis of PoM and PoC clinical trials. This presentation proposes a communication tool to help statisticians in communicating various risks with non-statisticians regarding risks.