

# DSS Statistics Seminar

June 4, 2020, 2:00 p.m.

<https://meet.google.com/pqk-fbgh-ftz>

## Key features in clinical trials design

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The properly designed randomized controlled trial is recognized as providing the highest level of evidence in determining guidelines for therapeutic practice. However, numerous pitfalls in trial design may occur, which, if succumbed to, may seriously impair the reliability of or confidence in the results. In this context, key features have been examined to determine whether a trial's design is "up to scratch." These fundamentals include: selection of patients, treatments, and outcomes; randomization methods; appropriate blinding; and determination of trial size. Of course, one first has to propose a relevant clinical question for which true equipoise exists. The focus is on major, pivotal (phase III) trials that are intended to directly influence clinical practice. Randomized controlled trials are the cornerstone of clinical guidelines; however, their design and interpretation may be complex and nuanced. Specifically, the intention is to provide guidance on how to design and interpret non inferiority trials and factorial trials and finally, due to the growing interest, particular attention is paid to adaptive sample size re-estimation and enrichment strategies, with guidance on when and how they may be used. All of these issues are showed by experiences from actual real trials, demonstrating the real world implications of trial design decisions.



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