ESR Researcher Project: Non-technical Summary

"Interval estimation for dose-finding studies"

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Dose-finding trials are clinical studies with the objective of determining the optimal dose of a drug. The objective of these studies is to determine the dose with highest potential for efficacy in the patient population that still meets safety criteria or the toxicity threshold.

Two typical analytical approaches for dose finding are: multiple comparison procedures and modelling techniques. These approaches are heavily dependent on the model assumptions and do not give robust estimates. The first objective of this project was to explore different statistical methods for characterizing the dose-response relationship and for identifying the optimum dose that overcome the shortcoming of the historical approaches. A review article elaborating the pros and cons of some of the new dose-finding approaches has been submitted to the Biometrical Journal and is currently under review.

The calculations of confidence intervals for the optimal doses have not been thoroughly investigated in literature. Currently the ESR is focused on devising an estimation method for the optimal dose in a clinical trial based on modelling approach and then comparing it with the existing estimation procedures. She also aims to extend the estimation problem so that it can provide solutions to the confidence interval problem stated above. This procedure will not only help clinicians in getting an accurate estimate of the optimal dose but also give an idea on the range of the optimal dose that is quite desirable in many situations.

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