

Tim Holland-Letz, Abteilung Biostatistik, Deutsches Krebsforschungszentrum DKFZ

Optimal experimental design in preclinical dose-response studies for single compounds and combination treatments

In practical dose response trials, especially in the cell culture context, experimental designs to establish functional relationships between dose and effect of a substance are generally chosen based on simple rules of thumb. These designs are generally reasonably effective, but not optimal.

In this talk we give an overview over the statistical optimal design approach in this context. We show how the optimal designs can be constructed, covering both all-purpose designs (D-optimality) and more specific designs (c-optimality). We discuss optimal designs under constraints, and introduce a graphical representation. Next, we show how these concepts can be extended to trials aiming to estimate the interaction between two different substances when given simultaneously.

Finally, we also present an R-Shiny application which allows construction of optimal designs in most of these contexts with a minimum of theoretical knowledge.