

## POSTER SESSION 3: Clinical trial design

### P3.1

#### Criteria for futility and efficacy evaluation in interim analyses and final evaluation of phase II-trials

Annette Kopp-Schneider, Manuel Wiesenfarth, Ruth Witt, Olaf Witt

*German Cancer Research Center (DKFZ), Heidelberg, Germany*

A phase II trial is typically a small-scale study to determine whether an experimental treatment should continue further clinical evaluation. In this setting, interim analyses are commonly performed to allow for early stopping for futility and/or efficacy. The use of Bayesian posterior probability as decision rule for early stopping and for final analysis has been suggested, especially in the context of biomarker-targeted therapies with small numbers of patients. In comparison to traditional hypothesis testing-based approaches the advantage is the flexibility with respect to number and timing of interim analyses as well as the final number of patients included in the study. Using a Bayesian hierarchical model, borrowing of information across similar study arms is possible.

The INFORM2 phase I/II trial series addresses individualized therapy for relapsed malignancies in childhood using next-generation diagnostics. The trials have a dichotomous endpoint and will include interim futility and/or efficacy evaluations. They are one-arm trials or have several arms run in parallel. Sample size is restricted by recruitment rate and duration and hence identical evaluation criteria for interim and final analyses will be used. We will show a workflow for planning of the trials and show the impact of the choice of the Bayesian model and the prior distributions for decision making.