

## Using value of information methodology to determine the sample size for a randomized clinical trial from an industry perspective

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**Objectives:** To illustrate, with the use of an example, the application of value of information (VOI) methodology for determining optimal sample size in the design of a randomized clinical trial (RCT) from an industry perspective.

**Methods:** Using a societal perspective VOI methodology has been used previously to determine the sample size which maximizes the difference between the cost of an RCT and the value of the information it provides. From a societal perspective the value of the information relates to the reduction in the expected opportunity loss provided by the trial data. The cost of the trial has an opportunity in addition to a financial component. From an industry perspective the value of the information relates to the probability of regulatory approval and the affect this has on profits. The costs of the trial are solely financial. To determine optimal sample size from an industry perspective one must specify the profit per prescription, the incidence, the time horizon and the relationship between the strength of the evidence and the probability of approval.

**Results:** The methodology is applied to a specific example. It is demonstrated that for highly profitable trials (*i.e.* value greatly exceeds cost) the optimality is very robust to the specification of the profit per prescription, the incidence, the time horizon and the relationship between the strength of the evidence and the probability of approval. Robustness diminishes with the profitability of the trial.

**Conclusions:** VOI methodology can be used to provide optimal robust sample size determinations for industry based RCTs.