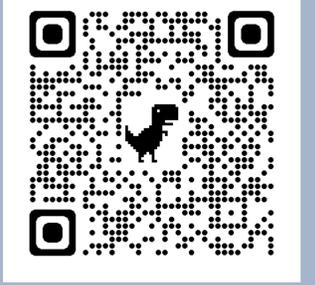


# Delivering Value for Money in Clinical Trials: Value-Adaptive Designs for Efficient Delivery of Publicly Funded Trials



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## 1 Value-adaptive designs

- Aim to deliver evidence-based, **value for money research** for the NHS.
- Involve **adaptive** data collection processes.
- Consider the **cost-effectiveness** of the research process, accounting for estimated effectiveness, its precision and the cost of carrying out the clinical trial.

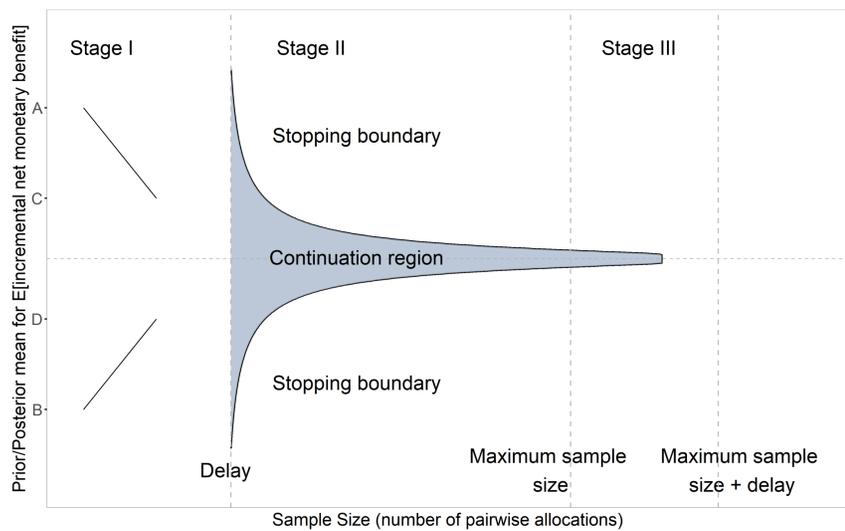


Figure 1: Value-based sequential two-arm design with adaptive stopping

## 2 Methods

- Engaged with stakeholders from across the NIHR on the **potential use and implementation** of value-adaptive methods in NIHR research.
- Applied the value-based sequential design with adaptive stopping to **two retrospective case studies**.

|   | CACTUS case study<br>(% increase over original trial) | HERO case study<br>(% increase over original trial) |
|---|---|---|
| <b>Expected sample size (maximum sample size)</b>                         |   |   |
| Original trial  | 95  | 124   |
| Value-based one-stage   | 132 (+39%)  | 177 (+43%)  |
| Value-based sequential  | 100 (+5.3%)   | 174 (+40%)  |
| <b>Expected cost associated with conducting the proposed trial design</b> |   |   |
| Original trial  | £1.22m  | £0.84m  |
| Value-based one-stage   | £1.39m (+14%)   | £0.92m (+11%)                                       |
| Value-based sequential  | £1.24m (+1.6%)  | £0.92m (+10%)                                       |
| <b>Expected net monetary benefit</b>                                      |   |   |
| Original trial  | £3.54m  | £52.0m  |
| Value-based one-stage   | £3.60m (+1.7%)  | £52.0m (+0.01%)                                     |
| Value-based sequential  | £3.85m (+8.8%)  | £52.1m (+0.19%)                                     |

Table 1: Summary of results from case studies using a value-adaptive design

Value-adaptive designs have the potential to deliver **cost-effective** and **innovative** studies that give **robust** evidence to inform practice and policy

## 3 Opportunities



More effective and cost-effective treatments to the NHS sooner.



Inform the choice of which research to pursue to maximise expected health economic benefit.



Facilitate more trials for a fixed budget.



Complement existing approaches to inform optimal trial design.

## 4 Challenges



Appropriate computing and management processes required e.g. at interim analyses.



Additional support for clinicians interpreting and implementing results.



Flexibility required in planned budgets.



Addressing the perceived learning curve to implement methods.

## 5 Discussion

With increased experience and application of value-adaptive designs there is great promise for **more efficient** publicly funded research. Further work is required to address identified challenges.