Practical help for specifying the target difference in sample size calculations for RCTs: the DELTA² five-stage study, including a workshop

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†In memoriam

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Plain English summary

The DELTA^2 five-stage study, including a workshop

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Plain English summary

This Difference ELicitation in TriAls[2] (DELTA[2]) advice and recommendations document aims to help researchers choose the 'target difference' in a type of research study called a randomised controlled trial. The number of people needed to be involved in a study – the sample size – is usually based on a calculation aimed to ensure that the difference in benefit between treatments is likely to be detected. The calculation also accounts for the risk of a false-positive finding. No more patients than necessary should be involved.

Choosing a 'target difference' is an important step in calculating the sample size. The target difference is defined as the amount of difference in the participants' response to the treatments that we wish to detect. It is probably the most important piece of information used in the sample size calculation.

How we decide what the target difference should be depends on various factors. One key decision to make is how we should measure the benefits that treatments offer. For example, if we are evaluating a treatment for high blood pressure, the obvious thing to focus on would be blood pressure. We could then proceed to consider what an important difference in blood pressure between treatments would be, based on experts' views or evidence from previous research studies.

This document seeks to provide assistance to researchers on how to choose the target difference when designing a trial. It also provides advice to help them clearly present what was done and why, when writing up the study proposal or reporting the study's findings. The document is also intended to be read by those who decide whether or not a proposed study should be funded.

Clarifying a study's aim and getting a sensible sample size is important. It can affect not only those involved in the study, but also future patients who will receive treatment.

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This report

This issue of the Health Technology Assessment journal series contains a project commissioned by the MRC–NIHR Methodology Research Programme (MRP). MRP aims to improve efficiency, quality and impact across the entire spectrum of biomedical and health-related research. In addition to the MRC and NIHR funding partners, MRP takes into account the needs of other stakeholders including the devolved administrations, industry R&D, and regulatory/advisory agencies and other public bodies. MRP supports investigator-led methodology research from across the UK that maximises benefits for researchers, patients and the general population – improving the methods available to ensure health research, decisions and policy are built on the best possible evidence.

To improve availability and uptake of methodological innovation, MRC and NIHR jointly supported a series of workshops to develop guidance in specified areas of methodological controversy or uncertainty (Methodology State-of-the-Art Workshop Programme). Workshops were commissioned by open calls for applications led by UK-based researchers. Workshop outputs are incorporated into this report, and MRC and NIHR endorse the methodological recommendations as state-of-the-art guidance at time of publication.

The authors have been wholly responsible for all data collection, analysis and interpretation, and for writing up their work. The HTA editors and publisher have tried to ensure the accuracy of the authors' report and would like to thank the reviewers for their constructive comments on the draft document. However, they do not accept liability for damages or losses arising from material published in this report.

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