Health Technology Assessment Programme

NHS National Institute for Health Research

HTA no 15/162

Intervention to reduce challenging behaviour and improve quality of life of young children with moderate to profound learning disabilities

Introduction

The aim of the HTA Programme is to ensure that high quality research information on the effectiveness, costs and broader impact of health technology is produced in the most efficient way for those who use, manage, provide care in or develop policy for the NHS. Topics for research are identified and prioritised to meet the needs of the NHS. Health technology assessment forms a substantial portfolio of work within the National Institute for Health Research and each year about fifty new studies are commissioned to help answer questions of direct importance to the NHS. The studies include both primary research and evidence synthesis.

Research Question:

What is the clinical and cost-effectiveness of an intervention to reduce challenging behaviour and improve quality of life of young children with moderate, severe or profound learning disabilities.

1. **Intervention:** A psychosocial intervention tailored to the patient group to reduce challenging behaviour and increase quality of life. The intervention should be acceptable to children's service commissioners, providers and families. All components of the intervention should be clearly described including their setting, format, and who would deliver them.
2. **Patient group:** Young children (for example, aged 3 – 5 years) with moderate, severe or profound learning disability and comorbid challenging behaviour and their parent/carer – exact inclusion criteria to be defined and justified by applicants. Consideration should be given to any comorbid conditions.
3. **Setting:** To be defined and justified by applicants.
4. **Control:** Usual care - to be defined by applicants. Issues of contamination between usual care and the intervention should be addressed. Applicants may wish to consider an attention control.
5. **Study design:** A randomised controlled trial (RCT) with an internal pilot RCT, to test recruitment, with clear progression criteria to the full trial. Applicants should describe any preliminary work needed to adapt the chosen intervention for use in the trial. If appropriate, a process evaluation should be carried out.
6. **Important outcomes:** Challenging behaviour; health related quality of life.

   Other outcomes: Inclusion and/or participation; parenting outcomes; patient, family and carer satisfaction; process indicators; adverse effects, cost-effectiveness.
7. **Minimum duration of follow-up:** One year to ascertain whether any short-term benefits are maintained.

NHS decision problem to be addressed by this research:

Children with learning disabilities are at greatly increased risk of challenging behaviours such as aggressive behaviour, destructive, disruptive and self-injurious behaviour compared to children without learning disabilities, but are less likely to receive intervention. Public policy supports early intervention as a strategy for resolving problems quickly and preventing long-term poor outcomes. While challenging behaviour is the product of a complex interaction between biological, psychological, social and environmental factors, there is evidence that some of the key factors causing and/or maintaining challenging behaviour are amenable to change, and that change in these factors can be associated with reductions in challenging behaviour. An intervention tailored to young children with learning disabilities and comorbid challenging behaviour therefore has the potential to significantly enhance the children's quality of life and that of their family members or carers.

The Department of Health has recognised that children with learning disabilities and challenging behaviour need the right support early in childhood. As such they have funded the Challenging Behaviour Foundation to create a vision of what the support should look like. According to the recent NICE clinical guideline, psychosocial interventions are the most commonly reported forms of intervention cited for this patient group including behavioural interventions based on functional assessment, and various approaches to parent training. The Challenging Behaviour Foundation has advocated the use of positive behaviour support for delivering a range of evidence-based supports to increase quality of life and reduce the occurrence, severity, or impact of behaviours that challenge. However, other groups have advocated the use of parent training programmes specifically adapted for children with learning disabilities and behaviours that challenge. High quality evidence is scarce for young children with moderate to profound learning disabilities and in the UK these children and their families rarely receive any intervention as part of usual care. Research is urgently needed to help progress care for this very vulnerable group of children and their families.

Notes to Applicants

The NIHR Health Technology Assessment Programme is funded by the NIHR, with contributions from the CSO in Scotland, NISCHR in Wales, and the Public Health Agency in Northern Ireland.

For many of the research questions posed by the HTA Programme, a randomised controlled trial is the most appropriate method of providing an answer. Suggestions for how a randomised controlled trial could be designed and constructed most efficiently are encouraged. Where the study design has been left open for applicants to specify, please note that the HTA Programme welcomes any study design which is well justified as the most appropriate approach to answer the research question.

Applicants are asked to:

1. Follow the Medical Research Council's (MRC) Good Clinical Practice guidelines (http://www.mrc.ac.uk/documents/pdf/good-clinical-practice-in-clinical-trials/) when planning how studies, particularly RCTs, will be supervised. Further advice specific to each topic will be given by the HTA Programme at full proposal and contract stages.

2. Note that trials involving medicinal products must comply with "The Medicines for Human Use (Clinical Trials) Regulations 2004". In the case of such trials, the DH expects the employing institution of the chief investigator to be nominated as the sponsor. Other institutions may wish to take on this responsibility or agree co-sponsorship with the employing institution. The DH is prepared to accept the nomination of multiple sponsors. Applicants who are asked to submit a full proposal will need to obtain confirmation of a sponsor(s) to complete their application. The DH reserve the right to withdraw from funding the project if they are not satisfied with the arrangements put in place to conduct the trial.

The MHRA (info@mhra.gsi.gov.uk, http://www.mhra.gov.uk) can provide guidance as to whether your trial would be covered by the regulations. The NIHR website (http://www.ct-toolkit.ac.uk/) also contains the latest information about Clinical Trials regulations and a helpful FAQ page.

In line with the government's transparency agenda, any contract resulting from this tender may be published in its entirety to the general public. Further information on the transparency agenda is at: http://transparency.number10.gov.uk/#

Applicants are recommended to seek advice from suitable methodological support services, at an appropriate stage in the development of their research idea and application. It is advisable to make contact at an early a stage as possible to allow sufficient time for discussion and a considered response.

The NIHR Research Design Service (http://www.rds.nihr.ac.uk/) can advise on appropriate NIHR Programme choice, and developing and designing high quality research grant applications.

Clinical Trials Toolkit

Researchers designing or undertaking clinical trials are encouraged to consult the Clinical Trials Toolkit (www.ct-toolkit.ac.uk). This NIHR resource is a website designed to help researchers navigate through the complex landscape of setting up and managing clinical trials in line with regulatory requirements. Although primarily aimed at those involved in publicly funded Clinical Trials of Investigational Medicinal Products (CTIMPs), the Toolkit will also benefit researchers and R&D staff working on trials in other areas, who will find useful information and guidance of relevance to the wider trials environment.

Research networks

The HTA Programme expects, where appropriate, that applicants will work with the relevant research network.

Making an application

If you wish to submit an Expression of Interest proposal on this topic, complete the on-line application form at www.nets.nihr.ac.uk/funding/hta-commissioned and submit it on line by **21 January 2016**. Applications will be considered by the HTA Commissioning Board at its meeting in **March 2016**.
IMPORTANT: For outline applications, if shortlisted, investigators will be given a minimum of **eight weeks to submit a full proposal**. The full proposal will be considered at the Commissioning Board in **July 2016**.

Applications received electronically after 1300 hours on the due date will not be considered.

Please see GUIDANCE ON APPLICATIONS overleaf.

Should you have any queries please contact htacmsng@soton.ac.uk

Guidance on applications

Required expertise

HTA is a multidisciplinary enterprise. It needs to draw on the expertise and knowledge of clinicians and of those trained in health service research methodologies such as health economics, medical statistics, study design, behavioural science and qualitative approaches. The HTA Programme expects teams proposing randomised controlled trials to include input from an accredited clinical trials unit, or one with equivalent experience. Applicants are also expected to engage a qualified Trial Manager for appropriate projects. A commitment to team working must be shown and applicants may wish to consider a collaborative approach between several institutions.

Public involvement in research

The HTA Programme recognises the benefit of increasing active involvement of members of the public in research and would like to support research projects appropriately. The HTA Programme encourages applicants to consider *how* the scientific quality, feasibility or practicality of their proposal *could* be improved by involving members of the public. Examples of how this has been done for health technology assessment projects can be found at www.nets.nihr.ac.uk/ppi. Research teams wishing to involve members of the public should include in their application: the aims of active involvement in this project; a description of the members of the public (to be) involved; a description of the methods of involvement; and an appropriate budget. Applications that involve members of the public will not, for that reason alone, be favoured over proposals that do not but it is hoped that the involvement of members of the public will improve the quality of the application.

Outcomes

Wherever possible, the results of HTA should provide information about the effectiveness and cost-effectiveness of care provided in its usual clinical setting and for the diverse subjects who would be eligible for the interventions under study. The endpoints of interest will in most cases include disease specific measures, health related quality of life and costs (directly and indirectly related to patient management). Wherever possible, these measurements should be made by individuals who are unaware of the treatment allocation of the subjects they are assessing. We encourage applicants to involve users of health care in the preparation of their proposal, for instance in selecting patient-oriented outcomes. Where established Core Outcomes exist they should be included amongst the list of outcomes unless there is good reason to do otherwise. Please see The COMET Initiative website at www.comet-initiative.org to identify whether Core Outcomes have been established. A period of follow up should be undertaken which is sufficient to ensure that a wider range of effects are identified other than those which are evident immediately after treatment. Where relevant, researchers should explore the effect of the intervention in relation to health inequalities. These factors should guide applicants in their choice of subjects, settings and measurements made.

Longer-term follow up

Researchers to consider building in provision, if appropriate, for a simple mechanism for long-term follow up using routine data bases/sets; including obtaining consent for this from participants at trial entry.

Sample size

A formal estimate should be made of the number of subjects required to show important differences in the chosen primary outcome measure. Justification of this estimate will be expected in the application.

Communication

Communication of the results of research to decision makers in the NHS is central to the HTA Programme. Successful applicants will be required to submit a single final report for publication by the HTA Programme. They are also required to seek peer-reviewed publication of their results elsewhere and may also be asked to support NETSCC, HTA in further efforts to ensure that results are readily available to all relevant parties in the NHS. Where findings demonstrate continuing uncertainty, these should be highlighted as areas for further research.

Timescale

There are no fixed limits on the duration of projects or funding and proposals should be tailored to fully address the problem (including long-term follow-up if necessary). Applicants should consider however that there is a pressing need within the NHS for this research, and so the duration of the research needs to be timely.

Feasibility and Pilot studies

We expect that when pilot or feasibility studies are proposed by applicants, or specified in commissioning briefs, a clear route to the substantive study will be described. This applies whether the brief or proposal describes just the preliminary study or both together. Whether preliminary and main studies are funded together or separately may be decided on practical grounds.

Feasibility Studies are pieces of research done before a main study. They are used to estimate important parameters that are needed to design the main study. Feasibility studies for randomised controlled trials may not themselves be randomised. Crucially, feasibility studies do not evaluate the outcome of interest; that is left to the main study. If a feasibility study is a small randomised controlled trial, it need not have a primary outcome and the usual sort of power calculation is not normally undertaken. Instead the sample size should be adequate to estimate the critical parameters (e.g. recruitment rate) to the necessary degree of precision.

Pilot studies are a version of the main study that is run in miniature to test whether the components of the main study can all work together. It is focused on the processes of the main study, for example to ensure recruitment, randomisation, treatment, and follow-up assessments all run smoothly. It will therefore resemble the main study in many respects. In some cases this will be the first phase of the substantive study and data from the pilot phase may contribute to the final analysis; this can be referred to as an internal pilot. Or at the end of the pilot study the data may be analysed and set aside, a so-called external pilot.

For a full definition of the terms 'feasibility study' and 'pilot study' visit the NETSCC website glossary page www.nets.nihr.ac.uk/glossary

In preparing for a substantive evaluation attention should be paid to appropriate guidance on how to develop interventions (such as the MRC guidance on developing and evaluating complex interventions and the IDEAL framework: www.ideal-collaboration.net/framework/).

Diagnostics and Imaging

In evaluating diagnostic and imaging techniques, the emphasis of the HTA Programme is to assess the effect on patient management and outcomes (particularly where changes in management can be shown to have patient benefits). Improvements in diagnostic accuracy, whilst relevant, are not the primary interest of this commissioned research programme. Applicants should justify where they consider improvements in diagnostic accuracy to be relevant to these objectives. Where there is poor

evidence to link diagnostic improvements to patient benefits, part of the primary research may be to assess the effects of such changes on patient outcome.

An assessment should also be made of changes in other resources (particularly other subsequent therapies) used as a result of changes in diagnostic methods.