Scoliosis-specific exercises in adolescent idiopathic scoliosis

Introduction

The aim of the HTA programme is to ensure that high quality research information on the effectiveness, costs and broader impact of health technologies 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.

Question

What is the clinical and cost-effectiveness of scoliosis-specific exercise treatment in adolescent idiopathic scoliosis, in comparison to standard care?

1 **Technology:** Scoliosis-specific exercises. The exact nature of the exercises is to be described and justified by the applicants.
2 **Patient group:** Children from 10-16 years with mild/primary adolescent idiopathic scoliosis (AIS) with a Cobb angle less than 50 degrees (i.e. are not eligible for surgery, and would otherwise enter watchful waiting or bracing).
3 **Setting:** Hospital outpatients or community setting.
4 **Control or comparator treatment:** Standard practice.
5 **Design:** Feasibility study with main trial broadly scoped. The feasibility study should:
   - Assess the acceptability and adherence to the chosen technology.
   - Identify the available number of participants and surgeons willing to participate, by a sufficient survey of orthopaedic surgeons.
   - Assess and describe training requirements of therapists.
   - Establish appropriate eligibility criteria for the study. These may include the requirement for bracing and how this will be considered in the design and analysis of the main study.
   - Continue to main trial with successful applicant dependent on clear indicators of success.
6 **Outcomes of the feasibility study:** Feasibility of progressing to a trial. The researchers should produce an outline protocol for a trial, which will then be separately assessed for funding. **Important outcomes for main trial:** Pain, exercise tolerance, psychological symptoms, quality of life, scoliosis severity. **Other outcomes:** Requirement for surgery/brace, patient acceptability, adverse effects, respiratory function, cosmetic appearance, costs and cost-effectiveness.
7 **Minimum duration of follow-up for the main trail:** To stabilisation (e.g. age 16 years).

Background to commissioning brief:

Scoliosis affects two children out of every 1,000 in the UK, but in 90% of cases of scoliosis, treatment is not required because the condition corrects itself as the child grows.

There is no NICE guidance on this subject; but advice from the National Scoliosis Foundation is that generally adults with scoliosis can engage in exercise and recreational sports without undue risk. Recent reviews conclude that the actual evidence on exercises for adolescent idiopathic scoliosis (AIS) is of level 1b, according to the Oxford Centre for Evidence-Based Medicine. However, only one randomised controlled trial (RCT) has been conducted in China, which compared two groups of 40 Chinese patients, showing an improvement of curvature in all treated patients after six months. Although there is some evidence for non surgical treatments, much of the quality is weak and therefore RCTs are urgently needed.

A larger, long-term RCT is required to confirm the results of the Chinese RCT; with a cost-effectiveness analysis in order for this to be incorporated as standard practice by the NHS.

Notes to Applicants

The NIHR Health Technology Assessment programme is funded by the NIHR, with contributions from the CSO in Scotland and WORD in Wales. Researchers from Northern Ireland should contact NETSCC to discuss their eligibility to apply.

For many of the questions posed by the HTA programme, a randomised controlled trial is likely to be the most appropriate method of providing an answer. However, there may be practical or ethical reasons why this might not be possible. Applicants proposing other research methods are invited to justify these choices.

Applicants are asked to:

1. Follow the Medical Research Council's Good Clinical Practice guidelines (http://www.mrc.ac.uk/Utilities/Documentrecord/index.htm?d=MRC002416) 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 DH/MRC website (http://www.ct-toolkit.ac.uk/) also contains the latest information about Clinical Trials regulations and a helpful FAQ page.

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 outline proposal on this topic, complete the on-line application form at http://www.hta.ac.uk/funding/standardcalls/index.shtml and submit it on line by 30/6/10. Applications will be considered by the HTA Commissioning Board at its meeting in late-September. For outline applications, if shortlisted, investigators will be given a minimum of eight weeks to submit a full proposal.

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

Please see GUIDANCE ON APPLICATIONS overleaf.

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 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 http://www.hta.ac.uk/PPIguidance/. 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. 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. These factors should guide applicants in their choice of subjects, settings and measurements made.

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 http://www.netscc.ac.uk/glossary/