Treating children with idiopathic steroid-resistant nephrotic syndrome

Introduction

The aim of the HTA programme is to ensure that high quality research information on the costs, effectiveness and broader impact of health technologies is produced in the most efficient way for those who use, manage and work in the NHS. Health technology assessment forms the largest portfolio of work in the NHS Research and Development Programme and each year about forty new studies are commissioned to help answer questions of direct importance to the NHS. The studies include primary and secondary research and cost about £10 million a year. Questions are identified and prioritised to meet the needs of the NHS and its patients.

Question

What is the most clinically and cost-effective way to treat idiopathic steroid-resistant nephrotic syndrome in children?

1 **Technology:** Single drugs: High-dose steroids (methylprednisolone); immunosuppressive agents (ciclosporin, tacrolimus, mycophenolate mofetil); alkylating agents (cyclophosphamide or chlorambucil). Combinations of drugs: methylprednisolone with chlorambucil or cyclophosphamide; methylprednisolone with ciclosporin. Plasma exchange as an alternative to drug-based therapy.
2 **Patient group:** Children aged 1 year to 18 years.
3 **Setting:** Paediatric nephrology centres.
4 **Control or comparator treatment:** Standard treatment.
5 **Design:** Evidence synthesis in the form of a systematic review and cost effectiveness modelling. The review should look at all treatments and include predictive studies if they exist. It is expected that most evidence will come from randomised controlled trials but other levels of evidence may need to be considered if there are insufficient long-term RCTs to inform the economic model. Modelling should include the long term implications of any proxy outcomes. The review will identify the priorities for future primary research.
6 **Outcomes:** Remission rates; relapse rates; renal function including proteinuria; adverse effects; long-term renal survival.

Summary of research need:

Few children have steroid-resistant nephrotic syndrome (SRNS), but of those that do, 50% develop renal failure within 5 years, and lifetime costs of renal failure and transplantation are considerable.

There is anecdotal evidence of a wide variation in treatment regimes for SRNS in the UK, but there is uncertainty about the most effective regimes for children with SRNS.

Synthesis of available evidence is required to identify and evaluate the wide range of current treatment regimes for SRNS in terms of clinical and cost-effectiveness. International evidence is needed as the condition is relatively rare, and there are ethnic factors and genetic traits that may be important.

Making an application

If you wish to submit a proposal on this topic, complete the electronic application form and return it, along with a detailed project description, to the Commissioning Manager at the National Coordinating Centre for Health Technology Assessment, Mailpoint 728, Boldrewood, University of Southampton, Southampton SO16 7PX by on **Thursday 14 July 2005**.

Your full proposal will be assessed by designated Commissioning Board members and clinical experts and a commissioning decision will be made by the Chair of the HTA Commissioning Board and the HTA Programme Director by Monday 15 August 2005.

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

Guidance on applications

Methods

Applicants should demonstrate knowledge of current research in the field and of systematic review methods and state how these would apply to the question posed. Valid and reliable methods should be proposed for identifying and selecting relevant material, assessing its quality and synthesising the results. Guidance on choice of appropriate methods is contained in NHS CRD Report 4 *Undertaking systematic reviews of research on effectiveness* (www.york.ac.uk/inst/crd/report4.htm). Where policy implications are considered, the emphasis should be on assessing the likely effects of a range of policy options open to decision makers rather than a judgement on any single strategy. Where epidemiological modelling or economic evaluation is required, the range of uncertainty associated with the results should be assessed. In the assessment of cost-effectiveness, further data collection may be required to estimate resource use and costs. If there is evidence that the ratio of costs and benefits may differ between readily identifiable groups, applicants are encouraged to state how they will identify these differences.

Updating

In order to inform decisions on whether and when to update the review, researchers will be expected to give some indication of how fast the evidence base is changing in the field concerned, based on the nature and volume of ongoing work known at the time the review is completed. Applicants should note that they will not be expected to carry out any future updating as part of the contract to complete the review.

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 communicate their work through peer-reviewed journals and may also be asked to support the NCCHTA 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

Your final report should be submitted to NCCHTA by **5pm on Friday 31 March 2006**.