Julia Stallibrass
Specialised Services Team Leader

Department of Health

Wellington House
113-155 Waterloo Road
London
SE1 8UG

Tel: 020 7972 4862
Fax: 020 7972 4324

To:
Chief Executives of Primary Care Trusts
Chief Executives of NHS Trusts and Foundation Trusts
Chief Executives of Strategic Health Authorities
Heads of Commissioning for Specialised Commissioning Groups
Heads of Commissioning for Local Specialised Commissioning Groups

28 October 2004

Dear Colleagues

NATIONAL DESIGNATION AND FUNDING OF THE SERVICE FOR PATIENTS WITH LYSOSOMAL STORAGE DISORDERS

For a period of two years, from April 2005 to March 2007, six centres will be nationally designated and funded by the Department of Health, under the auspices of the National Specialised Commissioning Advisory Group (NSCAG), to provide a service for patients with lysosomal storage disorders (LSDs). The service will include diagnostic, assessment AND treatment services. This means that the cost of drug treatments, including enzyme replacement therapies (ERTs), will be funded on a national basis. The six NHS Trusts concerned are: Great Ormond Street, Manchester Children's, the Royal Free, Addenbrooke’s, University College and Hope hospitals.

Please note that there was an entry in the Chief Executives Bulletin of 3-10 September, issue no: 235, announcing the national designation and funding of a Diagnostic and Management Advice service for patients with LSDs. This announcement supersedes that announcement.

National designation and funding will ensure that services to diagnose, assess and treat patients with LSDs will be concentrated in a small number of centres to allow the clinical teams to develop and maintain expertise in the management of these rare diseases. However, much of the care of patients with LSDs will still remain locally provided and funded, for example, bone marrow transplantation services for patients with Hurler’s syndrome.

Interim Period

In the interim period between now and April 2005 when national funding commences, PCTs will be expected to respond in a timely fashion to consideration of requests from clinicians at the designated centres for funding for ERTs or equivalent treatments.

Service Standards

Prior to April 2005 the centres will be assessed against a set of service standards which NSCAG are currently developing in conjunction with key stakeholders. Patients' groups will be invited to comment on the draft standards.

Clinical Protocols

Building on the good work of the West Midlands and London specialised services commissioners with clinicians from the centres, clinical guidelines for the use of ERTs have been drawn up which are currently being peer reviewed by the Royal Colleges.

NICE assessment

NICE is assessing the methodology for the appraisal of expensive drugs for rare diseases, paying particular attention to the ethical and equity issues, as well as the scientific ones, involved in applying the principles of cost effectiveness to such treatments. This project, which will report in late Spring 2005, will advise on an appropriate methodological approach and will include research into the public perspective including advice from NICE's Citizens' Council. Thereafter, if agreed by the Department, the methodology will be applied with the first assessment unlikely to be completed until 2006.

Long term follow up and evaluation

The Department of Health is providing support to ensure robust longitudinal evaluation of ERTs takes place. The Director of the Health Technology Assessment Programme is setting up an expert advisory group to take this forward.

Further information may be obtained from:

Sarah Watson, Lead Commissioner for LSDs, NSCAG
(sarah.watson@dh.gsi.gov.uk)
Edmund Jessop, Medical Adviser for LSDs, NSCAG
(edmund.jessop@dh.gsi.gov.uk)

Yours sincerely

Julia Stallibrass
Specialised Services Team Leader