Growth Hormone therapy in Prader-Willi Syndrome – UK practice

Growth Hormone (GH) therapy is recommended for growth failure in children with Prader-Willi Syndrome (PWS) – this is supported by the National Institute of Health & Clinical Excellence (NICE) and this guidance is published as ‘Technology Appraisal 188’ by NICE. The published guidance can be found at http://www.nice.org.uk/guidance/TA188/Guidance/pdf. The guidance also suggests that response to GH therapy in PWS children should also consider body composition.

GH therapy is always initiated and monitored by a specialist – generally a paediatric endocrinologist or paediatrician with expertise in growth disorders. However, the actual steps involved in this process can widely vary between different regions and hospitals. GPs generally do not initiate treatment, although in many areas they do prescribe the GH on the instructions of the specialist.

The generic process is as follows:

- Specialist with expertise – generally based at the hospital, decides when to start GH for a child with PWS. Specific tests to prove GH deficiency is not required, but it is essential to prove the diagnosis of PWS by genetic analysis.

- There are a number of GH preparations licensed for use in UK. The family decides which GH preparation to use based on their own personal preferences and the devices available to administer GH – the specialist provides information to the family and guides them in making their own choice.

- Following the specialists decision to start GH therapy and patients choice of the GH preparation, the specialist writes to the Primary Care Trust (PCT) or another subdivision of the PCT (like the pharmacy committee etc) seeking approval for the initiation of the GH therapy. Once approval is obtained, then the child's usual GP is authorised to prescribe the GH on a regular basis.

- In some areas, the specialist may also provide a GH prescription for the initial few months from the hospital, until formal approval from the PCT is obtained.

- The specialist will also organise training to the parents and if appropriate to the patient in the home administration of GH. The training is usually provided by the specialist nurse who works with the specialist. Sometimes the training is provided by an external agency or by the company itself.

- Some GH companies also provide additional facilities like collection of the GH prescription from the surgery & direct home delivery of the GH supplies.

- The actual steps involved in this process can vary widely. In addition, the NHS itself is undergoing a major restructuring including the process of commissioning; hence some of these steps are bound to change when new systems come into practice.

Although there appears to be a hugely confusing and long winding procedure to follow, almost all children with PWS were successful to obtain NHS funding for GH therapy following the specialists’ decision to start GH therapy. The NICE recommendations confirm that GH therapy for PWS children will continue to receive NHS funding in the future too, although the actual steps and processes in its implementation is likely to change.

(Written by Dr. Shankar Kanumakala, Consultant Paediatrician, Royal Alexandra Children's Hospital, Brighton, UK) February 2012