

19 February 2009

Changes to growth hormone access criteria for patients with Prader-Willi Syndrome

PHARMAC is pleased to announce changes to the current growth hormone access criteria for patients with Prader-Willi Syndrome (PWS), effective 1 March 2009.

These changes have resulted from a review of the current access criteria for patients with PWS carried out by PHARMAC between October 2007 and August 2008.

Patients are welcome to apply to the New Zealand Growth Hormone Committee (NZGHC) through their treating paediatrician in order to determine whether they will be eligible for subsidised growth hormone treatment under the revised criteria.

Families who have been self funding growth hormone therapy because their children did not previously meet the criteria may apply to the NZGHC to be considered under the revised criteria using growth data obtained prior to commencing growth hormone treatment. If the NZGHC determines that patients would have been eligible for growth hormone treatment under the revised criteria prior to commencing self funded treatment, PHARMAC will subsidise treatment for these patients from 1 March 2009.

Details of the decision

The revised growth hormone access criteria for patients with PWS are shown below:

CATEGORY FIVE PRADER-WILLI SYNDROME

ENTRY CRITERIA

In children with Prader-Willi Syndrome funding for growth hormone will be available where:

- a diagnosis of PWS has been confirmed by genetic testing; and
- growth velocity is <25th percentile for bone age over 12 months; and
- growth velocity in patients under two years of age has been assessed over a minimum six month period from the age of 12 months, with at least three supine length measurements over this period demonstrating clear and consistent evidence of linear growth failure (growth velocity < 25th percentile); and
- the bone age is <12 (girls) or <14 (boys); and

- sleep studies have been performed and there is no obstructive sleep disorder requiring treatment, or if an obstructive sleep disorder is found, it has been adequately treated under the care of a sleep physician and/or ENT surgeon; and
- there is no evidence of type II diabetes.

IGF-1 levels should be monitored to ensure the dose of growth hormone is not too high and, if IGF-1 levels are more than two standard deviations above the mean, the dose should be titrated down to bring IGF-1 levels closer to the normal range.

Patients approved for treatment with growth hormone should, wherever possible, undergo a DEXA scan prior to starting treatment and on an annual basis during treatment to better analyse the effects of growth hormone and therefore to provide evidence of its efficacy.

EXIT CRITERIA

Only one of the following criteria needs to be fulfilled to stop growth hormone therapy:

- Growth velocity fails to increase by at least 2cm/yr following at least a six month trial of growth hormone in those who do not have complete growth hormone deficiency.
- A growth velocity in the second and subsequent years of treatment in non growth hormone deficient patients that consistently falls below the 50th percentile for age for children with Prader-Willi Syndrome.
- A bone age of >14 years (female) or >16 years (male) and a growth velocity <2cm/yr as calculated over six months.
- An increase in bone age that exceeds the increase in height age in non growth hormone deficient patients with very delayed bone ages.
- BMI increases by 0.5 Standard Deviation Scores (SDS) or more in one year.
- A malignancy that develops after growth hormone therapy was commenced.
- A serious adverse reaction or complication that either the patient's specialist or New Zealand Growth Hormone Committee considers is likely to be attributable to growth hormone treatment.

Feedback received

We appreciate all the feedback we received and acknowledge the time people took to respond. All consultation responses received by 14 January 2009 were considered in their entirety in making a decision on the proposed changes.

As a result of this consultation, several minor changes were made to the proposed criteria. Other suggested changes will be considered further as part of an overall review of the Growth Hormone Access Criteria at a later date.

Further information

If you have any queries about this change, please contact the PHARMAC helpline on 0800 66 00 50 (9 am to 5 pm weekdays).