

PBS Growth Hormone Program (Paediatric Use) 1 February 2019 Listing Changes Quick Reference Guide for Prescribers

Changes to the Pharmaceutical Benefits Scheme (PBS) restrictions for growth hormone (GH) treatment (paediatric use), which set out the eligibility requirements for PBS subsidy, are being introduced on 1 February 2019.

These changes broaden the eligibility criteria for access to PBS-subsidised GH for paediatric patients in specific clinical circumstances. They relate to height percentile and growth velocity eligibility thresholds, growth velocity measurement intervals and associated growth data requirements. Specification of relevant criteria within the PBS restrictions has also been streamlined.

The changes affect six of the 10 listed indications under the PBS GH Program (and three of five treatment phases). Specific changes are summarised below by indication (full restriction text is available in the [Schedule of Pharmaceutical Benefits](#)).

Initial Treatment Phase

Short stature associated with biochemical growth hormone deficiency (BGHD)

Clinical Criteria

The 1 February 2019 changes:

- Increase the **height** percentile eligibility threshold for all patients from 'below the 1st percentile for age and sex' to '**at or below the 25th percentile** for age and sex';
- **Remove growth velocity eligibility thresholds for patients whose height is below the 1st percentile for age and sex (patients with a newly eligible height at the 1st percentile are also exempt);**
- Remove the requirement for growth velocity percentile for bone age and sex (**GVPBA**) to be calculated over both 12 and six-month intervals **for younger children¹** (instead it is **required to be calculated over the 12-month interval only**); and
- (For brands not restricted to patients aged 3 years or older) increase the **annual growth velocity** eligibility threshold **for patients whose chronological age is 2 years or less** from '8 cm per year or less' to '**14 cm per year or less**'.²

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children¹.**
(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).
- **Remove the requirement to provide growth data at the six and 12-month data points for patients whose height is below the 1st percentile for age and sex (instead only current height and weight measurements are required; this also applies for patients with a newly eligible height at the 1st percentile).**

Short stature associated with chronic renal insufficiency (CR)

Clinical Criteria

The 1 February 2019 changes:

- **Remove growth velocity eligibility thresholds for patients whose height is at or below the 1st percentile** for age and sex;
- Remove the requirement for **GVPBA** to be calculated over both 12 and six-month intervals **for younger children¹** (instead it is **required to be calculated over the 12-month interval only**); and
- (For brands not restricted to patients aged 3 years or older) increase the **annual growth velocity** eligibility threshold **for patients whose chronological age is 2 years or less** from '8 cm per year or less' to '**14 cm per year or less**'.²

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children¹.**
(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).
- **Remove the requirement to provide growth data at the six and 12-month data points for patients whose height is at or below the 1st percentile** for age and sex (instead **only current height and weight measurements are required**).

Growth retardation secondary to an intracranial lesion, or cranial irradiation (CL/CI)

Clinical Criteria

The 1 February 2019 changes:

- **Remove growth velocity eligibility thresholds for patients whose height is at or below the 1st percentile** for age and sex;
- Remove the requirement for **GVPBA** to be calculated over both 12 and six-month intervals **for younger children¹** (instead it is **required to be calculated over the 12-month interval only**); and
- (For brands not restricted to patients aged 3 years or older) increase the **annual growth velocity** eligibility threshold **for patients whose chronological age is 2 years or less** from '8 cm per year or less' to '**14 cm per year or less**'.²

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children¹.**
(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).
- **Remove the requirement to provide growth data at the six and 12-month data points for patients whose height is at or below the 1st percentile** for age and sex (instead **only current height and weight measurements are required**).

Hypothalamic-pituitary disease secondary to a structural lesion, with hypothalamic obesity driven growth (HO)

Clinical Criteria

The 1 February 2019 changes:

- Remove the requirement for **GVPBA** to be calculated over both 12 and six-month intervals **for younger children**¹ (instead it is **required to be calculated over the 12-month interval only**); and
- (For brands not restricted to patients aged 3 years or older) increase the **annual growth velocity** eligibility threshold **for patients whose chronological age is 2 years or less** from 'greater than 8 cm per year' to '**greater than 14 cm per year**'.^{2,3}

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children**¹.

(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).

Short stature and slow growth (SSSG)

Clinical Criteria

The 1 February 2019 changes:

- Increase the **height** percentile eligibility threshold for all patients from 'below the 1st percentile for age and sex' to '**at or below the 1st percentile** for age and sex'; and
- Remove the requirement for **GVPBA** to be calculated over both 12 and six-month intervals **for younger children**¹ (instead it is **required to be calculated over the 12-month interval only**).

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children**¹.

(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).

Short stature due to short stature homeobox (SHOX) gene disorders

Clinical Criteria

The 1 February 2019 changes:

- Increase the **height** percentile eligibility threshold for all patients from 'below the 1st percentile for age and sex' to '**at or below the 1st percentile** for age and sex';
- Remove the requirement for **GVPBA** to be calculated over both 12 and six-month intervals **for younger children**¹ (instead it is **required to be calculated over the 12-month interval only**); and
- (For brands not restricted to patients aged 3 years or older) increase the **annual growth velocity** eligibility threshold **for patients whose chronological age is 2 years or less** from '8 cm per year or less' to '**14 cm per year or less**'.²

Data Requirements

The 1 February 2019 changes:

- **Remove the requirement to provide 12 months of growth data at six-monthly intervals for younger children**¹.

(Note: while only current and 12-month data points are now required at each authority application for a younger child, as GH treatment periods are approximately six months in duration, this does not remove the need for clinical review on a six-monthly basis).

All Indications (BGHD, CR, CL/CI, HO, SSSG and SHOX)

Restriction Structure

The 1 February 2019 changes:

- Incorporate a reference definition for an 'older child'¹ (per the existing definition) to:
 - a) streamline the way GVPBA measurement intervals for older and younger children are specified within the clinical criteria (collapsing five criteria into one); and
 - b) streamline the way associated growth data requirements are specified within the prescriber instructions.

Reclassification Treatment Phases (Continuing or Recommencement of Treatment as a Reclassified Patient)

All Indications (BGHD, CR, CL/CI, HO, SSSG and SHOX)

Clinical Criteria

The 1 February 2019 changes **mirror the changes to the initial treatment phase clinical criteria for each respective indication** as described above. The **affected criteria relate to height percentile and growth velocity immediately prior to commencement of treatment**. These criteria establish whether the patient would have met initiation criteria for the new indication prior to commencing GH treatment.

Data Requirements

The 1 February 2019 changes **mirror the changes to the initial treatment phase data requirements for each respective indication** as described above. The **affected requirements relate to growth data from immediately prior to commencement of treatment**. These requirements enable assessment of whether the patient would have met initiation criteria for the new indication prior to commencing GH treatment.

Restriction Structure

The 1 February 2019 changes:

- Incorporate a reference definition for an 'older child'¹ (per the existing definition) to:
 - a) streamline the way GVPBA measurement intervals for older and younger children for the period *immediately prior to commencement of treatment* are specified within the clinical criteria (collapsing five criteria into one); and
 - b) streamline the way associated growth data requirements are specified within the prescriber instructions.

¹ An older child is defined as a male with a chronological age of at least 12 years or a bone age of at least 10 years, or a female with a chronological age of at least 10 years or a bone age of at least 8 years. References to younger children throughout this document refer to children who do not meet this definition (and have a bone age greater than 2.5 years in the context of clinical criteria relating to GVPBA).

² Because the increased annual growth velocity eligibility threshold of 14 cm per year applies only to patients with a chronological age of 2 years or less, the existing eligibility threshold of 8 cm per year is being maintained for patients with a chronological age greater than 2 years who have a bone age of 2.5 years or less. (The cohort to which the existing eligibility threshold applies has been described in the relevant criteria as patients with a 'bone *or chronological* age of 2.5 years or less', with the insertion of the words 'or chronological' reflecting the existing exemption from providing a bone age result for patients with a chronological age of 2.5 years or less).

³ The increase to the annual growth velocity eligibility threshold from 8 cm to 14 cm per year for patients with a chronological age of 2 years or less narrows eligibility for access to PBS-subsidised GH in one specific instance. This is because of the unique criteria for the HO indication, where growth velocity eligibility thresholds are reversed by comparison to all other indications for treatment under the GH Program (in this instance the annual growth velocity eligibility threshold is 'greater than 14 cm per year' rather than '14 cm per year or less').