

# PBS Indications & regulations: Paediatric use of Growth Hormone in Australia (June 2020)

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## SHORT STATURE AND SLOW GROWTH

Treatment Phase: Initial treatment

### **Clinical criteria:**

- Patient must have a current height at or below the 1st percentile for age and sex,

AND

- Patient must have a growth velocity below the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child),

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must not have a bone age of 2.5 years or less,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more,

AND

- Patient must be male and must not have a height greater than or equal to 167.7 cm; OR
- Patient must be female and must not have a height greater than or equal to 155.0 cm,

AND

- Patient must be male and must not have maturational or constitutional delay in combination with an estimated mature height equal to or above 160.1 cm; OR
- Patient must be female and must not have maturational or constitutional delay in combination with an estimated mature height equal to or above 148.0 cm.

### **Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

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.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; AND
4. A bone age result performed within the last 12 months; AND
5. Confirmation of the patient's maturational or constitutional delay status; AND
6. If the patient has maturational or constitutional delay, confirmation that the patient has an estimated mature height below the 1st adult height percentile; AND
7. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

## SHORT STATURE ASSOCIATED WITH BIOCHEMICAL GROWTH HORMONE DEFICIENCY

Treatment Phase: Initial treatment

### Clinical criteria:

- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 2 pharmacological growth hormone stimulation tests (e.g. arginine, clonidine, glucagon, insulin); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 pharmacological growth hormone stimulation test (e.g. arginine, clonidine, glucagon, insulin) and 1 physiological growth hormone stimulation test (e.g. sleep, exercise); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) with other evidence of growth hormone deficiency, including septo-optic dysplasia (absent corpus callosum and/or septum pellucidum), midline abnormality including optic nerve hypoplasia, cleft lip and palate, midfacial hypoplasia and central incisor, ectopic and/or absent posterior pituitary bright spot, absent empty sella syndrome, hypoplastic anterior pituitary gland and/or pituitary stalk/infundibulum, and genetically proven biochemical growth hormone deficiency either isolated or as part of hypopituitarism in association with pituitary deficits (ACTH, TSH, GnRH or vasopressin/ADH deficiency); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGF-1 levels; OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGFBP-3 levels,

AND

- Patient must have a current height at or below the 1st percentile for age and sex; OR
- Patient must have a current height above the 1st and at or below the 25th percentiles for age and sex and a growth velocity below the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child); OR
- Patient must have a current height above the 1st and at or below the 25th percentiles for age and sex and an annual growth velocity of 14 cm per year or less if the patient has a chronological age of 2 years or less; OR
- Patient must have a current height above the 1st and at or below the 25th percentiles for age and sex and an annual growth velocity of 8 cm per year or less if the patient has a bone or chronological age of 2.5 years or less,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

**Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

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.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. (a) A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; OR
4. (b) Height and weight measurements, not more than three months old at the time of application, for a patient whose current height is at or below the 1st percentile for age and sex; AND
5. A bone age result performed within the last 12 months (except for a patient whose chronological age is 2.5 years or less); AND
6. Evidence of biochemical growth hormone deficiency, including the type of tests performed and peak growth hormone concentrations; AND
7. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

Biochemical growth hormone deficiency should not be secondary to an intracranial lesion or cranial irradiation for applications under this category.

## GROWTH RETARDATION SECONDARY TO AN INTRACRANIAL LESION, OR CRANIAL IRRADIATION

Treatment Phase: Initial treatment

### Clinical criteria:

- Patient must have had an intracranial lesion and have undergone a 12 month period of observation following completion of treatment for the intracranial lesion (all treatment); OR
- Patient must have had an intracranial lesion, have received medical advice that it is unsafe to treat the intracranial lesion, and have undergone a 12 month period of observation since initial diagnosis of the intracranial lesion; OR
- Patient must have received cranial irradiation without having had an intracranial lesion, and have undergone a 12 month period of observation following completion of treatment for the condition for which cranial irradiation was received,

AND

- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 2 pharmacological growth hormone stimulation tests (e.g. arginine, clonidine, glucagon, insulin); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 pharmacological growth hormone stimulation test (e.g. arginine, clonidine, glucagon, insulin) and 1 physiological growth hormone stimulation test (e.g. sleep, exercise); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) with other evidence of growth hormone deficiency, including septo-optic dysplasia (absent corpus callosum and/or septum pellucidum), midline abnormality including optic nerve hypoplasia, cleft lip and palate, midfacial hypoplasia and central incisor, ectopic and/or absent posterior pituitary bright spot, absent empty sella syndrome, hypoplastic anterior pituitary gland and/or pituitary stalk/infundibulum, and genetically proven biochemical growth hormone deficiency either isolated or as part of hypopituitarism in association with pituitary deficits (ACTH, TSH, GnRH or vasopressin/ADH deficiency); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGF-1 levels; OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGFBP-3 levels,

AND

- Patient must have a current height at or below the 1st percentile for age and sex; OR
- Patient must have a current height above the 1st percentile for age and sex and a growth velocity below the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child); OR
- Patient must have a current height above the 1st percentile for age and sex and an annual growth velocity of 14 cm per year or less if the patient has a chronological age of 2 years or less; OR
- Patient must have a current height above the 1st percentile for age and sex and an annual growth velocity of 8 cm per year or less if the patient has a bone or chronological age of 2.5 years or less,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

**Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

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.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. (a) A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; OR
  1. (b) Height and weight measurements, not more than three months old at the time of application, for a patient whose current height is at or below the 1st percentile for age and sex; AND
4. A bone age result performed within the last 12 months (except for a patient whose chronological age is 2.5 years or less); AND
5. Evidence of biochemical growth hormone deficiency, including the type of tests performed and peak growth hormone concentrations; AND
  - a. Confirmation that the patient has had an intracranial lesion and has undergone a 12 month period of observation following completion of treatment for the intracranial lesion (all treatment); OR
  - b. Confirmation that the patient has had an intracranial lesion, has received medical advice that it is unsafe to treat the intracranial lesion, and has undergone a 12 month period of observation since initial diagnosis of the intracranial lesion; OR
  - c. Confirmation that the patient has received cranial irradiation without having had an intracranial lesion, and has undergone a 12 month period of observation following completion of treatment for the condition for which cranial irradiation was received; AND
6. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

## RISK OF HYPOGLYCAEMIA SECONDARY TO GROWTH HORMONE DEFICIENCY IN NEONATES/INFANTS

Treatment Phase: Initial treatment

### **Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

### **Clinical criteria:**

- Patient must have a chronological age of less than 2 years,

AND

- Patient must have a documented clinical risk of hypoglycaemia,

AND

- Patient must have documented evidence that the risk of hypoglycaemia is secondary to biochemical growth hormone deficiency,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. Recent growth data (height and weight, not older than three months); AND
4. Confirmation that the patient has a documented clinical risk of hypoglycaemia; AND
5. Confirmation that the patient has documented evidence that the risk of hypoglycaemia is secondary to biochemical growth hormone deficiency; AND
6. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.



## BIOCHEMICAL GROWTH HORMONE DEFICIENCY AND PRECOCIOUS PUBERTY

Treatment Phase: Initial treatment

### Clinical criteria:

- Patient must be male and have commenced puberty (demonstrated by Tanner stage 2 genital or pubic hair development or testicular volumes greater than or equal to 4 mL) before the chronological age of 9 years; OR
- Patient must be female and have commenced puberty (demonstrated by Tanner stage 2 breast or pubic hair development) before the chronological age of 8 years; OR
- Patient must be female and menarche occurred before the chronological age of 10 years,

AND

- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 2 pharmacological growth hormone stimulation tests (e.g. arginine, clonidine, glucagon, insulin); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 pharmacological growth hormone stimulation test (e.g. arginine, clonidine, glucagon, insulin) and 1 physiological growth hormone stimulation test (e.g. sleep, exercise); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) with other evidence of growth hormone deficiency, including septo-optic dysplasia (absent corpus callosum and/or septum pellucidum), midline abnormality including optic nerve hypoplasia, cleft lip and palate, midfacial hypoplasia and central incisor, ectopic and/or absent posterior pituitary bright spot, absent empty sella syndrome, hypoplastic anterior pituitary gland and/or pituitary stalk/infundibulum, and genetically proven biochemical growth hormone deficiency either isolated or as part of hypopituitarism in association with pituitary deficits (ACTH, TSH, GnRH or vasopressin/ADH deficiency); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGF-1 levels; OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGFBP-3 levels,

AND

- Patient must be undergoing Gonadotrophin Releasing Hormone agonist therapy for pubertal suppression,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

**Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
  - a. A minimum of 12 months of recent growth data (height and weight) at intervals no greater than six months. The most recent data must not be older than three months; OR
  - b. A minimum of 6 months of recent growth data (height and weight) for older children (males chronological age 12 and over or bone age 10 and over, females chronological age 10 and over or bone age 8 and over). The most recent data must not be older than three months; AND
3. A bone age result performed within the last 12 months (except for a patient whose chronological age is 2.5 years or less); AND
4. Evidence of biochemical growth hormone deficiency, including the type of tests performed and peak growth hormone concentrations; AND
5. Confirmation that the patient has precocious puberty; AND
6. Confirmation that the patient is undergoing Gonadotropin Releasing Hormone agonist therapy, for pubertal suppression; AND
7. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

## HYPOTHALAMIC-PITUITARY DISEASE SECONDARY TO A STRUCTURAL LESION, WITH HYPOTHALAMIC OBESITY DRIVEN GROWTH

Treatment Phase: Initial treatment

### Clinical criteria:

- Patient must have a structural lesion that is not neoplastic; OR
- Patient must have had a structural lesion that was neoplastic and have undergone a 12 month period of observation following completion of treatment for the structural lesion (all treatment); OR
- Patient must have a structural lesion that is neoplastic, have received medical advice that it is unsafe to treat the structural lesion, and have undergone a 12 month period of observation since initial diagnosis of the structural lesion,

AND

- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 2 pharmacological growth hormone stimulation tests (e.g. arginine, clonidine, glucagon, insulin); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 pharmacological growth hormone stimulation test (e.g. arginine, clonidine, glucagon, insulin) and 1 physiological growth hormone stimulation test (e.g. sleep, exercise); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) with other evidence of growth hormone deficiency, including septo-optic dysplasia (absent corpus callosum and/or septum pellucidum), midline abnormality including optic nerve hypoplasia, cleft lip and palate, midfacial hypoplasia and central incisor, ectopic and/or absent posterior pituitary bright spot, absent empty sella syndrome, hypoplastic anterior pituitary gland and/or pituitary stalk/infundibulum, and genetically proven biochemical growth hormone deficiency either isolated or as part of hypopituitarism in association with pituitary deficits (ACTH, TSH, GnRH or vasopressin/ADH deficiency); OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGF-1 levels; OR
- Patient must have evidence of biochemical growth hormone deficiency, with a peak serum growth hormone concentration less than 10 mU/L or less than or equal to 3.3 micrograms per litre in response to 1 growth hormone stimulation test (pharmacological or physiological e.g. arginine, clonidine, glucagon, insulin, sleep, exercise) and low plasma IGFBP-3 levels,

AND

- Patient must have other hypothalamic/pituitary hormone deficits (includes ACTH, TSH, GnRH and/or vasopressin/ADH deficiencies),

AND

- Patient must have hypothalamic obesity,

AND

- Patient must have a growth velocity above the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child); OR
- Patient must have an annual growth velocity of greater than 14 cm per year if the patient has a chronological age of 2 years or less; OR
- Patient must have an annual growth velocity of greater than 8 cm per year if the patient has a bone or chronological age of 2.5 years or less,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

**Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

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.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; AND
4. A bone age result performed within the last 12 months (except for a patient whose chronological age is 2.5 years or less); AND
5. Evidence of biochemical growth hormone deficiency, including the type of tests performed and peak growth hormone concentrations; AND
  - a. Confirmation that the patient has a structural lesion that is not neoplastic; OR
  - b. Confirmation that the patient had a structural lesion that was neoplastic and has undergone a 12 month period of observation following completion of treatment for the structural lesion (all treatment); OR
  - c. Confirmation that the patient has a structural lesion that is neoplastic, has received medical advice that it is unsafe to treat the structural lesion, and has undergone a 12 month period of observation since initial diagnosis of the structural lesion; AND
6. Confirmation that the patient has other hypothalamic/pituitary hormone deficits; AND
7. Confirmation that the patient has hypothalamic obesity; AND
8. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

Testing for biochemical growth hormone deficiency must have been performed at a time when all other pituitary hormone deficits were being adequately replaced.

## SHORT STATURE ASSOCIATED WITH TURNER SYNDROME

Treatment Phase: Initial treatment

### Treatment criteria:

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.
- Clinical criteria:
- Patient must have a current height at or below the 95th percentile for age on the Turner syndrome growth curve for girls,

AND

- Patient must have diagnostic results consistent with Turner syndrome (the condition must be genetically proven), defined as a loss of a whole X chromosome in all cells (4,5X), and gender of rearing is female; OR
- Patient must have diagnostic results consistent with Turner syndrome (the condition must be genetically proven), defined as a loss of a whole X chromosome in some cells (mosaic 46XX/45X), and gender of rearing is female; OR
- Patient must have diagnostic results consistent with Turner syndrome (the condition must be genetically proven), defined as genetic loss or rearrangement of an X chromosome (such as isochromosome X, ring-chromosome, or partial deletion of an X chromosome), and gender of rearing is female,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must not have a bone age of 2.5 years or less,

AND

- Patient must not have a height greater than or equal to 155.0cm,

AND

- Patient must not have a bone age of 13.5 years or greater.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND

3. (a) A minimum of 12 months of recent growth data (height and weight) at intervals no greater than six months. The most recent data must not be older than three months; OR
1. (b) A minimum of 6 months of recent growth data (height and weight) for older children (females chronological age 10 and over or bone age 8 and over). The most recent data must not be older than three months; AND
4. A bone age result performed within the last 12 months; AND
5. Confirmation that the patient has diagnostic results consistent with Turner syndrome; AND
6. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

## SHORT STATURE DUE TO SHORT STATURE HOMEBOX (SHOX) GENE DISORDERS

Treatment Phase: Initial treatment

### **Clinical criteria:**

- Patient must have diagnostic results consistent with a SHOX mutation/deletion, defined as a karyotype confirming the presence of a SHOX mutation/deletion without the presence of mixed gonadal dysgenesis; OR
- Patient must have diagnostic results consistent with a SHOX mutation/deletion, defined as mixed gonadal dysgenesis (45X mosaic karyotype with the presence of any Y chromosome material and/or SRY gene positive by FISH study) and have an appropriate plan of management in place for the patient's increased risk of gonadoblastoma,

AND

- Patient must have a current height at or below the 1st percentile for age and sex,

AND

- Patient must have a growth velocity below the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child); OR
- Patient must have an annual growth velocity of 14 cm per year or less if the patient has a chronological age of 2 years or less; OR
- Patient must have an annual growth velocity of 8 cm per year or less if the patient has a bone or chronological age of 2.5 years or less,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes (excluding gonadoblastoma secondary to mixed gonadal dysgenesis),

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a height greater than or equal to 167.7cm; OR
- Patient must be female and must not have a height greater than or equal to 155.0cm,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

### **Treatment criteria:**

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

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.



The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; AND
4. A bone age result performed within the last 12 months (except for a patient whose chronological age is 2.5 years or less); AND
5. Confirmation that the patient has diagnostic results consistent with a short stature homeobox (SHOX) gene disorder; AND
6. If the patient's condition is secondary to mixed gonadal dysgenesis, confirmation that an appropriate plan of management for the patient's increased risk of gonadoblastoma is in place; AND
7. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.

## SHORT STATURE ASSOCIATED WITH CHRONIC RENAL INSUFFICIENCY

Treatment Phase: Initial treatment

### Treatment criteria:

- Must be treated by a specialist or consultant physician in paediatric endocrinology; OR
- Must be treated by a specialist or consultant physician in general paediatrics in consultation with a nominated specialist or consultant physician in paediatric endocrinology.

### Clinical criteria:

- Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m<sup>2</sup> measured by creatinine clearance, excretion of radionuclides such as DTPA, or by the height/creatinine formula, and not have undergone a renal transplant; OR
- Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m<sup>2</sup> measured by creatinine clearance, excretion of radionuclides such as DTPA, or by the height/creatinine formula, have undergone a renal transplant, and have undergone a 12 month period of observation following the transplant,

AND

- Patient must have a current height at or below the 1st percentile for age and sex; OR
- Patient must have a current height above the 1st and at or below the 25th percentiles for age and sex and a growth velocity less than or equal to the 25th percentile for bone age and sex measured over a 12 month interval (or a 6 month interval for an older child); OR
- Patient must have a current height above the 1st and at or below the 25th percentiles for age and sex and an annual growth velocity of 8 cm per year or less if the patient has a bone age of 2.5 years or less,

AND

- Patient must not have diabetes mellitus,

AND

- Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,

AND

- Patient must not have an active tumour or evidence of tumour growth or activity,

AND

- Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,

AND

- Patient must be male and must not have a height greater than or equal to 167.7 cm; OR
- Patient must be female and must not have a height greater than or equal to 155.0 cm,

AND

- Patient must be male and must not have a bone age of 15.5 years or more; OR
- Patient must be female and must not have a bone age of 13.5 years or more.

Population criteria:

- Patient must be aged 3 years or older.

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.

The maximum duration of the initial treatment phase is 32 weeks. Prescribers must determine an appropriate weekly dose in accordance with the dosing arrangements detailed in the National Health (Growth Hormone Program) Special Arrangement 2015 and request the appropriate number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

The authority application must be in writing and must include:

1. A completed authority prescription form; AND
2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND
3. (a) A minimum of 12 months of recent growth data (height and weight measurements) or a minimum of 6 months of recent growth data for an older child. The most recent data must not be more than three months old at the time of application; OR
  1. (b) Height and weight measurements, not more than three months old at the time of application, for a patient whose current height is at or below the 1st percentile for age and sex; AND
4. A bone age result performed within the last 12 months; AND
5. Confirmation that the patient has an estimated glomerular filtration rate less than 30mL/minute/1.73m<sup>2</sup> ; AND
6. If a renal transplant has taken place, confirmation that the patient has undergone a 12 month period of observation following transplantation; AND
7. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 16 weeks' worth of treatment (with up to 1 repeat allowed).

Prescribers must keep a copy of any clinical records relating to the prescription, including such records required to demonstrate that the prescription was written in compliance with any relevant circumstances and/or purposes. These records must be kept for 2 years after the date the prescription to which the records relate is written.