12.01 SOMATROPIN
All forms and strengths,
All brands,

1 Purpose of Item

1.1 To ratify the amended eligibility criteria for the Section 100 (Growth Hormone) Authority Required listing of adult use somatropin arising from Endocrine Society of Australia (ESA) feedback following the July 2019 PBAC meeting regarding clarification of PBAC intent around Childhood Onset Growth Hormone Deficiency (CO-GHD) patients transitioning to the adult growth hormone (GH) program.

2 Background

2.1 Somatropin for the treatment of adults with severe growth hormone deficiency was listed on the PBS on 1 December 2018.

2.2 The adult somatropin eligibility criteria includes patients with CO-GHD due to a congenital, genetic or structural cause.

2.3 In July 2019, the PBAC ratified changes to the PBS eligibility restrictions for adult use somatropin which will provide that CO-GHD patients with congenital, genetic or structural causes who have previously received PBS-subsidised therapy as children are no longer required to provide provocation tests to meet the eligibility criteria for adult use somatropin.

2.4 The PBAC also ratified in July 2019 that CO-GHD patients who have previously received non-PBS therapy as children will be required to demonstrate the same level of evidence as adult onset growth hormone deficiency patients. *This may require some patients to have a break in therapy in order to perform the necessary tests to meet the requirements.*

2.5 The Quality of Life Assessment of Growth Hormone Deficiency in Adults was recommended for removal at the March 2019 PBAC meeting due to accessibility issues. This change was implemented 1 September 2019.

2.6 *Further work is underway to amend the definitions of adult and child in the National Health (Growth Hormone Program) Special Arrangement 2015 to ensure continued access under the paediatric eligibility criteria for patients with delayed bone age regardless of chronological age. PBAC will be advised once the legislative instrument has been amended.*

3 Current situation

3.1 Following the ratification of the amended restriction text for adult use somatropin at the July 2019 PBAC, advice was sought from the ESA to ensure clarity of the restriction wording in line with PBAC policy intent.

3.2 ESA commented that a paediatric patient with CO-GHD due to a congenital, genetic or structural cause may reach skeletal maturity at the age of 15-17 years and as such would be unable to access further GH under the PBS until they reach 18. The ESA suggested that the eligibility criteria should recognise that GH replacement for patients with CO-GHD due to a congenital, genetic or structural cause should continue during the transition between skeletal maturity and the chronological age of 18.

3.3 Summary of changes for initial treatment phase adult use somatropin:

Clinical Criteria	Evidence of provocation testing	Age eligibility
CO-GHD with previous PBS subsidised GH therapy as a child	Not required	Skeletal maturity
CO-GHD with previous non-PBS funded GH therapy as a child	Required, historical tests allowed	Skeletal maturity
Adult onset GHD	Required	18 years and older

3.4 A further option was proposed by ESA members following the July 2019 meeting for the PBAC to look at the opportunity to reduce unnecessary stimulation testing of adults with established hypopituitarism by allowing evidence of alternate test results. *The Department of Health (the Department) was unable to give full consideration to this prior to the August 2019 meeting. A number of factors need to be navigated including the potential for opening the eligibility criteria and modelling the cost impact, broader consultation with clinicians and review of the clinical evidence. Should the PBAC wish to pursue this body of work, the Department could work with sponsors to bring forward a submission.*

4 Requested PBAC advice

4.1 For PBAC to ratify the amended eligibility criteria set out in paragraph 1.1 for the Section 100 (Growth Hormone) Authority Required listing of somatropin for the treatment of adults with severe growth hormone deficiency.

4.2 The Department sought advice from the PBAC on its preferred approach to pursuing the additional body of work relating to amending the restriction eligibility criteria for provocation tests for adults with established hypopituitarism.

5 PBAC Outcome

5.1 The PBAC ratified amendments to the adult-use somatropin restrictions for CO-GHD patients with a congenital, genetic or structural cause to commence from when this cohort reaches skeletal maturity rather than the chronological age of 18 years. The PBAC noted that this would remove the potential of a lapse in PBS funded access for patients after reaching skeletal maturity until the age of 18 years.

5.2 The PBAC advised that should the ESA wish to explore further changes to the eligibility criteria of somatropin for the treatment of severe growth hormone deficiency in adults it would welcome submissions from the ESA. The PBAC noted that submissions would need to include clear proposal/s with evidence to support the proposals and draft restrictions in line with the proposal/s.

6 Recommended listing

6.1 Amend existing/recommended listing as follows. Changes are identified in italics (additions) and strikethrough (deletions):

Powder for injection 5 mg (15i.u.) with diluent in pre-filled pen (with preservative) Powder for injection 12 mg (36 i.u.) with diluent in pre-filled pen (with preservative) Genotropin GoQuick®, Pfizer Australia Pty Ltd

Solution for injection 10 mg (30 i.u.) in 2 mL cartridge (with preservative) NutropinAq®, Ipsen Pty Ltd

Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Proprietary Name and Manufacturer	
SOMATROPIN Somatropin (Recombinant human growth hormone) Powder for injection 5 mg (15 i.u.) with diluent in pre-filled pen (with preservative), 1	1	5	Genotropin GoQuick ®	Pfizer Australia Pty Ltd
Somatropin (Recombinant human growth hormone) Powder for injection 12 mg (36 i.u.) with diluent in pre-filled pen (with preservative), 1		5		
SOMATROPIN somatropin 10 mg/2 mL injection, 2 mL cartridge	1	5	NutropinAq®	Ipsen Pty Ltd

Category / Program	Section 100 – Growth Hormone Program
Prescriber type:	☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives
PBS Indication:	Severe growth hormone deficiency
Treatment phase:	Initial treatment
Restriction:	☐Restricted benefit ☒Authority Required - In Writing ☐Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	Must be treated by an endocrinologist.
Clinical criteria:	Patient must have a documented childhood onset growth hormone deficiency due to a congenital, genetic or structural cause; *AND* *Patient must have previously received PBS-subsidised treatment with this drug for this condition as a child.*

Population criteria:	~~Patient must be aged 18 years or older~~ *Patient must have a mature skeleton.*
Prescriber Instructions:	The authority application must be in writing and must include: 1. A completed authority prescription form; 2. A completed Severe Growth Hormone Deficiency supporting information form; 3. Confirmation of childhood onset growth hormone deficiency due to a congenital, genetic or structural cause, previous PBS-subsidised therapy with this drug as a child, and a mature skeleton; 4. A ~~baseline~~ serum IGF-1 measurement, including the date of testing and laboratory reference range for age and sex, of less than 12 weeks old at the time of application.
Administrative Advice:	Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au Applications for authority to prescribe should be forwarded to: Department of Human Services Complex Drugs Programs Reply Paid 9826 HOBART TAS 7001 *Note* No increase in the maximum number of repeats may be authorised.

Category / Program	Section 100 – Growth Hormone Program
Prescriber type:	☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives
PBS Indication:	Severe growth hormone deficiency
Treatment phase:	Initial treatment
Restriction:	☐Restricted benefit ☒Authority Required - In Writing ☐Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	Must be treated by an endocrinologist.
Clinical criteria:	Patient must have a documented childhood onset growth hormone deficiency due to a congenital, genetic or structural cause; *AND* *Patient must have previously received non-PBS subsidised treatment with this drug for this condition as a child;* *AND* *Patient must have current or historical evidence of one of the following*: (i) ~~Patient must have~~ an insulin tolerance test with maximum serum growth hormone (GH) less than 2.5 µg/l;

	(ii) ~~Patient must have~~ an arginine infusion test with maximum serum GH less than 0.4 µg/l; (iii) ~~Patient must have~~ a glucagon provocation test with maximum serum GH less than 3 µg/l;
Population criteria:	~~Patient must be aged 18 years or older~~ *Patient must have a mature skeleton.*
Prescriber Instructions:	The authority application must be in writing and must include: 1. A completed authority prescription form; 2. A completed Severe Growth Hormone Deficiency supporting information form; 3. Confirmation of childhood onset growth hormone deficiency due to a congenital, genetic or structural cause, previous non-PBS therapy with this drug as a child, and a mature skeleton; 4. Results of the growth hormone stimulation testing, including the date of testing, the type of test performed, the peak growth hormone concentration, and laboratory reference range for age/gender; 5. A ~~baseline~~ serum IGF-1 measurement, including the date of testing and laboratory reference range for age and sex, of less than 12 weeks old at the time of application.~~; AND~~
Administrative Advice:	Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au Applications for authority to prescribe should be forwarded to: Department of Human Services Complex Drugs Programs Reply Paid 9826 HOBART TAS 7001 *Note* No increase in the maximum number of repeats may be authorised.

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PBS Indication:	Severe growth hormone deficiency
Treatment phase:	Initial treatment
Restriction:	☐Restricted benefit ☒Authority Required - In Writing ☐Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	Must be treated by an endocrinologist.

Clinical criteria:	Patient must have adult onset growth hormone deficiency secondary to organic hypothalamic or pituitary disease; *AND* *Patient must have current evidence of one of the following*; (i) ~~Patient must have~~ an insulin tolerance test with maximum serum growth hormone (GH) less than 2.5 µg/l; (ii) ~~Patient must have~~ an arginine infusion test with maximum serum GH less than 0.4 µg/l; (iii) ~~Patient must have~~ a glucagon provocation test with maximum serum GH less than 3 µg/l;
Population criteria:	Patient must be aged 18 years or older.
Prescriber Instructions:	The authority application must be in writing and must include: 1. A completed authority prescription form; 2. A completed Severe Growth Hormone Deficiency supporting information form; 3. Confirmation of adult onset growth hormone deficiency due to organic hypothalamic or pituitary disease; 4. Results of the growth hormone stimulation testing, including the date of testing, the type of test performed, the peak growth hormone concentration, and laboratory reference range for age/gender; 5. A baseline serum IGF-1 measurement, including the date of testing and laboratory reference range for age and sex, of less than 12 weeks old at the time of application. Grandfathered patient who has previously received non-PBS subsidised treatment with this drug for this condition prior to 1 December 2018 must have met all the initial restriction criteria prior to initiating non-PBS subsidised treatment. Additional information of a baseline serum IGF-1 measurement, including the date of testing and laboratory reference range for age and sex, of less than 12 weeks prior to initiating non-PBS subsidised treatment with this drug for this condition must be provided at the time of application. A Grandfathered patient may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a Grandfathered patient must qualify under the Continuing treatment criteria.
Administrative Advice:	Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au Applications for authority to prescribe should be forwarded to: Department of Human Services Complex Drugs Programs Reply Paid 9826 HOBART TAS 7001 *Note* No increase in the maximum number of repeats may be authorised.

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PBS Indication:	Severe growth hormone deficiency
Treatment phase:	Continuing treatment
Restriction:	☐Restricted benefit ☒Authority Required - In Writing ☐Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	Must be treated by an endocrinologist.
Clinical criteria:	Patient must have previously received PBS-subsidised therapy with this drug for this condition *under an* initial treatment *restriction applying to a documented childhood onset growth hormone deficiency due to a congenital, genetic or structural cause; or adult onset growth hormone deficiency secondary to organic hypothalamic or pituitary disease*; AND Patient must maintain IGF-1 levels within the normal range for age and sex.
~~**Population criteria:**~~	~~Patient must be aged 18 years or older.~~
Prescriber Instructions:	The authority application must be in writing and must include: 1. A completed authority prescription form; 2. A completed Severe Growth Hormone Deficiency supporting information form; 3. A serum IGF-1 measurement, including the date of testing and laboratory reference range for age and sex, of less than 12 weeks old at the time of application.
Administrative Advice:	Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au Applications for authority to prescribe should be forwarded to: Department of Human Services Complex Drugs Programs Reply Paid 9826 HOBART TAS 7001 *Note* No increase in the maximum number of repeats may be authorised.