14.08 SOMATROPIN,

Powder for injection 5 mg (15 i.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

1 Purpose of Item

1.1 To seek PBAC advice on the requirement for prescribers to use the Quality of Life in Adult Growth Hormone Deficiency Assessment (QoL-AGHDA) questionnaire in order to assess whether patients meet the eligibility criteria for the Section 100 (Growth Hormone) Authority Required listing of somatropin for the treatment of adults with severe growth hormone (GH) deficiency.

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 Somatropin was previously only available on the PBS for a range of paediatric indications.

2.3 The listing for adults with severe growth hormone deficiency was based on the recommendations from the PBAC at its July 2017 meeting.

2.4 In its April 2017 resubmission, the ESA claimed that the factors that contribute to the QoL of an adult with GH deficiency include a wide range of emotional, cognitive and social functioning areas, which generic QoL assessment tools are likely to miss important aspects of the patient's experience of the disease, consequently reducing reliability and responsiveness. The ESA noted that the QoL-AGHDA questionnaire was selected on the basis that it is the only disease specific instrument that has been developed for the assessment of GH deficiency in adults and is extensively used in the UK and New Zealand.

3 Current situation

3.1 On 28 December 2018, the PBAC Chair received correspondence from the ESA advising of a potential issue for some prescribers (endocrinologists) when assessing their patients against the eligibility criteria for somatropin, with particular regard to the QoL-AGHDA questionnaire.

3.2 In summary, the QoL-AGHDA questionnaire was developed by a UK company called Galen Research Ltd and they own the rights to its use. The cost of a non-commercial license, as is required in the Australian context, is US$150 per annum.

3.3 The ESA has signed a license agreement to allow its members to use the questionnaire and start prescribing somatropin, but are concerned that not all endocrinologists in Australia are members and therefore will not be unable to access the QoL-AGHDA questionnaire. If some endocrinologists cannot access the questionnaire then patients will not meet the restriction requirements resulting in a negative impact on eligibility of treatment.

4 PBAC Outcome

4.1 The PBAC recommended the removal of the requirement for prescribers to use the QoL-AGHDA questionnaire from the current somatropin restrictions for the treatment of severe growth hormone deficiency.

4.2 In making its decision, the PBAC noted that not all endocrinologists in Australia have access to the questionnaire given it is privately owned. Further, the PBAC considered there was some uncertainty around the validity and reliability of the questionnaire given it comprises a set of self-administered binary (yes/no) questions.

4.3 The PBAC considered that removal of the requirement would unlikely have a significant impact on utilisation as it considered that it was unlikely that patients who do not receive a benefit from somatropin would continue with a treatment involving a daily injection.

5 Recommended listing

5.1 Amend existing listings as follows:

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	1	5		

Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Proprietary Name and Manufacturer	
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; or Patient must have adult onset growth hormone deficiency secondary to organic hypothalamic or pituitary disease, AND Patient must have an insulin tolerance test with maximum serum growth hormone (GH) less than 2.5 micrograms per litre; or Patient must have an arginine infusion test with maximum serum GH less than 0.4 micrograms per litre; or Patient must have a glucagon provocation test with maximum serum GH less than 3 micrograms per litre, ~~AND~~ ~~Patient must have a quality of life (QoL) score on the Quality of Life Assessment of Growth Hormone Deficiency in Adults (QoL-AGHDA) instrument of 16 or greater,~~
Population criteria:	Patient must be aged 18 years or older.
Prescriber Instructions:	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~~; and QoL score on the QoL-AGHDA instrument of 16 or greater, 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. The authority application must be in writing and must include: A completed authority prescription form; AND A completed Severe Growth Hormone Deficiency supporting information form; AND Confirmation of childhood onset growth hormone deficiency due to a congenital, genetic or structural cause; OR Confirmation of adult onset growth hormone deficiency due to organic hypothalamic or pituitary disease; AND 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; AND 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~~ ~~The patient's QoL score on the QoL-AGHDA instrument, including the date of testing, 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 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:	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 at the age of 18 years or older, AND Patient must maintain IGF-1 levels within the normal range for age and sex, ~~AND~~ ~~Patient must maintain a quality of life (QoL) score on the Quality of Life Assessment of Growth Hormone Deficiency in Adults (QoL-AGHDA) instrument with a reduction of more than 7 points from baseline.~~
Population criteria:	Patient must be aged 18 years or older.
Prescriber Instructions:	The authority application must be in writing and must include: A completed authority prescription form; AND A completed Severe Growth Hormone Deficiency supporting information form; AND 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~~; AND~~ ~~The patient's QoL score on the QoL-AGHDA instrument, including the date of testing, 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 No increase in the maximum number of repeats may be authorised.