# 6.13 SOMATROPIN
## Solution for injection 6 mg (18 i.u.) in 1.03 mL cartridge (with preservative),
## Solution for injection 12 mg (36 i.u.) in 1.5 mL cartridge (with preservative),
## Solution for injection 20 mg (60 i.u.) in 2.5 mL cartridge (with preservative),
## Saizen®,
## Merck Serono Australia Pty Ltd.

### 1 Purpose of Application

1.1 The minor submission requested an Authority Required listing for the treatment of growth disturbance (growth retardation) in pre-pubertal children due to chronic renal insufficiency (CRI).

### 2 Requested listing

2.1 The submission requested listing with the same restrictions as for the brands of somatropin currently listed for the "treatment of growth retardation due to CRI" on the Section 100 Human Growth Hormone Program. This comprises restrictions for the following treatment phases:

- Initial treatment
- Continuing treatment
- Continuing treatment as a reclassified patient
- Recommencement of treatment
- Recommencement of treatment as a reclassified patient

2.2 The new TGA registered indication for Saizen® is for "growth disturbance (growth retardation) in pre-pubertal children due to CRI", which is broadly consistent with the TGA registered indications for other PBS-listed somatropin brands, which is "for treatment of growth disturbance in children with CRI".

2.3 The submission requested the same listing as the currently listed brands for "growth retardation due to CRI"; however, the PBS listed indication is for "short stature due to CRI". The secretariat proposed that the current wording for the other PBS-listed brands apply for the purposes of consistency.

*For more detail on PBAC's view, see section 6 "PBAC outcome"*

### 3 Background

3.1 Somatropin was first TGA registered in 1992. Saizen® received TGA registration for the *"treatment of growth disturbance in pre-pubertal children due to CRI"* in September 2015, after an initial application was withdrawn in 2000 due to inadequate evidence on efficacy.

3.2 Saizen® received TGA designation as an Orphan Drug for the proposed indication in September 2011.

3.3 The PBAC has not considered Saizen® for the treatment of growth disturbance due to CRI for this particular brand.

3.4 Saizen® is currently listed for the following indications:

- Short stature and slow growth
- Short stature associated with biochemical growth hormone deficiency
- Growth retardation secondary to an intracranial lesion, or cranial irradiation
- Risk of hypoglycaemia secondary to growth hormone deficiency in neonates/infants
- Biochemical growth hormone deficiency and precocious puberty
- Hypothalamic-pituitary disease secondary to a structural lesion, with hypothalamic obesity driven growth
- Short stature associated with Turner syndrome
- Short stature due to short stature homeobox (SHOX) gene disorders

3.5 The following brands of somatropin are currently listed on the Section 100 Human Growth Program for the treatment of "short stature associated with CRI":

- Genotropin®
- Omnitrope®
- Humatrope®
- Norditropin®
- Nutropin®

*For more detail on PBAC's view, see section 7 "PBAC outcome"*

## 4 Comparator

4.1 As a minor submission, there was no economic comparison.

4.2 The PBAC noted that Saizen® has the same recommended daily dosage, 0.045-0.05 mg/kg body weight or 1.4 mg/m$^2$ body surface area (BSA), as other somatropin brands PBS-listed for this indication.

## 5 Consideration of the evidence

### *Sponsor hearing*

5.1 There was no hearing for this item as it was a minor submission.

### *Consumer comments*

5.2 The PBAC noted that no consumer comments were received for this item

### *Clinical trials*

5.3 As a minor submission, no clinical trials were presented in the submission.

5.4 The basis of the minor submission's request was to present changes to the approved Product Information which now includes the requested restriction.

***Estimated PBS usage & financial implications***

5.5 The PBAC considered that there would be no financial implications to the PBS as Saizen® is expected to substitute for other brands of somatropin, which have an equivalent price per milligram ($[redacted]/mg).

*For more detail on PBAC's view, see section 6 "PBAC outcome"*

## 6 PBAC Outcome

6.1 The PBAC recommended the listing of Saizen® to include an Authority Required listing for the treatment of 'short stature associated with chronic renal insufficiency (CRI)', consistent with the currently listed brands of somatropin, on the basis that it should be available only under special arrangements under Section 100 Human Growth Hormone Program, at an equivalent price per milligram as other brands of somatropin.

6.2 The PBAC recommended that Saizen® for the treatment of CRI should be treated as interchangeable on an individual patient basis with the other brands of somatropin listed for short stature associated with CRI.

6.3 The PBAC noted that there would be no financial impact as a result of this listing, as Saizen will substitute for other brands of somatropin currently listed for this indication, at the same price per mg.

6.4 The PBAC noted that this submission is not eligible for an Independent Review as it was recommended.

**Outcome:**
Recommended

## 7 Recommended listing

7.1 Amend existing listing as follows:

| Name, Restriction, Manner of administration and form | Max. Qty | №.of Rpts | Proprietary Name and Manufacturer | |
|---|---|---|---|---|
| SOMATROPIN | | | | |
| 60 units (20 mg/2.5 mL) injection: solution, 2.5 mL cartridge | 1 | 1 | Saizen® | Merck Serono Australia Pty Ltd |
| 18 units (6 mg/1.03 mL) injection, 1.03 mL cartridge | 1 | 1 | Saizen® | |
| 36 units (12 mg/1.5 mL) injection, 1.5 mL cartridge | 1 | 1 | Saizen® | |

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| **Category / Program** | Section 100 – Growth Hormone Programme |
| **Prescriber type:** | ☐ Dental ☒ Medical Practitioners ☐ Nurse practitioners ☐ Optometrists ☐ Midwives |
| **Condition:** | Short stature associated with chronic renal insufficiency |
| **PBS Indication:** | Short stature associated with chronic renal insufficiency |
| **Treatment phase:** | Initial |
| **Restriction Level / Method:** | ☐ Restricted benefit<br>☒ Authority Required - In Writing<br>☐ Authority Required - Telephone<br>☐ Authority Required – Emergency<br>☐ Authority Required - Electronic<br>☐ Streamlined |
| **Treatment criteria:** | • Must be treated by a specialist or consultant physician in paediatric endocrinology; OR<br>• 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 25th percentile for age and sex,<br>AND<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ measured by creatinine clearance, excretion of radionuclides such as DTPA, or by the height/creatinine formula, and not have undergone a renal transplant; OR<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ 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,<br>AND<br>• Patient must be male, have a chronological age of at least 12 years and a growth velocity equal to or less than the 25th percentile for bone age and sex measured over a 6 month interval; OR<br>• Patient must be male, have a bone age of at least 10 years and a growth velocity equal to or less than the 25th percentile for bone age and sex measured over a 6 month interval; OR<br>• Patient must be female, have a chronological age of at least 10 years and a growth velocity equal to or less than the 25th percentile for bone age and sex measured over a 6 month interval; OR<br>• Patient must be female, have a bone age of at least 8 years and a growth velocity equal to or less than the 25th percentile for bone age and sex measured over a 6 month interval; OR |

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| | • Patient must have a growth velocity equal to or less than the 25th percentile for bone age and sex measured over both 12 and 6 month intervals; OR<br>• Patient must have a bone age of 2.5 years or less and an annual growth velocity of 8 cm per year or less,<br>AND<br>• Patient must not have diabetes mellitus,<br>AND<br>• Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,<br>AND<br>• Patient must not have an active tumour or evidence of tumour growth or activity,<br>AND<br>• Patient must not have previously received treatment under the PBS S100 Growth Hormone Program,<br>AND<br>• Patient must be male and must not have a height greater than or equal to 167.7 cm; OR<br>• Patient must be female and must not have a height greater than or equal to 155.0 cm,<br>AND<br>• Patient must be male and must not have a bone age of 15.5 years or more; OR<br>• 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. |
| **Prescriber Instructions** | 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).<br><br>The authority application must be in writing and must include:<br>1. A completed authority prescription form; AND<br>2. A completed Growth Hormone Authority Application Supporting Information Form for initial treatment; AND<br>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<br>(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<br>4. A bone age result performed within the last 12 months; AND<br>5. Confirmation that the patient has an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ ; AND<br>6. If a renal transplant has taken place, confirmation that the patient has undergone a 12 month period of observation following transplantation; AND<br>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). |

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| **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).<br><br>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<br><br>Applications for authority to prescribe should be forwarded to:<br><br>Department of Human Services<br>Prior Written Approval of Complex Drugs<br>Reply Paid 9826<br>HOBART TAS 7001 |

| **Name, Restriction, Manner of administration and form** | **Max. Qty** | **№.of Rpts** | **Proprietary Name and Manufacturer** |
|---|---|---|---|
| SOMATROPIN | | | |
| 18 units (6 mg/1.03 mL) injection, 1.03 mL cartridge | 1 | 1 | Saizen® Merck Serono Australia Pty Ltd |
| 36 units (12 mg/1.5 mL) injection, 1.5 mL cartridge | 1 | 1 | Saizen® |
| 60 units (20 mg/2.5 mL) injection, 2.5 mL cartridge | 1 | 1 | Saizen® |

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| **Category / Program** | Section 100 – Growth Hormone Programme |
| **Prescriber type:** | ☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives |
| **Condition:** | Short stature associated with chronic renal insufficiency |
| **PBS Indication:** | Short stature associated with chronic renal insufficiency |
| **Treatment phase:** | Continuing treatment |
| **Restriction Level / Method:** | ☐Restricted benefit<br>☒Authority Required - In Writing<br>☐Authority Required - Telephone<br>☐Authority Required – Emergency<br>☐Authority Required - Electronic<br>☐Streamlined |
| **Treatment criteria:** | • Must be treated by a specialist or consultant physician in paediatric endocrinology; OR<br>• 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 previously received treatment under the PBS S100 Growth Hormone Program under the short stature associated with chronic renal insufficiency category,<br>AND |

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| | • Patient must not have been on the maximum dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• Patient must have achieved the 50th percentile growth velocity for bone age and sex while on the maximum dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• Patient must have achieved an increase in height standard deviation score for chronological age and sex while on the maximum dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• Patient must have achieved a minimum growth velocity of 4cm/year while on the maximum dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• Patient must have achieved and maintained mid parental height standard deviation score while on the maximum dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies),<br>AND<br>• Patient must not have diabetes mellitus,<br>AND<br>• Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,<br>AND<br>• Patient must not have an active tumour or evidence of tumour growth or activity,<br>AND<br>• Patient must not have undergone a renal transplant within the 12 month period immediately prior to the date of application,<br>AND<br>• Patient must not have an eGFR equal to or greater than 30mL/min/1.73m$^2$,<br>AND<br>• Patient must be male and must not have a height greater than or equal to 167.7 cm; OR<br>• Patient must be female and must not have a height greater than or equal to 155.0 cm,<br>AND<br>• Patient must be male and must not have a bone age of 15.5 years or more; OR<br>• 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. |

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| **Prescriber Instructions** | The maximum duration of each continuing treatment phase is 26 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 13 weeks' worth of treatment (with up to 1 repeat allowed).<br><br>The authority application must be in writing and must include:<br>1. A completed authority prescription form; AND<br>2. A completed Growth Hormone Authority Application Supporting Information Form for continuing treatment; AND<br>3. Growth data (height and weight) for the most recent 6 month treatment period, including data at both the start and end of the treatment period. The most recent data must not be older than three months; AND<br>4. A bone age result performed within the last 12 months; AND<br>5. The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 13 weeks' worth of treatment (with up to 1 repeat allowed).<br><br>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. |
| **Administrative Advice** | If a patient receiving treatment under the indication short stature due to chronic renal insufficiency undergoes a renal transplant and 12 months post-transplant has an eGFR of equal to or greater than 30mL/min/1.73m$^2$ prescribers should seek reclassification to the indication short stature and slow growth.<br><br>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).<br><br>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<br><br>Applications for authority to prescribe should be forwarded to:<br><br>Department of Human Services<br>Prior Written Approval of Complex Drugs<br>Reply Paid 9826<br>HOBART TAS 7001 |

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| **Category / Program** | Section 100 – Growth Hormone Programme |
| **Prescriber type:** | ☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives |
| **Condition:** | Short stature associated with chronic renal insufficiency |
| **PBS Indication:** | Short stature associated with chronic renal insufficiency |
| **Treatment phase:** | Continuing treatment as reclassified patient |
| **Restriction Level / Method:** | ☐Restricted benefit<br>☒Authority Required - In Writing<br>☐Authority Required - Telephone<br>☐Authority Required – Emergency<br>☐Authority Required - Electronic<br>☐Streamlined |
| **Treatment criteria:** | • Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in paediatric endocrinology; OR<br>• Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in general paediatrics. |
| **Clinical criteria:** | • Patient must have previously received treatment under the PBS S100 Growth Hormone Program under a category other than short stature associated with chronic renal insufficiency,<br>AND<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by a significant medical illness; OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by major surgery (e.g. renal transplant); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by an adverse reaction to growth hormone; OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by non-compliance due to social/family problems,<br>AND<br>• Patient must be male, had a chronological age of at least 12 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be male, had a bone age of at least 10 years at commencement of |

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| | growth hormone treatment, a growth velocity equal to or less than the 25thpercentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25th percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be female, had a chronological age of at least 10 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25th percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25th percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be female, had a bone age of at least 8 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25thpercentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25th percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must have had a growth velocity equal to or less than the 25th percentile for bone age and sex measured over both the 12 month and 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25th percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must have had a bone age of 2.5 years or less immediately prior to commencement of growth hormone treatment, an annual growth velocity of 8cm per year or less in the twelve month period immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25thpercentile for age and sex immediately prior to commencing growth hormone treatment,<br>AND<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ measured by creatinine clearance, excretion of radionuclides such as DTPA, or by the height/creatinine formula, and not have undergone a renal transplant; OR<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ 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,<br>AND<br>• Patient must not have diabetes mellitus,<br>AND<br>• Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,<br>AND<br>• Patient must not have an active tumour or evidence of tumour growth or activity,<br>AND<br>• Patient must be male and must not have a height greater than or equal to 167.7cm; OR<br>• Patient must be female and must not have a height greater than or equal to 155.0cm,<br>AND<br>• Patient must be male and must not have a bone age of 15.5 years or more; OR<br>• 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. |

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| **Prescriber Instructions** | The maximum duration of each continuing treatment phase is 26 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 13 weeks' worth of treatment (with up to 1 repeat allowed).<br><br>The authority application must be in writing and must include:<br>1. A completed authority prescription form; AND<br>2. A completed Growth Hormone Authority Application Supporting Information Form for continuing treatment as a reclassified patient; AND<br>3. (a) A minimum of 12 months of growth data (height and weight) from immediately prior to commencement of growth hormone treatment, at intervals no greater than six months, and a bone age result performed within the 12 months immediately prior to commencement of growth hormone treatment; OR<br>(b) If the patient was an older child (males chronological age 12 and over or bone age 10 and over, females chronological age 10 and over or bone age 8 and over) at the time of commencement of growth hormone treatment, a minimum of 6 months of growth data (height and weight) from immediately prior to commencement of growth hormone treatment, and a bone age result performed within the 12 months immediately prior to commencement of growth hormone treatment; AND<br>4. Confirmation that the patient has an estimated glomerular filtration rate less than 30ml/minute/1.73m$^2$ ; AND<br>5. If a renal transplant has taken place, confirmation that the patient has undergone a 12 month period of observation following transplantation; AND<br>6. Growth data (height and weight) for the most recent 6 month treatment period, including data at both the start and end of the treatment period. The most recent data must not be older than three months; AND<br>7. A bone age result performed within the last 12 months; AND<br>The proprietary name (brand), form and strength of somatropin requested, and the number of vials/cartridges required to provide sufficient drug for 13 weeks worth of treatment (with up to 1 repeat allowed).<br><br>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. |
| **Administrative Advice** | If a patient receiving treatment under the indication short stature due to chronic renal insufficiency undergoes a renal transplant and 12 months post-transplant has an eGFR of equal to or greater than 30mL/min/1.73m$^2$ prescribers should seek reclassification to the indication short stature and slow growth.<br><br>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).<br><br>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<br><br>Applications for authority to prescribe should be forwarded to:<br><br>Department of Human Services<br>Prior Written Approval of Complex Drugs<br>Reply Paid 9826<br>HOBART TAS 7001 |

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| **Category / Program** | Section 100 – Growth Hormone Programme |
| **Prescriber type:** | ☐ Dental ☒ Medical Practitioners ☐ Nurse practitioners ☐ Optometrists ☐ Midwives |
| **Condition:** | Short stature associated with chronic renal insufficiency |
| **PBS Indication:** | Short stature associated with chronic renal insufficiency |
| **Treatment phase:** | Recommencement of treatment |
| **Restriction Level / Method:** | ☐ Restricted benefit<br>☒ Authority Required - In Writing<br>☐ Authority Required - Telephone<br>☐ Authority Required – Emergency<br>☐ Authority Required - Electronic<br>☐ Streamlined |
| **Treatment criteria:** | • Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in paediatric endocrinology; OR<br>• Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in general paediatrics. |
| **Clinical criteria:** | • Patient must have previously received treatment under the PBS S100 Growth Hormone Program under the short stature associated with chronic renal insufficiency category,<br>AND<br>• Patient must have had a lapse in growth hormone treatment,<br>AND<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by a significant medical illness; OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by major surgery (e.g. renal transplant); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at |

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| | a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by an adverse reaction to growth hormone; OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by non-compliance due to social/family problems,<br>AND<br>• Patient must not have diabetes mellitus,<br>AND<br>• Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,<br>AND<br>• Patient must not have an active tumour or evidence of tumour growth or activity,<br>AND<br>• Patient must not have undergone a renal transplant within the 12 month period immediately prior to the date of application,<br>AND<br>• Patient must not have an eGFR equal to or greater than 30mL/min/1.73m$^2$,<br>AND<br>• Patient must be male and must not have a bone age of 15.5 years or more; OR<br>• Patient must be female and must not have a bone age of 13.5 years or more,<br>AND<br>• Patient must be male and must not have a height greater than or equal to 167.7cm; OR<br>• Patient must be female and must not have a height greater than or equal to 155.0cm. |
| **Population criteria:** | Patient must be aged 3 years or older. |

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| **Prescriber Instructions** | The maximum duration of each recommencement 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).<br><br>The authority application must be in writing and must include:<br>1. A completed authority prescription form; AND<br>2. A completed Growth Hormone Authority Application Supporting Information Form for recommencement of treatment; AND<br>3. Recent growth data (height and weight, not older than three months); AND<br>4. A bone age result performed within the last 12 months; AND<br>5. Confirmation that the patient has an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ ; AND<br>6. If a renal transplant has taken place, confirmation that the patient has undergone a 12 month period of observation following transplantation; AND<br>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).<br><br>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.<br><br>If a patient receiving treatment under the indication 'short stature associated with chronic renal insufficiency' undergoes a renal transplant and 12 months post-transplant has an eGFR of equal to or greater than 30mL/min/1.73m$^2$ prescribers should seek reclassification to the indication short stature and slow growth. |
| **Administrative Advice** | If recommencement of treatment is sought under a different indication than that under which the patient was previously receiving treatment an application for *recommencement of treatment as a reclassified patient* should be submitted.<br><br>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).<br><br>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<br><br>Applications for authority to prescribe should be forwarded to:<br><br>Department of Human Services<br>Prior Written Approval of Complex Drugs<br>Reply Paid 9826<br>HOBART TAS 7001 |

| Name, Restriction, Manner of administration and form | Max. Qty | №.of Rpts | Proprietary Name and Manufacturer | |
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| SOMATROPIN | | | | |
| 60 units (20 mg/2.5 mL) injection, 2.5 mL cartridge | 1 | 1 | Saizen® | Merck Serono Australia Pty Ltd |
| 18 units (6 mg/1.03 mL) injection, 1.03 mL cartridge | 1 | 1 | Saizen® | |
| 36 units (12 mg/1.5 mL) injection, 1.5 mL cartridge | 1 | 1 | Saizen® | |

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| **Category / Program** | Section 100 – Growth Hormone Programme |
| **Prescriber type:** | ☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives |
| **Condition:** | Short stature associated with chronic renal insufficiency |
| **PBS Indication:** | Short stature associated with chronic renal insufficiency |
| **Treatment phase:** | Recommencement of treatment as a reclassified patient |
| **Restriction Level / Method:** | ☐Restricted benefit<br>☒Authority Required - In Writing<br>☐Authority Required - Telephone<br>☐Authority Required – Emergency<br>☐Authority Required - Electronic<br>☐Streamlined |
| **Treatment criteria:** | • Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in paediatric endocrinology; OR<br>• Must be treated by a medical practitioner in consultation with a nominated specialist or consultant physician in general paediatrics. |
| **Clinical criteria:** | • Patient must have previously received treatment under the PBS S100 Growth Hormone Program under a category other than short stature associated with chronic renal insufficiency,<br>AND<br>• Patient must have had a lapse in growth hormone treatment,<br>AND<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by a significant medical illness; OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by major surgery (e.g. renal transplant); OR<br>• The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by an adverse reaction to growth hormone; OR |

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| | • The treatment must not have lapsed due to failure to respond to growth hormone at a dose of 9.5mg/m$^2$/week or greater for the most recent treatment period (32 weeks for an initial or recommencement treatment period and 26 weeks for a continuing treatment period, whichever applies), unless response was affected by non-compliance due to social/family problems,<br>AND<br>• Patient must be male, had a chronological age of at least 12 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be male, had a bone age of at least 10 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be female, had a chronological age of at least 10 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must be female, had a bone age of at least 8 years at commencement of growth hormone treatment, a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over the 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must have had a growth velocity equal to or less than the 25$^{th}$ percentile for bone age and sex measured over both the 12 month and 6 month interval immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$percentile for age and sex immediately prior to commencing growth hormone treatment; OR<br>• Patient must have had a bone age of 2.5 years or less immediately prior to commencement of growth hormone treatment, an annual growth velocity of 8cm per year or less in the twelve month period immediately prior to commencement of growth hormone treatment, and a height equal to or less than the 25$^{th}$ percentile for age and sex immediately prior to commencing growth hormone treatment,<br>AND<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ measured by creatinine clearance, excretion of radionuclides such as DTPA, or by the height/creatinine formula, and not have undergone a renal transplant; OR<br>• Patient must have an estimated glomerular filtration rate less than 30mL/minute/1.73m$^2$ 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,<br>AND<br>• Patient must not have diabetes mellitus,<br>AND<br>• Patient must not have a condition with a known risk of malignancy including chromosomal abnormalities such as Down and Bloom syndromes,<br>AND |

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| | • Patient must not have an active tumour or evidence of tumour growth or activity,<br>AND<br>• Patient must be male and must not have a height greater than or equal to 167.7cm; OR<br>• Patient must be female and must not have a height greater than or equal to 155.0cm,<br>AND<br>• Patient must be male and must not have a bone age of 15.5 years or more; OR<br>• 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. |
| **Prescriber Instructions** | The maximum duration of each recommencement 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).<br><br>The authority application must be in writing and must include:<br>1. A completed authority prescription form; AND<br>2. A completed Growth Hormone Authority Application Supporting Information Form for recommencement of treatment as a reclassified patient; AND<br>3. (a) A minimum of 12 months of growth data (height and weight) from immediately prior to commencement of growth hormone treatment, at intervals no greater than six months, and a bone age result performed within the 12 months immediately prior to commencement of growth hormone treatment; OR<br>(b) If the patient was an older child (males chronological age 12 and over or bone age 10 and over, females chronological age 10 and over or bone age 8 and over) at the time of commencement of growth hormone treatment, a minimum of 6 months of growth data (height and weight) from immediately prior to commencement of growth hormone treatment, and a bone age result performed within the 12 months immediately prior to commencement of growth hormone treatment; AND<br>4. Confirmation that the patient has an estimated glomerular filtration rate less than 30mL/minute/1.73$m^2$ ; AND<br>5. If a renal transplant has taken place, confirmation that the patient has undergone a 12 month period of observation following transplantation; AND<br>6. Recent growth data (height and weight, not older than three months); AND<br>7. A bone age result performed within the last 12 months; AND<br>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).<br><br>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.<br><br>If a patient receiving treatment under the indication short stature due to chronic renal insufficiency undergoes a renal transplant and 12 months post-transplant has an eGFR of equal to or greater than 30mL/min/1.73$m^2$ prescribers should seek reclassification to the indication short stature and slow growth. |

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| **Administrative Advice** | If a patient receiving treatment under the indication short stature due to chronic renal insufficiency undergoes a renal transplant and 12 months post-transplant has an eGFR of equal to or greater than 30mL/min/1.73m$^2$ prescribers should seek reclassification to the indication short stature and slow growth.<br><br>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).<br><br>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<br><br>Applications for authority to prescribe should be forwarded to:<br><br>Department of Human Services<br>Prior Written Approval of Complex Drugs<br>Reply Paid 9826<br>HOBART TAS 7001 |

## 8 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised in Australia. It considers submissions in this context. A PBAC decision not to recommend listing or not to recommend changing a listing does not represent a final PBAC view about the merits of the medicine. A company can resubmit to the PBAC or seek independent review of the PBAC decision.

## 9 Sponsor's Comment

Merck is pleased that the PBAC recognises that Saizen provides an alternative treatment option for patients with growth retardation due to CRI.