EXPLANATORY STATEMENT

National Health Act 1953

National Health (Growth Hormone Program) Special Arrangement Amendment (Mecasermin and Somatrogon) Instrument 2022

PB 92 of 2022

Authority

The Pharmaceutical Benefits Scheme (PBS) is established under the *National Health Act 1953* (the Act) and provides Australians with timely, reliable and affordable access to necessary and cost-effective medicines. The Act regulates the listing, pricing, prescribing, supply, and claiming and payment of subsidies for supply of drugs and medicinal preparations as pharmaceutical benefits.

Subsection 100(1) of the Act provides that the Minister may make special arrangements for, or in relation to, providing that an adequate supply of pharmaceutical benefits will be available to certain persons. These are persons: who are living in isolated areas; or who are receiving treatment in circumstances in which pharmaceutical benefits are inadequate for that treatment; or if the pharmaceutical benefits covered by the arrangements can be more conveniently or efficiently supplied under the arrangements.

Subsection 100(2) of the Act provides that the Minister may vary or revoke a special arrangement made under subsection 100(1).

Subsection 100(3) of the Act provides that Part VII of the Act, and instruments made for the purposes of Part VII, have effect subject to a special arrangement made under subsection 100(1).

Purpose

The *National Health (Growth Hormone Program) Special Arrangement Amendment (Mecasermin and Somatrogon) Instrument 2022* (the Amendment Instrument) amends the *National Health (Growth Hormone Program) Special Arrangement 2015* (PB 85 of 2015) (the Growth Hormone Special Arrangement). The purpose of the amendments is to include provision for the supply of two new medicines (mecasermin and somatrogon) for the treatment of children with particular conditions, consistent with recommendations of the Pharmaceutical Benefits Advisory Committee (PBAC) made in March 2022.

The Amendment Instrument provides for the following changes:

- the addition of two new definitions in section 4 of the Growth Hormone Special Arrangement for an '*authorised prescriber*' to account for the two new treatment phases for somatrogon, somatropin and mecasermin;
- the addition of a new definition for '*changing drug treatment phase for a child'* to allow patients to move from somatropin to somatrogon and somatrogon to somatropin, where being prescribed for a common indication;
- the addition of a new definition for *'pharmaceutical benefit has a drug'* for ease when applying to particular drugs without having to refer to table items in Schedule 1;
- specific references to mecasermin, somatrogon and somatropin, to clarify which treatment phases, dosing regimens, maximum quantities and maximum number of repeats apply to a particular pharmaceutical benefit that has these drugs. Currently the Growth Hormone Special Arrangement operates so that only somatropin can be prescribed.
- the repeal and substitution of Section 11 to remove duplication as well as including a new table to incorporate the dosing restrictions that authorised prescribers must comply with when prescribing mecasermin and somatrogon.

The amendments made by the Amendment Instrument are consistent with amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2012* (the Listing Instrument), which provides for the addition of pharmaceutical benefits containing the drugs mecasermin, and somatrogon, which commence on the same day. The Listing Instrument is made under sections 84AF, 84AK, 85, 85A, 88 and 101 of the Act.

Separate amendments to the *National Health (Growth Hormone Program) Special Arrangement 2015* to include the addition of the two new drugs to Schedule 1, and the amendments being made to list pharmaceutical benefits containing the two new drugs in the Listing Instrument, has been undertaken and these amending instruments will be registered to commence on 1 October 2022.

Background

Somatropin is currently the only medicine available for supply under the Pharmaceutical Benefits Scheme (PBS) Growth Hormone Program for the treatment of a range of paediatric and adult growth hormone deficiency (GHD) conditions.

At its March 2022 meeting, the Pharmaceutical Benefits Advisory Committee (PBAC) recommended two new medicines be made available on the Growth Hormone Program to paediatric and adolescent patients – mecasermin (Increlex®) and somatrogon (Ngenla®).

The Growth Hormone Special Arrangement details the dose restrictions for children and adults that authorised prescribers must comply with when prescribing the pharmaceutical benefit for each condition during the various treatment phases. Currently, these dosing restrictions apply to somatropin only.

Both mecasermin and somatrogon have different upper dosing limits/requirements to the already listed somatropin, for children.

Somatrogon

The PBAC recommended the listing of somatrogon for the treatment of paediatric patients with (1) short stature associated with biochemical growth hormone deficiency, or (2) short stature and slow growth. The PBS restrictions for somatrogon are consistent with the current restrictions for the somatropin brand Genotropin ® (currently five treatment phases) where the stated PBS indication is in common, however it includes two new treatment phases:

- changing drug treatment phase; and
- non-PBS subsidised to PBS subsidised supply treatment phase.

The PBAC noted the consumer comments which noted the improved patient satisfaction and treatment convenience of weekly dosing with somatrogon compared to daily dosing with somatropin. The PBAC agreed with the consumer comments that there is clinical need for a once-weekly growth hormone treatment for patients with paediatric GHD.

Mecasermin

The PBAC recommended the listing of mecasermin for the long-term treatment of growth failure in children and adolescents from 2 years to 18 years with severe primary insulin-like growth factor 1 deficiency (Primary IGFD). This is a genetic condition. Mecasermin is administered via subcutaneous injection. Mecasermin is available for three treatment phases (initial treatment phase, continuing treatment phase and non-PBS subsidised to PBS subsidised supply treatment phase). The PBAC considered that mecasermin would address a high and urgent unmet clinical need, as there are no other reimbursed or Therapeutic Goods Administration (TGA) registered options for this patient population.

Consultation

The amendments accord with recommendations made by the PBAC.

The PBAC conducted targeted consultation with members of the Endocrine Society of Australia and the Australasian Paediatric Endocrine Group (APEG) regarding eligibility criteria, dosage regimes and authorised prescribers for access to somatrogon and mecasermin.

The involvement of interested parties through the membership of the PBAC constitutes a formal and ongoing process of consultation in relation to matters relevant to the Growth Hormone Special Arrangement.

The PBAC is an independent expert body, established by section 100A of the Act, which makes recommendations to the Minister about which drugs and medicinal preparations should be available to Australians as pharmaceutical benefits.

PBAC members are appointed following nomination by prescribed organisations and associations from consumers, health economists, practising community pharmacists, general practitioners, clinical pharmacologists and specialists, with at least one member selected from each of these interests or professions. Remaining members are persons whom the Minister is satisfied have qualifications and experience in a field relevant to the functions of the PBAC, and that would enable them to contribute meaningfully to the deliberations of the PBAC. In addition, an industry nominee has been appointed to the PBAC membership under the PBS Access and Sustainability Package of reforms announced in May 2015. When recommending the listing of a medicine on the PBS, the PBAC takes into account the medical condition for which listing is sought and the medicine's clinical effectiveness, safety and cost-effectiveness compared with other treatments. Pharmaceutical companies are consulted throughout the process of the listing of their medicines on the PBS and in relation to changes to those listings. This includes consultation through the PBAC process.

Further consultation on the Amendment Instrument was considered unnecessary due to the nature of the consultation that had already taken place throughout the listing process.

This instrument commences on 1 October 2022.

This instrument is a legislative instrument for the purposes of the *Legislation Act 2003.*

Details of this instrument are set out in the Attachment.

DETAILS OF THE NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT AMENDMENT (MECASERMIN AND SOMATROGON) INSTRUMENT 2022

Section 1 Name

This section provides the name of the instrument is the *National Health (Growth Hormone Program) Special Arrangement Amendment (Mecasermin and Somatrogon) Instrument 2022*. It can also be cited as PB 92 of 2022.

Section 2 Commencement

This section provides that the Amendment Instrument commences on 1 October 2022.

Section 3 Authority

This section provides that the Amendment Instrument is made under subsection 100(2) of the *National Health Act 1953* (the Act).

Section 4 Schedules

This section provides that each instrument that is specified in a Schedule to the Amendment Instrument is amended or repealed as set out in the applicable items in the Schedule concerned, and any other item in a Schedule to the Amendment Instrument has effect according to its terms.

Schedule 1 – Amendments

Schedule 1 sets out the amendments to the *National Health (Growth Hormone Program) Special Arrangement 2015* (the Growth Hormone Special Arrangement) made by the Amendment Instrument.

Items 1- 3 – Subsection 4(1) Definitions

Item 1 inserts two new definitions of "authorised prescriber" in subsection 4(1) of the Growth Hormone Special Arrangement. This reflects the implementation of two new treatment phases: (ca) '*changing drug treatment phase*' and (cb) '*non-PBS subsidised to PBS subsidised supply treatment phase*'. The changing drug treatment phase applies to somatrogon or somatropin (see item 2). The non-PBS subsidised to PBS subsidised supply treatment phase applies to mecasermin or somatrogon.

Item 2 inserts a new definition of '*changing drug treatment phase for a child'* to allow for a child to move from somatropin to somatrogon and somatrogon to somatropin. Allowing a child to move from somatropin to somatrogon means that instead of having daily injections (somatropin) they can have weekly injections (somatrogon).

Item 3 inserts the definition of "*pharmaceutical benefit has a drug"* to state that it has the same meaning as in Part VII of the Act. It is included so that provisions of the instrument can be limited in their application to particular drugs without referring to table items of Schedule 1 - as Schedule 1 does not include numbered table items by which the reader could cross-refer to particular drugs without naming them.

Item 4 – Sections 8 and 9

Item 4 repeals and substitutes Sections 8 and 9. Section 8 is repealed and substituted in order to specify the maximum quantity or number of units of the pharmaceutical benefit that may be directed to be supplied by an authorised prescriber when treating a child with somatropin, somatrogon or mecasermin during the different treatment phases.

Subsections 8(1) to 8(4) apply to somatrogon and somatropin. For somatrogon and somatropin the maximum quantity that may be directed to be supplied in one prescription for a child is an amount that is sufficient for either 13 or 16 weeks depending on the treatment phase. Subsections 8(1) to (4) read in conjunction with section 9, which allows for a maximum of one repeat, provides that the total quantity of somatrogon and somatropin that an authorised prescriber may prescribe is up to 26 weeks or 32 weeks for each treatment phase.

Subsection 8(5) applies to mecasermin. The maximum quantity that an authorised prescriber can prescribe for a pharmaceutical benefit that has the drug mecasermin in any treatment phase is an amount sufficient for one month of treatment (see section 9 for maximum number of repeats).

Item 4 also repeals and substitutes section 9 in order to specify the maximum number of repeats that an authorised prescriber may include on the prescription when treating a child for the different treatment phases of somatropin and somatrogon (subsection 9(1)) and mecasermin (subsection 9(2)).

Item 5 – Section 10 (heading)

Item 5 removes the current heading 'Definitions' to avoid confusion around the different dosing regimens for mecasermin, somatrogon and somatropin when an authorised prescriber is treating children. It is substituted with the new heading 'Definitions relating to doses of somatropin' to clearly specify to authorised prescribers that the definitions are specific to somatropin only.

Item 6 – Subsection 10(1)

Item 6 removes the words "In this Part" and replaces them with "For the purposes of table items 1 to 4 of the table in section 11". This limits the application of the definition of 'dose' in subsection 10(1) to somatropin.

Item 7 - Subsection 10(2)

Item 7 removes the word "The" and replaces it with "For the purposes of table items 1 to 3 of the table in section 11, the". This clarifies that the measurement defined in subsection 10(2) only relates to the doses of somatropin for the particular conditions listed in table items 1 to 3 in Section 11. The table in Section 11 has been completely redrafted to include the two new pharmaceutical benefits - mecasermin and somatrogon (see item 9).

Item 8 – Section 10(3)

Item 8 removes the word "The" and replaces it with "For the purposes of table item 4 ('somatropin') of the table in section 11, the". This clarifies that the measurement 'kg' defined in subsection 10(3) only relates to the doses of somatropin for the particular condition listed in table item 4 in Section 11.

Item 9 – Section 11

Item 9 repeals and substitutes Section 11, which details the dose restrictions that authorised prescribers must comply with when prescribing the pharmaceutical benefit for each condition during the relevant treatment phase. The table in Section 11 includes the two new dosing restrictions for mecasermin and somatrogon. A new column titled 'Drug' has been added to the table in Section 11 to identify which dosing requirements apply to pharmaceutical benefits containing each drug. Subsection 11(4) has been removed as it was a duplication of 10(2)(b) and 10(3)(b).

Statement of Compatibility with Human Rights

Prepared in accordance with Part 3 of the Human Rights (Parliamentary Scrutiny) Act 2011

National Health (Growth Hormone Program) Special Arrangement Amendment (Mecasermin and Somatrogon) Instrument 2022

This Legislative Instrument is compatible with the human rights and freedoms recognised or declared in the international instruments listed in section 3 of the *Human Rights (Parliamentary Scrutiny) Act 2011*.

Overview of the Legislative Instrument

The *National Health (Growth Hormone Program) Special Arrangement Amendment (Mecasermin and Somatrogon) Instrument 2022* (PB 92 of 2022) (the Amendment Instrument) amends the *National Health (Growth Hormone Program) Special Arrangement 2015* (PB 85 of 2015) (Growth Hormone Special Arrangement). The purpose of the amendments is to include provision for the supply of two new medicines (mecasermin and somatrogon) for the treatment of children with particular conditions, consistent with recommendations of the Pharmaceutical Benefits Advisory Committee (PBAC) made in March 2022.

The purpose of this instrument is to enable subsidised access to two specific drugs for children, with the drugs promoting growth in height and not any non-gender affirming hormones.

Growth hormone is produced by our brain's pituitary gland and governs our height, bone length and muscle growth. Our bones need enough growth hormone during our childhood and adolescence in order to lengthen to adult proportions. Growth hormone prompts our liver to make a substance called insulin-like growth factor (IGF-1). This and other similar compounds are involved in bone growth. Taking synthesised growth hormone can help people reach their full height.

A person's age, sex, body weight and skeletal maturity are some of the factors taken into consideration when prescribing growth hormone medicines and determining correct dosage. The Growth Hormone Special Arrangement defines these terms for the purposes of patient eligibility based on clinical evidence and the expert opinion of the PBAC.

There are checks and balances on provision of the drugs to prevent harmful effects on children by restricting who can be an authorised prescriber and providing for a maximum dosage for each pharmaceutical benefit.

The amendments will ensure that the appropriate pharmaceutical benefits listed in Schedule 1 to the Growth Hormone Special Arrangement are available for eligible patients who require treatment with mecasermin or somatrogon in line with their diagnosis and clinical circumstances. The amendments made by the Amendment Instrument are consistent with amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2012* (PB 71 of 2012) (the Listing Instrument) concerning mecasermin and somatrogon, which commence on the same day. The Listing Instrument is made under sections 84AF, 84AK, 85, 85A, 88 and 101 of the Act.

Human Rights Implications

This Legislative Instrument engages Articles 9 and 12 of the International Covenant on Economic, Social and Cultural Rights (ICESCR) by assisting with the progressive realisation by all appropriate means of the right of everyone to social security and to the enjoyment of the highest attainable standard of physical and mental health. It also engages Article 24 of the Convention on the Rights of the Child. Article 24(1) relevantly provides for the right of the child to the highest attainable standard of health and ensuring no child is deprived of his or her right to access to such health care services. Article 24(2) goes on to provide that States Parties to the Convention shall take appropriate measures to (b) ensure the provision of necessary medical assistance and health care to all children.

This Legislative Instrument assists with the advancement of these rights by ensuring access to PBS subsidised mecasermin and somatrogon (growth hormone) treatment for paediatric patients in line with their diagnosis and appropriate age measure.

Conclusion

This Legislative Instrument is compatible with human rights because it promotes the protection of human rights.

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