

EXPLANATORY STATEMENT

NATIONAL HEALTH ACT 1953

NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT AMENDMENT INSTRUMENT 2019 (No. 3)

PB 109 of 2019

Authority

Subsection 100(1) of the *National Health Act 1953* (the Act) enables the Minister to 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: live in isolated areas; or 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 purpose of the *National Health (Growth Hormone Program) Special Arrangement Amendment Instrument 2019 (No. 3)* (the Amendment Instrument) is to amend the *National Health (Growth Hormone Program) Special Arrangement 2015* (PB 85 of 2015) (the Primary Instrument) to make changes to the definitions of adult and child in the Primary Instrument that implements special arrangements under section 100 of the Act relating to the Growth Hormone Program.

The amendments will ensure that the appropriate pharmaceutical benefits listed in either Schedule 1 or 2 to the Primary Instrument are available for eligible patients who require treatment with somatropin (growth hormone) in line with their diagnosis and appropriate age measure.

Background

Somatropin was originally listed on the Pharmaceutical Benefits Scheme (PBS) Growth Hormone Program for the treatment of a range of paediatric growth hormone deficiency (GHD) conditions. On 1 December 2018, the Australian Government extended the PBS listing for growth hormone to eligible adults. This means that children who received growth hormone treatment in childhood may access PBS-subsidised treatment as adults if they meet the PBS restriction criteria. People diagnosed with severe GHD in adulthood are also able to access PBS-subsidised growth hormone treatment.

Subsequent to the PBS listing of growth hormone for eligible adults, access issues were identified for childhood onset growth hormone deficiency (CO-GHD) patients due to the way child and adult are defined in the *National Health (Growth Hormone Program) Special Arrangement 2015 (PB 85 of 2015)*. This is an unanticipated consequence of the *National Health (Growth Hormone Program) Special Arrangement Amendment (Adult Use) Instrument (PB 96 of 2018)* which introduced provisions for the supply under the PBS of the pharmaceutical benefit somatropin for adults.

At its August 2019 meeting, the Pharmaceutical Benefits Advisory Committee (PBAC) recommended amendments be made to the adult-use growth hormone PBS restriction criteria for patients with CO-GHD due to a congenital, genetic or structural cause, to allow PBS-subsidised growth hormone treatment to commence from when this cohort reaches skeletal maturity rather than from the chronological age of 18 years. The PBAC noted that this would remove the potential lapse in PBS-subsidised access to growth hormone for CO-GHD patients between reaching skeletal maturity and the age of 18 years.

To support this PBAC recommendation, the current definitions of adult and child will be amended in order to assist in determining which persons can appropriately access growth hormone as listed in either Schedule 1 or 2 to the Primary Instrument.

The current definition distinguishes a child or an adult on the basis of the person having reached or not reached the age of 18 years. However, this definition is not always appropriate as maturity of skeleton can be reached before the age of 18, and some conditions that require treatment with growth hormone are dependent on skeletal maturity, rather than the chronological age of the person.

For most paediatric patients, access to pharmaceutical benefits under Schedule 1 to the Primary Instrument ceases upon reaching maturity of skeleton. As skeletal maturity may occur prior to the age of 18, the current definitions of adult and child are not sufficient to allow for patients with CO-GHD due to a congenital, genetic or structural cause to continue to access PBS-subsidised growth hormone treatment between reaching skeletal maturity and the age of 18 years.

Prader-Willi syndrome (PWS), a genetic disorder often accompanied by GHD, is the only PBS eligible paediatric GHD condition where chronological age, rather than maturity of skeleton is the limiting factor for eligibility to PBS-subsidised growth hormone treatment. Therefore, the amended definitions of adult and child allow PWS patients continued access to Schedule 1 to the Primary Instrument until the age of 18, even if skeletal maturity has been reached.

The definitions of adult and child have been amended to reflect reference to skeletal maturity as occurs in CO-GHD. As skeletal maturity does not form a basis in determining the treatment of GHD in adults, the previous definition of an adult as “a person who has turned 18” is still considered applicable for persons with adult-onset GHD.

Commencement

The Amendment Instrument commences on 1 January 2020.

Details

Details of the Amendment Instrument are set out in the **Attachment**.

Consultation

The amendments accord with recommendations made by the PBAC. The PBAC conducted targeted consultation with members of the Endocrine Society of Australia regarding the eligibility criteria for access to somatropin for the treatment of severe growth hormone deficiency taking into consideration the impact of skeletal maturity and chronological age on access to treatment.

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 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 those interests or professions.

Remaining members are persons whom the Minister is satisfied have qualifications and experience in a field relevant to the functions of PBAC, and that would enable them to contribute meaningfully to the deliberations of PBAC. In addition, an industry nominee has been appointed to the PBAC membership. When recommending the listing of a medicine on the PBS, PBAC takes into account the medical conditions for which the medicine has been approved for use in Australia, its clinical effectiveness, safety and cost-effectiveness compared with other treatments.

The Amendment Instrument is a legislative instrument for the purposes of the *Legislation Act 2003*.

DETAILS OF THE NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT AMENDMENT INSTRUMENT 2019 (No. 3)

Section 1 Name

This section provides the name of the instrument is the *National Health (Growth Hormone Program) Special Arrangement Amendment Instrument 2019 (No. 3)*. It can also be cited as PB 109 of 2019.

Section 2 Commencement

This section provides that the Instrument commences on 1 January 2020.

Section 3 Authority

This section provides that the Instrument is made under section 100 of the *National Health Act 1953*.

Section 4 Schedule

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

Schedule 1 – Amendments

National Health (Growth Hormone Program) Special Arrangement 2015 (PB 85 of 2015) (the Primary Instrument)

A number of expressions used in the Primary Instrument are defined in Part 1, section to assist in the interpretation of provisions in the Primary Instrument. These include the definitions of “adult” and “child” in order to assist in determining which persons can access the appropriate growth hormone listed in either Schedule 1 or 2 to the Primary Instrument. The current definition distinguishes a child or an adult on the basis of the person having reached or not reached the age of 18 years. However, this definition is not always appropriate as maturity of skeleton can be reached before the age of 18, and that some conditions that require treatment with growth hormone are dependent on skeletal maturity, rather than the age of the person.

Item 1 repeals and substitutes a new definition of “adult” in subsection 4(1) of the Primary Instrument. An adult is now defined as any of the following: “a person who has turned 18 and has adult onset growth hormone deficiency” or a person who has a mature skeleton, or a person who has been diagnosed with Prader-Willis syndrome and is aged 18 years or older.”

Item 2 repeals and substitutes a new definition of “child” in subsection 4(1) of the Primary Instrument. A child is now defined as a person who is any of the following: a person that is not an adult, or a person who has been diagnosed with Prader-Willis syndrome and is less than 18 years of age.

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 Instrument 2019 (No. 3)

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 purpose of this Legislative Instrument, made under section 100 of the *National Health Act 1953* (the Act), is to make changes to the definitions of adult and child relating to the Growth Hormone Program. This ensures that the appropriate pharmaceutical benefits listed in either Schedule 1 or 2 to the Primary Instrument are available for patients who require treatment with growth hormone (somatropin) in line with their diagnosis and appropriate age measure.

The current definition distinguishes a child or an adult on the basis of the person having reached or not reached the age of 18 years. However, this definition is not always appropriate as maturity of skeleton can be reached before the age of 18, and some conditions that require treatment with growth hormone are dependent on skeletal maturity, rather than the chronological age of the person.

For most paediatric patients, access to pharmaceutical benefits under Schedule 1 to the Primary Instrument ceases upon reaching maturity of skeleton. As skeletal maturity may occur prior to the age of 18, the current definitions of adult and child are not sufficient to allow for patients with CO-GHD due to a congenital, genetic or structural cause to continue to access PBS-subsidised growth hormone treatment between reaching skeletal maturity and the age of 18 years.

Prader-Willi syndrome (PWS), a genetic disorder often accompanied by GHD, is the only PBS eligible paediatric GHD condition where chronological age, rather than maturity of skeleton is the limiting factor for eligibility to PBS subsidised growth hormone treatment. Therefore, the amended definitions of adult and child allow PWS patients continued access to Schedule 1 to the Primary Instrument until the age of 18, even if skeletal maturity has been reached.

The definitions of adult and child have been amended to reflect reference to skeletal maturity as occurs in CO-GHD. As skeletal maturity does not form a basis in determining the treatment of GHD in adults, the previous definition of an adult as “a person who has turned 18” is still considered applicable for persons with adult-onset GHD.

The definitions of adult and child set out in this Legislative Instrument for the purposes of patient eligibility of paediatric and adult patients to access PBS subsidised somatropin (growth hormone) treatment have been based on clinical evidence and the expert opinion of the Pharmaceutical Benefits Advisory Committee (PBAC).

Human Rights Implications

This Legislative Instrument engages Article 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 the enjoyment of the highest attainable standard of physical and mental health.

This Instrument assists with the advancement of this right by ensuring continued access to PBS subsidised somatropin (growth hormone) treatment for paediatric and adult patients in line with their diagnosis and appropriate age measure.

Conclusion

This Legislative Instrument is compatible with human rights because it continues to promote the right to health.

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