

EXPLANATORY STATEMENT

NATIONAL HEALTH ACT 1953

NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT AMENDMENT INSTRUMENT 2023 (No. 2)

PB 96 of 2023

Purpose

This is the *National Health (Growth Hormone Program) Special Arrangement Amendment Instrument 2023 (No. 2)* (this Instrument). The purpose of this Instrument, made under subsection 100(2) of the *National Health Act 1953* (the Act), is to amend the *National Health (Growth Hormone Program) Special Arrangement 2015* (PB 85 of 2015) (the Special Arrangement), to make changes to the Special Arrangement relating to the Growth Hormone Program.

The amendments made by this Instrument reflect amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2012* (PB 71 of 2012) (the Listing Instrument), which commence on the same day. The changes in the Listing Instrument provide for the removal of references to Prader-Willi Syndrome (PWS) from three circumstance codes for severe Growth Hormone Deficiency (existing circumstances codes C13516, C13637 and C12601). These are consequential changes due to the amendment of the definitions of ‘adult’ and ‘child’ in the Special Arrangement (as described below). The Listing Instrument is made under sections 84AF, 84AK, 85, 85A, 88 and 101 of the Act.

Schedule 1 to this Instrument provides for the following changes to the Special Arrangement:

- the repeal and substitution of three definitions in Part 1, Section 4, “Definitions” for:
 - o ‘adult’ and ‘child’ to align with the Pharmaceutical Benefits Advisory Committee’s (PBAC) earlier recommendations which moved away from the chronological age of a patient and towards skeletal maturity as the determining factor.
 - o *CDC 2000* to reflect the latest version of the US Growth Charts in effect at the time a Pharmaceutical Benefits Scheme (PBS) authority application is made.

The amendments will ensure that the appropriate pharmaceutical benefits listed in either Schedule 1 or 2 of the Special Arrangement are available for eligible patients who require treatment with growth hormone medicines in line with their diagnosis and appropriate age measure.

These changes are summarised, by subject matter, in the Attachment.

Authority

Subsection 100(1) of the Act enables the Minister to make special arrangements for the supply of pharmaceutical benefits.

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).

Background

The Australian Government provides subsidised access to growth hormone (mecasermin, somatrogon and somatropin) for eligible paediatric and adult patients through the Pharmaceutical Benefits Scheme (PBS) Growth Hormone Program, established under section 100 of the Act. There are currently 14 different treatment categories included under the Program. Growth Hormone restriction criteria is clinically complex. Patients must be treated by an endocrinologist or a specialist in consultation with an endocrinologist, to be eligible for PBS-subsidised treatment with growth hormone medicines. Growth Hormone medicines can be dispensed by a community pharmacy, a public hospital pharmacy, a private hospital pharmacy and a dispensing doctor.

1. Definition of ‘adult’ and ‘child’ in the Special Arrangement

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 childhood onset growth hormone deficiency (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. The broader intent of the July 2019 and August 2019 PBAC recommendations for adult severe Growth Hormone Deficiency listings was to ensure that patients diagnosed with Growth Hormone deficiency in childhood (i.e., under the age of 18 years) and commenced on Growth Hormone replacement prior to the age of 18 years, but who do not have documented evidence that they meet the current diagnostic requirements of the restriction, are not disadvantaged and may continue to access Growth Hormone replacement.

To support the PBAC recommendation, the current definitions of ‘adult’ and ‘child’ were amended on 1 January 2020 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 amended definition for ‘adult’ included ‘has a diagnosis of Prader-Willi syndrome and is aged 18 years or older’.

However, there was no PBAC recommendation from the August 2019 meeting specifically referring to patients with PWS aged 18 years or older.

This Instrument amends the Special Arrangement definitions of ‘adult’ and ‘child’ to better align with the intent of previous PBAC recommendations (July and August 2019).

Similarly, references to PWS in three of the circumstance codes for severe Growth Hormone Deficiency (C13516, C13637 and C12601), are being removed (through separate amendments to the Listing Instrument also commencing 1 October 2023) in line with the relevant PBAC recommendation in August 2019 which makes no mention of PWS in the PBAC recommended listing.

2. Definition of ‘CDC 2000’ in the Special Arrangement

CDC 2000 is a chart used to assess what a person’s ideal body weight is. The update to this definition is to reflect the latest version of the US Growth Charts, to include the new CDC Extended BMI-for-age Growth Charts issued in December 2022.

Consultation

The amendments made by this Instrument accord with recommendations made by the Pharmaceutical Benefits Advisory Committee (PBAC). The PBAC conducted targeted consultation with members of the Endocrine Society of Australia regarding the eligibility criteria for access to growth hormone medicines 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 Pharmaceutical Benefits Scheme (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.

General

A provision-by-provision description of this instrument is contained in the Attachment.

This Instrument commences on 1 October 2023.

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

PROVISION-BY-PROVISION DESCRIPTION OF NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT AMENDMENT INSTRUMENT 2023 (No. 2)

Section 1 Name of Instrument

This section provides the name of this instrument as the *National Health (Growth Hormone Program) Special Arrangement Amendment Instrument 2023 (No. 2)* and may also be cited as PB 96 of 2023.

Section 2 Commencement

This section provides that this instrument commences on 1 October 2023.

Section 3 Authority

This section states that this instrument is made under subsection 100(2) of the *National Health Act 1953*.

Section 4 Schedules

Section 4 provides that each instrument that is specified in a Schedule to the 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 Instrument has effect according to its terms.

Schedule Amendments

The amendments in Schedule 1 involve changes to the definitions for “adult”, “CDC 2000” and “child”. These changes are summarised below.

SUMMARY OF CHANGES TO THE NATIONAL HEALTH (GROWTH HORMONE PROGRAM) SPECIAL ARRANGEMENT 2015 MADE BY THIS INSTRUMENT

Items 1- 3 – Subsection 4(1) Definitions

A number of expressions used in the Special Arrangement are defined in Part 1 to assist in the interpretation of provisions. 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 Special Arrangement.

Items 1 & 3 repeal and substitute the definitions of *adult* and *child* to remove the specific reference to Prader-Willi Syndrome (PWS) in patients aged 18 years or older, consistent with the Pharmaceutical Benefits Advisory Committee recommendations which do not specifically refer to patients with PWS aged 18 years or older. The new definitions are:

adult means a person, who:

- (a) is not a child; or
- (b) is 18 years of age or older and has late onset growth hormone deficiency.

child means a person, who:

- (a) has a non-mature skeleton; or
- (b) has a diagnosis of Prader-Willi syndrome, with a mature skeleton and is less than 18 years of age.

Item 2 – Subsection 4(1) Definitions

Item 3 repeals and substitutes the definition of *CDC 2000* to include the new ‘CDC Extended BMI-for-age Growth Charts’ published in December 2022, and to indicate where the document can be obtained. The new definition is:

CDC 2000 means the growth charts in the document entitled *2000 CDC Growth Charts for the United States: Methods and Development* dated May 2002 and *CDC Extended BMI-for-Age Growth Charts* dated December 2022, published by the Centers for Disease Control and Prevention, US Department of Health and Human Services and available on that Department’s website at <http://www.cdc.gov/GROWTHcharts>

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 2023 (No. 2)

(PB 96 of 2023)

This 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 Instrument

The purpose of this Instrument, made under subsection 100(2) of the *National Health Act 1953* (the Act), is to amend the *National Health (Growth Hormone Program) Special Arrangement 2015* (PB 85 of 2015) (the Special Arrangement), to make changes to the Special Arrangement relating to the Growth Hormone Program. This Instrument makes changes to the definitions of adult, child and CDC 2000 in the Primary Instrument.

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

The purpose of the amendments is to better align the definitions of ‘adult’ and ‘child’ with the intent of previous Pharmaceutical Benefits Advisory Committee (PBAC) recommendations (July and August 2019). The definition of ‘CDC 2000’ is being updated to reflect the latest version of the US Growth Charts, to include the new CDC Extended BMI-for-age Growth Charts published in December 2022.

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 and adults 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 growth hormone medicines in line with their diagnosis and clinical circumstances.

The Australian Government provides subsidised access to growth hormone medicines for eligible paediatric and adult patients through the Pharmaceutical Benefits Scheme (PBS) Growth Hormone Program. Growth Hormone restriction criteria is clinically complex. Different restriction criteria apply to the writing of a prescription for growth hormone for adults and children respectively. Prescribers ensure the appropriate restriction criteria are met at the time of prescribing.

Patients with Child-Onset Growth Hormone Deficiency (CO-GHD) due to a congenital, genetic or structural cause are able to access PBS subsidised growth hormone through community, public or private hospital pharmacies if they meet the relevant PBS restriction criteria. The usual PBS co-payment will apply for each dispensing of growth hormone.

The amendments made by this Instrument reflect amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2012* (PB 71 of 2012) (the Listing Instrument), which commence on the same day. The changes in the Listing Instrument provide for references to Prader-Willi Syndrome (PWS) in three circumstance codes for severe Growth Hormone Deficiency to be removed. The Listing Instrument is made under sections 84AF, 84AK, 85, 85A, 88 and 101 of the Act.

Human rights implications

This 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 Articles 3, 23 and 24 of the Convention on the Rights of the Child (CRC). Article 3 provides that for all actions concerning children, the best interests of the child shall be a primary consideration, including conformity with established standards in the areas of safety and health. Article 23 recognises that a mentally or physically disabled child should enjoy a full and decent life, in conditions which ensure dignity, promote self-reliance and facilitate the child's active participation in the community. This includes having available resources and effective access to healthcare services which are appropriate to the child's condition and in a manner conducive to the child achieving the fullest possible social integration and individual development, including his or her cultural and spiritual development. 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 Instrument further engages Article 25 of the Convention on the Rights of Persons with Disabilities by recognising that persons with disabilities have the right to the enjoyment of the highest attainable standards of health without discrimination on the basis of disability. States parties shall take all appropriate measures to ensure access for persons with disabilities to health services that are gender-sensitive, including health-related rehabilitation. This includes providing persons with disabilities the same range, quality and standard of free or affordable health care as provided to other persons, providing these services as close as possible to people's own communities (including in rural areas), specific and appropriate health care designed to minimise and prevent further disabilities (including among children and older persons) and prevent discriminatory denial of health care or health services or food and fluids on the basis of disability.

Analysis

Growth Hormone (GH) is very important in the body. It is needed for children to grow normally. It helps make sure there is enough muscle and fat in the body. It keeps bones healthy. Some children do not make enough GH. A low level of GH can cause children to grow more slowly. In adults, a low level of GH can cause problems with fitness and health.

Although growth hormone's main function is to promote growth in childhood, it is still important once adulthood is reached. In adults as well as in children, growth hormone helps regulate metabolism — a critical chemical process through which the body turns food into energy, tissue or waste products.

Growth hormone treatment provides both health benefits and significant increases in height. Children and adults can benefit from treatment with PBS subsidised growth hormone, which can help:

- increase bone density, thereby preventing fractures;
- increase muscle mass;
- increase energy levels;
- increase the capacity for exercise; and
- decrease body fat.

Treatment with PBS subsidised growth hormone may also improve energy levels, cognition, and quality of life improvements. Patients may see improvements in self-esteem, well-being and social interaction.

This Instrument assists with the advancement of these rights by ensuring access to PBS subsidised growth hormone treatment for paediatric and adult patients in line with their diagnosis and appropriate age measure. The PBS is a benefit scheme which assists with advancement of these human rights by providing for subsidised access by patients to medicines. The recommendatory role of the Pharmaceutical Benefits Advisory Committee (PBAC) ensures that decisions about subsidised access to medicines on the PBS are evidence-based.

Conclusion

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

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