**EXPLANATORY STATEMENT**

***NATIONAL HEALTH ACT 1953***

***NATIONAL HEALTH (HIGHLY SPECIALISED DRUGS PROGRAM)
SPECIAL ARRANGEMENT AMENDMENT (MAY UPDATE) INSTRUMENT 2025***

**PB 44 of 2025**

**Purpose**

This is the *National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (May Update) Instrument 2025* (PB 44 of 2025) (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 (Highly Specialised Drugs Program) Special Arrangement 2021* (PB 27 of 2021) (the Special Arrangement), to make changes to the Special Arrangement relating to the Highly Specialised Drugs (HSD) Program.

The amendments made by this Instrument reflect amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2024* (PB 26 of 2024), which commence on the same day. The *National Health (Listing of Pharmaceutical Benefits) Instrument 2024* (PB 26 of 2024), and amendments to that instrument, are made under sections 84AF, 84AK, 85, 85A, 88 and 101 of the Act.

Schedule 1 to this Instrument provides for the addition of the drugs edaravone, esketamine, macitentan with tadalafil, and vutrisiran, the addition of brands of the listed drugs azacitidine, bosentan, and tenofovir with emtricitabine, the deletion of a form of the listed drug adefovir, and the alteration of circumstances in which a prescription may be written for the listed drugs difelikefalin, eltrombopag, infliximab, and vedolizumab under the Special Arrangement. These changes are summarised, by subject matter, in the Attachment.

**Authority**

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

Subsection 100(2) of the Act provides that the Minister may, by legislative instrument, 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 for edaravone amendment**

The HSD Program is being amended upon a recommendation by the Pharmaceutical Benefits Advisory Committee (PBAC) to include edaravone for the treatment of amyotrophic lateral sclerosis (ALS). Access to initial treatment with edaravone will occur through prescriptions written by a specialist affiliated with a public or private hospital under the HSD Program. Edaravone will be available via the HSD Program Community Access arrangements for continuing treatment. Enabling access to edaravone for continuing treatment through the HSD Program Community Access arrangements means that any medical practitioner may prescribe edaravone for continuing treatment, including for patients receiving treatment in a community setting. Additionally, Community Access prescriptions written by medical practitioners for the continuing treatment of patients receiving treatment in, at, or from a public or private hospital can be dispensed from any Pharmaceutical Benefits Scheme (PBS) Approved Supplier, including community pharmacies. As well as providing subsidised access to a new treatment for ALS, this amendment will enable patients receiving continuing treatment with edaravone to have a greater choice as to where they access their treatment, particularly those in rural and remote areas.

**Consultation**

The amendments made by this Instrument accord with recommendations made by the PBAC.

An ongoing and formal process of consultation in relation to matters relevant to the Special Arrangement includes the involvement of interested parties through the membership of the PBAC.

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.

Pharmaceutical companies were consulted throughout the process of changes to the listings on the PBS. This includes consultation through the PBAC process.

Further consultation for this Instrument was considered unnecessary due to the nature of the consultation that has already taken place in the decision to list the medications outlined under ‘Purpose’.

Details of this Instrument are set out in the Attachment.

This Instrument commences on 1 May 2025.

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

**ATTACHMENT**

**DETAILS OF THE *NATIONAL HEALTH (HIGHLY SPECIALISED DRUGS PROGRAM) SPECIAL ARRANGEMENT AMENDMENT (MAY UPDATE) INSTRUMENT 2025***

**Section 1 Name of Instrument**

This section provides that the name of the Instrument is the *National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (May Update) Instrument 2025* and may also be cited as PB 44 of 2025.

**Section 2 Commencement**

This section provides that this Instrument commences on 1 May 2025.

**Section 3 Authority**

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

**Section 4 Schedules**

This section 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 1 Amendments**

*National Health (Highly Specialised Drugs Program) Special Arrangement 2021* (PB 27 of 2021)

**Item 1 Definition of *CAR drug***

This item amends the definition of *CAR drug* in section 6 of the Special Arrangement to include the drugs macitentan with tadalafil, and vutrisiran in the list of drugs which are CAR drugs.

**Item 2 Definition of *community access medication***

This item amends the definition of *community access medication* in section 6 of the Special Arrangement to include the drug edaravone limited to where the use is for continuing treatment.

**Item 3 Definition of *authorised prescriber***

This item amends the definition of *authorised prescriber* in subsection 7(4) of the Special Arrangement. The effect of this amendment is to make a medical practitioner an *authorised prescriber* for HSD pharmaceutical benefits that have the drug edaravone, regardless of the form of the drug, for the continuing phase of the patient’s treatment.

The reference to “continuing treatment” in the amended definition of *community access medication* as it relates to edaravone, and in subsection 7(4) (as amended), limits community access edaravone to a patient’s continuing treatment phase.

The prescription circumstances for edaravone in Schedule 3 to the Special Arrangement (as amended by this Instrument) clarify that, for a patient’s initial treatment phase, the patient’s condition must be or have been diagnosed by a neurologist. Under existing subsection 7(1) of the Special Arrangement, a specialist affiliated with the hospital in, at, or from which the patient is receiving treatment is an authorised prescriber for edaravone for initial treatment. The effect of existing subsection 7(1), operating in conjunction with the prescription circumstances for edaravone in Schedule 3 to the Special Arrangement (as amended by this Instrument), is that any specialist affiliated with the hospital in, at, or from which the patient is receiving treatment is authorised to prescribe edaravone for the initial treatment of ALS, as long as the condition is or has been diagnosed by a neurologist.

**Items 4 to 25 Amendments to Schedules 1, 2, and 3**

Items 4 to 25 in Schedule 1 involve the addition of drugs, the addition of brands of listed drugs, the deletion of a form of a listed drug, and the alteration of circumstances in which a prescription may be written for listed drugs available under the Special Arrangement. These changes are summarised below.

**SUMMARY OF CHANGES TO THE *HIGHLY SPECIALISED
DRUGS PROGRAM* MADE BY THIS INSTRUMENT**

Drug Addition

| **Listed Drug** |
| --- |
| Edaravone |
| Esketamine |
| Macitentan with tadalafil |
| Vutrisiran |

Form Deletion

|  |  |
| --- | --- |
| **Listed Drug** | **Form** |
| Adefovir | Tablet containing adefovir dipivoxil 10 mg (S19A) |

**Brand Added**

|  |  |
| --- | --- |
| ***Listed Drug*** | ***Form and Brand*** |
| Azacitidine | Powder for injection 100 mg *(Azacitidine SXP)* |
| Bosentan | Tablet 125 mg (as monohydrate) *(Bosentan Viatris)* |
| Tenofovir with emtricitabine | Tablet containing tenofovir disoproxil fumarate 300 mg with emtricitabine 200 mg *(TENOFOVIR/EMTRICITABINE 300/200 APX)* |

**Alteration of Circumstances in Which a Prescription May be Written**

|  |
| --- |
| ***Listed Drug*** |
| Difelikefalin |
| Eltrombopag |
| Infliximab |
| Vedolizumab |

Documents Incorporated by Reference

|  |  |  |
| --- | --- | --- |
| ***Listed Drug*** | ***Document Incorporated*** | ***Document access*** |
| Edaravone | **ALS Functional Rating Scale – Revised (ALSFRS‑R) score.**The document is incorporated as in force on the day this Instrument takes effect, pursuant to paragraph 14(1)(b) of the *Legislation Act 2003*.The ALSFRS-R questionnaire is routinely administered to patients with ALS by neurologists at every clinic visit, typically 3 monthly. It is not proprietary and can be accessed online, being reproduced on multiple medical websites (e.g. https://www.mdcalc.com/calc/10166/revised-amyotrophic-lateral-sclerosis-functional-rating-scale-alsfrs-r). There are also websites that allow the neurologist to input a patient’s ratings to obtain a printable or digital copy of their score (https://neurotoolkit.com/alsfrs-r/). | An ALSFRS-R calculator is available via the MiNDAUS ALS registry: https://www.mindaus.org/wp-content/uploads/2023/09/22094546/DataDictionaryPROMMindausHansenJuly2023V01.1.pdf |
| Vutrisiran | **Familial Amyloid Polyneuropathy (FAP) stages.**The document is incorporated as in force on the day this Instrument takes effect, pursuant to paragraph 14(1)(b) of the *Legislation Act 2003*.FAP stages are used to determine the severity of hereditary transthyretin amyloidosis and thus patient eligibility for PBS subsidised vutrisiran. The stages are described below:*Familial Amyloid Polyneuropathy (FAP) stages*Stage 0 - No symptoms.Stage 1 - Unimpaired ambulation.Stage 2 - Assistance with ambulation required.Stage 3 - Wheelchair-bound or bedridden. | The FAP stages can be accessed free of charge at the Orphanet Journal of Rare Diseases website: https://ojrd.biomedcentral.com/articles/10.1186/1750-1172-8-31 |
| Vutrisiran | **New York Heart Association (NYHA) classification.** The document is incorporated as in force on the day this Instrument takes effect, pursuant to paragraph 14(1)(b) of the *Legislation Act 2003*.The NYHA classification system is used to define the degree of heart failure. The different classes in the NYHA Functional Classification for heart failure are described below: *Class/Patient Symptoms*Class I: No limitation of physical activity. Ordinary physical activity does not cause undue fatigue, palpitation or shortness of breath.Class II: Slight limitation of physical activity. Comfortable at rest. Ordinary physical activity results in fatigue, palpitation, shortness of breath or chest pain.Class III: Marked limitation of physical activity. Comfortable at rest. Less than ordinary activity causes fatigue, palpitation, shortness of breath or chest pain.Class IV: Symptoms of heart failure at rest. Any physical activity causes further discomfort. | The NYHA classification system is available for download for free from the Heart Foundation website (contained within the heart failure clinical guidelines): https://www.heartfoundation.org.au/Conditions/Heart-failure-clinical-guidelines |
| Vutrisiran | **Polyneuropathy Disability (PND) score/stages.**The document is incorporated as in force on the day this Instrument takes effect, pursuant to paragraph 14(1)(b) of the *Legislation Act 2003*.PND stages are used to determine the severity of hereditary transthyretin amyloidosis and thus patient eligibility for PBS subsidised vutrisiran. The stages are described below:*Polyneuropathy Disability (PND) stages*Stage 0 - No symptoms.Stage I - Sensory disturbances but preserved walking capability.Stage II - Impaired walking capacity but able to walk without stick or crutches.Stage IIIA - Walking with help of one stick or crutch.Stage IIIB - Walking with help of two sticks or crutches.Stage IV - Confined to wheelchair or bedridden. | The PND stages can be accessed free of charge at the Orphanet Journal of Rare Diseases website: https://ojrd.biomedcentral.com/articles/10.1186/1750-1172-8-31 |

**Statement of Compatibility with Human Rights**

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

***National Health (Highly Specialised Drugs Program) Special Arrangement
Amendment (May Update) Instrument 2025***

**(PB 44 of 2025)**

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 (Highly Specialised Drugs Program) Special Arrangement 2021* (PB 27 of 2021) (the Special Arrangement), to make changes to the Special Arrangement relating to the Highly Specialised Drugs Program.

The pharmaceutical benefits supplied under the Special Arrangement are for the treatment of chronic conditions which, because of their clinical use or other special features, may only be supplied to patients receiving specialised treatment.

**Human Rights Implications**

This Instrument engages Articles 9 and 12 of the International Covenant on Economic, Social and Cultural Rights (ICESCR), specifically the rights to social security and health.

*The Right to Social Security*

The right to social security is contained in Article 9 of the ICESCR. It requires that a country must, within its maximum available resources, ensure access to a social security scheme that provides a minimum essential level of benefits to all individuals and families that will enable them to acquire at least essential health care. Countries are obliged to demonstrate that every effort has been made to use all resources that are at their disposal in an effort to satisfy, as a matter of priority, this minimum obligation.

The UN Committee on Economic Social and Cultural Rights reports that there is a strong presumption that retrogressive measures taken in relation to the right to social security are prohibited under ICESCR. In this context, a retrogressive measure would be one taken without adequate justification that had the effect of reducing existing levels of social security benefits, or of denying benefits to persons or groups previously entitled to them. However, it is legitimate for a government to re-direct its limited resources in ways that it considers to be more effective at meeting the general health needs of all society, particularly the needs of the more disadvantaged members of society.

*The Right to Health*

The right to the enjoyment of the highest attainable standard of physical and mental health is contained in Article 12(1) of the ICESCR. The UN Committee on Economic, Social and Cultural Rights (the Committee) has stated that the right to health is not a right for each individual to be healthy, but is a right to a system of health protection which provides equality of opportunity for people to enjoy the highest attainable level of health.

The Committee reports that the ‘highest attainable standard of health’ takes into account the country’s available resources. This right may be understood as a right of access to a variety of public health and health care facilities, goods, services, programs, and conditions necessary for the realisation of the highest attainable standard of health.

**Analysis**

This Instrument advances the right to health and the right to social security by ensuring that the amendments to the *National Health (Listing of Pharmaceutical Benefits) Instrument 2024* (the Listing Instrument), that affect the pharmaceutical benefits that may be supplied under the Special Arrangement, are made concurrently. This Instrument provides for the addition of the drugs edaravone, esketamine, macitentan with tadalafil, and vutrisiran, the addition of brands of the listed drugs azacitidine, bosentan, and tenofovir with emtricitabine, and the deletion of a form of the listed drug adefovir.

The inclusion of edaravone for the treatment of amyotrophic lateral sclerosis (ALS) on the Highly Specialised Drugs (HSD) Program, including the details of the circumstances in which this drug can be prescribed, is a result of a recommendation by the Pharmaceutical Benefits Advisory Committee (PBAC). This amendment will enable prescriptions written by a specialist affiliated with a public or private hospital for patients to access initial treatment with edaravone through the HSD Program. It will also enable patients to access continuing treatment with edaravone through the HSD Program Community Access arrangements. Enabling access to edaravone for continuing treatment through the HSD Program Community Access arrangements means that any medical practitioner may prescribe edaravone for continuing treatment, including for patients receiving treatment in a community setting. Additionally, Community Access prescriptions written by medical practitioners for the continuing treatment of patients receiving treatment in, at, or from a public or private hospital can be dispensed from any Pharmaceutical Benefits Scheme (PBS) Approved Supplier, including community pharmacies. As well as providing subsidised access to a new treatment for ALS, this amendment will enable patients receiving continuing treatment with edaravone to have a greater choice as to where they access their treatment, particularly those in rural and remote areas. This increase in choice promotes Article 12 of the ICESCR.

The Listing Instrument determines the pharmaceutical benefits that are on the PBS through declarations of drugs and medicinal preparations, and determinations of forms, manners of administration and brands. 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 PBAC ensures that decisions about subsidised access to medicines on the PBS are evidence-based.

When a sponsor submits a request to delist a drug from the PBS, subsection 101(4AAB) of the *National Health Act 1953* requires that the Minister or their delegate obtain advice from the Pharmaceutical Benefits Advisory Committee (PBAC), an independent and expert advisory body, before varying or revoking declarations under subsection 85(2) so as to delist the drug. In these instances, one of the matters which the PBAC provides advice on is whether the delisting of a drug will result in an unmet clinical need for patients. PBAC also considers whether the delisting of a form of a drug will result in an unmet clinical need for patients.

Written advice from the PBAC is tabled with the monthly amendments to the Listing Instrument. An unmet clinical need would arise when a currently treated patient population would be left without treatment options once a delisting occurs. Alternative treatment options could include using a different: form, strength or drug. The PBAC considered the delisting of forms of drugs in the above-mentioned instrument would not result in an unmet clinical need, except where indicated for a particular form of drug below. Where the PBAC has identified an unmet clinical need, a Supply Only period will be/has been instituted as outlined below to allow opportunity for patients to transition to an alternative treatment option. The delisting of these items will not affect access to the drugs (or an alternative treatment if required), as affected patients will be able to access alternative medicines through the PBS, and the delisting is unlikely to have an effect on the amount patients pay for those drugs, as co-payment amounts are capped, ensuring their rights to social security are maintained. From 1 January 2024, these amounts are $31.60 for general patients and $7.70 for concession card holders.

The drug adefovir in the form tablet containing adefovir dipivoxil 10 mg (S19A) (Adefovir Dipivoxil Tablets 10 mg (SigmaPharm Laboratories)) was requested to be delisted from the PBS Schedule by the sponsor. This item was listed under section 19A of the *Therapeutic Goods Act 1989* to address the shortage of adefovir in the form tablet containing adefovir dipivoxil 10 mg. The temporary approval granted in respect of this drug for importation and supply of a medicine not on the Australian Register of Therapeutic Goods lapsed on 31 January 2025. Patient access has not been affected as the approved form of the drug is available on the PBS Schedule under Supply Only arrangements for a period of 6 months, allowing patients with a pre-existing valid prescription to access this item pending transition to an alternative treatment. When reviewing the request to delist adefovir in the form tablet containing adefovir dipivoxil 10 mg (APO-Adefovir), the PBAC noted the low number of services in the last financial year and that there are alternatives on the PBS. The PBAC advised the delisting of this product would not result in an unmet clinical need.

**Conclusion**

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

**Rebecca Richardson
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**Pricing and PBS Policy Branch**

**Technology Assessment and Access Division**

**Department of Health and Aged Care**