



PB 141 of 2024

National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (January Update) Instrument 2024

National Health Act 1953

I, EDEN SIMON, Assistant Secretary (Acting), Pricing and PBS Policy Branch, Technology Assessment and Access Division, Department of Health and Aged Care, delegate of the Minister for Health and Aged Care, make this Instrument under subsection 100(2) of the *National Health Act 1953*.

Dated 19 December 2024

EDEN SIMON
Assistant Secretary (Acting)
Pricing and PBS Policy Branch
Technology Assessment and Access Division

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1 Name

- (1) This instrument is the *National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (January Update) Instrument 2024*.
- (2) This instrument may also be cited as PB 141 of 2024.

2 Commencement

- (1) Each provision of this instrument specified in column 1 of the table commences, or is taken to have commenced, in accordance with column 2 of the table. Any other statement in column 2 has effect according to its terms.

Commencement information		
Column 1	Column 2	Column 3
Provisions	Commencement	Date/Details
1. <i>The whole of this instrument</i>	<i>1 January 2025</i>	<i>1 January 2025</i>

Note: This table relates only to the provisions of this instrument as originally made. It will not be amended to deal with any later amendments of this instrument.

- (2) Any information in column 3 of the table is not part of this instrument. Information may be inserted in this column, or information in it may be edited, in any published version of this instrument.

3 Authority

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

4 Schedules

Each instrument that is specified in a Schedule to this instrument is amended or repealed as set out in the applicable items in the Schedule concerned, and any other item in a Schedule to this instrument has effect according to its terms.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 (Highly Specialised Drugs Program) Special Arrangement 2021 (PB 27 of 2021)

[1] Schedule 1, entry for Abacavir with Lamivudine

omit:

	Abacavir/ Lamivudine Mylan	C4527 C4528	60	5
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[2] Schedule 1, after entry for Adalimumab in the form Injection 40 mg in 0.4 mL pre-filled pen [Brand: Humira]

insert:

	Hyrimoz	C12120 C14061 C14063 C14064 C14107 C14136	See Schedule 2	See Schedule 2
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[3] Schedule 1, entry for Mycophenolic acid in the form Capsule containing mycophenolate mofetil 250 mg

omit:

	Ceptolate	C5600 C5653 C9689 C9690	600	5
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[4] Schedule 1, entry for Risdiplam

(a) *omit from the column headed "Circumstances":* **C14458**

(b) *insert in numerical order in the column headed "Circumstances":* **C16257**

[5] Schedule 1, entry for Vedolizumab

(a) *omit from the column headed "Circumstances":* **C16170**

(b) *insert in numerical order in the column headed "Circumstances":* **C16239**

[6] Schedule 2, entry for Risdiplam [Maximum quantity: 1; Maximum repeats: 0]

(a) *omit from the column headed "Circumstances":* **C14458**

(b) *insert in numerical order in the column headed "Circumstances":* **C16257**

[7] Schedule 2, entry for Vedolizumab [Maximum quantity: 1; Maximum repeats: 2]

- (a) omit from the column headed “Circumstances”: **C16170**
- (b) insert in numerical order in the column headed “Circumstances”: **C16239**

[8] Schedule 3, entry for Risdiplam

- (a) omit:

	C14458	<p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment with this drug of pre-symptomatic spinal muscular atrophy (SMA) Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of deletion of one copy of the SMN1 gene in addition to a pathogenic/likely pathogenic variant in the remaining single copy of the SMN1 gene; AND The condition must have genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND The condition must be pre-symptomatic; AND The treatment must be given concomitantly with best supportive care for this condition; AND Patient must be untreated with gene therapy. Patient must be aged under 36 months prior to commencing treatment. Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include: (a) a completed authority prescription form; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: (i) confirmation of genetic diagnosis of SMA; and (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA) The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing. The approved Product Information recommended dosing is as follows: (i) 16 days to less than 2 months of age: 0.15 mg/kg (ii) 2 months to less than 2 years of age: 0.20 mg/kg (iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg (iv) 2 years of age and older weighing 20 kg or more: 5 mg In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to</p>	Compliance with Written Authority Required procedures
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			(iv), prescribe up to: 1 unit where (i) applies; 2 units where (ii) applies; 3 units where (iii) applies; 3 units where (iv) applies.	
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(b) insert in numerical order after existing text:

	C16257		<p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment with this drug of pre-symptomatic spinal muscular atrophy (SMA) Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of deletion of one copy of the SMN1 gene in addition to a pathogenic/likely pathogenic variant in the remaining single copy of the SMN1 gene; AND The condition must have genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND The condition must be pre-symptomatic; AND The treatment must be given concomitantly with best supportive care for this condition; AND Patient must be untreated with gene therapy. Patient must be aged under 36 months prior to commencing treatment. Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include: (a) details of the proposed prescription; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: (i) confirmation of genetic diagnosis of SMA; and (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA) The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing. The approved Product Information recommended dosing is as follows: (i) 16 days to less than 2 months of age: 0.15 mg/kg</p>	Compliance with Written Authority Required procedures
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			<p>(ii) 2 months to less than 2 years of age: 0.20 mg/kg (iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg (iv) 2 years of age and older weighing 20 kg or more: 5 mg</p> <p>In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:</p> <p>1 unit where (i) applies; 2 units where (ii) applies; 3 units where (iii) applies; 3 units where (iv) applies.</p>	
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[9] Schedule 3, entry for Vedolizumab

(a) omit:

	C16170		<p>Moderate to severe chronic pouchitis Initial 2 treatment (Recommencement of treatment after a break in biological medicine) Patient must have previously received PBS-subsidised treatment with this drug for this condition; AND Patient must not have already failed, or ceased to respond to, PBS-subsidised treatment with this drug for this condition; AND Patient must not receive more than 14 weeks of treatment under this restriction. Must be treated by a gastroenterologist (code 87); OR Must be treated by a consultant physician [internal medicine specialising in gastroenterology (code 81)]; OR Must be treated by a consultant physician [general medicine specialising in gastroenterology (code 82)]. The treatment must be initiated in combination with standard of care antibiotic. The assessment of a patient's response to this initial course of treatment must be made after the third dose of vedolizumab so there is adequate time for a response to be demonstrated. The assessment must be made prior to obtaining a PBS authority for continuing treatment from the dose at week 14. Where a response assessment is not conducted within the required timeframe, the patient will be deemed to have failed to respond to treatment with this drug, unless the patient has experienced a serious adverse reaction of a severity resulting in the necessity for permanent withdrawal of treatment. Application for authorisation of initial treatment must be in writing and must include: (a) details of the proposed prescription; and</p>	Compliance with Written Authority Required procedures
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		<p>(b) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice) which includes the following:</p> <p>(i) the patient's baseline Modified Pouchitis Disease Activity Index (mPDAI) score and minimum endoscopic mPDAI sub-score; and</p> <p>(ii) details of prior biological medicine therapy for this condition, [date of commencement and duration of therapy].</p> <p>The endoscopic assessment contributing to the Modified Pouchitis Disease Activity Index score to confirm the patient's condition at baseline must have been performed no more than 4 weeks prior to the application.</p> <p>Applications for treatment of this condition must be received within 4 weeks of the endoscopy to confirm diagnosis.</p> <p>The prescriber must exclude secondary causes of pouchitis, for example:</p> <p>(a) Ischaemia;</p> <p>(b) Crohn's disease (CD) or CD of the pouch;</p> <p>(c) Irritable pouch syndrome;</p> <p>(d) Predominant cuffitis;</p> <p>(e) Pouch stricture or pouch fistula;</p> <p>(f) Active infection;</p> <p>(g) NSAIDs;</p> <p>(h) Coeliac disease.</p>	
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(b) omit entry for Circumstances Code "C16217" and substitute:

	C16217	<p>Moderate to severe chronic pouchitis</p> <p>Transitioning from non-PBS to PBS-subsidised supply - Grandfather arrangements</p> <p>Patient must have received non-PBS-subsidised treatment with this drug for this PBS indication prior to 1 December 2024; AND</p> <p>Patient must be receiving treatment with this drug for this condition at the time of application; AND</p> <p>Patient must have undergone ileal pouch anal anastomosis (IPAA) due to ulcerative colitis at least one year prior to initiating non-PBS-subsidised treatment with this drug for this condition; AND</p> <p>The condition must be confirmed based on the patient's symptoms, treatment history and baseline endoscopic examination of the pouch (pouchoscopy); AND</p> <p>Patient must have had a Modified Pouchitis Disease Activity Index (mPDAI) score of at least 5 at the time of initiating treatment with this drug for this condition; AND</p> <p>Patient must have had a minimum endoscopic mPDAI sub-score of at least 2 at the time of initiating treatment with this drug for this condition; AND</p>	Compliance with Written Authority Required procedures
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		<p>Patient must have had at least 3 recurrent episodes of pouchitis within the year prior to initiating treatment with this drug for this condition, each of which was treated with at least 2 weeks of antibiotic or other prescription therapy; OR</p> <p>The condition must have required maintenance antibiotic therapy taken continuously for at least 4 weeks before commencing treatment with this drug; AND</p> <p>Patient must not receive more than 24 weeks of treatment under this restriction; AND</p> <p>The treatment must have been initiated in combination with standard of care antibiotic; AND</p> <p>Patient must have demonstrated a partial or complete response to treatment with this drug as determined by the treating clinician, for this condition if the patient has received non-PBS-subsidised treatment for the first three doses of induction.</p> <p>Must be treated by a gastroenterologist (code 87); OR</p> <p>Must be treated by a consultant physician [internal medicine specialising in gastroenterology (code 81)]; OR</p> <p>Must be treated by a consultant physician [general medicine specialising in gastroenterology (code 82)].</p> <p>The assessment of a patient's response to this course of treatment must be made after the third dose of vedolizumab so there is adequate time for a response to be demonstrated. The assessment must be made prior to obtaining a PBS authority for continuing treatment from the dose at week 14.</p> <p>Where a response assessment is not conducted within the required timeframe, the patient will be deemed to have failed to respond to treatment with this drug.</p> <p>The application for authorisation of treatment must be in writing and must include:</p> <p>(a) details of the proposed prescription; and</p> <p>(b) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice) which includes the following:</p> <p>(i) the patient's baseline Modified Pouchitis Disease Activity Index (mPDAI) score and minimum endoscopic mPDAI sub-score; and</p> <p>(ii) details of prior drug therapy for the condition [dosage, date of commencement and duration of therapy]; and</p> <p>(iii) the date of commencement of this drug for this condition.</p> <p>The endoscopic assessment contributing to the Modified Pouchitis Disease Activity Index score to confirm the patient's condition at baseline must have been performed no more than 4 weeks prior to initiation with non-PBS-subsidised treatment with this drug.</p> <p>The prescriber must have excluded secondary causes of pouchitis, for example:</p> <p>(a) Ischaemia;</p> <p>(b) Crohn's disease (CD) or CD of the pouch;</p>	
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			<p>(c) Irritable pouch syndrome;</p> <p>(d) Predominant cuffitis;</p> <p>(e) Pouch stricture or pouch fistula;</p> <p>(f) Active infection;</p> <p>(g) NSAIDs;</p> <p>(h) Coeliac disease.</p>	
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(c) insert in numerical order after existing text:

	C16239		<p>Moderate to severe chronic pouchitis</p> <p>Initial 2 treatment (Recommencement of treatment after a break in biological medicine)</p> <p>Patient must have previously received PBS-subsidised treatment with this drug for this condition;</p> <p>AND</p> <p>Patient must not have already failed, or ceased to respond to, PBS-subsidised treatment with this drug for this condition; AND</p> <p>Patient must not receive more than 14 weeks of treatment under this restriction.</p> <p>Must be treated by a gastroenterologist (code 87); OR</p> <p>Must be treated by a consultant physician [internal medicine specialising in gastroenterology (code 81)]; OR</p> <p>Must be treated by a consultant physician [general medicine specialising in gastroenterology (code 82)].</p> <p>The treatment must be initiated in combination with standard of care antibiotic.</p> <p>The assessment of a patient's response to this initial course of treatment must be made after the third dose of vedolizumab so there is adequate time for a response to be demonstrated. The assessment must be made prior to obtaining a PBS authority for continuing treatment from the dose at week 14.</p> <p>Where a response assessment is not conducted within the required timeframe, the patient will be deemed to have failed to respond to treatment with this drug, unless the patient has experienced a serious adverse reaction of a severity resulting in the necessity for permanent withdrawal of treatment.</p> <p>Application for authorisation of initial treatment must be in writing and must include:</p> <p>(a) details of the proposed prescription; and</p> <p>(b) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice) which includes the details of prior biological medicine therapy for this condition [date of commencement and duration of therapy].</p> <p>The prescriber must have excluded secondary causes of pouchitis, for example:</p>	<p>Compliance with Authority Required procedures</p>
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			(a) Ischaemia; (b) Crohn's disease (CD) or CD of the pouch; (c) Irritable pouch syndrome; (d) Predominant cuffitis; (e) Pouch stricture or pouch fistula; (f) Active infection; (g) NSAIDs; (h) Coeliac disease.	
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