

6.16 TEDUGLUTIDE, Powder for injection 5 mg with diluent, Revestive[®], Takeda Pharmaceuticals Australia Pty Ltd

1 Purpose of Application

- 1.1 The minor submission requested amending the Section 100 (Highly Specialised Drugs Program) Authority required listing of teduglutide for the treatment of paediatric patients with Type III (chronic) short bowel syndrome (SBS) with intestinal failure (SBS-IF) to include specific response criteria for paediatric patients.

2 Background

- 2.1 The current TGA indication is for “the treatment of adult and paediatric patients 2 years of age and above with SBS who are dependent on parenteral support. Patients should be stable for at least 4 weeks on their parenteral support regimen before initiating teduglutide therapy.”

Previous PBAC consideration

- 2.2 At its March 2019 meeting, the PBAC recommended the listing of teduglutide as a Section 100 (Highly Specialised Drug Program) benefit for the treatment of patients with Type III (chronic) intestinal failure associated with short bowel syndrome (paragraph 7.1, teduglutide Public Summary Document (PSD), March 2019).
- 2.3 During the March 2019 evaluation, two teduglutide trials in paediatric patients were identified. The results of a 12-week, Phase 3, open-label, pharmacokinetic, pharmacodynamic, safety study of teduglutide 0.025 or 0.05 mg/kg/day in paediatric subjects with short bowel syndrome associated with intestinal failure were reported by Carter et al. (2017). The authors concluded that treatment with teduglutide was well tolerated, and associated with a trend towards a reduction in parenteral nutrition requirements (page 48, teduglutide Commentary, March 2019). Results of a Phase 3, 24-week, safety, efficacy, and pharmacodynamics study investigating teduglutide 0.025 or 0.05 mg/kg/day or standard of care in paediatric subjects with short bowel syndrome who are dependent on parenteral support were reported by Hill et al. (2018; abstract only). The authors stated that treatment with teduglutide was associated with clinically meaningful parenteral support reductions. The responder rate for parenteral support volume reduction and enteral autonomy was largest in the patient group treated with 0.05 mg/kg teduglutide. The safety profile was favourable in both teduglutide dosing groups, and no new safety signals were identified.

- 2.4 The PBAC considered that the restriction should not specify any age criteria, noting that teduglutide has been studied in paediatric patients and was shown to be well tolerated (paragraph 7.6, teduglutide PSD, March 2019).
- 2.5 Teduglutide was first listed on 1 October 2019.

Current situation

- 2.6 The current teduglutide restrictions were developed based on input from expert clinicians.
- 2.7 The submission noted that although the current teduglutide restrictions allow use in both adults and children, the restriction criteria were developed based only on the expected response to therapy for adult patients.
- 2.8 The submission stated that input from a paediatric advisory board attended by key multi-disciplinary team members forms the basis of and justification for the restriction wording proposed in this submission.

For more detail on PBAC's view, see section 5 PBAC outcome.

3 Requested listing

- 3.1 The submission requested changing the restrictions for paediatric patients stating that the current restrictions may inadvertently disadvantage some paediatric patients. The submission explained that as children's nutritional requirements continue to increase with continued body growth, it may not always be possible to demonstrate a benefit in terms of reduced parenteral support (PS) days within the specified timeframes even in circumstances when the child is benefiting from treatment with teduglutide.
- 3.2 The submission proposed that alternative definitions for treatment response, treatment stability, treatment failure and deterioration during a trial cessation period be added to the current restrictions for children. The proposed additions to the restrictions are based around a 20% reduction in PS volume (measure in mL per kg of body weight). The submission noted that a 20% reduction in weekly PS volume was a primary outcome in the pivotal trials in adult patients presented in the previous teduglutide submission as well as the trials in paediatric patients presented in this submission.
- 3.3 Changes to the existing listing proposed by the sponsor are grey-shaded. Suggestions and additions proposed by the Secretariat are in italics and deletions are in strikethrough.

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MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
TEDUGLUTIDE					
teduglutide 5 mg injection [28 vials] (& inert substance diluent [28 x 0.5 mL syringes], 1 pack	11793Q (Public) 11795T (Private)	1	28	11	Revestive
Category / Program: Section 100 – Highly Specialised Drugs Program (Public & Private hospitals)					
Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners					
Restriction type: <input checked="" type="checkbox"/> Authority Required – non-immediate assessment by Services Australia (In-writing only via mail/postal service or electronic lodgement)					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Type III Short bowel syndrome with intestinal failure					
Treatment Phase: Initial treatment					
Treatment criteria:					
Must be treated by a gastroenterologist; or					
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit					
AND					
Clinical criteria:					
Patient must have short bowel syndrome with intestinal failure following major surgery					
AND					
Clinical criteria:					
Patient must have a history of dependence on parenteral support for at least 12 months					
AND					
Clinical criteria:					
Patient must have received a stable parenteral support regimen for at least 3 days per week in the previous 4 weeks					
AND					
Clinical criteria:					
Patient must not have active gastrointestinal malignancy or history of gastrointestinal malignancy within the last 5 years					
AND					
Clinical criteria:					
The treatment must not exceed 12 months under this restriction					
AND					
Clinical criteria:					
Patient must not have previously received PBS-subsidised treatment with this drug for this condition					
Prescribing Instructions:					
Baseline is the mean number of days of parenteral support per week over the four weeks immediately prior to initiating treatment with teduglutide under the PBS initial treatment restriction or four weeks immediately prior to initiating treatment with non-PBS subsidised teduglutide for grandfathered patients.					
Prescribing Instructions:					
Provide a baseline value in this authority application of the amount of parenteral support per week, expressed as either: (i) for a patient of any age, the mean number of days of parenteral support per week (ii) for a patient aged less than 18 years, the mean volume of parenteral support per week in mL per kg. Determine the mean over the four weeks immediately prior to this authority application. For patients aged less than 18 years, both (i) and (ii) may be supplied, but provide at least (i). Measurement of treatment response/failure/stability in future authority applications are to be compared against the baseline value(s).					
Prescribing Instructions:					
A stable parenteral support regimen is defined as a minimum of 3 days of parenteral support (parenteral nutrition with or without IV fluids) per week for 4 consecutive weeks to meet caloric, fluid or electrolyte needs.					
Prescribing Instructions:					
Baseline number of days of parenteral support should be documented in the patient's medical records.					
Prescribing Instructions:					

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<p>The authority application must be made in writing and must include:</p> <p>(1) a completed authority prescription form; and</p> <p>(2) a completed Short bowel syndrome with intestinal failure form; and</p> <p>(3) details of baseline mean number of days on parenteral support per week for 4 consecutive weeks immediately preceding this application; and</p> <p>(4) Patients aged 17 years or less: documentation of the average weekly volume of PS in terms of mL per kg of body weight for the 4 consecutive weeks immediately preceding this application; and</p> <p>(5) documented duration in months of prior dependence on parenteral support.</p>
<p>Prescribing Instructions:</p> <p>The authority application will be assessed manually by the relevant Government agency and must include:</p> <p>(1) a completed authority prescription; and</p> <p>(2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)</p>
<p>Administrative Advice: A patient may only qualify for PBS-subsidised treatment under this restriction once in a lifetime.</p>
<p>Administrative Advice:</p> <p>Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).</p> <p>Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au</p> <p>Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos</p> <p>Or mailed to:</p> <p>Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>

- 3.4 The PBAC considered that the Prescribing Instruction, ‘Baseline number of days of parenteral support should be documented in the patient’s medical records’, could be removed as this information is currently captured by the authority application form assessed by Services Australia.
- 3.5 The PBAC noted that the proposed changes to the Prescribing Instructions aims to provide a baseline measure of weight-normalised PS volume requirements in patients aged less than 18 years to measure treatment response in this patient cohort.

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Category / Program: Section 100 – Highly Specialised Drugs Program (Public & Private hospitals)					
Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners					
Restriction type: <input checked="" type="checkbox"/> Authority Required – non-immediate assessment by Services Australia (In-writing only via mail/postal service or electronic lodgement)					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Type III Short bowel syndrome with intestinal failure					
Treatment Phase: First continuing treatment					
Treatment criteria:					
Must be treated by a gastroenterologist; or					
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit					
AND					
Clinical criteria:					
Patient must have previously received PBS-subsidised initial treatment with this drug for this condition; or					
Patient must have received PBS-subsidised treatment with this drug for this condition as a grandfathered patient					
AND					
Clinical criteria:					
Patient (any age) must have a reduction in parenteral support frequency of at least one day per week compared to the mean number of days per week at baseline; or					
Patient is less than 18 years of age and has a reduction in weekly PS volume of at least 20% (mL per kg of body weight) from baseline					
Patient must have, as a patient aged less than 18 years, a reduction in the mean weekly parenteral support volume of at least 20% (mL per kg of body weight) relative to baseline					
Prescribing Instructions:					
Baseline is the mean number of days of parenteral support per week over the four weeks immediately prior to initiating treatment with teduglutide under the PBS initial treatment restriction or four weeks immediately prior to initiating treatment with non-PBS subsidised teduglutide for grandfathered patients.					
OR					
the mean volume (mL per kg of body weight) of PS per week over the four weeks immediately prior to initiating treatment under the PBS initial treatment restriction					
Prescribing Instructions:					
Refer to the measurement(s) stated in the Initial treatment authority application for the baseline dependence on parenteral support. Determine the current mean use per week of parental support in days (for a patient of any age) and/or in mean volume per week in mL per kg (for a patient less than 18 years of age). State these values in this authority application.					
Prescribing Instructions:					
The current mean number of days of parenteral support is calculated as the mean number of days in which any parenteral support is required (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period					
Prescribing Instructions:					
The current mean weekly parenteral support volume is calculated as the mean mL per kg of body weight of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.					
From [insert listing date here]					
Where the mean weekly volume of parenteral support in terms of mL per kg of body weight for 4 consecutive weeks has not been stated in an Initial treatment authority application for a patient currently aged less than 18 years, provide both: (1) a known or estimated retrospective baseline value that would have applied to the patient immediately before commencing treatment with this drug, and, (2) the current value in this authority application, if mean weekly volume is being used to assess response in a child. Do not provide these values if response assessment relies solely on the mean number of days of parenteral support.					

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<p>Prescribing Instructions: Treatment failure For applications for first continuing treatment, failure of treatment is defined as no change compared to baseline in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs.</p>
<p>Prescribing Instructions: Treatment failure Adults: For applications for first continuing treatment, failure of treatment is defined as no change compared to baseline in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs.</p> <p>Children less than 18 years of age: For applications for first continuing treatment, failure of treatment is defined as: (i) no change compared to baseline in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs; or (ii) a reduction of less than 20% compared to baseline in weekly parenteral support volume (mL per kg of body weight; parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs</p>
<p>Prescribing Instructions: Patients who experience failure of treatment must permanently discontinue treatment.</p>
<p>Prescribing Instructions: Current mean number of days of parenteral support should be documented in the patient's medical records.</p>
<p>Prescribing Instructions: The authority application must be made in writing and must include: (1) a completed authority prescription form; and (2) a completed Short bowel syndrome with intestinal failure Form; and (3) details of the mean number of days reduction of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs from baseline; and (4) the current mean number of days per week of parenteral support over the preceding 4 week period.</p>
<p>Prescribing Instructions: The authority application will be assessed manually by the relevant Government agency and must include: (1) a completed authority prescription; and (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)</p>
<p>Administrative Advice: A patient may only qualify for PBS-subsidised treatment under this restriction once in a lifetime.</p>
<p>Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).</p> <p>Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au</p> <p>Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>

- 3.6 The PBAC considered the requested addition of an alternative definition of baseline may introduce practical administrative difficulties for Services Australia for existing patients aged less than 18 years who do not have baseline figures based on the submission's requested new measures. The PBAC considered that adding new Prescribing Instructions that allow estimated retrospective baseline figures (mean volume in mL per kg) to be provided to Services Australia would allow patients aged less than 18 years to receive continuing treatment.
- 3.7 The current definition of treatment failure in the First continuing treatment restriction is: 'no change compared to baseline in the mean number of days per week in

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parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs'. This definition does not technically cover patients who have an improvement of less than one day per week in PS compared to baseline (for example, 0.5 days improvement), or patients who have an increase in PS compared to baseline.

- 3.8 At the March 2019 meeting, the PBAC considered that patients whose PS requirements increase (i.e. at least one day per week increase in PD over a consecutive 4-week period) while on teduglutide under the continuing restriction should be required to cease teduglutide therapy permanently (paragraph 7.4, teduglutide PSD, March 2019).
- 3.9 The PBAC noted that patients who do not meet the clinical criterion "Patient must have a reduction in parenteral support frequency of at least one day per week compared to the mean number of days per week at baseline" or the proposed clinical criterion "Patient must have, as a patient aged less than 18 years, a reduction in the mean weekly parenteral support volume of at least 20% (mL per kg of body weight) relative to baseline" would not be approved for treatment under the first continuing treatment restriction. The intent of the first continuing restriction is that if the minimum improvements specified in the clinical criteria are not met, PBS subsidy be discontinued permanently. This is different to the subsequent continuing eligibility criteria, where a small, absolute improvement not meeting the definition of a response, requires a trial cessation period, but not permanent discontinuation of PBS subsidy. In this regard, the PBAC considered that a definition for treatment failure in the first continuing treatment restriction was not required.

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Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Type III Short bowel syndrome with intestinal failure
Treatment Phase: Subsequent continuing treatment
Treatment criteria:
Must be treated by a gastroenterologist; or
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit
AND
Clinical criteria:
Patient must have received PBS-subsidised first-continuing treatment with this drug for this condition and achieved a treatment response in the preceding treatment period; or
Patient must have received PBS-subsidised recommencement of treatment following a trial cessation period and not have previously experienced a failure to respond to treatment with this drug for this condition
Prescribing Instructions:
Treatment response
For applications for subsequent continuing treatment, treatment response is when there was a reduction in the mean number of days of parenteral support of at least 1 day per week since the last assessment for PBS-subsidised treatment, OR where a patient has completely ceased treatment with parenteral support for a period of at least 4 consecutive weeks.
Prescribing Instructions:
Treatment response
Adult:
For applications for subsequent continuing treatment, treatment response is when there was a reduction in the mean number of days of parenteral support of at least 1 day per week since the last assessment for PBS-subsidised treatment; or Where a patient has completely ceased treatment with parenteral support for a period of at least 4 consecutive weeks
Child (age less than 18 years):
For applications for subsequent continuing treatment, treatment response is when there was a reduction in the mean number of days of parenteral support of at least 1 day per week since the last assessment for PBS-subsidised treatment; or Where there is a reduction in the mean weekly volume of parenteral support (mL per kg of body weight) of at least 20% since the last assessment for PBS-subsidised treatment; or Where a patient has completely ceased treatment with parenteral support for a period of at least 4 consecutive weeks
Prescribing Instructions:
The current mean number of days of parenteral support is calculated as the mean number of days in which any parenteral support is required (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.
Prescribing Instructions:
The current mean weekly parenteral support volume is calculated as the mean mL per kg of body weight of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.
Prescribing Instructions:
Treatment failure
For applications for subsequent continuing treatment, failure of treatment is defined as an increase in the mean number of days per week of parenteral support requirements of at least 1 day per week over the preceding 4 week period compared to the last assessment for PBS-subsidised treatment of parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs.
Prescribing Instructions:
Treatment failure
Adult:
For applications for subsequent continuing treatment, failure of treatment is defined as an increase in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs.
Child (age less than 18 years):
For applications for subsequent continuing treatment, failure of treatment is defined as an increase in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs; or An increase of 20% or more in the mean weekly volume of parenteral support (mL per kg of body weight; parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs
Prescribing Instructions: All Patients
Patients who experience failure of treatment must permanently discontinue treatment.

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Prescribing Instructions:

Treatment stability

Patients who neither demonstrate a treatment response nor a treatment failure since the last assessment for PBS-subsidised treatment are considered to have a stable parenteral support regimen, defined as the same mean number of days of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the 4 weeks preceding treatment period, where the number of days is greater than zero and the mean number of days of parenteral support is less than baseline. Patients with a stable parenteral support regimen over 6 months must undertake a trial cessation period. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Prescribing Instructions:

Treatment stability

Adult:

Patients who neither demonstrate a treatment response nor a treatment failure since the last assessment for PBS-subsidised treatment are considered to have a stable parenteral support regimen, defined as the same mean number of days of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the 4 weeks preceding treatment period, where the number of days is greater than zero and the mean number of days of parenteral support is less than baseline. Patients with a stable parenteral support regimen over 6 months must undertake a trial cessation period. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Child (aged less than 18 years):

Patients who neither demonstrate a treatment response nor a treatment failure since the last assessment for PBS-subsidised treatment are considered to have a stable parenteral support regimen. A stable parenteral support regimen is defined as either the same mean number of days of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the 4 weeks preceding treatment period, where the number of days is greater than zero and the mean number of days of parenteral support is less than baseline; or
A change (increase or decrease) of less than 20% in mean weekly parenteral support volume (mL per kg of body weight) to meet caloric, fluid or electrolyte needs over the 4 weeks preceding treatment period

Prescribing Instructions:

Trial cessation period

Patients who demonstrate a stable frequency of mean days per week of parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Prescribing Instructions:

Trial cessation period

Adult:

Patients who demonstrate a stable frequency of mean days per week in parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Child (age less than 18 years):

Patients who demonstrate a stable frequency of mean days or volume (mL per kg body weight) per week in parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Prescribing Instructions:

The authority application must be made in writing and must include:

- (1) a completed authority prescription form; and
- (2) a completed Short bowel syndrome with intestinal failure Form; and
- (3) details of the mean number of days reduction of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the preceding treatment period or confirmation the patient has had 4 consecutive weeks without parenteral support (if applicable); and
- (4) the current mean number of days per week of parenteral support over the preceding 4 week period.

Prescribing Instructions:

The authority application will be assessed manually by the relevant Government agency and must include:

- (1) a completed authority prescription; and
- (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)

Administrative Advice:

Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).

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Or mailed to:

Services Australia

Complex Drugs

Reply Paid 9826

HOBART TAS 7001

Administrative Advice: No increase in the maximum number of repeats may be authorised.

Administrative Advice: Special Pricing Arrangements apply.

Indication: Type III Short bowel syndrome with intestinal failure

Treatment Phase: Recommencement of treatment

Treatment criteria:

Must be treated by a gastroenterologist; or

Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit

AND

Clinical criteria:

Patient must have received PBS-subsidised treatment with this drug for this condition

AND

Clinical criteria:

Patient must have undertaken a trial cessation period due to experiencing a stable parenteral support regimen in the first continuing or subsequent continuing treatment phase, and not due to a treatment failure

AND

Clinical criteria:

Patient must have experienced deterioration during a trial cessation period

Prescribing Instructions:

Trial cessation period

Patients who demonstrate a stable frequency of mean days per week of parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Prescribing Instructions:

Trial cessation period

Adult:

Patients who demonstrate a stable frequency of mean days per week of parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Child (age less than 18 years):

Patients who demonstrate a stable frequency of mean days or volume (mL per kg body weight) per week in parenteral support in a 6-month period commencing after the initial 12 months of treatment with this drug for this condition are required to undertake a trial of treatment cessation. Patients who have re-commenced after a trial cessation period are exempt from further trial cessation.

Prescribing Instructions:

Deterioration during the trial cessation period includes an increase in parenteral support frequency of more than or equal to one day per week from the pre-cessation level, or other clinical parameters suggestive of deterioration including changes in renal function or urinary sodium levels or changes in body weight.

Prescribing Instructions:

Deterioration during the trial cessation period includes:

Adult:

An increase in parenteral support frequency of more than or equal to one day per week from the pre-cessation level, or other clinical parameters suggestive of deterioration including changes in renal function or urinary sodium levels or changes in body weight.

Child (age less than 18 years):

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(i) an increase in parenteral support frequency of more than or equal to one day per week from the pre-cessation level; or
(ii) an increase in average weekly parenteral support volume of more than 20% (mL per kg of body weight); or
(iii) other clinical parameters suggestive of deterioration including changes in renal function or urinary sodium levels, serum electrolyte, urea or creatinine disturbances or reduction in body weight

Prescribing Instructions:

The authority application must be made in writing and must include:

- (1) a completed authority prescription form; and
- (2) a completed Short bowel syndrome with intestinal failure Form; and
- (3) details of the reason for recommencement after trial cessation; and
- (4) the current mean number of days per week of parenteral support over the preceding 4 week period
- (5) details of completion of the trial cessation period including the start and end date.

Prescribing Instructions:

The authority application will be assessed manually by the relevant Government agency and must include:

- (1) a completed authority prescription; and
- (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)

Administrative Advice:

Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).

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- 3.10 The PBAC noted that the Grandfather listings [attached to PBS item codes: 11808L (Pub) / 11812Q (Priv)] are now more than 12 months old. The PBAC considered it was appropriate to delete these listings.

For more detail on PBAC's view, see section 5 PBAC outcome.

4 Consideration of the evidence

Sponsor hearing

- 4.1 There was no hearing for this item.

Consumer comments

- 4.2 The PBAC noted and welcomed the input from Parenteral Nutrition Down Under (PNDU) via the Consumer Comments facility on the PBS website. The comment described the significant effects on quality of life for children with SBS-IF. PNDU stated that any reduction in the total volume of PS equates to a reduction in the total number of hours of PS, thereby improving the child's quality of life, and considered that a reduction in the number of hours of PS should be incorporated into the current restrictions.

Clinical trials

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- 4.3 The submission stated that for paediatric patients, there are only non-randomised open-label trials available, where the duration of follow-up is short and patient numbers are low.
- 4.4 The submission provided six studies from a systematic literature search, identifying all trials where teduglutide is used in paediatric patients diagnosed with SBS-IF.
- 4.5 Details of the trials presented in the submission are provided in the table below.

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Table 1: Trials and associated reports presented in the submission

Trial ID	Protocol title/ Publication title	Type of report	Inclusion/exclusion
Included			
TED-C13-003	A 12-week Pharmacokinetic, Safety, and Pharmacodynamic Study of Teduglutide in Pediatric Subjects Aged 1 Year Through 17 Years, with Short Bowel Syndrome who are Dependent of Parenteral Support; Final report: 15 June 2015. <i>Carter, B.A., Cohran, V.C., Cole, C.R., et al. (2017). "Outcomes from a 12-week, open-label, multicentre clinical trial of teduglutide in pediatric short bowel syndrome." Journal of Pediatrics 181: 102-111.</i>	Clinical study report Full publication	Included
SHP633-303	A Retrospective and Prospective, Open-label, Long-term Safety and Efficacy Study of Teduglutide in Pediatric Subjects with Short Bowel Syndrome Who Completed TED-c13-003. Interim report: 04 June 2018. (Final expected Q1 2021)	Interim clinical study report	Included
TED-C14-006	A 24-week Double-blind, Safety, Efficacy, and Pharmacodynamic Study Investigating Two Doses of Teduglutide in Pediatric Subjects Through 17 Years of Age with Short Bowel Syndrome who are Dependent on Parenteral Support; Final report: 06 February 2018. Kocoshis, P. B., Merritt, R. J., Hill, S., et al. (2020). "Safety and efficacy of teduglutide in pediatric patients with intestinal failure due to short bowel syndrome: a 24-week, phase III study." <i>Journal of Parenteral and Enteral Nutrition</i> 44: 621-631. <i>Kocoshis, S., Carter, B., Hill, S., et al (2018). "Efficacy, growth, and safety outcomes of teduglutide in children with short bowel syndrome-associated intestinal failure (SBS-IF): a phase 3 study." Journal of Pediatric Gastroenterology and Nutrition 67 (Suppl 1): S271-2.</i>	Clinical study report Full publication Conference abstract	Included
SHP633-304	A Prospective, Open-Label, Long-term, Safety and Efficacy Study of Teduglutide in Pediatric patients with Short Bowel Syndrome Who Completed TED-c14-006 Interim Report; 05 July 2018. (Final expected Q1 2021)	Interim clinical study report	Included
SHP633-302	A 24-week Safety, Efficacy, Pharmacodynamic, and Pharmacokinetic Study of Teduglutide in Japanese Pediatric Subjects, Aged 4 Months through 15 Years, with Short Bowel Syndrome who are Dependent on Parenteral Support. Final report: 27 May 2020.	Clinical study report	Included
TED-R13-002	A prospective, multi-center registry for pediatric patients with short bowel syndrome. Interim report: 22 March 2017.	Interim clinical study report	Included
Included (identified after the literature searches were conducted)			
Ramos et al., 2020	Ramos Boluda, E., Redecillas Ferreiro, S., Manrique Moral, O. et al. "Experience with teduglutide in paediatric short bowel syndrome: first real-life data." <i>J Ped Gastro Nut</i> : publish ahead of print. DOI: 10.1097/MPG.0000000000002899	Full publication	Included

Source: Tables 5 and 6, Appendix A of the minor submission

SLR = Systematic literature review

Italics indicate the two trials identified during the March 2019 evaluation.

Comparative effectiveness

- 4.6 The submission claimed that, overall, the body of evidence presented confirms that:
- “Teduglutide treatment at the approved dose of 0.05 mg/kg/day is associated with clinically significant reductions in weight-normalised PS volume, days per week and hours per day.
 - Teduglutide treatment at the approved dose of 0.05 mg/kg/day is associated with complete weaning from PS in some patients (20% of patients by the end of 12 weeks in TED-C13-003 and 11.5% of patients by the end of week 24 in TED-C14-006).
 - The clinical trials efficacy data are supported by a longer-term (1-year) real world evidence study.
 - Interim data from long-term open-label extension studies suggest that efficacy is maintained during long-term treatment with teduglutide.”

Comparative harms

- 4.7 The submission stated that the available clinical evidence indicates that teduglutide is generally safe and well tolerated in the paediatric SBS-IF population.
- 4.8 The submission provided the latest periodic safety update report for teduglutide (reporting period 31 August 2018 to 30 August 2019) which concluded there were no newly identified risks and no important changes to the previous knowledge regarding the efficacy and safety of teduglutide.

Estimated PBS usage & financial implications

- 4.9 The submission presented updated financial estimates for 2019 to 2023 to align with the years specified in the existing Risk Sharing Arrangement (RSA).
- 4.10 The submission noted that since the March 2019 submission, patient co-payment fees have increased from \$40.30 for general ordinary services and \$6.50 for general safety net and concessional ordinary services, to \$41.00 and \$6.60 respectively. These are the only inputs that differ between the estimates presented in this submission and those within the current RSA.
- 4.11 The revised total impact to the PBS is presented in Table 2. Based on the effective price of teduglutide, the submission estimated the net cost to the PBS to be \$0 to < \$10 million in 2019, increasing to \$0 to < \$10 million in 2021, and tapering to \$0 to < \$10 million in 2023.

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Table 2: Estimated use and financial implications

	2019	2020	2021	2022	2023
TED eligible adult patients	■ ¹	■ ¹	■ ¹	■ ¹	■ ¹
TED eligible paediatric patients	■ ¹	■ ¹	■ ¹	■ ¹	■ ¹
Uptake rate	40%	50%	70%	95%	100%
Net new patients electing treatment on TED	■ ¹	■ ¹	■ ¹	■ ¹	■ ¹
Total number of patients receiving TED (after mortality and discontinuation adjustment)	■ ¹	■ ¹	■ ¹	■ ¹	■ ¹
Total number of TED scripts dispensed (13.04/patient/year)	■ ¹	■ ²	■ ²	■ ²	■ ²
Cost to PBS/RPBS	\$■ ³	\$■ ³	\$■ ³	\$■ ³	\$■ ³
Less co-payments	-\$■ ³	-\$■ ³	-\$■ ³	-\$■ ³	-\$■ ³
Net cost to PBS/RPBS^a	\$■ ³	\$■ ³	\$■ ³	\$■ ³	\$■ ³
Net cost to PBS/RPBS (from RSA)^b	\$■ ³	\$■ ³	\$■ ³	\$■ ³	\$■ ³

Source: Tables 10, 11, 12 and 14 of the minor submission (pages 45-48); REVESTIVE_paeds_UCM-Release-3-Workbook-v106; "2b.1 epi workings", 2b. patients prevalent", "3a. Scripts proposed", "3c. Impact – proposed (eff)"

TED, teduglutide

^a The difference between the minor submission's net cost values and those in the current RSA (in the row below) is due to patient co-payment fees, which have increased from \$40.30 for general ordinary services and \$6.50 for general safety net and concessional ordinary services, to \$41.00 and \$6.60 respectively.

^b Net cost values as per the current RSA. The paediatric patient numbers were added post-PBAC recommendation.

The redacted values correspond to the following ranges:

¹ < 500

² 500 to < 5,000

³ \$0 to < \$10 million

Financial Management - Risk Sharing Arrangements

4.12 No changes are proposed to any aspect of the current RSA for teduglutide.

For more detail on PBAC's view, see section 5 PBAC outcome.

5 PBAC Outcome

5.1 The PBAC recommended amending the Section 100 (Highly Specialised Drugs Program) Authority required listing of teduglutide for the treatment of paediatric patients with Type III (chronic) short bowel syndrome (SBS) with intestinal failure (SBS-IF) to include specific response criteria for paediatric patients.

5.2 The PBAC recalled it considered that the restriction should not specify any age criteria given teduglutide has been studied in paediatric patients and was shown to be well tolerated (paragraph 7.6, teduglutide PSD, March 2019).

5.3 The PBAC acknowledged that, as children's nutritional requirements continue to increase with continued body growth, it may not always be possible to demonstrate a benefit in terms of reduced PS days within the specified timeframes of the current

restriction, even in circumstances when the child is benefiting from treatment with teduglutide. The PBAC noted that input from a paediatric advisory board formed the basis of, and justification for, the submission's request to include a 20% reduction in weekly PS volume as a response criterion for paediatric patients. The PBAC noted that a 20% reduction in weekly PS volume is a primary outcome of the teduglutide clinical trials in adult and paediatric patients. Overall, the PBAC considered that for patients aged less than 18 years, a reduction in the mean weekly PS volume of at least 20% (mL per kg of body weight) relative to baseline is clinically meaningful.

- 5.4 The PBAC considered that for existing paediatric patients who would not have a documented baseline parenteral support amount expressed in mean weekly volume (mL/kg per), it would be reasonable for Services Australia to accept a retrospective estimate of the baseline value for the purpose of demonstrating a clinical benefit in continuing treatment (either first continuing or subsequent continuing) authority applications.
- 5.5 The PBAC considered that the clinical criteria in the first continuing treatment restriction clearly specifies the treatment response required for patients to be approved for treatment under this restriction. As such, the PBAC considered that a specific definition of treatment failure is not required (see paragraph 3.9).
- 5.6 The PBAC considered it was appropriate to provide clear administrative handling arrangements for paediatric patients who were under 18 years of age at the last authority application and who have since turned 18. For these patients, it would be appropriate to allow either mean days of PS, or mean volume of PS per week to be used for the first authority application after turning 18. In future authorities following the first authority application after turning 18, reversion to mean days per week is expected.
- 5.7 The PBAC considered it may not be possible to capture all signs and symptoms which indicate that a patient has deteriorated during a trial cessation period in an exhaustive list. The PBAC advised that the treating clinician should confirm deterioration during a trial cessation period based on any factors that may be relevant. The PBAC advised that this should be clarified in the recommencement of treatment restriction.
- 5.8 The PBAC noted that the current recommencement of treatment restriction does not allow for patients who take a treatment break from teduglutide to recommence treatment through this restriction. The PBAC noted there may be medical reasons for which a treatment break may be clinically appropriate and advised that the current restriction should be amended to allow these patients to recommence treatment.
- 5.9 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

Outcome:

Recommended

6 Recommended listing

6.1 Amend existing listing as follows:

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	No. of Rpts	Available brands
TEDUGLUTIDE					
teduglutide 5 mg injection [28 vials] (& inert substance diluent [28 x 0.5 mL syringes], 1 pack	11793Q (Public) 11795T (Private)	1	28	11	Revestive
Edit Restriction Summary 9569 / Treatment of Concept: 9569 (as at 1 March 2021)					
Category / Program: Section 100 – Highly Specialised Drugs Program (Public & Private hospitals)					
Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners					
Restriction type: <input checked="" type="checkbox"/> Authority Required (non-immediate/delayed assessment by Services Australia)					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Type III Short bowel syndrome with intestinal failure					
Treatment Phase: Initial treatment					
Treatment criteria:					
Must be treated by a gastroenterologist; or					
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit					
AND					
Clinical criteria:					
Patient must have short bowel syndrome with intestinal failure following major surgery					
AND					
Clinical criteria:					
Patient must have a history of dependence on parenteral support for at least 12 months					
AND					
Clinical criteria:					
Patient must have received a stable parenteral support regimen for at least 3 days per week in the previous 4 weeks					
AND					
Clinical criteria:					
Patient must not have active gastrointestinal malignancy or history of gastrointestinal malignancy within the last 5 years					
AND					
Clinical criteria:					
The treatment must not exceed 12 months under this restriction					
AND					
Clinical criteria:					
Patient must not have previously received PBS-subsidised treatment with this drug for this condition					
Prescribing Instructions:					
Provide a baseline value in this authority application of the amount of parenteral support per week, expressed as either:					
(i) for a patient of any age, the mean number of days of parenteral support per week					
(ii) for a patient aged less than 18 years, the mean volume of parenteral support per week in mL per kg.					
Determine the mean over the four weeks immediately prior to this authority application. For a patient aged less than 18 years, both (i) and (ii) may be supplied, but provide at least (i). Assessment of treatment response/non-response in the 'First continuing treatment' authority application will be compared against the baseline value(s) submitted in this application.					
Prescribing Instructions:					
A stable parenteral support regimen is defined as a minimum of 3 days of parenteral support (parenteral nutrition with or without IV fluids) per week for 4 consecutive weeks to meet caloric, fluid or electrolyte needs.					

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<p>Prescribing Instructions: The authority application must include: (1) a completed authority prescription; and (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)</p>
<p>Administrative Advice: A patient may only qualify for PBS-subsidised treatment under this restriction once in a lifetime.</p>
<p>Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	No. of Rpts	Available brands
TEDUGLUTIDE					
teduglutide 5 mg injection [28 vials] (& inert substance diluent [28 x 0.5 mL syringes], 1 pack	11794R (Pub) 11806J (Priv)	1	28	5	Revestive
Edit Restriction Summary 9684 / Treatment of Concept: 9793 (as at 1 March 2021)					
Category / Program: Section 100 – Highly Specialised Drugs Program (Public & Private hospitals)					
Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners					
Restriction type: <input checked="" type="checkbox"/> Authority Required (non-immediate/delayed assessment by Services Australia)					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Type III Short bowel syndrome with intestinal failure					
Treatment Phase: First continuing treatment					
Treatment criteria:					
Must be treated by a gastroenterologist; or					
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit					
AND					
Clinical criteria:					
Patient must have previously received PBS-subsidised initial treatment with this drug for this condition; or					
Patient must have received PBS-subsidised treatment with this drug for this condition as a grandfathered patient					
AND					
Clinical criteria:					
Patient must have a reduction in parenteral support frequency of at least one day per week compared to the mean number of days per week at baseline; or					
Patient must have, as a patient aged less than 18 years, a reduction in the mean weekly parenteral support volume of at least 20% (mL per kg of body weight) relative to baseline					
Prescribing Instructions: Refer to the measurement(s) stated in the Initial treatment authority application for the baseline dependence on parenteral support. Determine the current mean use per week of parental support in days (for a patient of any age) and/or the mean volume per week in mL per kg (for a patient less than 18 years of age). State these values in this authority application.					
Prescribing Instructions:					

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<p>The current mean number of days of parenteral support is calculated as the mean number of days in which any parenteral support is required (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period</p>
<p>Prescribing Instructions: The current mean weekly parenteral support volume is calculated as the mean mL per kg of body weight of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.</p>
<p>Prescribing Instructions: From [insert listing date here] Where the mean weekly volume of parenteral support in terms of mL per kg of body weight for 4 consecutive weeks has not been stated in an Initial treatment authority application for a patient currently aged less than 18 years, provide in this authority application both: (i) a known or estimated retrospective baseline value that would have applied to the patient immediately before commencing treatment with this drug, and (ii) the current value (observed over the preceding 4 weeks) Provide these values for a child only where mean weekly volume is to be used as an alternative response assessment to mean days of parenteral support per week. Otherwise, continue to use mean days per week.</p>
<p>Prescribing Instructions: A patient who has turned 18 years of age since their last authority application may be assessed for response using either the mean number of days of parenteral support or mean volume of parenteral support. Any subsequent authority application after this application must be assessed using the mean number of days of parenteral support.</p>
<p>Prescribing Instructions: Patients who do not meet the clinical criteria with respect to demonstrating the minimum reduction in parenteral support must permanently discontinue PBS subsidy.</p>
<p>Prescribing Instructions: The authority application must include: (1) a completed authority prescription; and (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)</p>
<p>Administrative Advice: A patient may only qualify for PBS-subsidised treatment under this restriction once in a lifetime.</p>
<p>Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>
<p>Edit Restriction Summary 9761 / ToC: 9740 (as at 1 March 2021)</p>
<p>Administrative Advice: No increase in the maximum number of repeats may be authorised.</p>
<p>Administrative Advice: Special Pricing Arrangements apply.</p>
<p>Indication: Type III Short bowel syndrome with intestinal failure</p>
<p>Treatment Phase: Subsequent continuing treatment</p>
<p>Treatment criteria:</p>
<p>Must be treated by a gastroenterologist; or</p>
<p>Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit</p>
<p>AND</p>
<p>Clinical criteria:</p>
<p>Patient must have received PBS-subsidised first-continuing treatment with this drug for this condition and achieved a treatment response in the preceding treatment period; or</p>

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<p>Patient must have received PBS-subsidised recommencement of treatment following a trial cessation period and not have previously experienced a failure to respond to treatment with this drug for this condition</p>
<p>Prescribing Instructions: <u>Treatment response</u> Adult: In a subsequent continuing treatment application, treatment response in a patient aged at least 18 years is one of: (i) a reduction in the mean number of days of parenteral support of at least 1 day per week since the last assessment for PBS-subsidised treatment; or (ii) parenteral support completely ceasing (due to clinical benefit from this drug) for a period of at least 4 consecutive weeks</p> <p>Child (age less than 18 years): In a subsequent continuing treatment application, treatment response in a child is one of: (iii) a reduction in the mean number of days of parenteral support of at least 1 day per week since the last assessment for PBS-subsidised treatment; or (iv) a reduction in the mean weekly volume of parenteral support (mL per kg of body weight) of at least 20% since the last assessment for PBS-subsidised treatment; or (v) parenteral support completely ceasing (due to clinical benefit from this drug) for a period of at least 4 consecutive weeks</p>
<p>Prescribing Instructions: A patient who has turned 18 years of age since their last authority application may be assessed for response using either the mean number of days of parenteral support or mean volume of parenteral support. Any subsequent authority application after this application must be assessed using the mean number of days of parenteral support.</p>
<p>Prescribing Instructions: The current mean number of days of parenteral support is calculated as the mean number of days in which any parenteral support is required (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.</p>
<p>Prescribing Instructions: The current mean weekly parenteral support volume is calculated as the mean mL per kg of body weight of parenteral support (parenteral nutrition with or without IV fluids) per week to meet caloric, fluid or electrolyte needs over the immediately preceding 4 week treatment period.</p>
<p>Prescribing Instructions: <u>Treatment failure</u> Adult: In a subsequent continuing treatment application, treatment failure is defined as an increase in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs, compared to the most recent authority application.</p> <p>Child (age less than 18 years): In a subsequent continuing treatment application, treatment failure is defined as at least one of the following occurring: (i) an increase in the mean number of days per week in parenteral support (parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs, compared to the most recent authority application; or (ii) an increase of 20% or more in the mean weekly volume of parenteral support (mL per kg of body weight; parenteral nutrition with or without IV fluids) to meet caloric, fluid or electrolyte needs, compared to most recent authority application</p>
<p>Prescribing Instructions: Patients who experience failure of treatment must permanently discontinue treatment.</p>
<p>Prescribing Instructions: <u>Treatment stability</u> A patient who neither demonstrates a treatment response as defined above nor a treatment failure as defined above since the last assessment for PBS-subsidised treatment is considered to have 'treatment stability'.</p> <p><u>Trial cessation period</u> A patient with treatment stability must undertake a trial cessation period. A patient who re-commences treatment after a trial cessation period is exempt from further trial cessation periods.</p>
<p>Prescribing Instructions: The authority application must include: (1) a completed authority prescription; and (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)</p>
<p>Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).</p>

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<p>Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au</p> <p>Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos</p> <p>Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>
Edit Restriction Summary 9685 / ToC: 9829 (as at 1 March 2021)
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Type III Short bowel syndrome with intestinal failure
Treatment Phase: Recommencement of treatment
Treatment criteria:
Must be treated by a gastroenterologist; or
Must be treated by a specialist within a multidisciplinary intestinal rehabilitation unit
AND
Clinical criteria:
Patient must have received PBS-subsidised treatment with this drug for this condition
AND
Clinical criteria:
Patient must have undertaken a trial cessation period due to experiencing a stable parenteral support regimen in the first continuing or subsequent continuing treatment phase, and not due to a treatment failure; or
Patient must have undertaken a trial cessation period for any medical reason other than lack of treatment efficacy
AND
Clinical criteria:
Patient must have experienced deterioration during a trial cessation period
Prescribing Instructions:
Deterioration during the trial cessation period includes an increase in parenteral support use, as well as changes in renal function, urinary sodium levels and changes in body weight in the absence of an increase in parenteral support use. This is not an exhaustive list of the signs/symptoms of disease deterioration – the treating physician must be satisfied that in the absence of treatment with this drug, the patient's condition has deteriorated.
Prescribing Instructions:
The authority application must include: (1) a completed authority prescription; and (2) the relevant completed authority application form for this condition and treatment phase (location referenced in the administrative Note)
Administrative Advice:
Authority applications to continue treatment after this application should occur under the 'Subsequent continuing treatment' phase.
Administrative Advice:
Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).
<p>Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au</p> <p>Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos</p> <p>Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001</p>

This restriction may be subject to further review. Should there be any changes made to the restriction the Sponsor will be informed.

7 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

8 Sponsor's Comment

The sponsor had no comment.