

7.13 PROTEIN FORMULA WITH VITAMINS AND MINERALS, AND LOW IN POTASSIUM, PHOSPHORUS, CALCIUM, CHLORIDE AND VITAMIN A

Oral liquid 125 mL, 24,
Renastep[®],
Vitaflo Australia Pty Ltd

1 Purpose of Application

- 1.1 The minor resubmission requested Authority Required (STREAMLINED) listing of protein formula with vitamin and minerals and low in potassium, phosphorus, calcium, chloride and vitamin A (hereafter referred to as Renastep[®]) for the treatment of chronic renal failure (CRF) for patients aged 3 to 18 years on a cost minimisation basis per calorie of energy equivalent against Renastart[®].

2 Background

- 2.1 At the November 2019 PBAC meeting, the Committee recommended Authority Required (STREAMLINED) listing of Renastep for the treatment of CRF for infants and young children on a cost minimisation basis per calorie of energy equivalent against the cheapest alternative PBS listed whey protein formula indicated for CRF (paragraph 7.1, Renastep Public Summary Document (PSD), November 2019 PBAC meeting). At the time of this recommendation, Kindergen[®] was the cheapest alternative PBS listed whey protein formula indicated for CRF.
- 2.2 At its November 2019 meeting, the PBAC agreed with the Nutritional Products Working Party (NPWP) that Renastart was an appropriate comparator for Renastep, but considered that Kindergen was also a relevant pricing comparator (paragraph 7.4, Renastep PSD, November 2019 PBAC meeting).

3 Requested listing

- 3.1 The requested listing is presented below. Suggestions and additions proposed by the Secretariat are in italics and deletions are in strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Dispensed Price for Max. Qty	Proprietary Manufacturer	Name and
CASEIN PROTEIN FORMULA SUPPLEMENTED WITH AMINO ACIDS, DOCOSAHEXAENOIC ACID, VITAMINS AND MINERALS, AND LOW IN CALCIUM, PHOSPHATE, POTASSIUM, CHLORIDE AND VITAMIN A Oral liquid, 24 × 125 mL bottle	8	5	\$1,369.73	Renastep [®]	Vitaflo Australia Pty Ltd

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Restriction Summary 6190 / Treatment of Concept: 6190

Category / Program: GENERAL – General Schedule (Code GE)
Prescriber type: <input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input checked="" type="checkbox"/> Nurse practitioners (CTO) <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives
Restriction type/ Method: <input type="checkbox"/> Unrestricted benefit <input type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required – In Writing <input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency <input checked="" type="checkbox"/> Authority Required – Streamlined
Indication: Chronic renal failure
Population criteria: Patient must be a child aged 3 to 18 years inclusive years or older
AND
Clinical criteria: Patient must require treatment with a low modified protein, low phosphorus diet
Or
Clinical criteria: Patient must require treatment with a low modified -protein, low phosphorus, low potassium diet

3.2 At its November 2019 meeting, the PBAC noted that the requested indication for Renastep was for children aged 3 to 18 years inclusive, whereas the comparator Renastart was listed for infants and young children. The PBAC considered the PBS population for Renastep should be consistent with Renastart to offer flexibility to young patients (paragraph 7.7, Renastart PSD, November 2019 PBAC meeting).

3.3 In the resubmission, the sponsor reiterated its previous request to list Renastep for children aged 3 to 18 years inclusive.

For more detail on PBAC’s view, see section 7 PBAC outcome.

4 Comparator

4.1 The resubmission nominated Renastart as the main comparator for Renastep. The reasons provided were that Renastep is therapeutically equivalent to Renastart and contains the critical nutrient, DHA. The resubmission also listed the following similarities between Renastart and Renastep:

- Both are exclusively for use in the dietary management of paediatric CRF;
- Both contain added vitamins, minerals and trace elements to meet Australian Nutritional Reference Values (with the exception of those that are commonly restricted in CRF);
- Both contain low levels of calcium, chloride, potassium, phosphorus and Vitamin A; and
- Both contain DHA.

4.2 The PBAC previously considered that Kindergen was also a relevant pricing comparator (paragraph 7.4, Renastep PSD, November 2019 PBAC meeting).

4.3 The resubmission presented no new evidence as to why Kindergen should not be considered an appropriate pricing comparator.

4.4 As in the original November 2019 submission, the resubmission nominated Kindergen as a secondary comparator, but claimed that it is less likely for Renastep to displace Kindergen as the latter does not contain DHA or AHA. [REDACTED]

4.5 The sponsor indicated that if Renastep was not recommended for PBS listing on a cost-minimisation basis against Renastart, the sponsor would not proceed with PBS listing for Renastep.

For more detail on PBAC’s view, see section 7 PBAC outcome.

5 Consideration of the evidence

Sponsor hearing

5.1 There was no hearing for this item as it was a minor submission.

Consumer comments

5.2 The PBAC noted that no consumer comments were received for this resubmission.

Clinical trials

5.3 The resubmission provided no new clinical data.

Economic analysis

5.4 The resubmission requested to list Renastep on a cost minimisation basis to Renastart, as outlined in Table 1.

5.5 The proposed dispensed price for maximum quantity (DPMQ) is \$1,369.73.

Table 1: Proposed pricing structure for Renastep

	Renastart price (\$)	Renastep price (\$)
AEMP (per carton)	\$309.34 (11856 kcal) 6 x 400 g tins	\$156.55 (6000kcal) 24 x125 mL Bottles
AEMP (per max qty)	\$1,237.36 (47424 kcal) 6 x 400 g tins x 4 packs	\$1,252.40 (48000kcal) 24 x125 mL x 8
Wholesaler mark up (7.52%)	\$69.94	\$69.94
Price to pharmacy	\$1,307.30	\$1,322.34
Pharmacy mark-up (\$40 for medicines with AEMP over \$1000.01)	\$40.00	\$40.00
Dispensing fee	\$7.39	\$7.39
DPMQ	\$1,358.27	\$1,369.73

Source: Table 1 of the submission July 2020

Drug cost/patient/year: \$7,816.55

- 5.6 The estimated Renastep cost/patient/year, as per the minor resubmission, would be \$7,816.55, based on 5.75 scripts per year and the proposed DPMQ of \$1,369.73 less the average patient copayment of \$10.33.

Estimated PBS usage & financial implications

- 5.7 In order to estimate the number of patients likely to transfer from Renastart to Renastep, the resubmission included the following assumptions in the financial calculations (these assumptions have not changed from the November 2019 submission):
- 10 (/20) patients (50%) are aged 3-10 years and these are the only patients who would potentially switch from Renastart to Renastep.
 - 5 (50%) of these patients are aged 3-5 years and therefore the remaining 5 patients are aged 5 to ≤10 years.
- 5.8 The minor resubmission estimated a cost to the PBS of less than \$10 million in Year 6 of listing, with a total cost to the PBS of less than \$10 million over the first 6 years of listing.
- 5.9 In the pre-PBAC response, the sponsor clarified that the estimated cost to the PBS in Year 6 was less than \$10 million, with a total net save to the PBS of less than \$10 million over the first 6 years of listing. This is summarised in the table below as well as the sponsor's expected patient and prescription numbers, which were also updated in the pre-PBAC response.

Table 2: Estimated use and financial implications

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of patients treated ^a	█	█	█	█	█	█
Number of scripts dispensed ^{a,b}	█	█	█	█	█	█
Estimated financial implications of Renastep						
Cost to PBS/RPBS	\$█	\$█	\$█	\$█	\$█	\$█
Copayments	\$█	\$█	\$█	\$█	\$█	\$█
Cost to PBS/RPBS (without co-payment)	\$█	\$█	\$█	\$█	\$█	\$█
Estimated financial implications for replacement of Renastart (proposed comparator)						
Cost to PBS/RPBS ^a	\$█	\$█	\$█	\$█	\$█	\$█
Copayments	\$█	\$█	\$█	\$█	\$█	\$█
Cost to PBS/RPBS (without co-payment) ^a	\$█	\$█	\$█	\$█	\$█	\$█
Net financial implications						
Net cost to PBS/RPBS ^a	-\$█	\$█	\$█	\$█	\$█	\$█

^a Figures updated following receipt of pre-PBAC response

^b Assuming █ scripts per patient per year as estimated by the submission (Appendix 7). Estimates assume Renastep will replace Renastart at 1:1 ratio.

Source: Summary Page – Renastep utilisation and cost model spreadsheet; pre-PBAC Response

The redacted table shows that at Year 6, the estimated number of patients treated was less than 10,000 and the estimated number of scripts dispensed was less than 10,000.


5.10 As a minor submission, the financial estimates have not been independently evaluated.

For more detail on PBAC’s view, see section 7 PBAC outcome.

6 NPWP Consideration

6.1 The NPWP noted that the sponsor disagreed with the PBAC’s recommendation at the November 2019 meeting for Renastep to be cost-minimised to the lowest-cost PBS-listed whey protein formula for chronic kidney disease. The NPWP reiterated its previous advice to the PBAC that Renastart was an appropriate comparator for Renastep.

6.2 The NPWP noted the resubmission’s claim that Kindergen was not an appropriate comparator, as it did not contain DHA and AHA. █

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- 6.3 The NPWP advised that the cost minimisation should be based on price per kcal as Renastart is a renal feed. The NPWP advised that renal feeds are generally low in protein and clinically the dose is based on the energy provided, whereas some other PBS listed foods for special medical purposes are priced per gram of protein because the dose prescribed is based on the protein provided.
- 6.4 The NPWP advised that Renastep meets a clinical gap in nutritional requirements for children aged 10-18 years as it provides twice the calories of Renastart and Kindergen. Traditionally, children in this age bracket use adult renal feeds.
- 6.5 The NPWP considered that some clinicians and patients may prefer Renastep, as it is a ready-to-use formulation (rather than a powder) and suitable for use in children over the age of 3 years.
- 6.6 The NPWP disagreed with the PBAC's recommendation that the PBS population for Renastep should be consistent with that for Renastart (infants and young children) to provide flexibility to young patients. The NPWP considered that the PBS population for Renastep should be children aged 3 years and over.
- 6.7 The NPWP reiterated its previous advice that the submission had underestimated the likely use of Renastep and that patients aged 10-18 years were a new population which would grow the market and result in a cost to the PBS. However, the NPWP noted a substantial portion of the cost would result from shifting current treatment costs from patients and hospitals to the PBS.
- 6.8 The NPWP advised that the inclusion of DHA and AHA should not be considered a reason for a price premium as there is no strong evidence for therapeutic benefit in older children. The comparison of Renastep to the Nutrient Reference Values for infants was not provided in the application.

For more detail on PBAC's view, see section 7 PBAC outcome.

7 PBAC Outcome

- 7.1 The PBAC recommended the Authority Required (STREAMLINED) listing of protein formula with vitamins and minerals and low in potassium (Renastep®) for the treatment of chronic renal failure for patients aged 3 to 18 years on a cost minimisation basis per calorie of energy equivalent against Kindergen.
- 7.2 The PBAC agreed with the NPWP that Renastart was an appropriate comparator for Renastep, but considered that Kindergen was also a relevant pricing comparator.
- 7.3 The PBAC noted the NPWP advice that the cost minimisation should be based on price per kcal.

- 7.4 The PBAC noted the resubmission’s claim that Kindergen was not an appropriate comparator, as it did not contain DHA and AHA. [REDACTED]
- [REDACTED] The PBAC noted the NPWP advice that the inclusion of DHA and AHA should not be considered a reason for a price premium as there is no strong evidence for therapeutic benefit in older children. The PBAC thus considered Kindergen was an appropriate comparator.
- 7.5 The PBAC noted the NPWP advice that Renastep meets a clinical gap in nutritional requirements for children aged 10 to 18 years, providing twice the calories of Renastart and Kindergen. The PBAC recommended a small price premium be applied to recognise the nutritional value of Renastep for patients aged 10 to 18 years.
- 7.6 The PBAC noted the NPWP advice that some clinicians and patients may prefer Renastep, as it is a ready-to-use formulation (rather than a powder) and suitable for use in children over the age of 3 years who are otherwise treated with adult protein formulas through hospital access programs.
- 7.7 The PBAC noted the advice of the NPWP on the population criteria and advised that the PBS population should be patients aged 3 years or older.
- 7.8 The PBAC maintained its November 2019 advice that Renastep is suitable for prescribing by nurse practitioners, and the Early Supply Rule should not apply.
- 7.9 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that because Renastep is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over the comparator, or not expected to address a high and urgent unmet clinical need given the presence of an alternative therapy, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2009* for pricing Pathway A were not met.
- 7.10 The PBAC noted that this submission is not eligible for an Independent Review because it received a positive recommendation.

Outcome:

Recommended

8 Recommended listing

- 8.1 Add new item:

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Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Manufacturer	Name and
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Oral liquid, 24 × 125 mL bottle

Restriction Summary 6190 / Treatment of Concept: 6190

Category / Program: GENERAL – General Schedule (Code GE)
Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners <input checked="" type="checkbox"/> Nurse practitioners (CTO)
Restriction type / Method: <input type="checkbox"/> Authority Required – Streamlined
Indication: Chronic renal failure
Population criteria: Patient must be a child aged 3 years or older
AND
Clinical criteria: Patient must require treatment with a low protein, low phosphorus diet
Or
Clinical criteria: Patient must require treatment with a low protein, low phosphorus, low potassium diet

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

9 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.

10 Sponsor's Comment

The sponsor had no comment.