

**5.23 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 submission requested an Authority Required (STREAMLINED) listing of casein protein formula for the treatment of chronic renal failure for patients aged 3 to 18 years.

2 Requested listing

2.1 The submission requested the following new listing.

2.2 Suggestions and additions proposed by the Secretariat to the requested listing are in italics and deletions are in strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Dispensed Price for Max. Qty	Proprietary Manufacturer	Name and
PROTEIN FORMULA WITH VITAMINS AND MINERALS, AND LOW IN POTASSIUM, PHOSPHORUS, CALCIUM, CHLORIDE AND VITAMIN A Oral liquid, 24 × 125 mL bottles	8	5	\$1,627	Renastep [®]	Vitaflo Australia Pty Ltd

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 <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives
PBS Indication:	Chronic kidney disease renal failure
Restriction Level / Method:	<input checked="" type="checkbox"/> Streamlined
Clinical criteria:	Patient must require treatment with a modified protein, low phosphorus diet OR Patient must require treatment with modified protein, low phosphorus, low potassium diet
Population criteria:	Patient must be <i>an infant or a young child.</i>

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

3 Current situation

- 3.1 The Sponsor of Renastep® confirmed that it meets the requirements of foods that have medical purposes as set out under The Australia New Zealand Food Standards Code – Standard 2.9.5: Food for Special Medical Purposes.
- 3.2 The submission claimed that Renastep has a nutritional profile tailored to patients from 3 years of age with chronic kidney disease (CKD). The formulation has a higher protein concentration and double the caloric density of the main comparator, Renastart. The minor submission claimed that these patients can receive adequate protein and calories despite a limited fluid allowance, and taking a lower volume may also help to alleviate the nausea, vomiting and poor appetite which often occur with CKD.
- 3.3 Renastep® has not been considered by PBAC previously.

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

4 Comparator

- 4.1 The minor submission nominated Renastart (VitaFlo Australia) as the main comparator as it is therapeutically similar to Renastep and contains long-chain polyunsaturated fatty acid and docosahexaenoic acid (DHA).
- 4.2 The submission also nominated another comparator, KinderGen, but claimed that it is less likely for Renastep to displace KinderGen as the latter does not contain DHA or arachidonic acid.
- 4.3 A comparison of the energy and protein composition of Renastep compared to the nominated comparators is provided in Table 1.

Table 1: Nutritional composition of Renastep and comparators

Nutrient per 100ml	Renastep	Renastart*	KinderGen**
Energy (kJ)	836	414	421
(kcal)	200	99	101
Protein (g)	4.0	1.5	1.5

*Renastart Standard dilution as per manufacturer’s recommendation: 20% w/v (20g powder made up to 100ml with water)

**KinderGen Standard dilution as per manufacturer’s recommendation: 20% w/v (20g powder made up to 100ml with water)

[Source: Extracted from submission appendix 1]

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 item.

Clinical trials

5.3 The minor submission included a clinical trial examining the acceptability of Renastep in 11 patients aged between 3 and 18 years with CKD. The trial was conducted over three specialist paediatric renal centres in the UK: Southampton Children’s Hospital, Alder Hey Children’s Hospital and Birmingham Children’s Hospital.

5.4 The trial was a descriptive study, which did not provide a direct comparison between Renastep and Renastart, the main comparator. The study did not investigate the potential benefits from increased protein concentration and caloric density as claimed by the submission. The study found that Renastep was useful in helping patients adhere to their complex dietary regime, due to its ready-to-use formulation.

Economic analysis

5.5 The submission claimed there is an unmet clinical need for the proposed listing, as there is no product listed on the PBS for use in the dietary management of CKD for children aged between 10 and 18 years. For this reason, the Sponsor requested to list Renastep on a cost minimisation basis to Renastart on a per calorie equivalent basis, plus a ■■■% price increase, as shown in Table 2.

Table 2: Pricing Structure for Renastep

	Renastart price (\$)	Renastep price (with requested 20% price increase) (\$)	Renastep price (without requested 20% price increase) (\$)
AEMP (per carton)	309.34 (11856 kcal) 6 x 400 g tins	██████ (6000 kcal) 24 x 125 mL bottles	██████
AEMP (per max qty)	1237.36 (47424 kcal) 6 x 400 g tins x 4 packs	██████ (48000 kcal) 24 x 125 mL bottles x 8 packs	██████
Wholesaler mark up	+69.94	+69.94	+69.94
Price to pharmacy	1307.30	██████	██████
Pharmacy mark up (\$40 for medicines with AEMP over \$1000.01)	+40.00	+40.00	+40.00
Dispensing fee	+7.39	+7.39	+7.39
DPMQ	54.69	██████	██████

Source: Table 1 of the submission. Price without requested 20% price increase calculated by the PBAC Secretariat.

Estimated PBS usage & financial implications

5.6 In order to estimate the number of patients likely to transfer from Renastart to Renastep, the submission included the following assumption in its financial calculations:

- 10 (out of 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.

5.7 The minor submission 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 based on the proposed price. This is summarised in Table 3 with the Sponsor's expected patient and prescription numbers.

Table 3: 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						
Number of scripts dispensed ^a						
Estimated financial implications of Renastep						
Cost to PBS/RPBS						
Copayments						
Cost to PBS/RPBS (without co-payment)						
Estimated financial implications for replacement of Renastart						
Cost to PBS/RPBS						
Copayments						
Cost to PBS/RPBS (without co-payment)						
Net financial implications						
Net cost to PBS/RPBS						

^a Assuming 5.75 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

The redacted table shows that at Year 6, the estimated number of scripts dispensed was less than 10,000 and the net cost to the PBS would be less than \$10 million.

5.8 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 Nutritional Products Working Party Consideration (and sponsor’s further clarification)

6.1 The Nutritional Products Working Party (NPWP) noted the requested listing of Renastep® for the dietary management of paediatric patients with chronic kidney disease (CKD) aged 3 – 18 years old. The NPWP considered that Renastart® was the appropriate comparator.

6.2 At present, the PBS subsidises protein formulas for paediatric CKD designed for patients who are infants or young children. The NPWP noted that patients aged 10 years and above are individually managed with protein formulas indicated for adult patients, often provided through access arrangements in treating hospitals. The NPWP noted that there are inequities in current arrangements for patients in the target age group, as not all hospitals assume the costs of these formulas and some families rely on financial assistance from charities to assist with the cost of managing the condition.

6.3 Furthermore, the NPWP considered that treating paediatric patients with adult dietary formulas for CKD was not optimal, as their nutritional profiles and calorie densities are not designed for this patient group. While the calorie density of

Renastep® was designed for paediatric patients, the NPWP noted the product could not be the sole source of nutrition for most patients due to insufficient levels of some vitamins and minerals (including iron and vitamin A) to meet dietary requirements in the target age group.

- 6.4 The NPWP considered the submission underestimated the likely use of Renastep® and noted 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.
- 6.5 The NPWP supported the listing of Renastep® on a cost minimisation basis with Renastart® at the same price per kcal of energy. The NPWP considered that there was no clinical rationale for the requested 20% price premium over the comparator's price per kcal of energy.
- 6.6 The Sponsor's Pre-PBAC response acknowledged the views of the NPWP, and provided a revised request to list Renastep on a cost-minimisation basis with Renastart per kilocalorie of energy.

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 infant and young children on a cost minimisation basis per calorie of energy equivalent against the cheapest alternative PBS listed whey protein formula indicated for chronic renal failure.
- 7.2 The PBAC noted that the Sponsor has withdrawn the requested 20% price premium over the comparator's price per kilocalorie of energy in the pre-PBAC response.
- 7.3 The PBAC noted the NPWP advice that patients aged 10-18 years with chronic renal failure are individually managed with protein formulas indicated for adult patients in a hospital setting. The PBAC acknowledged there are inequities in the current arrangement (as noted in paragraph 6.2), and that the listing of Renastep on the PBS could provide an alternative for these patients.
- 7.4 The PBAC agreed with the NPWP that Renastart was an appropriate comparator for Renastep, but considered that Kindergen was also a relevant pricing comparator.
- 7.5 The PBAC noted that the total number of calories in the proposed maximum quantity (8 packs with 5 repeats) aligns with that for Renastart®, but that the patient population for Renastep (3 to 18 years) is broader than for Renastart (0-10 years), and some of the older patients may require a higher protein and energy intake. However, the PBAC considered the requested maximum quantity and number of repeats were appropriate as Renastep is most likely to be used in younger patients.

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- 7.6 The PBAC considered it would be appropriate to update the proposed indication to chronic renal failure to align with the indication of the main comparator, Renastart.
- 7.7 The PBAC noted that the comparator, Renastart is currently listed for infants and young children, but the restriction is silent on age. The PBAC considered the PBS population for Renastep should be consistent with Renastart to offer flexibility to young patients.
- 7.8 The PBAC recommended that, under Section 101(3BA) of the National Health Act 1953, Renastep should be treated as interchangeable with Renastart on an individual patient basis.
- 7.9 The PBAC advised that Renastep is suitable for prescribing by nurse practitioners, similar to other nutritional products currently listed for the dietary management of patients with chronic renal failure.
- 7.10 The PBAC recommended that the Early Supply Rule should not apply to Renastep, since it has previously been the PBAC's view that general nutrients be exempt.
- 7.11 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 Renastart, 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.
- 7.12 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:

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name and Manufacturer	
PROTEIN FORMULA WITH VITAMINS AND MINERALS, AND LOW IN POTASSIUM, PHOSPHORUS, CALCIUM, CHLORIDE AND VITAMIN A Oral liquid, 24 x 125 mL bottles	8	5	Renastep®	Vitaflo Australia Pty Ltd
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 <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
PBS Indication:	Chronic renal failure			
Restriction Level / Method:	<input checked="" type="checkbox"/> Streamlined			

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Clinical criteria:	Patient must require treatment with a modified protein, low phosphorus diet OR Patient must require treatment with modified protein, low phosphorus, low potassium diet
Population criteria	Patient must be an infant or a young child.

The restriction is subject to further review. Should there be any substantial changes 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.