

5.11 AMINO ACID FORMULA WITH VITAMINS AND MINERALS, LOW PHENYLALANINE AND SUPPLEMENTED WITH DOCOSAHEXAENOIC ACID AND ARACHIDONIC ACID

Sachets containing oral powder 12.5 g, 30 (PKU Explore 5)

Sachets containing oral powder 25 g, 30 (PKU Explore 10)

PKU Explore[®], Vitaflo Australia Pty Ltd.

1 Purpose of Application

- 1.1 The minor submission requested Restricted Benefit listings for two new forms of amino acid formula with vitamins and minerals, low phenylalanine and supplemented with docosahexaenoic acid and arachidonic acid (PKU Explore) for the dietary management of phenylketonuria (PKU).

2 Requested Listing

- 2.1 The submission requested the following new listing. Suggestions and additions proposed by the Secretariat to the requested listing are added in italics and suggested deletions are crossed out with strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Dispensed Price for Max. Qty	Proprietary Name and Manufacturer
AMINO ACID FORMULA WITH VITAMINS AND MINERALS, LOW PHENYLALANINE AND SUPPLEMENTED WITH DOCOSAHEXAENOIC ACID AND ARACHIDONIC ACID				
powder for oral liquid, 30 x 12.5 g sachets	8	5	\$1000.62	PKU Explore5 [®] Vitaflo Australia Pty Ltd
powder for oral liquid, 30 x 25 g sachets	4	5	\$1000.75	PKU Explore10 [®] 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
Condition:	Phenylketonuria
PBS Indication:	Phenylketonuria

Restriction Method:	Level /	<input checked="" type="checkbox"/> Restricted benefit
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- 2.2 The requested maximum quantity and repeats provide approximately one month of supply with five repeats, which is consistent with the main comparator PKU Anamix First Spoon®.

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

3 Background

- 3.1 The sponsor of PKU Explore confirmed that it meets the requirements for 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 PBAC has not considered PKU Explore previously.
- 3.3 PKU Explore was designed as a second stage protein substitute and will be used in the dietary management of older infants and young children with PKU to meet their increasing protein requirements as they grow.
- 3.4 The nominated comparator, PKU Anamix First Spoon, is low in phenylalanine and contains amino acids, vitamins, minerals and docosahexaenoic acid, and was considered and recommended by the PBAC at its March 2017 meeting.
- 3.5 The Secretariat noted that PKU Gel, a similar product to PKU Explore, is also available on the PBS. The submission stated that PKU Gel is an earlier generation product, which was formulated before long chain polyunsaturated fatty acids (arachidonic acid and docosahexaenoic acid) were routinely added to the protein substitute for infants and children from 6 months to 5 years of age, and hence is no longer ideal for these older infants and children with PKU. The submission stated that PKU Explore is intended to replace PKU Gel, and that PKU Gel will be delisted once PKU Explore is PBS listed.

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

4 Comparator

- 4.1 The minor submission nominated PKU Anamix First Spoon (PBS code: 11185Q) as the main comparator.
- 4.2 PKU Explore products contain the same protein equivalent (PE) per sachet as the PKU Anamix First Spoon, at 5 g of PE in a 12.5 g sachet, and 10 g of PE in a 25 g sachet.
- 4.3 The Secretariat noted that while PKU Gel has a different formulation to PKU Explore, it might also be an appropriate comparator, as the intention is to replace the former with the latter.

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 submission presented a study which evaluated the acceptability and tolerance of PKU Explore 5 over a 28-day time period in the dietary management of children aged from a weaning-age to 5 years with PKU.

5.4 The study observed 19 children with PKU aged from 5 months to 4 years, all diagnosed by newborn screening. The submission claimed that 13 out of 19 children (68%) were able to take PKU Explore 5 during the 28-day intervention period, and 58% of children continued to take this product long term.

5.5 The clinical significance of the study was not evaluated in the context of the minor submission.

Estimated PBS usage & financial implications

5.6 The submission proposed that PKU Explore be cost-minimised to PKU Anamix First Spoon at an equivalent price-per-gram of PE to PKU Anamix First Spoon. The submission stated that PKU Explore would have an identical cost-per-gram of PE when compared to PKU Anamix First Spoon, and a slightly higher cost-per-gram of PE when compared to PKU Gel. Therefore, if PKU Explore were to replace PKU Anamix Infant First Spoon, it would be cost-neutral to the PBS; whereas if PKU Explore were to replace PKU Gel, the cost to the PBS would increase by approximately \$1,179.02 per patient over 12 months. The pricing structures between the three products are summarised in Table 1.

Table 1: Pricing Structure for PKU Explore and PKU Gel

	PKU Explore5	PKU Explore10	PKU Gel
AEMP (per carton)	\$111.83 (150g PE)	\$223.66 (300g PE)	\$191.24 (300g PE)
AEMP (per Max Qty)	111.83 x 8 = \$894.64 (1200g PE)	223.66 x 4 = \$894.64 (1200g PE)	191.24 x 4 = \$764.96 (1200g PE)
DPMQ	\$1000.62	\$1000.62	\$856.30

PE = protein equivalent

Source: extracted from Appendix 8 of the minor submission and Table 1 of minor submission, p17.

5.7 The submission claimed that the listing of PKU Explore should not result in any new patients commencing dietary therapy for PKU. The submission indicated that sales of

PKU Anamix First Spoon and PKU Gel would decline as sales of PKU Explore increase; and that the sponsor intends to delist PKU Gel once PKU Explore is PBS listed.

5.8 Table 2 presents the estimated number of patients treated and net costs to PBS over the next six years for PKU Explore.

Table 2: Estimated number of patients treated and net costs to PBS over next 6 years

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
PKU Explore5						
No. Patients						
No. Prescriptions						
Net Cost to PBS** (\$)						
PKU Explore10						
No. Patients						
No. Prescriptions						
Net Cost to PBS** (\$)						
Total net cost to PBS (\$)						

Note: Estimate based on new patient commencing. The submission stated the course of treatment for PKU Explore 5 is not likely to be longer than 6 months for any 1 patient and so the Cost to the PBS is estimated to be less than the amount listed for each of the estimated number of patients per year.

** DPMQ less patient co-payment

Source: Tables from pp18-19 of the submission.

The redacted table shows that the total estimated number of patients over 6 years was in the range of 10,000 – 50,000, and the net cost to the PBS was less than \$10 million.

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

6 NPWP Consideration

6.1 The Nutritional Product Working Party (NPWP) supported the listing of PKU Explore as it provides an alternative treatment for some patients as part of their dietary management of PKU. It noted that the levels of vitamins and minerals such as manganese and potassium were lower in PKU Explore than PKU Anamix First Spoon. The iron quantity of the product provides around 80% of dietary requirements, which is clinically considered to be on the lower end of reasonable limits for patients across all ages. However, the NPWP considered that these nutrients could be obtained from daily dietary intake.

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

7 PBAC Outcome

7.1 The PBAC recommended the listings for two new forms of amino acid formula with vitamins and minerals, low phenylalanine and supplemented with docosahexaenoic acid and arachidonic acid (PKU Explore 5 and PKU Explore 10) for the dietary management of phenylketonuria (PKU) on a cost minimisation basis to PKU Anamix First Spoon at an equivalent cost per gram of protein equivalent (PE).

- 7.2 The PBAC noted that the NPWP supported the proposed listing of the products as they provide an alternative treatment for some patients as part of their dietary management of PKU.
- 7.3 The PBAC accepted that PKU Explore would substitute for the currently listed product, PKU Anamix First Spoon. The PBAC accepted that this substitution would be cost neutral to the PBS as PKU Explore and PKU Anamix First Spoon have an equivalent cost per gram PE.
- 7.4 The PBAC noted that PKU Explore is suitable for inclusion in the PBS medicines for prescribing by nurse practitioners, similar to other nutritional product listings for PKU.
- 7.5 The PBAC noted that the Early Supply Rule should not apply to PKU Explore, as it has generally been the PBAC’s view that nutritional product listings be exempt.
- 7.6 In accordance with Section 101 (3BA) of the *National Health Act, 1953*, the PBAC advised it is of the opinion that PKU Explore should be treated as interchangeable on an individual patient basis with PKU Gel, PKU Anamix and PKU Anamix Infant.
- 7.7 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because PKU Explore is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over PKU Anamix First Spoon, 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.
- 7.8 The PBAC noted that this submission is not eligible for an Independent Review, as it received a positive recommendation.

Outcome:

Recommended

8 Recommended listing

- 8.1 Add two new items:

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amino acid formula with vitamins and minerals, low phenylalanine and supplemented with docosahexaenoic acid and arachidonic acid powder for oral liquid, 30 x 12.5 g sachets				

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Restriction Level / Method:	<input checked="" type="checkbox"/> Restricted benefit

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amino acid formula with vitamins and minerals, low phenylalanine and supplemented with docosahexaenoic acid and arachidonic acid powder for oral liquid, 30 x 25 g sachets	4	5	PKU Explore10® Vitaflo Australia Pty Ltd

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9 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised in Australia. It considers submissions in this context. A PBAC decision not to recommend listing or not to recommend changing a listing does not represent a final PBAC view about the merits of the medicine. A company can resubmit to the PBAC or seek independent review of the PBAC decision.

10 Sponsor's Comment

Vitaflo Australia notes that PBAC has accepted the amendments to the PSD.