

5.12 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 Anamix First Spoon), PKU Anamix First Spoon®, Nutricia Australia Pty Ltd.

1 Purpose of Application

1.1 The minor submission requested a Restricted Benefit listing for the treatment of phenylketonuria (PKU).

2 Requested Listing

2.1 The submission requested the following new listing:

2.2 No changes to the proposed listing were suggested by the Secretariat.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Dispensed Price for Max. Qty	Proprietary Manufacturer	Name and
AMINO ACID FORMULA WITH VITAMINS AND MINERALS, LOW PHENYLALANINE AND SUPPLEMENTED WITH DOCOSAHEXAENOIC ACID AND ARACHIDONIC ACID oral powder, 30 × 12.5 g sachets	8	5	\$ [REDACTED]	PKU Anamix First Spoon®	Nutricia 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
Restriction Level / Method:	<input checked="" type="checkbox"/> Restricted benefit

3 Background

3.1 The sponsor of PKU Anamix First Spoon® 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 PKU Anamix First Spoon® has not been considered by PBAC previously and no product containing amino acid formula with vitamins and minerals, low phenylalanine, and supplemented with docosahexaenoic acid has been considered by the PBAC previously.

4 Comparator

- 4.1 The minor submission nominated PKU Gel® as the comparator, as it is a powdered protein substitute in a 24g sachet providing 10g protein equivalent (PE) for PKU patients aged 6 months to 10 years old.

For more detail on PBAC's view, see section 6 "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 As a minor submission, no clinical trials were presented in the submission.
- 5.4 The basis of the request was a claim from the sponsor of a clinical need for a concentrated, low volume protein substitute that can be prepared with a semi-solid consistency for older infants to support the weaning process.
- 5.5 In consideration of the submission, the Nutritional Product Working Party (NPWP) noted that:
- The nominated comparator, PKU Gel®, was appropriate.
 - The level of phenylalanine in PKU Anamix First Spoon® was 13 mg/100 g powder (1.6 mg per 5 g protein equivalent/12.5 g sachet). The comparator PKU Gel® contains no detectable level of phenylalanine. The NPWP did not consider that the low level of phenylalanine in the product was a clinical issue.
 - The submission determined its maximum quantity (MQ) and repeats based on a five year old boy (with classical PKU) consuming 80% of protein requirements (or 36.8 g per day) from PKU Anamix First Spoon®. The submission stated that PKU Anamix First Spoon® must be given in conjunction with another protein source (e.g. breast milk, infant formula). The NPWP considered these assumptions were clinically appropriate.
 - Based on the above assumption regarding the protein requirement of 36.8 g PE per day, a five-year-old PKU patient would require 7.36 sachets each day (rounded up to 8). The product was packaged in 30 sachets per carton that would require a maximum quantity of 8 cartons to supply the needs of a five

year old for one month. The NPWP considered the requested maximum quantity was appropriate.

- The comparison data of nutrient reference values (NRV) provided by the sponsor (Appendix B of the Submission) contained very high percentages of chromium and manganese for a 6-month-old infant, in comparison to the acceptable intake (AI). However, the levels of chromium and manganese were similar to the comparator and were therefore not considered to be of clinical concern. The NPWP also advised that it might be more appropriate for the sponsor to compare their data using the NRV levels for patients at both 6 months and between 7 to 12 months (using a 7 month old as the reference value).
- This product was suitable for use only in patients aged 6 months to 5 years. However, the NPWP considered that as patients will be under the care of a specialist there was no need to include an age limit in the restriction.

The NPWP supported the listing of PKU Anamix First Spoon® as a Restricted Benefit listing for the treatment of phenylketonuria on a cost-minimisation basis against PKU Gel® at an equivalent price per gram of protein equivalent.

Estimated PBS usage & financial implications

- 5.6 The minor submission proposed that PKU Anamix First Spoon® be cost-minimised to PKU Gel® at an equivalent price per gram of PE as PKU Gel®.
- 5.7 Each 12.5 g sachet of PKU Anamix First Spoon® contains 5 g of protein equivalent (PE) with a total of 150 g per carton and 1200 g per proposed maximum quantity of 8 cartons. By comparison, PKU Gel® provides 10 g of PE per 24 g sachet with a total of 300 g per carton and 1200 g per maximum quantity of 4 cartons.
- 5.8 Based on the annual prescriptions of PKU Gel® in 2015 (i.e. 287), and assuming each patient used ■■ prescriptions per year, the submission estimated that there were ■■ prevalent patients who use PKU Gel® (■■■■■■■■■■). The Department reviewed the patient data and determined that there were actually ■■ prevalent patients during this time-frame.
- 5.9 The submission stated that by year 5 of listing, approximately ■■% of the patients of its comparator PKU Gel® would switch to PKU Anamix First Spoon®, which equates to approximately ■■ patients. If the Department's prevalent patient figures from paragraph 5.8 were used to calculate expected switching this would equate to ■■ patients.
- 5.10 The submission claimed that the introduction of PKU Anamix First Spoon® would not result in any new patients commencing dietary treatment for PKU, nor would it increase patient's usage of protein substitutes. If this was the case, the net cost to the PBS of listing Anamix First Spoon® was estimated to be nil.

For more detail on PBAC's view, see section 6 "PBAC outcome"

6 PBAC Outcome

- 6.1 The PBAC recommend the listing of PKU Anamix First Spoon® as a Restricted Benefit for the treatment of phenylketonuria (PKU) on a cost-minimisation basis to PKU Gel® at an equivalent cost per gram of protein equivalent (PE).
- 6.2 The PBAC noted that the NPWP supported the decision to list PKU Anamix First Spoon® on the PBS.
- 6.3 The PBAC considered PKU Gel® an appropriate comparator for PKU Anamix First Spoon®.
- 6.4 The PBAC noted that the submission estimated a nil cost to the PBS as it was expected to substitute for currently listed products on an equivalent cost per gram PE.
- 6.5 In accordance with subsection 101(3BA) of the *National Health Act 1953*, the PBAC advised it is of the opinion that, on the basis of the material available to it at its March 2017 meeting, PKU Anamix First Spoon® should be treated as interchangeable on an individual patient basis with similar nutritional products. Similar products may include PKU Gel® and PKU Anamix Infant®.
- 6.6 The PBAC advised that PKU Anamix First Spoon® was suitable for prescribing by nurse practitioners, as nutritional products are currently included for prescribing by nurse practitioners.
- 6.7 The PBAC recommended that the Early Supply Rule should not apply as it has been the PBAC's view that general nutrients be exempt.
- 6.8 The PBAC noted that this submission was not eligible for an Independent Review, as it had received a positive recommendation.

Outcome:

Recommended

7 Recommended listing

- 7.1 Add new item:

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name and Manufacturer
AMINO ACID FORMULA WITH VITAMINS AND MINERALS, LOW PHENYLALANINE AND SUPPLEMENTED WITH DOCOSAHEXAENOIC ACID AND ARACHIDONIC ACID oral powder, 30 × 12.5 g sachets	8	5	PKU Anamix Nutricia Australia Pty Ltd First Spoon®

Category / Program	GENERAL – General Schedule (Code GE)
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Public Summary Document – March 2017 PBAC Meeting

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
Restriction Level / Method:	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required - In Writing <input type="checkbox"/> Authority Required - Telephone <input type="checkbox"/> Authority Required – Emergency <input type="checkbox"/> Authority Required - Electronic <input type="checkbox"/> Streamlined

8 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.

9 Sponsor's Comment

The sponsor had no comment.