

4.02 AMINO ACID FORMULA WITH CARBOHYDRATE, VITAMINS MINERALS AND TRACE ELEMENTS WITHOUT PHENYLALANINE

Oral liquid: powder for, 30 x 20 g sachets;
 PKU Go, Orpharma Pty Ltd.

1 Purpose of Application

1.1 The minor submission requested a Restricted Benefit listing for phenylketonuria.

2 Requested listing

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 WITHOUT PHENYLALANINE Amino acid formula with vitamins and minerals without phenylalanine containing 15 g protein oral liquid: powder, 30, 20g sachets	4	5	\$ [REDACTED]	PKU Go	Orpharma 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
Episodicity:	-
Severity:	-
Condition:	Phenylketonuria
PBS Indication:	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

3 Background

3.1 PKU Go does not require registration with the TGA. The sponsor 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 Go was considered by the Nutritional Products Working Party (NPWP) in September 2015 and the PBAC in November 2015.

- In September 2015, The NPWP deferred its consideration of the submission until such time that clarification from the sponsor could be sought regarding: whether the product meets “Australia New Zealand Food Standards Code - Standard 2.9.1 - Infant Formula Products”; and the vitamin and mineral values for the product.
- In November 2015, the PBAC noted that the sponsor provided additional information post-submission to address the NPWP concerns. The PBAC then deferred its recommendation until further advice could be provided by the NPWP on the submission in view of this additional information.

3.3 The NPWP considered the additional information for this submission in January 2016, and its updated advice is noted below under ‘Consideration of the evidence’.

4 Comparator

4.1 The submission nominates PKU Gel and PKU Anamix Junior as the main comparators. All 3 products are supplied as a powder sachet and contain 10g protein equivalent per single use pack.

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 In September 2015, in consideration of the submission, the NPWP noted:

- This product has a similar proportion of amino acids to the comparators, PKU Gel® and PKU Anamix Infant®; but it does not contain choline. There is an RDI level for choline, and patients on a PKU-suitable diet are likely to have low levels of choline. The sponsor was therefore encouraged to consider adding choline to future formulations of the product.
- This product can be used across the age range of 6 months to 10 years of age.
- Although the sponsor stated that this product meets “Australia New Zealand Food Standards Code - Standard 2.9.5 - Food for Special Medical Purposes”, the NPWP noted that the submission, for a formula proposed to be used in infants younger than 12 months, did not provide a comparison with the requirements of the “Australia New Zealand Food Standards Code - Standard 2.9.1 - Infant Formula Products”, as recommended in the PBAC Guidelines.
- The sponsor did not provide any information about the osmolality when this product is mixed with 20mL of water. This is important in clinical practice as high

levels can cause gastrointestinal upsets in young children (for example, the 3000 mOsm/kg per 30mL water measured in PKU Gel®).

- The sponsor presented different vitamin and mineral levels in Table A.17 (p. 10) and Attachment 3 of the submission.
- At the price proposed, the submission estimated no net cost to the PBS.

5.5 In January 2016, in consideration of information received post-submission, the NPWP noted that:

- The sponsor provided a suitable comparison against the requirements of the “Australia New Zealand Food Standards Code - Standard 2.9.1 - Infant Formula Products”.
- The sponsor provided corrected vitamin and minerals values for Attachment 3, to align with the values in Attachment A.1.7, page 10 of the original submission, which were accurate. The NPWP noted that the product adequately meets the recommended daily intake for risk nutrients. Whilst the levels were viewed as low among the ‘toddler’ age group, the NPWP considered that higher amounts of product would be used in practice than suggested by the submission.
- The sponsor advised that the manufacturer has begun work to add choline to future formulations of the product, in line with NPWP advice.
- The sponsor provided osmolality results for this product. The NPWP noted that the osmolality was higher than for the comparator PKU Gel®, clinicians prescribing such products, and their colleague dietitians, will be generally aware of recommended water consumption levels.

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

Estimated PBS usage & financial implications

5.7 The DPMQ has been calculated based on the same cost per protein equivalent of the comparator PKU Anamix Junior, consistent with the pricing of all similar nutritional products for PKU.

5.8 The submission assumes less than 10,000 dispensings per year for this product.

5.9 Based on the DPMQ in the submission, there was estimated to be no net cost to the PBS as the submission expects PKU Go to only replace the use of other PBS listed nutritional products for PKU.

6 PBAC Outcome

6.1 The PBAC recommended listing amino acid formula with vitamins carbohydrate, vitamins, and minerals and trace elements without phenylalanine, sachets containing oral powder 20 g, 30 (PKU Go) as a Restricted Benefit for phenylketonuria on a cost-minimisation basis against amino acid formula with vitamins and minerals without phenylalanine, sachets containing oral powder 24 g, 30 (PKU Gel) at an equivalent price per gram of protein.

6.2 The PBAC noted the advice of the NPWP that supported the listing of PKU Go® on

the PBS, following its consideration of additional information received from the sponsor post-submission.

- 6.3 In accordance with subsection 101(3BA) of the Act the PBAC advised that it is of the opinion that, on the basis of the material available to it at its March 2016 meeting, PKU Go® should not be treated as interchangeable on an individual patient basis with any other drugs.
- 6.4 The PBAC recommended that PKU Go® is suitable for inclusion in the PBS medicines for prescribing by nurse practitioners within collaborative arrangements.
- 6.5 The PBAC recommended that the Early Supply Rule should not apply as it has been the PBAC's view that general nutrients be exempt.

Outcome:

Recommended

7 Recommended listing

7.1 Add new item:

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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.