

**11.02 AMINO ACID FORMULA WITH VITAMINS, MINERALS AND LONG CHAIN POLYUNSATURATED FATTY ACIDS WITHOUT PHENYLALANINE, Oral Powder 400 g (PKU Start), PKU Start™, Vitaflo International Pty Ltd**

**1 Purpose of Submission**

- 1.1 The Committee Secretariat submission requested that amino acid formula with vitamins, minerals and long chain polyunsaturated fatty acids without phenylalanine (PKU Start™) with a new formulation continue to be listed on the PBS for the dietary management of PKU under the existing conditions.

**2 Background**

- 2.1 In July 2018, the PBAC recommended the General Schedule Restricted Benefit listing of PKU Start for the dietary management of PKU on a cost-minimisation basis with PKU Anamix Infant at an equivalent price per gram of PE.
- 2.2 The submission stated that PKU Start meets the requirements for foods that have special medical purposes as set out under *The Australia New Zealand Food Standards Code (ANZFSC) – Standard 2.9.5: Food for Special Medical Purposes*, and therefore does not require registration with the Therapeutic Goods Administration.
- 2.3 The submission stated that PKU Start’s reformulation complies with international regulations for infant formula including the published regulations in the European Union and CODEX guidelines. Additionally, the submission stated that PKU Start’s minor formulation changes comply with *ANZFSC Standard 2.9.1: Infant Formula Standards Code* with very minor discrepancies.
- 2.4 As PKU Start is marketed as a nutritional product and not a therapeutic good, it is not registered in the Australian Register of Therapeutic Goods.

**3 Requested listing**

- 3.1 The submission requested no changes to the existing listing of PKU Start under PBS item code 11653H.

**4 Comparator**

- 4.1 The submission nominated PKU Anamix Infant as the main comparator. This was appropriate as PKU Anamix was the accepted comparator for the initial CMA at the

July 2018 PBAC meeting. The current formulation of PKU Start, recommended at the same meeting, is also a relevant comparator (para. 4.1, amino acid formula with vitamins, minerals and long chain polyunsaturated fatty acids without phenylalanine July 2018 Public Summary Document). No claims of comparative safety or efficacy were made in the submission.

## **5 Consideration of the evidence**

### ***Sponsor hearing***

5.1 There was no sponsor hearing for this item.

### ***Consumer comments***

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

### ***Nutritional profile***

5.3 A comparison of the new and current formulation of PKU Start, including comparison to the nominated comparator, is provided in Table 1.

5.4 The submission stated that the following changes have been made and provided an explanation:

- coconut oil has been removed to reduce the risk of emerging contaminants.
- potato starch has been added for the purpose of stabilising the formulation prior to spray drying.

5.5 The submission stated the that the following changes had been made without providing a specific explanation:

- Several nutrient sources were replaced.
- Amino acid profile altered insignificantly (no change to protein (equivalent) content).
- Increase to carbohydrate content (insignificant).
- Increase to iron content, and zinc contents.
- DHA source change and content adjustment. The submission states that the oil from microalgae *schizochytrum sp* provides the source for DHA. Tuna oil is the source of DHA in the current PKU Start product.
- Reduction in arachidonic acid (ARA)content.
- Vitamin E and fatty acid profile alterations.

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Table 1: Comparison of nutritional composition of current and new formulation for PKU Start and nominated comparator.

Nutrient	Product nutritional content (per 100 mL can)		
	PKU Start new formulation	PKU Start current formulation	PKU Anamix Infant
Energy kJ	288	287	293
kcal	69	68	70
Protein Equivalent g	2.0	2.0	2.0
Carbohydrate g	7.3	7.2	7.5
Of which (OW) sugars	0.6	0.7	1.1
Fat g	3.5	3.5	3.5
OW saturates g	0.3	0.9	1.2
OW monounsaturates g	2.4	1.8	1.7
OW polyunsaturates g	0.6	0.6	0.6
OW DHA mg	17	14	18
OW ARA mg	17	28	2.1
OW Linoleic Acid mg	510	536	481
OW α-Linolenic Acid mg	42	48	48
<b>Vitamins</b>			
Vitamin A mcg RE	65	65	61
Vitamin D3 mcg	1.6	1.6	1.7
Vitamin E mg	1.4	1.0	1.4
Vitamin C mg	8.6	8.6	7.3
Vitamin K mcg	6	5.6	5.6
Thiamin mg	0.06	0.06	0.08
Riboflavin mg	0.07	0.07	0.08
Niacin mg (mg NE)	0.48/ (1.3)	0.47/ (1.2)	0.35/ (1.2)
Vitamin B6 mg	0.06	0.06	0.08
Folic acid mcg (folate)	11/ (18)	11	8.3
Vitamin B12 mcg	0.17	0.17	0.18
Biotin mcg	2.7	2.7	2.7
Pantothenic Acid mg	0.3	0.35	0.42
Choline mg	21	21	22
Inositol mg	8.5	8.5	14.7
<b>Minerals</b>			
Sodium mg	27	27	28.7
Potassium mg	72	71	75.8
Chloride mg	54	51	53.3
Calcium mg	56	56	61.5
Phosphorus mg	42	42	45
Magnesium mg	6	6.3	8.8
<b>Trace Elements</b>			
Iron mg	1.1	0.82	1.2
Copper mg	0.06	0.06	0.06
Zinc mg	0.9	0.49	0.84
Manganese mg	0.04	0.04	0.004
Iodine mcg	15	15	14.7
Selenium mcg	3.0	3.0	2.7
Chromium mcg	2.1	2.0	2.1
Molybdenum mcg	2.3	2.3	1.8

Source: Submission Appendix 1

- 5.6 The submission made further dietetic assessment of the formulation changes in Table 1 of the main body and in Appendices 1 to 6 of the submission.
- 5.7 The submission claimed that PKU Start complies with *Standard 2.9.1 – Infant formula products*. The submission stated that all nutrient sources comply, except chromium, which is included as chromium trichloride, which is permitted by international standards. The submission further stated that nutrients exist in PKU Start in permitted amounts, except choline. The submission stated that the sponsor does not understand this to be detrimental to infants taking PKU Start.
- 5.8 The submission states that PKU Start complies with ANZFSC - Schedule 27 of *Standard 1.6.1 - Microbiological limits in food* and limits specified within the Compendium of Microbiological Criteria.

### ***Estimated PBS usage and financial implications***

- 5.9 The submission did not present economic or financial evaluations. The submission requested no change to the current pricing arrangements.
- 5.10 The submission considered the formulation changes will have nil financial impact to Government, as no change in price is requested and it did not estimate any changes in utilisation will occur.

## **6 NPWP Consideration**

- 6.1 The NPWP advised that the new formulation is expected to provide non-inferior clinical benefit for the treatment of phenylketonuria.
- 6.2 The NPWP supported the new formulation of PKU Start continuing to be listed on the PBS under the same conditions as the current formulation.
- 6.3 The NPWP noted the differences between the previous and new formulations of PKU Start, and the comparison with PKU Anamix. The NPWP noted that the increased iron and zinc in the new formulation of PKU Start brings it more in line with PKU Anamix. The NPWP noted that whilst chromium in PKU start and PKU Anamix infant is high compared to NRV it meets FSANZ infant formula guidelines. The NPWP considered that the other changes to components such as fatty acids were not significant.
- 6.4 The NPWP noted that, for much of the past year, PKU Start was the only PBS-listed infant formula for the treatment of PKU available in Australia due to product shortages.
- 6.5 The NPWP considered that there is a paucity of products on the PBS for the treatment of PKU in each age group, and expressed concern that many PKU treatments have either been delisted from the PBS or are experiencing ongoing supply issues. The NPWP emphasised that there is a strong clinical need for PKU treatments on the PBS and that adequate patient care relies heavily on the availability of a range of PKU treatments for each age group.

## **7 PBAC Outcome**

- 7.1 The PBAC recommended the new formulation of amino acid formula with vitamins, minerals and long chain polyunsaturated fatty acids without phenylalanine (PKU Start™) continue to be listed on the PBS for the dietary management of PKU under the existing conditions of the current formulation.
- 7.2 The PBAC agreed with the NPWP advice that the new formulation is expected to provide a non-inferior clinical benefit for the management of PKU.
- 7.3 The PBAC noted that the sponsor requested no changes to the current pricing arrangement, and that this recommendation will result in a nil cost to Government.
- 7.4 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 No change to the existing listing

## **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.