

## 6.11 GLYCOMACROPEPTIDE AND ESSENTIAL AMINO ACIDS WITH VITAMINS AND MINERALS

**Sachets containing oral powder 16 g, 60,  
Sachets containing oral powder 32 g, 30,  
PKU Build 10<sup>®</sup>, PKU Build 20<sup>®</sup>,  
Cortex Health Pty Ltd.**

### 1 Purpose of Application

- 1.1 The minor submission requested a change to the formulation of the glycomacropeptide formula, PKU Build, to include docosahexaenoic acid (DHA), a lower level of phenylalanine and other changes to the nutritional profile.

### 2 Requested listing

- 2.1 The submission requested no changes to the PBS listing of PKU Build. Current listing information is presented below.
- 2.2 Suggestions and additions proposed by the Secretariat to the current 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 Name and Manufacturer	
GLYCOMACROPEPTIDE AND ESSENTIAL AMINO ACIDS WITH VITAMINS AND MINERALS					
powder for oral liquid, 60 x 16 g sachets	2	5	\$1,073.17	PKU Build <sup>®</sup> 10	Cortex Health Pty Ltd
powder for oral liquid, 30 x 32 g sachets	4	5	\$2,068.79	PKU Build <sup>®</sup> 20	

<b>Category / Program:</b>	GENERAL – General Schedule (Code GE)
<b>Prescriber type:</b>	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input checked="" type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives
<b>PBS Indication:</b>	Phenylketonuria
<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required – In Writing <input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency <input type="checkbox"/> Streamlined
<b>Administration Advice:</b>	<i>This product is low in folic acid, choline and methionine.</i>

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

### 3 Background

- 3.1 PKU Build was recommended by the PBAC at its November 2017 meeting, on a cost minimisation basis with Camino Pro Bettermilk and Camino Pro Bettermilk Lite. At that time, the Nutritional Products Working Party (NPWP) noted that PKU Build contains only 56% of the recommended daily intake (RDI) of iron for children aged one to three years old.
- 3.2 The basis of the minor submission’s request was that the product was reformulated in response to dietitian and clinician requests for DHA to be included in PKU Build.
- 3.3 The sponsor of PKU Build previously confirmed it meets the requirements for foods for medical purposes as set out under The Australia New Zealand Food Standards Code – Standard 2.9.5: Food for Special Medical Purposes when the PBAC considered the matter at its November 2017 meeting (PKU Build PSD, November 2017).

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

### 4 Comparator

- 4.1 The submission did not nominate a comparator. At the time of original recommendation (November 2017), the PBAC accepted Camino Pro Bettermilk and Camino Pro Bettermilk Lite as the comparators.

*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 As a minor submission, no clinical trials were presented in the submission.

#### ***Other relevant matters***

- 5.4 As well as the addition of DHA to the formulation, the levels of phenylalanine have decreased, however the nutritional profile has also changed. Table 1 below outlines changes in sachet size and levels of DHA, phenylalanine and iron (which the NPWP previously considered low) between the current and proposed formulations.

**Table 1: Comparison of sachet size and key nutrients**

	PKU Build 10		PKU Build 20	
Formulation change	Nov 2017 PBAC	Nov 2019 PBAC	Nov 2017 PBAC	Nov 2019 PBAC

	PKU Build 10		PKU Build 20	
Sachet size	16 g	15 g	32 g	30 g
Protein	10 g	10 g	20 g	20 g
Energy	226 kJ	210 kJ	452 kJ	420 kJ
Carbohydrate	2 g	0.4 g	4 g	0.7 g
Fat	0.6 g	0.6 g	1.1 g	1.3 g
Dietary fibre	0 g	0.1 g	0 g	0.2 g
Docosahexaenoic acid	0 mg	25 mg	0 mg	50 mg
Phenylalanine	15 mg	10 mg	30 mg	20 mg
Iron	3.6 mg	3.9 mg	7.2 mg	7.8 mg

Source: Table 1 of the submission

**Drug cost/patient/course: \$1,073.17 (PKU Build 10), \$2,068.79 (PKU Build 20).**

5.5 The submission did not request a change to the current dispensed prices for maximum quantities of PKU Build (presented above).

### ***Estimated PBS usage & financial implications***

5.6 The submission did not comment on potential changes to the utilisation and PBS cost of PKU Build. The addition of DHA to the formulation may increase its appeal and market share, however given the formulation is priced the same per gram of protein equivalent as its comparators, Camino Pro Bettermilk and Camino Pro Bettermilk Lite, switches from alternative glycomacropptide containing products to PKU Build are unlikely to result in any additional cost to the PBS.

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

## **6 NPWP Consideration (and sponsor's further clarification)**

6.1 The NPWP was concerned the nutritional profile of PKU Build had changed significantly, with some nutrient levels reducing substantially. The NPWP raised particular concern over the low levels of folic acid and essential amino acids, and considered that the new formulation of PKU Build would not provide paediatric patients with the necessary dietary intake of folic acid and methionine without additional supplementation.

6.2 The NPWP wondered if these reductions were the result of manufacturing processes designed to lower the phenylalanine content of the formula and requested the Sponsor provide justification and clarify whether other products from the same manufacturer were likely to be affected by similar changes to manufacturing processes.

6.3 The NPWP considered that it was appropriate to recommend to the PBAC an administrative note on the listing or other instruction regarding the low levels of some nutrients in the formulation, including folate, choline and methionine was appropriate. The Pre-PBAC Response also stated that folate levels had been converted measurement in dietary folate equivalents (DFE), and that choline had been reduced for palatability of the product.

- 6.4 In an out of session consideration, the NPWP also noted that even measured in DFE, dietary folate was still below recommended daily intake levels, and the Sponsor had provided no rationale for the reduction in methionine levels in the reformulation. The NPWP therefore revised its advice to the PBAC that the administrative note should be, ‘This product is low in folate DFE, methionine and choline compared to other PBS-listed glycomacropeptide products for this indication’.

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

## **7 PBAC Outcome**

- 7.1 The PBAC recommended continuing the Restricted Benefit PBS listing of the glycomacropeptide formula, PKU Build, following its reformulation, at the same price for which it is currently subsidised.
- 7.2 The PBAC noted the NPWP was concerned by a number of changes to the vitamin, mineral and micronutrient profile of the formulation, specifically the reduction in levels of choline and methionine, and the below recommended daily intake (RDI) levels of folate (measured in dietary folate equivalents). The PBAC agreed with the NPWP that the addition of an administrative note to advise of the low levels of folate, choline and methionine was appropriate.
- 7.3 The PBAC noted the submission did not request an increased price for PKU Build, and the product remained priced on a cost-minimisation basis to the previously accepted comparators, Camino Pro Bettermilk and Camino Pro Bettermilk Lite.
- 7.4 The PBAC advised its previous advice (November 2017 PBAC, PKU Build Public Summary Document) regarding Nurse Practitioner Prescribing, the Early Supply Rule and interchangeability under Section 101(3BA) of the *National Health Act 1953* remained appropriate.
- 7.5 The PBAC advised this submission is not eligible for an Independent Review as it received a positive recommendation.

### **Outcome:**

Recommended

## **8 Recommended listing**

- 8.1 Amend existing/recommended listing as follows:

Public Summary Document – November 2019 PBAC Meeting

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<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required – In Writing <input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency <input type="checkbox"/> Streamlined
<b>Administration Advice:</b>	<i>This product is low in folate DFE, methionine and choline compared to other PBS-listed glycomacropeptide products for this indication.</i>

*This restriction may be subject to further review. Should there be any changes made 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.