

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

Sachets containing oral powder 16 g, 60 (PKU Build 10)

Sachets containing oral powder 32 g, 30 (PKU Build 20)

PKU Build 10®

PKU Build 20®, Cortex Health Pty Ltd

### 1 Purpose of Application

- 1.1 The minor submission sought a Restricted Benefit listing of two new forms of glycomacropeptide (GMP) and essential amino acids with vitamins and minerals for the treatment of Phenylketonuria.

### 2 Requested Listing

- 2.1 The submission requested the following new listing:

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 containing 10 g protein equivalent powder for oral liquid, 60 x 16 g sachets	2	5	\$ [REDACTED]	PKU Build 10 Cortex Health 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 Level / Method:	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required <input type="checkbox"/> Streamlined

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 <i>containing 4520 g protein equivalent powder for oral liquid, 30 x 32 g sachets</i>	4	5	\$ [REDACTED]	PKU Build 20 Cortex Health Pty Ltd

<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>Condition:</b>	Phenylketonuria
<b>PBS Indication:</b>	Phenylketonuria
<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required <input type="checkbox"/> Streamlined

### 3 Background

- 3.1 The sponsor of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> confirms that they meet 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 Build 10 and 20<sup>®</sup> have not been considered by PBAC previously.

### 4 Comparator

- 4.1 The minor submission nominated Camino Pro<sup>®</sup> Bettermilk and PKU Bettermilk Lite<sup>®</sup> as comparators.
- 4.2 Camino Pro<sup>®</sup> Bettermilk and PKU Bettermilk Lite<sup>®</sup> contain 15 g and 20g protein equivalents (PE) respectively compared to 10 g and 20 g PE for PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup>.

### 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 minor submission presented the following clinical trials:

### Trials and associated reports presented in the submission

Trial ID	Protocol title/ Publication title	Publication citation
NCT01428258	Phase 2 study of glycomacropeptide versus amino acid diet for management of phenylketonuria	Ney, D.M., Stroup, B.M., et al. (2016). Am J Clin Nutr 104(2): 334-345.
	Nutritional status in patients with phenylketonuria using glycomacropeptide as their major protein source.	Pinto, A., Almeida, M.F., et al. (2017). Eur J Clin Nutr. DOI 10.1038/ejcn.2017.38

Source: Submission

- 5.4 In a comparative crossover study in 30 patients investigated the impact of 3 weeks of GMP vs. Amino Acid medical food (AA MF) therapy (with a 3-week washout period between treatments). The frequency of medical food intake was significantly higher with GMP compared with AA MFs (3.74 vs. 2.73 times/day, P=0.001). Study patients rated GMP as more acceptable than AA formulas, noting improved GI symptoms and less hunger. No statistically significant differences in plasma phenylalanine levels over time were noted between the GMP or AA treatments.
- 5.5 A retrospective review of long-term GMP use in 11 patients found adequate phenylalanine control, despite the small increase in phenylalanine consumed with GMP and improved phenylalanine/tyrosine ratios which may be associated with improved executive function.
- 5.6 In all the comparative clinical studies GMP was found to be effective at maintaining appropriate phenylalanine levels, while also improving protein synthesis and patient satiety. Patients also reported better palatability of the GMP products.

### Estimated PBS usage & financial implications

- 5.7 The requested price was calculated based on a cost per g protein equivalents (PE) (\$0.802/g PE) compared to the comparator products at the AEMP level.
- 5.8 The submission stated that the listing of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> is expected to be cost neutral to the PBS as it will directly substitute for other GMP containing products such as Camino Pro<sup>®</sup> Bettermilk and PKU Bettermilk Lite<sup>®</sup>.
- 5.9 In consideration of the submission, the Nutritional Products Working Party (NPWP) noted that:
- The sponsor of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> confirms that they meet 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*.
  - The levels of Vitamin D were high but below the upper limit specified in the NRV guidelines.

- PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> contains only 56% of the RDI of iron for children aged one to three years old. However, the NPWP considered that children over the age of one with PKU would be prescribed a greater quantity than specified in the appendix and therefore did not consider the amount of iron in these products was enough to not recommend the listing of the product.

The NPWP supported the Restricted Benefit listing of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> on a cost-minimisation basis against Camino Pro<sup>®</sup> Bettermilk<sup>®</sup> and Camino Pro<sup>®</sup> Bettermilk Lite<sup>®</sup> for the treatment of Phenylketonuria. The NPWP noted that the level of Vitamin D is high, but did not exceed the upper limit. The NPWP also noted the low level of iron, but, considered that patients over the age of one year would be prescribed a higher intake than specified.

## **6 PBAC Outcome**

- 6.1 The PBAC recommended the listings of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> as Restricted Benefits for the treatment of Phenylketonuria, on a cost-minimisation basis against the nominated comparators Camino Pro<sup>®</sup> Bettermilk and PKU Bettermilk Lite<sup>®</sup> at an equivalent price per gram of protein equivalent.
- 6.2 The PBAC noted the advice of the NPWP supported the listing of PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> on the PBS.
- 6.3 The PBAC considered that Camino Pro<sup>®</sup> Bettermilk and PKU Bettermilk Lite<sup>®</sup> were the appropriate comparators as they have a comparable amount of PE to PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup>.
- 6.4 The PBAC noted that the submission estimated a nil cost to the PBS as it is expected to substitute for other currently listed GMP containing products on an equivalent cost per gram of protein.
- 6.5 The PBAC advised that PKU Build 10<sup>®</sup> and PKU Build 20<sup>®</sup> are suitable for prescribing by nurse practitioners, as nutritional products are currently included for prescribing by nurse practitioners.
- 6.6 The PBAC recommended that the Safety Net 20 Day Rule should not apply as it has been the PBAC's view that general nutrients should be exempt.

### **Outcome:**

Recommended

## **7 Recommended listing**

- 7.1 Add new item:

Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Proprietary Name	Manufacturer
GLYCOMACROPEPTIDE AND ESSENTIAL AMINO ACIDS WITH VITAMINS AND MINERALS containing 10 g protein equivalent powder for oral liquid, 60 x 16 g sachets	2	5	PKU Build 10	Cortex Health Pty Ltd

<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
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Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Proprietary Name	Manufacturer
GLYCOMACROPEPTIDE AND ESSENTIAL AMINO ACIDS WITH VITAMINS AND MINERALS containing 20 g protein equivalent powder for oral liquid, 30 x 32 g sachets	4	5	PKU Build 20	Cortex Health Pty Ltd

<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>Condition:</b>	Phenylketonuria
<b>PBS Indication:</b>	Phenylketonuria
<b>Restriction Level / Method:</b>	<input checked="" type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required <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.