5.28 GLYCOMACROPEPTIDE FORMULA WITH AMINO ACIDS, VITAMINS, MINERALS, TRACE ELEMENTS, CARBOHYDRATE, FAT AND LOW PHENYLALANINE Sachets containing oral powder 33.4 g, 30 (PKU GMPro ULTRA), PKU GMPro ULTRA®, Nutricia Australia Pty Limited

1 Purpose of Submission

1.1 The Category 3 submission requested a General Schedule Restricted Benefit listing of glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine, sachets containing oral powder 33.4 g (PKU GMPro ULTRA®) for the treatment of phenylketonuria (PKU) in children and adults.

2 Background

2.1 PKU GMPro ULTRA had not been considered by the Pharmaceutical Benefits Advisory Committee (PBAC) previously.

2.2 Glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine is not PBS-listed.

2.3 The submission confirmed PKU GMPro ULTRA 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*.

3 Requested listing

3.1 The submission requested the following new listing.

Add new medicinal product as follows:

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
GLYCOMACROPEPTIDE FORMULA WITH AMINO ACIDS, VITAMINS, MINERALS, TRACE ELEMENTS, CARBOHYDRATE, FAT AND LOW PHENYLALANINE					
glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine powder for oral liquid, 30 x 33.4 g sachets	*NEW*	*4*	*4*	*5*	*PKU GMPro ULTRA*
Restriction Summary [new] / Treatment of Concept:					
	Category / Program: GENERAL – General Schedule (Code GE)				
	Prescriber type: ☒Medical Practitioners ☒Nurse practitioners				
	Restriction type: ☒Restricted benefit				
	Indication: Phenylketonuria				
	Population criteria: ~~For children from 3 years of age, and adults~~				

3.2 The submission proposed a population criterion, which is inconsistent with the proposed comparators.

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

4 Comparator

4.1 The submission nominated PKU Bettermilk Lite® and PKU Build 20® as the main comparators. The PBAC considered this was appropriate.

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.

Consumer comments

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

Clinical trials

5.3 As a Category 3 submission, no clinical trials were presented in the submission. The submission considered PKU GMPro ULTRA nutritionally very similar to the main comparators.

5.4 The submission presented a summary of the results of acceptability case studies evaluating the tolerance, compliance, acceptability and safety of PKU GMPro ULTRA. The submission considered PKU GMPro ULTRA well tolerated and accepted, with good compliance to the dietary regimen.

Pricing consideration

5.5 The submission presented a cost-minimisation analysis of PKU GMPro ULTRA compared with comparators based on the same price of protein equivalent (PE) per gram of $0.8016 at the approved ex-manufacturer price (AEMP).

5.6 The submission proposed an AEMP ($480.97) equivalent to the comparators.

5.7 The PBAC noted that as PKU GMPro ULTRA is a new drug it would be placed on the F1 formulary. Drugs in the F1 formulary are subject to anniversary price reductions with the first reduction being a 5% reduction on the fifth anniversary of listing. The comparators are listed as the drug glycomacropeptide and essential amino acids with vitamins and minerals and have already been subject to a 5% five-year anniversary price reduction (on 1 April 2019).

Drug cost/patient/year: $25,008.24

5.8 The above calculation of nutritional product cost per patient per year for the treatment of PKU is derived from the dispensed price for maximum quantity (DPMQ) for one month's supply ($2,084.02) over 12 months. The submission stated that the maximum quantity is calculated based on the highest expected usage, which was calculated for an adult male.

5.9 The submission claimed that for an adult male with reference weight 80 kg, the protein requirement per day would be 80 g, calculated based on weight (kg) times the correspondent total protein (g/kg/day).

5.10 At the highest expected usage, the product could substitute 100% of daily protein requirements, equating to 80 g per day. In this case, each prescription of PKU GMPro ULTRA provides 2400 g of protein, which will provide 80 kg males a month's supply. Protein requirements will vary dependent on age, bodyweight, and medical condition of the patient.

Estimated PBS utilisation and financial implications

5.11 The submission did not present economic or financial evaluations.

5.12 The submission considered the listing of PKU GMPro ULTRA will not have a financial impact to Government because it will not result in any new patients commencing dietary treatment for PKU. The submission expects PKU GMPro ULTRA to only substitute for the comparators assuming 1:1 script equivalence.

5.13 The submission requested PKU GMPro ULTRA to have an equivalent DPMQ ($2,084.02) to both comparators.

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

6 NPWP Consideration

6.1 The Nutritional Products Working Party (NPWP) supported the listing of PKU GMPro ULTRA for the treatment of phenylketonuria (PKU) in children and adults.

6.2 The NPWP noted that the submission stated that PKU GMPro ULTRA is a reformulation of previously PBAC recommended PKU GMPro®. However, the NPWP noted the nutritional content in PKU GMPro ULTRA differed from that of PKU GMPro and advised it should not be considered a reformulation of PKU GMPro.

6.3 The NPWP advised that the comparators PKU Bettermilk Lite and PKU Build 20 are appropriate.

6.4 The NPWP advised to exclude the proposed population criterion 'For children from 3 years of age, and adults' from the restriction noting this is consistent with the comparators.

6.5 The NPWP advised the addition of a cow's milk allergy caution to the restriction is not necessary. The NPWP considered the nutritional label containing the allergy warning would be sufficient.

6.6 The NPWP advised that the addition of administration advice to the restriction was not necessary noting that prescribers will be familiar with the contemporary treatment factors.

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

7 PBAC Outcome

7.1 The PBAC recommended the General Schedule Restricted Benefit listing of glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine (PKU GMPro ULTRA®) for the treatment of phenylketonuria.

7.2 The PBAC noted and agreed with the NPWP advice detailed in Section 6 NPWP consideration.

7.3 The PBAC advised that PKU GMPro ULTRA is suitable for prescribing by nurse practitioners.

7.4 The PBAC recommended that the Early Supply Rule should not apply, as it has been the PBAC's view that general nutrients be exempt.

7.5 The PBAC considered the cost-minimisation analysis appropriate noting the AEMP of PKU GMPro ULTRA compared with the main comparators PKU Bettermilk Lite® and PKU Build 20® was based on the same price of protein equivalent (PE) per gram of $0.8016.

7.6 The PBAC noted that the substitution would only occur with the comparators (assuming 1:1 script equivalence) with an equivalent DPMQ of $2,084.02. The PBAC considered the estimation of nil financial cost to the Government reasonable.

7.7 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because PKU GMPro ULTRA is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over PKU Bettermilk Lite and PKU Build 20, or not expected to address a high and urgent unmet clinical need given the presence of an alternative therapy, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2009* for Pricing Pathway A were not met.

7.8 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 Add new item:

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
GLYCOMACROPEPTIDE FORMULA WITH AMINO ACIDS, VITAMINS, MINERALS, TRACE ELEMENTS, CARBOHYDRATE, FAT AND LOW PHENYLALANINE					
glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine powder for oral liquid, 30 x 33.4 g sachets	*NEW*	*4*	*4*	*5*	*PKU GMPro ULTRA*
Restriction Summary [new] / Treatment of Concept:					
	Category / Program: GENERAL – General Schedule (Code GE)				
	Prescriber type: ☒Medical Practitioners ☒Nurse practitioners				
	Restriction type: ☒Restricted benefit				
	Indication: Phenylketonuria				

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.