

5.16 AMINO ACID FORMULA WITH CARBOHYDRATE WITHOUT PHENYLALANINE

Tablet 1.25 g, 462

PKU Easy Tablet[®], Orpharma Pty Ltd

1 Purpose of Application

- 1.1 The minor submission requested a Restricted Benefit listing for a new amino acid formula with carbohydrate without phenylalanine, PKU Easy Tablet[®] (hereafter referred to as 'PKU Easy'), for the dietary management of phenylketonuria (PKU).

2 Requested listing

- 2.1 The minor submission requested the following new listing.
- 2.2 Suggestions and additions proposed by the Secretariat to the requested listing are added in italics and suggested deletions are crossed out with strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty (packs)	No. of Rpts	Dispensed Price for Max. Qty	Proprietary Name and Manufacturer
AMINO ACID FORMULA WITH CARBOHYDRATE WITHOUT PHENYLALANINE Tablet: modified release, 6 x 77g	4	5	\$1317.39*	PKU Easy Tablet [®] Orpharma Pty Ltd

* The DPMQ proposed in the minor submission for PKU Easy Tablet (\$██████████) is derived from the AEMP for Phlexy-10 for the dietary management of PKU prior to the application of any relevant Statutory Price Reductions. In the overview, the relevant F1 Statutory Price Reductions have been applied to the requested listing to enable a relevant comparison to be made with Phlexy-10 (see paragraphs 5.4-6).

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
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/Electronic/Emergency <input type="checkbox"/> Streamlined
Administrative Advice:	<i>This product does not contain any vitamins, minerals or trace elements.</i>

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

3 Background

- 3.1 The sponsor of PKU Easy 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*.

4 Comparator

- 4.1 The minor submission nominated Phlexy-10[®] tablets as the comparator, ‘as they are the most similar amino acid formula without phenylalanine that is also a tablet formulation’.
- 4.2 The minor submission also stated that there may be some replacement of other similar products by PKU Easy, for example PKU Easy Microtabs[®]. However, as the pricing of these similar products is the same per gram of protein content, the submission considered that replacement of formulations other than the comparator will not impact the overall cost to the PBS.

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.

Other relevant matters

- 5.3 The minor submission stated that there are some key benefits of PKU Easy over other oral tablets and liquid formulations (including the comparator, Phlexy-10 tablets):
- Convenient dosing option – only needs to be swallowed with liquid.
 - No odour and no taste due to the special coating, improving palatability.
 - Slow release tablet formulation allowing for more controlled, gradual tablet dissolution and release of amino acid contents over 3 hours, which may help maintain desired phenylalanine concentrations.
 - Low kilojoules – a lower energy content than many mixtures and other supplement types, with no fat.
- 5.4 The minor submission stated that an additional source of vitamins, minerals and trace elements would be required for patients using PKU Easy to ensure adequate nutrient intake.

Drug cost/patient/year: \$15,808.68

5.5 The minor submission proposed DPMQ based on a cost minimisation analysis versus Phlexy-10, based on cost per protein equivalent content (see Table 1 below).

Table 1: Calculation of DPMQ based on cost equivalence to comparator

Product (PBS item number)	PBS pack size	PE per PBS pack (g)	PE in PBS max qty (g)	AEMP	PBS Max. Qty.	DPMQ	DPMQ with average co-pay removed
PKU Easy tablets	Tablet, 0.92 g PE per tablet, 77 tablets, 6	425	1700	\$299.47 ^a	4	\$1,317.39	\$1,281.60
Phlexy-10 (8678R)	Tablets 1 g, 75	62	1494	\$43.86	24	\$1,166.83	\$1,131.04

^a Cost-minimised to Phlexy-10 tablets on 1 August 2019

PE: protein equivalent

Source: PKU Easy – Attachment 4 – financial estimates Microsoft Excel spreadsheet.

5.6 The submission sought PKU Easy to be considered under the pricing arrangements agreed under Clause 5.7 of the Strategic Agreement between Medicines Australia and the Commonwealth of Australia (the Strategic Agreement). Specifically, if the PBAC recommends listing of PKU Easy on a cost-minimisation basis to Phlexy-10 tablets the submission requested that PKU Easy not be subject to any statutory price reductions that have previously been applied to Phlexy-10 tablets.

5.7 The application of Clause 5.7 of the Strategic Agreement is a matter for the Minister (or delegate) and is not a matter for the PBAC. In the overview the relevant F1 Statutory Price Reductions have been applied to the requested listing to enable a relevant comparison to be made with Phlexy-10.

5.8 The DPMQ proposed in the submission for PKU Easy (\$ [REDACTED]) is derived from the AEMP for Phlexy-10 tablets for PKU prior to the application of any relevant Statutory Price Reductions.

Estimated PBS usage & financial implications

5.9 The minor submission stated that usage of PKU Easy would be in place of other PBS-listed formulas for PKU and, thus, there is ‘no real expected net financial impact of listing’. It was also stated that the cost to the PBS of PKU Easy is expected to be completely offset by a reduction in the cost of other relevant nutritional products for PKU, including some use of the comparator.

5.10 The submission projected negligible market growth over the 5 year forecast, and that PKU Easy Tablet would displace 20% of the comparator in 2020, increasing linearly to 80% in the 6th year of listing in 2025. It also stated that 0.88 scripts of PKU Easy Tablet were equivalent to 1 script of Phlexy-10 based on the different PE content in the PBS maximum quantity for each product.

5.11 Based on the estimates of utilisation in the minor submission and a DPMQ of \$1317.39, listing of PKU Easy tablet is expected to be cost-neutral to the PBS.

Table 2: Estimated use and financial implications

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of scripts dispensed ^a	█	█	█	█	█	█
Estimated financial implications of PKU Easy Tablet^b						
Cost to PBS/RPBS	\$█	\$█	\$█	\$█	\$█	\$█
Copayments	-\$█	-\$█	-\$█	-\$█	-\$█	-\$█
Cost to PBS/RPBS less copayments	\$█	\$█	\$█	\$█	\$█	\$█
Estimated financial implications for Phlexy-10						
Cost to PBS/RPBS	-\$█	-\$█	-\$█	-\$█	-\$█	-\$█
Copayments	\$█	\$█	\$█	\$█	\$█	\$█
Cost to PBS/RPBS less copayments	-\$█	-\$█	-\$█	-\$█	-\$█	-\$█
Net financial implications^b						
Net cost to PBS/RPBS	-\$█	-\$█	-\$█	-\$█	-\$█	-\$█

^aScripts per year as estimated by the submission, adjusted by the equivalent PE amount to the comparator.

^bThe relevant F1 Statutory Price Reductions have been applied to the price of the requested listing to enable a relevant comparison to be made with Phlexy-10 (see paragraphs 5.4-6). The submission estimated that if PKU Easy Tablet is listed at the DPMQ proposed in the submission (of \$█), the net cost to the PBS over the first 6 years of listing would be \$█. Source: Table 4.6, page 22 of minor submission and Attachment 4 – Financial Estimates

The redacted table shows that at Year 6, the estimated number of scripts dispensed was less than 10,000 and the net cost to the PBS would be less than \$10 million.

5.12 As a minor submission, the financial estimates have not been independently evaluated.

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

6 NPWP Consideration (and sponsor's further clarification)

6.1 The Nutritional Products Working Party (NPWP) accepted Phlexy-10 as the comparator for PKU Easy.

6.2 The NPWP noted that PKU Easy does not contain any vitamins, minerals or trace elements and therefore patients would have to achieve their recommended daily intake (RDI) of these by other means, such as through diet or supplements. Some of these vitamin, mineral and trace element supplements, such as Phlexy-Vits or Paediatric Seravit (and other alternatives), are subsidised on the PBS. Therefore, the NPWP considered that while the requested cost minimisation may be an appropriate basis for listing, this did not necessarily mean a listing would be cost neutral to either patients or the PBS as some patients would require PBS-subsidised supplements.

6.3 The NPWP considered the use of the tablet formulation was likely to be low because of the large number of tablets patients had to take and the need to obtain vitamins

and minerals from other sources. The NPWP considered that PKU Easy was likely to only substitute for Phlexy-10.

- 6.4 The NPWP supported the listing of the tablet formulation as an additional treatment option for the dietary management of PKU on a cost-minimisation basis with Phlexy-10 per gram of protein equivalent.
- 6.5 The NPWP supported a note be added to this listing to state that, 'This product does not contain any vitamins, minerals or trace elements'. The NPWP advised this change should flow on to the listing of Phlexy-10.

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

7 PBAC Outcome

- 7.1 The PBAC recommended the Restricted Benefit listing of amino acid formula with carbohydrate without phenylalanine, PKU Easy Tablet[®], for the dietary management of phenylketonuria (PKU).
- 7.2 The PBAC noted the Nutritional Products Working Party (NPWP) supported the proposed listing as a treatment option for the dietary management of PKU. The PBAC also noted the NPWP advice that PKU Easy does not contain any vitamins, minerals or trace elements, and that patients would therefore have to meet their dietary needs through other sources, which may include use of PBS-subsidised vitamin, mineral and trace element supplements such as Phlexy-vits and Paediatric Seravit.
- 7.3 The PBAC agreed with the NPWP that an administrative note should be added to the restriction advising prescribers of the lack of vitamins, minerals and trace elements to note the difference from all currently listed PBS products for the treatment of PKU.
- 7.4 The PBAC accepted that Phlexy-10 was the relevant comparator for PKU Easy.
- 7.5 The PBAC noted that use of the only similar PBS-listed formulation of amino acid formula tablets, Phelxy-10 (for dietary management of PKU), was very low. Given the large number of tablets in the standard dosage and low use of other amino acid tablet formulations, the PBAC considered it was likely the use of PKU Easy would also be low.
- 7.6 Given the likely low use of this products, the PBAC considered that while there may be an additional per-patient cost to the PBS if additional vitamin, mineral and trace element supplements are prescribed, any additional overall cost to the PBS was likely to be low. On balance, the PBAC considered that PKU Easy was a suitable alternative amino acid formula for some patients and would be acceptably cost-effective if it costs no more per gram of protein than the cheapest alternative amino acid formula currently available for PKU.
- 7.7 The PBAC advised that PKU Easy is suitable for prescribing by nurse practitioners, as is the case with other nutritional products.
- 7.8 The PBAC recommended that the Early Supply Rule should not apply to PKU Easy, since it has previously been the PBAC's view that in general, nutritional products be exempt.

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- 7.9 Under Section 101(3BA) of the *National Health Act, 1953*, the PBAC recommended that PKU Easy should be treated as interchangeable on an individual patient basis with other amino acid formulas used for the treatment of PKU (i.e. Phlexy-10).
- 7.10 The PBAC noted that this submission is not eligible for Pricing Pathway A under *National Health (Pharmaceutical and Vaccines – Cost Recovery) Regulations 2009* because PKU Easy is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over any alternative therapy and is not expected to address a high and urgent unmet clinical need.
- 7.11 The PBAC noted that this submission is not eligible for an Independent Review because it received a positive recommendation.

Outcome:

Recommended

8 Recommended listing

8.1 Add new item:

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name and Manufacturer	
AMINO ACID FORMULA WITH CARBOHYDRATE WITHOUT PHENYLALANINE Tablet: modified release, 6 x 77g	4	5	PKU Easy Tablet®	Orpharma Pty Ltd

Category / Program:	GENERAL – General Schedule (Code GE)
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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/Electronic/Emergency <input type="checkbox"/> Streamlined
Administrative Advice:	This product does not contain any vitamins, minerals or trace elements.

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.