

## 5.19 AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT VALINE, LEUCINE AND ISOLEUCINE

Sachets containing oral powder 12.5 g, 30,  
MSUD Explore 5<sup>®</sup>,  
Vitaflo Australia Pty Ltd

### 1 Purpose of Application

- 1.1 The minor submission sought listing of a new amino acid formula (MSUD Explore 5) for the dietary management of patients with maple syrup urine disease (MSUD).

### 2 Requested listing

- 2.1 The submission requested the following listing.

| Name, Restriction, Manner of administration and form  | Max. Qty | No. of Rpts | Dispensed Price for Max. Qty | Proprietary Name and Manufacturer                        |
|---|----------|-------------|------------------------------|--|
| AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT VALINE, LEUCINE AND ISOLEUCINE<br>Powder for oral liquid, 30 x 12.5 g sachets | 8        | 5           | \$1,980.23                   | MSUD Explore 5 <sup>®</sup><br>Vitaflo Australia 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<br><input type="checkbox"/> Midwives               |
| <b>PBS Indication:</b>             | Maple syrup urine disease  |
| <b>Restriction Level / Method:</b> | <input checked="" type="checkbox"/> Restricted benefit<br><input type="checkbox"/> Authority Required – In Writing<br><input type="checkbox"/> Authority Required – Telephone/Electronic/Emergency<br><input type="checkbox"/> Streamlined |

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

### 3 Background

- 3.1 The sponsor of MSUD Explore 5 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*.
- 3.2 The PBAC has not considered an application for MSUD Explore5 previously.

## 4 Comparator

- 4.1 The submission nominated MSUD Gel as the comparator due to similarities in the nutritional profile and place in therapy as a second-stage protein supplement suitable for infants.
- 4.2 The submission states the difference between the MSUD explore and the nominated comparator to be the addition of docosahexaenoic acid (DHA) and arachidonic acid (ARA).

*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.

### ***Drug cost/patient/year: \$23,762.76***

- 5.4 The estimated drug cost/patient/year would be \$23,762.76, assuming a monthly requirement of 1,200g of protein equivalent (PE), at a DPMQ of \$1,980.23 for 1,200g PE per month for 12 months.

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

- 5.5 The minor submission estimated a net cost to the PBS of less than \$10 million in Year 6 of listing, with a total net cost to the PBS of less than \$10 million over the first 6 years of listing. This is summarised in the table below as well as the expected patient/prescription numbers.

**Table 1: Estimated use and financial implications**

|  | Year 1 | Year 2 | Year 3 | Year 4 | Year 5 | Year 6 |
|--|--------|--------|--------|--------|--------|--------|
| <b>Estimated extent of use</b>                           |        |        |        |        |        |        |
| Number of patients treated                               | ■      | ■      | ■      | ■      | ■      | ■      |
| Number of scripts dispensed <sup>a</sup>                 | ■      | ■      | ■      | ■      | ■      | ■      |
| <b>Estimated financial implications of MSUD Explore5</b> |        |        |        |        |        |        |
| Cost to PBS/RPBS   | ■      | ■      | ■      | ■      | ■      | ■      |
| Copayments   | ■      | ■      | ■      | ■      | ■      | ■      |

|   | Year 1 | Year 2 | Year 3 | Year 4 | Year 5 | Year 6 |
|---|--------|--------|--------|--------|--------|--------|
| Cost to PBS/RPBS less copayments                                    | ██████ | ██████ | ██████ | ██████ | ██████ | ██████ |
| <b>Estimated financial implications for replacement of MSUD Gel</b> |        |        |        |        |        |        |
| Cost to PBS/RPBS  | ██████ | ██████ | ██████ | ██████ | ██████ | ██████ |
| Copayments  | ██████ | ██████ | ██████ | ██████ | ██████ | ██████ |
| Cost to PBS/RPBS less copayments                                    | ██████ | ██████ | ██████ | ██████ | ██████ | ██████ |
| <b>Net financial implications</b>                                   |        |        |        |        |        |        |
| Net cost to PBS/RPBS  | ██████ | ██████ | ██████ | ██████ | ██████ | ██████ |

<sup>a</sup> Assuming 9 prescriptions per year/patient as estimated by the submission.

Source: Compiled in the minor overview based on utilisation and cost model spreadsheet August 2019 of the submission. MSUD Gel prices from the PBS Schedule, item 8592F.

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.

- 5.6 The submission requested a 15% higher price per gram of protein equivalent than the comparator MSUD Gel, as MSUD Explore 5 contains DHA and ARA<sup>1</sup>. Based on the submission estimates, this results in an additional cost per patient per year to the PBS of \$2,068.50 per year. The projected net cost to the PBS/RPBS is driven by the requested price premium. The Secretariat notes that calculation of the cost per patient per year in years 2 to 6 equates to \$2,081.13.
- 5.7 The estimates assume patients will use 9 prescriptions per year, based on an analysis of utilisation of the comparator, MSUD Gel. The submission also states 1,200g PE equates to approximately 1 months' supply, which may equate to up to 12 prescriptions per year.
- 5.8 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 NPWP noted the requested listing of MSUD Explore5<sup>®</sup> as a second-stage protein supplement alternative to MSUD Gel<sup>®</sup> for the dietary management of patients with MSUD. The NPWP noted the Sponsor intends to delist MSUD Gel<sup>®</sup> following the listing of MSUD Explore5<sup>®</sup>, after which it will be the only second-stage protein supplement for patients aged 6 months to 5 years of age on the PBS for this condition.
- 6.2 MSUD Explore5<sup>®</sup> contains arachidonic acid (AA) and docosahexaenoic acid (DHA) which the NPWP agreed was beneficial. The NPWP noted the Sponsor requested a 15% price premium for the addition of AA and DHA, however the NPWP noted this

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<sup>1</sup> The requested price per gram of protein equivalent (at approved ex-manufacturer price) is \$1.533/g PE, which is 15% higher than the price of MSUD Gel at \$1.333/g PE.

may not be reasonable as other formulas containing AA and/or DHA, such as MSUD Anamix Junior®, do not have such a price premium.

- 6.3 The NPWP supported the listing of MSUD Explore5® on a cost minimisation basis with MSUD Gel® per gram of protein equivalent without the requested price premium.
- 6.4 The Pre-PBAC Response argued that the requested price premium was justified, as the addition of DHA and AA represents a patient-relevant improvement in the formulation over the comparator, MSUD Gel. In an out of session consideration, the NPWP advised the PBAC it considered that in this case, the requested price premium was not justified as the addition of DHA and AA in the target population was not clinically indicated.

*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 the amino acid formula, MSUD Explore 5®, for the dietary management of maple syrup urine disease (MSUD) on a cost minimisation basis with MSUD Gel®, at an equivalent cost per gram of protein equivalent (PE).
- 7.2 The PBAC noted the NPWP supported the proposed listing as an alternative second stage protein supplement for dietary management of MSUD in patients aged six months to five years old. The PBAC further noted the Sponsor's intention to delist the comparator, MSUD Gel from the PBS following the listing of MSUD Explore 5.
- 7.3 The PBAC accepted MSUD Gel was the appropriate comparator. The PBAC considered MSUD Explore 5 would directly substitute for MSUD Gel and was unlikely to grow the market for nutritional products for dietary management of MSUD for the target age group.
- 7.4 The PBAC agreed with the NPWP advice that the requested 15% price premium was not justified as the addition of docosahexaenoic acid (DHA) and arachidonic acid (AA) was not clinically indicated for the target population, and other nutritional products for the dietary management of MSUD which contain DHA and/or AA such as MSUD Anamix Junior were not listed with a price premium.
- 7.5 The PBAC considered that MSUD Explore 5 is suitable for inclusion in the PBS medicines for prescribing by nurse practitioners, similar to other nutritional products currently listed for the dietary management of patients with MSUD.
- 7.6 The PBAC advised, under Section 101(3BA) of the *National Health Act 1953* that MSUD Explore 5 should be treated as interchangeable on an individual patient basis with MSUD Gel.
- 7.7 The PBAC recommended that the Early Supply Rule should not apply to MSUD Explore 5, as it has generally been the PBAC's view that nutritional product listings be exempt.

- 7.8 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because MSUD Explore 5 is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over MSUD Gel, 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.
- 7.9 The PBAC noted that this is not eligible for an Independent Review as it received a positive recommendation.

**Outcome:**

Recommended

## 8 Recommended listing

### 8.1 Add new item:

| Name, Restriction,<br>Manner of administration and form   | Max.<br>Qty | No. of<br>Rpts | Proprietary Name and Manufacturer |                           |
|---|-------------|----------------|-----------------------------------|---------------------------|
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*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.