

## 5.19 DEFERIPRONE

### Tablets, 1000 mg, Ferriprox<sup>®</sup>, Apotex

#### 1 Purpose of Application

- 1.1 The submission sought a Section 100 Highly Specialised Drugs, Authority Required (STREAMLINED) listing for a higher strength tablet of deferiprone for the treatment of transfusional iron overload associated with thalassaemia major.

#### 2 Requested listing

- 2.1 The submission requested a Section 100 Highly Specialised Drugs, Authority Required (STREAMLINED) listing for deferiprone 1000 mg tablets. The submission requested the same restrictions as the current PBS listing for deferiprone 500 mg tablets.

#### 3 Background

- 3.1 Deferiprone is TGA registered for the treatment of iron overload in patients with thalassaemia major who are unable to take or in whom desferrioxamine therapy has proven ineffective.
- 3.2 Deferiprone 500 mg tablets, was PBS listed in February 2004 as a Section 100 Highly Specialised Drug (Private and Public Hospitals). Deferiprone oral solution 100 mg per ml, 250 ml was listed in June 2009. In November 2010, changes to the maximum quantity and number of repeats were implemented following the July 2010 PBAC meeting recommendations.

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

#### 4 Comparator

- 4.1 The submission nominated deferiprone 500 mg tablets as comparator as the submission argued that the 500 mg tablets are likely to be replaced in clinical practice by the 1000 mg tablets. The submission indicated that use of the deferiprone oral solution is extremely low and is not expected to change in response to the listing of the 1000 mg tablet formulation.

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

#### 5 Consideration of evidence

- 5.1 The submission included the results of the study LA33-BA, an open label comparative randomised, single dose 2-way crossover bioequivalence study which demonstrated

the bioequivalence of the 500 mg tablets and the 1000 mg tablets following a single 1000 mg dose of deferiprone’

## Sponsor hearing

There was no Sponsor hearing for this item as it was a minor submission.

## Consumer comments

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

## 6 Estimated PBS usage and financial implications

- 6.1 The submission proposed an equivalent price per milligram for the 1000 mg presentation of deferiprone compared to the currently listed 500 mg presentation.
- 6.2 The submission presented the estimated net costs to the PBS/RPBS across the forward projections associated with the proposed listing. Details are summarised in Table 2.

**Table 2 Estimated net cost to the PBS/RPBS associated with the proposed listing**

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6	Years 1-6 Cumulative
<b>Projected net cost of new listing</b>							
to PBS	\$455,836	\$638,171	\$820,506	\$911,673	\$911,673	\$911,673	\$4,649,532
to RPBS	\$0	\$0	\$0	\$0	\$0	\$0	\$0
to the PBS/RPBS	\$455,836	\$638,171	\$820,506	\$911,673	\$911,673	\$911,673	\$4,649,532
<b>Projected net costs of displaced medicines</b>							
to PBS	\$455,836	\$638,171	\$820,506	\$911,673	\$911,673	\$911,673	\$4,649,532
to RPBS	\$0	\$0	\$0	\$0	\$0	\$0	\$0
to the PBS/RPBS	\$455,836	\$638,171	\$820,506	\$911,673	\$911,673	\$911,673	\$4,649,532
<b>Overall net cost to the PBS/RPBS</b>							
to PBS	\$0	\$0	\$0	\$0	\$0	\$0	\$0
to RPBS	\$0	\$0	\$0	\$0	\$0	\$0	\$0
to the PBS/RPBS	\$0	\$0	\$0	\$0	\$0	\$0	\$0

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

## 7 PBAC Outcome

- 7.1 The PBAC recommended the listing of deferiprone 1000 mg as a Section 100 Highly Specialised Drugs, Private and Public Hospitals, Authority Required (STREAMLINED) for the treatment of transfusional iron overload associated with thalassaemia major.
- 7.2 The PBAC noted that the 1000 mg tablets would reduce the patient’s tablet burden.
- 7.3 The PBAC was satisfied that one tablet of the 1000 mg strength is bioequivalent to two of the currently listed 500 mg tablets.
- 7.4 The PBAC noted that the sponsor estimated that the proposed listing of the 1000 mg tablets would be cost-neutral against the current listing of the 500 mg tablets.
- 7.5 The PBAC considered that clinicians might opt to prescribe tablets of the two strengths at the same time to achieve the optimal dosage per body weight. As such, the PBAC recommended to reduce the maximum quantity for the 500 mg strength tablets to minimise the potential wastage of medicine and associated costs to the PBS.

**Outcome**

Recommended

**8 Recommended listing**

Add new item.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name	Manufacturer
DEFERIPRONE 1000 mg tablet, 50	6	5	Feriprox	Apotex
Category / Program	Section 100 – Highly Specialised Drugs Program (Private and Public Hospitals)			
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
Severity:	Chronic			
Condition:	Iron Overload			
PBS Indication:	Iron Overload			
Restriction Level / Method:	<input type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required - In Writing <input type="checkbox"/> Authority Required - Telephone <input type="checkbox"/> Authority Required - Emergency <input type="checkbox"/> Authority Required - Electronic <input checked="" type="checkbox"/> Streamlined			
Clinical criteria:	Patient must have thalassaemia major, AND  Patient must be unable to take desferrioxamine therapy,			

Flow-on changes.

Amend the current listing for deferiprone 500 mg tablet as follows.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	Proprietary Name	Manufacturer
DEFERIPRONE 500 mg tablet, 100	6 3	5	Feriprox	Apotex

Category / Program	Section 100 – Highly Specialised Drugs Program (Private and Public Hospitals)
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives
Severity:	Chronic
Condition:	Iron Overload
PBS Indication:	Iron Overload
Restriction Level / Method:	<input type="checkbox"/> Restricted benefit <input type="checkbox"/> Authority Required - In Writing <input type="checkbox"/> Authority Required - Telephone <input type="checkbox"/> Authority Required - Emergency <input type="checkbox"/> Authority Required - Electronic <input checked="" type="checkbox"/> Streamlined
Clinical criteria:	Patient must have thalassaemia major, AND  Patient must be one in whom desferrioxamine therapy has proven ineffective.

## 9 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.

## 10 Sponsor's Comment

The sponsor had no comment.