

**5.17 POMALIDOMIDE,  
Capsule 1 mg,  
Capsule 2 mg,  
Pomolide™,  
Juno Pharmaceuticals Pty Ltd**

**1 Purpose of Submission**

- 1.1 The Category 4 submission requested Section 100 (Highly Specialised Drugs Program) Authority Required Pharmaceutical Benefits Scheme (PBS) listings of new forms of pomalidomide (capsule 1 mg, capsule 2 mg; Pomolide™) for the treatment of multiple myeloma under the same conditions as the currently listed forms of pomalidomide.

**2 Background**

- 2.1 Pomalidomide 3 mg and 4 mg are listed on the PBS as Section 100 HSD Authority Required listing for multiple myeloma in multiple brands.
- 2.2 While the recommended starting dose for pomalidomide is 4 mg (in combination with dexamethasone with or without bortezomib), the submission stated that there is a need for lower strengths due to dose interruptions and dose reductions that may occur as per the Therapeutic Goods Administration (TGA) product information (PI).

**Registration status**

- 2.3 Pomalidomide 1 mg and 2 mg capsules were first registered by the TGA on 1 July 2014 for multiple myeloma under the brand name Pomalyst®.
- 2.4 Pomolide 1 mg and 2 mg capsules were TGA registered on 18 May 2021 for the treatment of multiple myeloma. The TGA considered Pomolide 1 mg, 2 mg, 3 mg and 4 mg to be bioequivalent to the respective strengths of Pomalyst.

**Previous PBAC consideration**

- 2.5 Pomalidomide was first considered by the PBAC at the July 2014 meeting for the treatment of multiple myeloma and was subsequently recommended at the November 2014 meeting. At its November 2017 meeting, the PBAC recommended changing the restriction to include the treatment of patients who have experienced severe intolerance or toxicity to lenalidomide and/or bortezomib, and at its July 2019 meeting recommended listing pomalidomide in combination with bortezomib and dexamethasone (PBd).
- 2.6 The PBAC has not considered the listing of 1 mg and 2 mg strengths of pomalidomide previously and these strengths are not currently listed on the PBS.

2.7 In an out-of-session consideration between its July 2022 and November 2022 meetings, the PBAC advised that bioequivalent brands of pomalidomide should not be considered equivalent for the purposes of substitution (i.e., 'a'-flagged). The PBAC noted potential for unnecessary administrative burden and confusion for prescribers and pharmacists that may delay patient access where an alternative brand is sought (paragraphs 4.2 and 4.3, lenalidomide and pomalidomide Public Summary Document, October 2022 PBAC OOS consideration).

### 3 Requested listing

3.1 The submission requested the following new listings, identical to the current restrictions of Pomolide 3 and 4 mg. For brevity, only the new content has been included. Suggested additions are in italics.

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	No. of Rpts	Available brands
POMALIDOMIDE					
pomalidomide 1 mg capsule, 14	<i>NEW (HSD Public)</i>	1	14	2	Pomolide
	<i>NEW (HSD Private)</i>				
pomalidomide 1 mg capsule, 21	<i>NEW (HB)</i>	1	21	0	
	<i>NEW (HS)</i>				
pomalidomide 2 mg capsule, 14	<i>NEW (HB)</i>	1	14	2	
	<i>NEW (HS)</i>				
pomalidomide 2 mg capsule, 21	<i>NEW (HB)</i>	1	21	0	
	<i>NEW (HS)</i>				

3.2 The submission requested the same maximum quantity and maximum repeats as the 3 mg and 4 mg strengths. The submission highlighted that the rationale for the requested listing was that dose adjustments (interruptions and reductions) occur during treatment with pomalidomide due to the occurrence of haematological toxicities (such as thrombocytopenia and neutropenia). As such, the submission considers that listing lower strengths on the PBS would allow for more efficient dosing. It was noted that the PI does not specify how long a patient would require treatment with lower strengths, but presumably it would be until the haematological toxicities resolve.

### 4 Comparator

4.1 The submission nominated PBS-listed Pomalyst as the main comparator. This was appropriate. The PBAC considered that the higher strength of Pomolide is also a relevant comparator.

### 5 Consideration of the evidence

#### *Sponsor hearing*

5.1 There was no hearing for this item

### **Consumer comments**

- 5.2 The PBAC noted and welcomed the input from Rare Cancers Australia via the Consumer Comments facility on the PBS website. While the comments did not address the addition of the new strengths of pomalidomide, they described a range of benefits of pomalidomide treatment in general. This included the ability to take the drug orally which improved quality of life by reducing hospital visits for infusions, and that pomalidomide was considered a less physically and psychologically tolling treatment alternative to other chemotherapy regimens. The comments also described the challenges long-term smokers face with pomalidomide treatment. The PBAC noted that this advice was supportive of the listing of these new strengths.

### **Clinical trials**

- 5.3 The submission's request was based on evidence from a clinical trial CC-4047-MM-003 to demonstrate the need for 1 mg and 2 mg capsules for more efficient dosing. The results concluded that high pomalidomide dose intensity resulted in dose interruptions in 58% of subjects due to treatment emergent adverse events (haematological toxicities) (Weisel et al. 2013).

### **Clinical claim**

- 5.4 The submission claimed non-inferior comparative effectiveness and non-inferior comparative safety of Pomolide compared with Pomalyst.
- 5.5 The PBAC considered that the claim of non-inferior comparative effectiveness and non-inferior comparative safety of Pomolide to Pomalyst was reasonable.

### **Economic analysis**

- 5.6 As a Category 4 submission, the economic analysis had not been independently evaluated.
- 5.7 The submission presented a cost-minimisation analysis of Pomolide compared with Pomalyst.
- 5.8 The submission estimated the equi-effective doses as 1 mg of Pomolide = 1 mg of Pomalyst. As Pomalyst 1 mg is not PBS-listed, the relevant pricing comparator would be the higher strengths of pomalidomide.
- 5.9 The proposed ex-manufacturer prices were based on the same price per mg as the currently listed 4 mg form of Pomolide.

### **Estimated PBS usage and financial implications**

- 5.10 The submission used a market-share approach to estimate the financial impact to the PBS/RPBS of the requested listing (see Table 1). The submission stated that the proposed listing was not expected to impact the overall market size.
- 5.11 The submission had assumed that some patients would transition from privately prescribed 1 mg and 2 mg capsules to the proposed listing, while others would be

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patients suitable for a 1 mg or 2 mg dose that have been prescribed a higher dose of 3 mg on the PBS rather than being prescribed the lower strengths privately. The submission assumed that the latter patients constitute 1.5% of the existing 3 mg capsule market. The submission assumed that 20% of these patients would move to the 1 mg capsule and 80% to the 2 mg capsule if listed. The pre-PBAC response clarified that 20% of patients requiring a 1 mg dose, instead of taking a 3 mg capsule once every 3 days, would take a 1 mg capsule daily, and likewise, 80% of patients requiring a 2 mg dose, instead of taking a 3 mg capsule daily for 2 days with a break on the 3<sup>rd</sup> day, would take a 2 mg capsule daily. This was appropriate.

- 5.12 It was estimated that there would be < 500 scripts supplied over the first six years of listing (< 500 in Year 1 to < 500 in Year 6).

**Table 1: Estimated use and financial implications for pomalidomide 1 mg and 2 mg capsules**

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
<b>Estimated extent of use (number of scripts)<sup>1</sup></b>						
Public – Capsule 1 mg, 21	1	1	1	1	1	1
Private – Capsule 1 mg, 21	1	1	1	1	1	1
Public – Capsule 1 mg, 14	1	1	1	1	1	1
Private – Capsule 1 mg, 14	1	1	1	1	1	1
Public – Capsule 2 mg, 21	1	1	1	1	1	1
Private – Capsule 2 mg, 21	1	1	1	1	1	1
Public – Capsule 2 mg, 14	1	1	1	1	1	1
Private – Capsule 2 mg, 14	1	1	1	1	1	1
<b>Estimated financial implications (\$)</b>						
New PBS listing	2	2	2	2	2	2
Changed PBS listing	3	3	3	3	3	3
Net cost to PBS	3	3	3	3	3	3
New RPBS listing	2	2	2	2	2	2
Changed RPBS listing	2	2	2	2	2	2
Net cost to RPBS	2	2	2	2	2	2
<b>Net cost to PBS/RPBS</b>	<b>3</b>	<b>3</b>	<b>3</b>	<b>3</b>	<b>3</b>	<b>3</b>

Source: Table 18 & 19, pp. 31-32, Table 4-8 p. 34 of the submission.

Abbreviations: PBS = Pharmaceutical Benefits Scheme; RPBS = Repatriation Pharmaceutical Benefits Scheme.

The redacted values correspond to the following ranges:

<sup>1</sup> < 500

<sup>2</sup> \$0 to < \$10 million

<sup>3</sup> net cost saving

- 5.13 The submission claimed that the cost of pomalidomide 1 mg and 2 mg capsules to the PBS/RPBS was expected to be \$0 to < \$10 million over six years (Year 1 \$0 to < \$10 million to Year 6 \$0 to < \$10 million).

- 5.14 The submission stated that the estimated net financial impact to the PBS/RPBS for the listing of pomalidomide 1 mg and 2 mg capsules would be a cost save over six years (Year 1 \$0 to < \$10 million to Year 6 \$0 to < \$10 million) due to an assumption that the less expensive lower strengths will substitute for the higher strengths in some patients who may require dose adjustments.

## 6 PBAC Outcome

- 6.1 The PBAC recommended the listing of pomalidomide (Pomolide) 1 mg and 2 mg capsules under the same circumstances as the 3 mg and 4 mg capsule listings. The PBAC considered that the claim of non-inferior comparative effectiveness and non-inferior comparative safety of Pomolide to Pomalyst was reasonable.
- 6.2 The PBAC considered the lower strengths to be therapeutically relative to the higher strengths on a per mg basis.
- 6.3 The PBAC considered that the listing of 1 mg and 2 mg of pomalidomide would likely not increase overall market utilisation.
- 6.4 The PBAC considered that the predicted cost saving presented in the submission may not be realised in practice but considered that the listing should be cost neutral.
- 6.5 The PBAC considered that the maximum quantity and repeats should be consistent with the listing of the higher strengths as a patient would likely require treatment with the lower strengths until the haematological toxicities resolve.
- 6.6 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because pomalidomide 1 mg and 2 mg are not expected to provide improvement in efficacy and reduction of toxicity over pomalidomide 2 mg and 3 mg, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met.
- 6.7 The PBAC noted that this submission is not eligible for an Independent Review because it received a positive recommendation.

### Outcome:

Recommended

## 7 Recommended listing

- 7.1 Add new forms of pomalidomide for the treatment of multiple myeloma under the same conditions as the currently listed forms of pomalidomide:
  - Pomalidomide 3 mg (21 capsules – PBS item codes 10406Q, 10417G; 14 capsules - PBS item codes 12666P, 12668R)
  - Pomalidomide 4 mg (21 capsules – PBS item codes 10386P, 10387Q; 14 capsules – PBS item codes 12661J, 12665N).
- 7.2 Add new listing as follows:
  - S100 HSD Authority Required (public/private) Pomolide™ 1 mg capsule
  - S100 HSD Authority Required (public/private) Pomolide™ 2 mg capsule

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	NEW (HSD Private)				
pomalidomide 1 mg capsule, 21	NEW (HSD Public)	1	21	0	
	NEW (HSD Private)				
pomalidomide 2 mg capsule, 14	NEW (HSD Public)	1	14	2	
	NEW (HSD Private)				
pomalidomide 2 mg capsule, 21	NEW (HSD Public)	1	21	0	
	NEW (HSD Private)				
<b>Restriction Summary 13756</b>					
	<b>Category / Program:</b> Section 100 – Highly Specialised Drugs Program (S100 HSD)				
	<b>Prescriber type:</b> <input checked="" type="checkbox"/> Medical Practitioners				
	<b>Restriction type:</b> <input checked="" type="checkbox"/> Authority Required (telephone/online PBS Authorities system)				
	<b>Administrative Advice:</b> Patients receiving pomalidomide under the PBS listing must be registered in the risk management program relevant for the brand of pomalidomide being prescribed and dispensed: Pomolide - Juno's Pregnancy Prevention Program; Pomalyst - i-access program; Pomalidomide Sandoz - Pregnancy Prevention Program.				

***This restriction may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed.***

## 8 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.

## 9 Sponsor's Comment

The sponsor had no comment.