

**7.09 PATIROMER,  
Sachet, 8.4 g powder for oral liquid,  
Sachet, 16.8 g powder for oral liquid,  
Veltassa<sup>®</sup>,  
Vifor Pharma Pty Limited.**

**1 Purpose**

- 1.1 The early re-entry resubmission requested an Authority Required (telephone) listing for patiromer for the initial treatment of adult patients with chronic kidney disease (CKD) Stage 3-4, with chronic hyperkalaemia (at least two episodes of serum potassium 6.0 mmol/L or higher in the previous 12 months), who are receiving at least one renin angiotensin aldosterone system inhibitor (RAASi) medicine or are indicated for a RAASi medicine but are unable to tolerate it due to prior occurrence of hyperkalaemia.
- 1.2 The PBAC did not recommend patiromer for this indication at the November 2022 meeting. This resubmission addressed the issues raised by the PBAC at the November 2022 meeting; see Table 1 below.

Public Summary Document – March 2023 PBAC Meeting

Table 1: Summary of key matters to be addressed, as per the November 2022 patiromer PSD

| Matter of concern  | Response  |
|--|---|
| <p><u>Economics:</u><br/>Presentation of an economic analysis that was based solely on the cost minimisation approach presented in the November 2022 resubmission (paragraph 7.14). The PBAC noted that the approach presented by the evaluators, which excluded acute hyperkalaemia patients was appropriate (paragraph 7.9). The PBAC considered that it would be appropriate for a small price premium to be applied to patiromer due to the potentially improved tolerability (paragraphs 7.11).</p> | <p>The resubmission presented an economic analysis that was based on the cost minimisation approach presented in the November 2022 resubmission and which excluded patients receiving acute treatment. The cost minimisation applied different equi-effective doses for patiromer and SPS/CPS compared to the base case presented in November 2022.</p>   |
| <p><u>Financials:</u><br/>Estimates based on the revised price of patiromer obtained from the cost minimisation approach (paragraph 7.14).</p>   | <p>The approach for estimating the financial impact estimates was unchanged from the November 2022 resubmission; however, 3 inputs were changed:</p> <ul style="list-style-type: none"> <li>• The effective AEMP of patiromer was reduced by █% (from \$█ to \$█);</li> <li>• The rate of CKD diagnosis was increased from a maximum of 35% in Year 6 to a flat 50% over each of the 6 years; and</li> <li>• Adherence to patiromer was reduced from 96.4% to 50%.</li> </ul> |
| <p><u>Risk Sharing Arrangement:</u><br/>Presentation of a RSA based on the revised financial estimates with expenditure caps beyond which a rebate of █% was applied (paragraph 7.14)</p>  | <p>No RSA was presented</p>   |

Source: Table 1, p9 of the March 2023 resubmission

AEMP = approved ex-manufacturer price; CKD = chronic kidney disease; CPS = calcium polystyrene sulfonate; PSD = public summary document; RSA = risk sharing arrangement; SPS = sodium polystyrene sulfonate

## 2 Background

2.1 Patiromer was listed on the Australian Register of Therapeutic Goods on 12 December 2017 for the treatment of hyperkalaemia in adults.

### **Previous PBAC considerations**

2.2 Patiromer was considered by the PBAC at the November 2019, November 2020 and November 2022 PBAC meetings. At the November 2022 meeting, the PBAC did not recommend patiromer as the additional clinical evidence presented in the resubmission did not adequately address the uncertainties surrounding the long-term benefits of patiromer. The PBAC considered that the cost utility analysis was unreliable for decision making and relied on long-term outcomes that were not supported by the clinical data. The PBAC advised that it would be more appropriate for the price of patiromer to be based on a cost-minimisation approach versus sodium polystyrene sulfonate (SPS) and calcium polystyrene sulfonate (CPS) resins given these could be used to treat the overall target population. (paragraph 7.1, patiromer Public Summary Document (PSD), November 2022).

### 3 Requested listing

3.1 The resubmission accepted all suggested amendments to the previously considered PBS restriction.

|  | PBS item code | Max. qty (packs) | Max. qty (units) | No. of repeats | DPMQ and AEMP                                   | Available brands |
|--|---------------|------------------|------------------|----------------|---|------------------|
| <b>Initial, continuing and grandfather</b>   |               |                  |                  |                |   |                  |
| PATIROMER  |               |                  |                  |                |   |                  |
| patiromer 8.4 g powder for oral liquid, 30 sachets   | New           | 1                | 30               | 5              | Published:<br>DPMQ: \$375.00;<br>AEMP: \$325.89 | Veltassa         |
| patiromer 16.8 g powder for oral liquid, 30 sachets  | New           | 1                | 30               | 5              | Effective:<br>DPMQ: \$<br>AEMP: \$              |                  |
| <b>Category / Program:</b> GENERAL – General Schedule (Code GE)  |               |                  |                  |                |   |                  |
| <b>Prescriber type:</b> <input checked="" type="checkbox"/> Medical Practitioners  |               |                  |                  |                |   |                  |
| <b>Episodicity:</b> Chronic  |               |                  |                  |                |   |                  |
| <b>Condition:</b> Hyperkalaemia  |               |                  |                  |                |   |                  |
| <b>PBS Indication:</b> Chronic hyperkalaemia   |               |                  |                  |                |   |                  |
| <b>Treatment phase:</b> Initial PBS-subsidised treatment   |               |                  |                  |                |   |                  |
| <b>Restriction type:</b> <input checked="" type="checkbox"/> Authority Required (telephone/online PBS Authorities system)  |               |                  |                  |                |   |                  |
| <b>Population criteria:</b><br>Patient must have stage 3 to stage 4 chronic kidney disease   |               |                  |                  |                |   |                  |
| <b>Clinical criteria:</b><br>The condition must <del>not</del> be inadequately controlled by a low potassium diet.   |               |                  |                  |                |   |                  |
| <b>AND</b>   |               |                  |                  |                |   |                  |
| <b>Clinical criteria:</b><br>Patient must have experienced at least 2 episodes of hyperkalaemia (defined as serum potassium levels of <i>at least</i> 6.0 mmol/L or higher) within the <del>previous</del> 12 months <i>prior to commencing this drug</i>  |               |                  |                  |                |   |                  |
| <b>AND</b>   |               |                  |                  |                |   |                  |
| <b>Clinical criteria:</b><br>The treatment must not be in place of emergency treatment of hyperkalaemia  |               |                  |                  |                |   |                  |
| <b>AND</b>   |               |                  |                  |                |   |                  |
| <b>Treatment criteria:</b><br>Patient must be undergoing treatment with a renin angiotensin aldosterone system inhibitor; OR<br>Patient must be indicated for treatment with a renin angiotensin aldosterone system inhibitor; but not able to tolerate this due to prior occurrence of hyperkalaemia                    |               |                  |                  |                |   |                  |
| <b>AND</b>   |               |                  |                  |                |   |                  |
| <b>Treatment criteria:</b><br>Must be treated by a specialist medical practitioner   |               |                  |                  |                |   |                  |
| <b>Administrative advice:</b><br>Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see <a href="http://www.servicesaustralia.gov.au/HPOS">www.servicesaustralia.gov.au/HPOS</a> ) or by telephone by contacting Services Australia on 1800 888 333 |               |                  |                  |                |   |                  |
| <b>Treatment phase:</b> Continuing treatment   |               |                  |                  |                |   |                  |
| <b>Restriction:</b> <input checked="" type="checkbox"/> Authority Required (Streamlined) [new]   |               |                  |                  |                |   |                  |

Public Summary Document – March 2023 PBAC Meeting

|  |
|--|
| <b>Clinical criteria:</b>  |
| Patient must have previously received PBS-subsidised treatment with this drug for this condition;  |
| <b>AND</b>   |
| <b>Clinical criteria:</b>  |
| The treatment must not be in place of emergency treatment of hyperkalaemia   |
| <b>Treatment criteria:</b>   |
| Patient must not be undergoing dialysis;   |
| <b>AND</b>   |
| <b>Treatment criteria:</b>   |
| Patient must be undergoing treatment with a renin angiotensin aldosterone system inhibitor   |
| <b>Treatment phase:</b> Grandfathered  |
| <b>Restriction:</b> <input checked="" type="checkbox"/> Authority Required (Streamlined) [new]   |
| <b>Clinical criteria:</b>  |
| Patient must have received non-PBS subsidised treatment with this drug for this condition before [date of listing];  |
| <b>AND</b>   |
| <b>Clinical criteria:</b>  |
| The condition must not be adequately controlled by a low potassium diet.   |
| <b>AND</b>   |
| <b>Clinical criteria:</b>  |
| Patient must have, prior to commencing non-PBS treatment with this drug, experienced at least 2 episodes of hyperkalaemia (defined as serum potassium levels of at least 6.0 mmol/L or higher) within the previous 12 months;                |
| <b>AND</b>   |
| <b>Clinical criteria</b>   |
| The treatment must not be in place of emergency treatment of hyperkalaemia   |
| <b>AND</b>   |
| <b>Treatment criteria:</b>   |
| Patient must be undergoing treatment with a renin angiotensin aldosterone system inhibitor;  |
| <b>AND</b>   |
| <b>Treatment criteria:</b>   |
| Patient must not be undergoing dialysis;   |
| <b>AND</b>   |
| <b>Treatment criteria:</b>   |
| <b>AND</b>   |
| Must be treated by a specialist medical practitioner   |
| <b>Administrative advice:</b>  |
| This grandfather restriction will cease to operate from 12 months after the date specified in the clinical criteria  |
| <b>Administrative advice:</b> Patients may qualify for PBS subsidised treatment under this restriction once only. For continuing PBS subsidised treatment, a 'Grandfathered' patient must qualify under the 'Continuing treatment' criteria. |

- 3.2 Flat pricing was proposed whereby the price for each strength is the same. The proposed effective price (AEMP) of patiomer of \$| for both the 8.4 g and 16.8 g sachets (30 sachets per pack) represented a |% reduction (from \$|) compared to the November 2022 submission. A flat pricing proposal was first considered in November 2020, as post-marketing data indicated that approximately 92% of patients commenced and continued treatment at a dose of 8.4 g/day.
- 3.3 The resubmission stated that, as of 19 December 2022, there were 95 patients in the compassionate access program, which the resubmission stated indicated the continued demand for patiomer. The Secretariat suggested access for these patients through the Initial treatment phase restriction by describing it as: 'Initial PBS-

subsidised treatment'. They will be required to meet the same eligibility requirements as any other patient.

*For more detail on PBAC's view, see section 5 PBAC outcome.*

## **4 Consideration of the evidence**

### ***Sponsor hearing***

4.1 There was no hearing for this item.

### ***Consumer comments***

4.2 The PBAC noted and welcomed the input from health care professionals (10) via the Consumer Comments facility on the PBS website. The comments described the benefits of treatment with patiomer including the improved tolerability and palatability compared to currently available therapies. The PBAC recalled that previous resubmissions had also received comments from individuals and health care professionals describing the side effects and significant palatability issues associated with SPS/CPS resins and the benefits provided by patiomer. The PBAC noted that this advice was supportive of the evidence provided in the submission.

### ***Clinical claim***

4.3 The resubmission described patiomer as non-inferior in terms of effectiveness and safety compared to SPS/CPS resins, noting patiomer is associated with fewer gastrointestinal adverse events (AEs) and improved tolerability. This was consistent with the PBAC's previous advice in November 2022, in which the PBAC considered that patiomer was:

- non-inferior compared to SPS/CPS resins in terms of reducing potassium levels (paragraph 6.48, patiomer PSD, November 2022 PBAC Meeting).
- non-inferior compared to SPS/CPS resins in terms of safety, noting that patiomer appeared to be more palatable and was possibly associated with fewer gastrointestinal adverse events and improved tolerability (paragraph 7.6, patiomer PSD, November 2022 PBAC Meeting).

4.4 Consistent with the previous PBAC advice, the resubmission did not make a clinical claim (nor present an economic evaluation) versus standard care.

### ***Economic analysis***

4.5 In November 2022, the PBAC considered 'it would be appropriate for any future resubmission to be based solely on the cost minimisation approach versus SPS/CPS resins presented by the evaluators in chronic hyperkalaemia patients (per Table 16, which resulted in a cost per pack of patiomer of \$| in the submission and \$| in the Pre-Sub-Committee Response (PSCR) (as the gastrointestinal adverse event costs were removed)). The PBAC considered it would be reasonable for a small price premium to

be applied to patiromer over SPS/CPS due to the potentially improved tolerability, as highlighted in the consumer comments' (paragraph 7.11, patiromer PSD, November 2022 PBAC Meeting).

- 4.6 The resubmission based the proposed price of patiromer solely on a cost minimisation approach (CMA), consistent with the PBAC's previous advice. However, compared with the CMA presented in the November 2022 submission, the key difference was that the average daily dose of SPS and CPS resins was based on the dose recommended in the Product Information of 15 g three to four times a day, with the resubmission applying a daily dose of 45 g (this had been presented as a sensitivity analysis in the previous submission). A 50% adherence rate was then applied to the Product Information doses to calculate the average daily dose of 22.5 g.
- 4.7 In November 2022, the average daily dose of SPS/CPS resins was 15 g, which was based on the results of a clinician survey (i.e. 30 g per day<sup>1</sup> with an adherence assumption of 50%).
- 4.8 The average daily dose of patiromer remained unchanged in the resubmission and was based on the dosing data from the patient access program (9.9 g per day, plus an adherence assumption of 50%). Unchanged from the previous submission, the resubmission proposed that 3.6% of patients would use two patiromer sachets per day (to achieve a 25.2 g dose), which was a key assumption (rather than the average dose per patient) given the sponsor had proposed the same price per pack of patiromer (regardless of dose).
- 4.9 The resubmission assumed a 50% adherence rate for both SPS/CPS resins and patiromer, consistent with the previous submission. In the previous submission, this was applied as two separate assumptions: a frequency of use of 5 days per week<sup>2</sup>; and 70% compliance<sup>3</sup>. The PBAC had previously considered that it may be simpler to combine the two assumptions into a total adherence estimate, which based on the resubmission's estimates would be 50% in both arms (paragraph 6.77, patiromer PSD, November 2022 PBAC Meeting).
- 4.10 The equi-effective doses proposed in the resubmission were:
- 4.95 g patiromer = 22.5 g SPS or CPS resin

This differs to the previous submission, where the proposed equi-effective doses

---

<sup>1</sup> In the survey presented in the previous submission ("Attachment 8B. Australian KOL Survey 2"), the question asked was "The Resonium (SPS or CPS) Product Information dosage recommendations indicate the usual dosage is 15g three to four times per day. Please indicate in clinical practice how you generally prescribe this medicine in terms of frequency and dosage (grams) you prescribe". There were 20 responses, with a mean of 28.2 g and median of 30 g (range 11.3 to 45 g).

<sup>2</sup> The November 2022 submission stated that the source was the 'KOL survey 2', but it is unclear how this value was derived.

<sup>3</sup> In the survey presented in the previous submission ("Attachment 8B. Australian KOL Survey 2"), the question asked was "Of the patients that you may prescribe patiromer in the future if reimbursed, what proportion of patients do you consider will take the medicine as prescribed? i.e. Please indicate the expected adherence of patients prescribed patiromer in the future. Note: reasons for non-compliance may include forgetting to take the medicine or reluctance to take medicine due to side-effects or palatability." There were 22 responses, with a mean of 70.2% and median of 70% (range 49 to 100%).

*Public Summary Document – March 2023 PBAC Meeting*

were:

- 4.95 g patiromer = 15 g SPS or CPS resin

In November 2022, the PBAC had ‘acknowledged that there was a lack of comparable data, and it was unlikely that better quality data would be forthcoming. In the absence of alternative information, the PBAC considered that the equi-effective doses proposed in the resubmission were likely reasonable’ (paragraph 7.10, patiromer PSD, November 2022 PBAC Meeting).

- 4.11 The duration of treatment was changed from 112 days in November 2022 to 361.76 days (11.9 months) to align with the financial estimates.
- 4.12 The resubmission stated that no changes were made to the cost offsets applied. However, the resubmission applied a cost offset for serious gastrointestinal (GI) adverse events of \$29.83 per patient per course (i.e. per 361.76 days of treatment, based on \$3,010 per hospitalisation and an incremental rate of 0.01 per patient year), despite this being removed in the previous PSCR given the clinical claim was changed from superior to non-inferior safety (paragraph 6.77, patiromer PSD, November 2022 PBAC Meeting). The previous evaluation had considered this cost offset was likely to be overestimated, given rates for SPS/CPS were derived from a study in patients who were predominantly in CKD Stage 5, including patients treated with dialysis, who are more likely to experience gastrointestinal adverse events.
- 4.13 The resubmission appropriately continued to apply a cost offset for treating constipation with SPS/CPS resins of \$47.43 per patient per course (i.e. per 361.76 days of treatment, based on 23% of patients requiring on-going lactulose).
- 4.14 The key inputs to the cost minimisation approach are presented in the table below.

Public Summary Document – March 2023 PBAC Meeting

Table 2: Key inputs to the cost minimisation approach

|                          | SPS/CPS resins  |  | Patiromer  |
|--------------------------|---|--|--|
|                          | November 2022   | March 2023   | Nov 2022 and March 2023  |
| Average daily dose       | 30 g per day (as per clinician survey)  | 45 g per day (as per Product Information)                  | 9.9g/day based on compassionate access program                       |
| Adherence                | 50%   | 50%  | 50%  |
| Equi-effective doses     | 15 g SPS or CPS = 4.95 g patiromer  | 22.5 g SPS or CPS = 4.95 g patiromer                       | 4.95 g<br>(3.6% of patients use 2 sachets/day to achieve 25.2g dose) |
| Duration of treatment    | 112 days  | 361.76 days  | Same as SPS/CPS resins arm   |
| % use SPS vs CPS         | SPS = % of use (CPS: %)   |  | -  |
| Direct medicine costs    | SPS = \$54.71 / pack (454 g) <sup>a</sup><br>CPS = \$259.67 / pack (300 g) <sup>b</sup>   |  | -  |
| AE costs: constipation   | Lactulose = \$9.50 / 500 mL<br>For treatment of SPS/CPS related constipation in 23% of patients   |  | -  |
| AE costs: serious GI AEs | Treatment for GI SAEs (AR-DRG G70) = \$3,009.81<br>Rate difference between patiromer and SPS/CPs = 1 per 100 patient years<br>GI AE costs were removed in the PSCR. | GI AE costs were re-included in cost minimisation approach | -  |

Source: Table 3.1.1, p18 of the March 2023 resubmission

AE = adverse event; AEMP = approved ex-manufacturer price; AR-DRG = Australian refined-diagnosis related groups; CPS = calcium polystyrene sulfonate; GI = gastrointestinal; PSCR = pre-sub-committee response; SAE = serious adverse event; SPS = sodium polystyrene sulfonate

<sup>a</sup> AEMP from the RPBS for item 4470G sodium polystyrene sulphonate (Resonium-A) oral powder 454 g

<sup>b</sup> Wholesaler price to pharmacy, less 7.52% mark-up to derive ex-manufacturer price

- 4.15 The table below presents the results of the cost minimisation approach from the resubmission. The resubmission stated that although the resultant ex-manufacturer price of patiromer was \$| (and \$| if a |% price premium was applied to account for improved tolerability), the price offered was \$|, which the resubmission stated represented the lowest price that the sponsor was able to offer.
- 4.16 The cost minimisation approach presented in November 2022 resulted in an ex-manufacturer price of patiromer of \$| in the submission and \$| in the PSCR (from which the gastrointestinal adverse event costs were removed).

Public Summary Document – March 2023 PBAC Meeting

Table 3: Cost minimisation approach between SPS/CPS and patiromer

| Input   | CPS        | SPS        | Patiromer  |
|---|------------|------------|--|
| Cost per pack (AEMP)                              | \$259.67   | \$54.71    | -  |
| Quantity per pack                                 | 300 g      | 454 g      | 30 sachets   |
| Dose per day                                      | 45 g       | 45 g       | 1.036 sachets  |
| Adherence   | 50%        | 50%        | 50%  |
| Sachets / day                                     | -          | -          | 0.52   |
| Final dose per day                                | 22.5 g     | 22.5 g     | 4.95 g   |
| Number of days of treatment                       | 361.76     | 361.76     | 361.76   |
| Number of packs per treatment                     | 27.13      | 17.93      | 6.24   |
| Lactulose cost per bottle                         | \$9.50     | \$9.50     | -  |
| Lactulose usage                                   | 23%        | 23%        | -  |
| Lactulose cost (per 361.76 day course)            | \$47.43    | \$47.43    | -  |
| AE cost (per 361.76 day course)                   | \$29.83    | \$29.83    | -  |
| Cost per treatment (per 361.76 day course)        | \$7,122.62 | \$1,058.13 | -  |
| Weighted average treatment cost (█% SPS & █% CPS) | \$█        |            | -  |
| Proposed cost per pack (AEMP)                     |            |            | Derived from CMA: \$█ <sup>a</sup><br>Proposed: \$█ <sup>b</sup> |

Source: Table 3.4.1, p20 of the March 2023 resubmission

AE = adverse event; AEMP = approved ex-manufacturer price; CPS = calcium polystyrene sulfonate; SPS = sodium polystyrene sulfonate

<sup>a</sup> Based on \$█ per treatment and 6.24 packs per treatment

<sup>b</sup> The resubmission stated this 'represents the lowest price that the sponsor is able to offer for patiromer'.

### Estimated PBS usage and financial implications

4.17 The usage and financial impact estimates were updated in the resubmission, as compared to the November 2022 resubmission, as follows:

- The effective AEMP of patiromer was reduced from \$309.60 to \$█;
- The rate of CKD diagnosis was increased from a maximum of 35% in Year 6 to a consistent rate of 50% over the first 6 years. In November 2022, the PBAC considered that the assumed rate was underestimated (paragraph 7.12, patiromer PSD, November 2022); and
- Adherence to patiromer was reduced from 96.4% to 50%.

4.18 Key assumptions applied in the financial estimates are outlined in Table 4.

Public Summary Document – March 2023 PBAC Meeting

Table 4: Key inputs used to estimate the utilisation and financial impact of patiromer

|  | Year 1   | Year 6         | Source and comments  |
|--|--|----------------|--|
| Australian adult population                          | 21,411,852   | 23,030,499     |  |
| Prevalence of CKD 3+                                 | 5.72%  | 6.27%          | Based on 2011-2012 National Health Measures Survey for all ages; annual increase of 0.11% per year.  |
| CKD diagnosis rate                                   | 50%  | 50%            | Basis for estimate of 50% is unclear. The previous submission applied 10% in Year 1 increasing to 35% in Year 6, based on CKD diagnosis rates used in the dapagliflozin CKD submission. The previous evaluation had noted this was based on a broader CKD population (i.e. the population requested for patiromer have more severe disease, thus may have higher diagnosis rate) (para 6.86).                                      |
| Patients with CKD 3-4                                | 98.2%  |                | Based on a retrospective review of Australian general practice data.   |
| Patients with ≥2 hyperkalaemia episodes in 12 months | 1.29%  |                | Weighted estimate assuming: 0.9% of patients with Stage 3 CKD experience HK (87% of pts are Stage 3) + 3.8% with Stage 4 CKD experience HK (13% of pts are Stage 4) based on 2 two studies.  |
| <b>Eligible patients</b>                             | █ <sup>1</sup>   | █ <sup>1</sup> |  |
| <b>Previous submission</b>                           | █ <sup>2</sup>   | █ <sup>1</sup> |  |
| Uptake rate  | 10%  | 50%            | Based on clinician survey (n = 11). Unchanged from previous submission.  |
| <b>Total treated patients</b>                        | █ <sup>2</sup>   | █ <sup>2</sup> |  |
| <b>Previous submission</b>                           | █ <sup>3</sup>   | █ <sup>2</sup> | Higher number of treated patients compared to previous submission due to increased CKD diagnosis rate.   |
| Treatment duration                                   | 11.9 months  |                | Based on compassionate access program (n = 56). In November 2022, the 'PBAC noted that with the prevalence approach applied in the resubmission, patients can receive 11.9 months of treatment in Year 1 then be re-treated in Year 2. With an uptake rate of 50%, half of all treated patients would be re-treated again the following year, which the PBAC considered was likely to be substantially overestimated' (para 6.88). |
| Patiromer adherence                                  | 50% per CMA  |                | Was 96.4% in the previous submission, based on AMETHYST-DN trial   |
| Patiromer dose distribution                          | 8.4 g: 89.3%,<br>16.8 g: 14.3%<br>(i.e. 3.6% use both) |                | Based on compassionate access program.   |
| Patiromer DPMQ                                       | \$█  |                | Was \$█ in previous submission   |

Notes: PSCR requested an additional 100 grandfather patients – not included in table above.

Resubmission assumed the use of RAASi therapies will increase, however the impact on the financials is negligible. Further info in Table 19 of ESC advice.

The redacted values correspond to the following ranges:

<sup>1</sup> 5,000 to < 10,000

<sup>2</sup> 500 to < 5,000

<sup>3</sup> < 500

4.19 The estimated use and financial implications of patiromer is summarised in Table 5.

Public Summary Document – March 2023 PBAC Meeting

Table 5: 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              | 1      | 1      | 1      | 1      | 1      | 1      |
| Number of scripts dispensed             | 1      | 4      | 5      | 6      | 6      | 6      |
| <b>Estimated financial implications</b> |        |        |        |        |        |        |
| Cost to MBS                             | 2      | 2      | 2      | 2      | 2      | 2      |
| Cost of patiromer to PBS/RPBS/MBS       | 2      | 2      | 2      | 2      | 2      | 2      |
| Cost of additional RAASi medicines      | 2      | 2      | 2      | 2      | 2      | 2      |
| Net cost of patiromer to PBS/RPBS/MBS   | 2      | 2      | 2      | 2      | 2      | 2      |
| <b>November 2022 resubmission</b>       |        |        |        |        |        |        |
| Number of patients treated              | 3      | 3      | 1      | 1      | 1      | 1      |
| Number of scripts dispensed             | 1      | 4      | 5      | 5      | 6      | 7      |
| Net cost to PBS/RPBS/MBS                | 2      | 2      | 2      | 2      | 2      | 2      |

Source: Tables 4.1.2, 4.1.5 and 4.3.2, pp 27, 29 and 33 of the March 2023 resubmission

MBS = Medical Benefits Schedule; PBS = Pharmaceutical Benefits Scheme; RPBS = Repatriation Pharmaceutical Benefits Scheme

The redacted values correspond to the following ranges:

<sup>1</sup> 500 to < 5,000

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

<sup>3</sup> < 500

<sup>4</sup> 5,000 to < 10,000

<sup>5</sup> 10,000 to < 20,000

<sup>6</sup> 20,000 to < 30,000

<sup>7</sup> 30,000 to < 40,000

- 4.20 The resubmission estimated that the net cost to the PBS/RPBS of listing patiromer would be \$0 to < \$10 million in Year 6 and \$20 million to < \$30 million over the first 6 years of listing. The previous submission estimated that the listing of patiromer would cost \$30 million to < \$40 million over the first 6 years.
- 4.21 Compared with the previous submission, patient numbers were higher in the resubmission (due to the higher CKD diagnosis rate applied) while prescription numbers were higher in Years 1 to 4, but lower in Years 5 and 6 (due to the interplay of the higher CKD diagnosis rate and lower adherence rate applied).
- 4.22 While the resubmission applied two changes to the utilisation estimates, the PBAC had previously considered that ‘some of the assumptions in the financial estimates were likely to underestimate the eligible population (e.g. applying a CKD diagnosis rate based on dapagliflozin, which is used in a broader CKD population, who may be less likely to be diagnosed) and others were likely to overestimate patiromer utilisation (e.g. the uptake rates applied and the application of these uptake rates in the context of a prevalence approach). In November 2022, the PBAC advised that, on balance, in the absence of more reliable estimates, the utilisation estimates may be appropriate in the context of an RSA and with a revised price of patiromer’ (paragraph 7.12, patiromer PSD, November 2022 PBAC Meeting). The resubmission applied a higher CKD diagnosis rate (which the PBAC previously considered would likely underestimate the population) but did not apply a lower uptake rate (which the PBAC previously considered would likely overestimate the population). Overall, the resubmission’s prevalence approach meant patients can receive 11.9 months of treatment in Year 1 then be re-treated in Year 2. With an uptake rate of  $\frac{1}{2}$ %, half of all treated patients

would be re-treated again the following year, which the PBAC previously considered was likely to be substantially overestimated (paragraph 6.88, patiromer PSD, November 2022 PBAC Meeting).

- 4.23 Although the economic analysis consisted of a cost minimisation approach, the resubmission stated that the listing of patiromer represented a net increase to the PBS as patiromer will not substitute for any currently PBS-listed therapies (given it is replacing SPS/CPS resins which are not PBS-listed). The resubmission stated this increase will potentially be further extended by the increased use of concomitant RAASi medicines and estimated an increase in the cost of RAASi medicines of \$0 to < \$10 million over six years. However, the PBAC previously considered that ‘it remained unknown as to whether patiromer confers a clinically important difference in terms of optimisation/maintenance of RAASi treatment’ (paragraph 7.4, patiromer PSD, November 2022 PBAC Meeting).

### ***Financial Management – Risk Sharing Arrangements***

- 4.24 The resubmission did not propose an RSA. The PBAC previously advised that a resubmission should include ‘an RSA based on the revised financial impact estimates with expenditure caps, beyond which a rebate of  $\frac{1}{2}$ % would be applied’ (paragraph 7.14, patiromer PSD, November 2022 PBAC Meeting).

*For more detail on PBAC’s view, see section 5 PBAC outcome.*

## **5 PBAC Outcome**

- 5.1 The PBAC recommended the listing of patiromer for the treatment of adult patients with chronic kidney disease (CKD) Stage 3-4, with chronic hyperkalaemia (at least two episodes of serum potassium  $\geq 6.0$  mmol/L in the previous 12 months), who are receiving at least one renin angiotensin aldosterone system inhibitor (RAASi) or are indicated for a RAASi but are unable to tolerate it due to prior occurrence of hyperkalaemia. The PBAC considered that the resubmission had addressed the substantive outstanding issues identified at the November 2022 PBAC meeting via providing a price for patiromer based solely on a cost minimisation approach between patiromer and sodium polystyrene sulfonate (SPS) and calcium polystyrene sulfonate (CPS) and presenting revised financial estimates which incorporated the reduced price proposed in the resubmission.
- 5.2 The PBAC acknowledged the consumer comments relating to patiromer which described the benefits of treatment and highlighted the need for safe, effective, and palatable therapies in this setting. The comments also highlighted the issues with the existing therapies such as gastrointestinal adverse events and lack of palatability.
- 5.3 The PBAC recalled that in November 2022 it concluded that although the results of the indirect treatment comparisons between patiromer and SPS/CPS resins were highly uncertain, it was likely that patiromer and SPS/CPS resins were similar in terms of potassium reduction outcomes. The PBAC also recalled that it previously considered

that patiromer was non-inferior compared to SPS/CPS resins in terms of safety, noting that patiromer appeared to be more palatable and was possibly associated with fewer gastrointestinal adverse events and improved tolerability.

- 5.4 The PBAC noted that the resubmission appropriately presented a cost minimisation approach between patiromer and SPS/CPS resins in chronic hyperkalaemia patients only. The PBAC noted that the approach differed slightly compared to that presented in November 2022 in that the average daily dose of the SPS/CPS resins was based on the recommended Product Information dose, rather than the clinician survey, to which an assumption of 50% adherence was applied. Although this increased the dose of SPS/CPS resin compared to the November 2022 resubmission, the PBAC considered that the approach was reasonable and determined that the equi-effective doses were:
- 4.95 g patiromer = 22.5 g SPS or CPS resin
- 5.5 The PBAC considered that the inclusion of cost offsets for serious gastrointestinal adverse events and constipation associated with SPS/CPS resins were reasonable. Overall, the PBAC considered that the cost minimisation approach adopted in the resubmission was appropriate (see Table 3).
- 5.6 In terms of the utilisation and financial impact estimates, the PBAC noted that the resubmission made changes to the assumptions for the rate of CKD diagnosis and the adherence to patiromer (see paragraph 4.17). The PBAC noted that these changes, in combination with the proposed ex-manufacturer price, reduced the estimated net cost to the PBS/RPBS over the first 6 years of listing to \$20 million to < \$30 million (from \$30 million to < \$40 million in November 2022). The PBAC noted that although the economic analysis consisted of a cost minimisation approach, there was a net cost to the PBS as patiromer will not substitute for any currently listed PBS therapies. The PBAC noted that although SPS resin is listed on the RPBS, the net cost to the RPBS was likely to be minimal.
- 5.7 The PBAC noted that it previously advised that a risk sharing arrangement would be required but considered that at the proposed price and with the revised utilisation estimates, this was no longer necessary, particularly as there was a low risk of utilisation outside of the recommended restriction. However, noting that some uncertainty remained in the utilisation estimates, the PBAC advised that the utilisation and financial impact of patiromer should be reviewed by DUSC two years after listing.
- 5.8 The PBAC advised that patiromer is suitable for prescribing by nurse practitioners for continuing treatment only.
- 5.9 The PBAC recommended that patiromer should not be exempt from the Early Supply Rule.
- 5.10 The PBAC advised that, under Section 101(3BA) of the *National Health Act 1953*, patiromer should not be treated as interchangeable with any other drugs on an individual patient basis.

Public Summary Document – March 2023 PBAC Meeting

- 5.11 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because patiromer is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over SPS/CPS resins, and not expected to address a high and urgent unmet clinical need, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met.
- 5.12 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

**Outcome:**

Recommended

## 6 Recommended listing

6.1 Add new item:

| MEDICINAL PRODUCT<br>Medicinal product pack                          | PBS item code  | Max. qty (packs) | Max. qty (units) | No. Rpts | Available brands |
|--|--|------------------|------------------|----------|------------------|
| PATIROMER  |  |                  |                  |          |                  |
| patiromer 8.4 g powder for oral liquid, 30 sachets                   | New MP   | 1                | 30               | 5        | Veltassa         |
| patiromer 16.8 g powder for oral liquid, 30 sachets                  | New MP   | 1                | 30               | 5        |                  |
| <b>Restriction Summary/ Treatment of Concept: Authority Required</b> |  |                  |                  |          |                  |
| Concept ID   | <b>Category / Program:</b> GENERAL – General Schedule (Code GE)  |                  |                  |          |                  |
|  | <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>Episodicity:</b> Chronic  |                  |                  |          |                  |
|  | <b>Severity:</b> [blank]   |                  |                  |          |                  |
|  | <b>Condition:</b> Hyperkalaemia  |                  |                  |          |                  |
|  | <b>Indication:</b> Chronic hyperkalaemia   |                  |                  |          |                  |
|  | <b>Treatment phase:</b> Initial PBS-subsidised treatment (including grandfathered patients)  |                  |                  |          |                  |
|  | <b>Population criteria:</b>  |                  |                  |          |                  |
|  | Patient must have stage 3 to stage 4 chronic kidney disease  |                  |                  |          |                  |
|  | <b>Clinical criteria:</b>  |                  |                  |          |                  |
|  | The condition must be inadequately controlled by a low potassium diet.   |                  |                  |          |                  |
|  | <b>AND</b>   |                  |                  |          |                  |
|  | <b>Clinical criteria:</b>  |                  |                  |          |                  |
|  | Patient must have experienced at least 2 episodes of hyperkalaemia (defined as serum potassium levels of at least 6.0 mmol/L) within the 12 months prior to commencing this drug |                  |                  |          |                  |
|  | <b>AND</b>   |                  |                  |          |                  |
|  | <b>Clinical criteria:</b>  |                  |                  |          |                  |
|  | The treatment must not be in place of emergency treatment of hyperkalaemia   |                  |                  |          |                  |
|  | <b>Treatment criteria:</b>   |                  |                  |          |                  |

Public Summary Document – March 2023 PBAC Meeting

|   |  |
|---|--|
|   | Patient must be undergoing treatment with a renin angiotensin aldosterone system inhibitor; or   |
|   | Patient must not be undergoing treatment with a renin angiotensin aldosterone system inhibitor because such treatment results in hyperkalaemia   |
|   | <b>AND</b>   |
|   | <b>Treatment criteria:</b>   |
|   | Must be treated by a specialist medical practitioner with experience in the diagnosis and management of chronic kidney disease   |
|   |  |
|   | <b>Administrative advice:</b><br>Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see <a href="http://www.servicesaustralia.gov.au/HPOS">www.servicesaustralia.gov.au/HPOS</a> ) or by telephone by contacting Services Australia on 1800 888 333 |
| <b>Restriction Summary / Treatment of Concept: Authority Required (STREAMLINED)</b> |  |
|   | <b>Category / Program:</b> GENERAL – General Schedule (Code GE)  |
|   | <b>Prescriber type:</b> <input checked="" type="checkbox"/> Medical Practitioners <input checked="" type="checkbox"/> Nurse Practitioners  |
|   | <b>Restriction:</b> <input checked="" type="checkbox"/> Authority Required (Streamlined) [new]   |
|   | <b>Administrative Advice:</b>  |
|   | Continuing Therapy Only:<br>For prescribing by nurse practitioners as continuing therapy only, where the treatment of, and prescribing of medicine for, a patient has been initiated by a medical practitioner. Further information can be found in the Explanatory Notes for Nurse Practitioners.                       |
|   |  |
|   | <b>Indication:</b> Chronic hyperkalaemia   |
|   |  |
|   | <b>Treatment phase:</b> Continuing treatment   |
|   |  |
|   | <b>Clinical criteria:</b>  |
|   | Patient must have previously received PBS-subsidised treatment with this drug for this condition   |
|   | <b>AND</b>   |
|   | <b>Clinical criteria:</b>  |
|   | The treatment must not be in place of emergency treatment of hyperkalaemia   |
|   |  |
|   | <b>Treatment criteria:</b>   |
|   | Patient must not be undergoing dialysis  |
|   | <b>AND</b>   |
|   | <b>Treatment criteria:</b>   |
|   | Patient must be undergoing treatment with a renin angiotensin aldosterone system inhibitor   |

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

## **7 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.

## **8 Sponsor's Comment**

The sponsor had no comment.