

6.01 Review of PBS restrictions for thrombopoietin receptor agonists (TPO-RA) romiplostim and eltrombopag for immune thrombocytopenia (ITP)

1 Purpose of item

- 1.1 To request the Pharmaceutical Benefits Advisory Committee (PBAC) consider extensions to the current Pharmaceutical Benefits Scheme (PBS) listings for the thrombopoietin receptor agonists (TPO-RA): romiplostim and eltrombopag.

2 Background

- 2.1 Romiplostim and eltrombopag were listed on the PBS via a series of applications between 2009 and 2014. Since then, confidence in the efficacy and safety of these medicines has progressively increased, including reduced concern about bone marrow fibrosis (reticulin formation).
- 2.2 The PBAC was requested to consider the following parts of the restriction:
- sequential use
 - use as an alternative to splenectomy
 - use in children
 - use of platelet counts as a criterion for commencing and continuing treatment
 - any other aspects of the restriction that may need refreshing to align with current standards of care
 - the requirement for written authority approval for initial treatment

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

3 Current situation

Sequential use

- 3.1 Switching between eltrombopag and romiplostim within the initial 24 weeks of treatment is explicitly mentioned in the current restrictions. As per the PBAC Public Summary Documents (PSDs) from 2009-2011 this was *"to allow flexibility for prescribers and patients to establish the most suitable treatment for each individual within this period"*. The restrictions for continuing treatment say that: *"Patient must not have previously received PBS-subsidised continuing treatment with romiplostim/eltrombopag for this condition."*
- 3.2 Recent Australian consensus guidelines for adult immune thrombocytopenia state that, "Switching from one TPO-RA to another has been shown to be effective for some patients and is permissible for PBS reimbursement with written application" (Choi,

Merriman et al. 2022)¹. It is unclear whether this refers to switching in the initial 24 weeks or to switching any time since the start of treatment.

- 3.3 Eltrombopag and romiplostim have distinct pharmacodynamics and pharmacokinetic properties and may have different tolerability and efficacy in individual patients.
- 3.4 There are no randomised data to establish a role for switching between TPO-RAs. Gonzalez-Porras and co-authors summarised and pooled data from the 18 published non-randomised studies of switching as at 2018 (González-Porras, Godeau et al. 2019)². Lack of efficacy was identified as the primary reason for switching in 58% of patients (172/295). Non-efficacy-related reasons for switching included adverse events (AEs), patient preference, and platelet count fluctuations (variously defined: $30 \times 10^9/L$ to $400 \times 10^9/L$ within a month or change of $> 200 \times 10^9/L$ in weekly counts without rescue treatment). Response rates (roughly defined as per the PBS continuation criteria, above) after switching were available in 209 patients, and 162 of those (78%) achieved or maintained a platelet response. Nearly all patients (93% [87/94]) who switched TPO-RAs due to reasons other than lack of efficacy maintained their response after switching. A high response rate (65% [72/111]) and improved platelet counts were observed even if switching was due to lack of efficacy with the first TPO-RA.
- 3.5 The outcomes of switching were similar regardless of the direction of the switch (i.e. eltrombopag to romiplostim or romiplostim to eltrombopag); however, the reasons for switching were different for eltrombopag and romiplostim. Although the rate of switching due to safety and tolerability considerations was comparable between the two TPO-RAs (20–30%), switching due to platelet count fluctuations was reported exclusively in patients who received romiplostim. Among 20 patients who switched to eltrombopag due to platelet count fluctuations, 14 (70%) attained a response. Patient preference was a major driver of switching from romiplostim (s/c) to eltrombopag (oral).
- 3.6 In short, most patients who switch due to lack of efficacy with the first TPO-RA respond to the alternate TPO-RA, demonstrating an absence of cross-resistance between the two medicines. Clinical practice guidelines recommend switching to the alternate TPO-RA should be considered before the use of a less-preferable (last-line) option (González-Porras, Godeau et al. 2019)³.

¹ Choi, P. Y., E. Merriman, A. Bennett, A. K. Enjeti, C. W. Tan, I. Goncalves, D. Hsu and R. Bird (2022). "Consensus guidelines for the management of adult immune thrombocytopenia in Australia and New Zealand." *Medical Journal of Australia* **216**(1): 43-52.

² González-Porras, J. R., B. Godeau and M. Carpenedo (2019). "Switching thrombopoietin receptor agonist treatments in patients with primary immune thrombocytopenia." *Therapeutic advances in hematology* **10**: 2040620719837906.

³ González-Porras, J. R., B. Godeau and M. Carpenedo (2019). "Switching thrombopoietin receptor agonist treatments in patients with primary immune thrombocytopenia." *Therapeutic advances in hematology* **10**: 2040620719837906.

- 3.7 PBS data showed that between 12 April 2011 and 31 January 2022, 1,809 patients were supplied with either romiplostim or eltrombopag. Of those 1,809 patients, 284 patients (15.7%) switched between the two TPO-RAs at least once.
- 3.8 The mean time-on-treatment (i.e. until treatment was either discontinued or switched to another TPO-RA) was 3.8 years and 3.4 years for romiplostim and eltrombopag respectively. Of those who switched, 174 patients (61%) switched within 24 weeks from the time of initiation and 110 patients (39%) switched after 24 weeks. A subgroup analysis of those who switched after 24 weeks showed that the estimated mean time-to-treatment was 5.2 years in this group, i.e., for patients who persisted treatment beyond 24 weeks, most stayed on treatment for about 5.2 years.

Use as an alternative to splenectomy

- 3.9 The current restrictions position TPO-RAs after splenectomy or where splenectomy is contraindicated.
- 3.10 The initial PBAC applications for TPO-RAs (2009-2011) specified placebo as the comparator in the economic model, not splenectomy (or off-label rituximab). The PSD from those PBAC meetings noted that identifying a patient group with an absolute contraindication to splenectomy would be difficult and that the risk of TPO-RAs being used outside the restriction as an alternative to splenectomy should be managed by a risk share arrangement.
- 3.11 Current clinical practice guidelines position TPO-RAs as second line, with splenectomy and rituximab as alternatives depending on patient preferences and values (Neunert, Terrell et al. 2019,⁴ Provan, Arnold et al. 2019,⁵ George and Arnold 2020,⁶ Choi, Merriman et al. 2022,⁷ Janssens, Selleslag et al. 2022⁸).
- 3.12 Although sustained remission rates with splenectomy are about 60% to 70%, some cases of ITP resolve allowing all treatment to be stopped. Further, splenectomy has long-term adverse effects such as an increased risk of infection/immunosuppression and thrombo-embolism.

⁴ Neunert, C., D. R. Terrell, D. M. Arnold, G. Buchanan, D. B. Cines, N. Cooper, A. Cuker, J. M. Despotovic, J. N. George and R. F. Grace (2019). "American Society of Hematology 2019 guidelines for immune thrombocytopenia." *Blood advances* **3**(23): 3829-3866.

⁵ Provan, D., D. M. Arnold, J. B. Bussel, B. H. Chong, N. Cooper, T. Gernsheimer, W. Ghanima, B. Godeau, T. J. González-López and J. Grainger (2019). "Updated international consensus report on the investigation and management of primary immune thrombocytopenia." *Blood advances* **3**(22): 3780-3817.

⁶ George, J. and D. Arnold (2020). Immune thrombocytopenia (ITP) in adults: Secondline and subsequent therapies–UpToDate, UpToDate.

⁷ Choi, P. Y., E. Merriman, A. Bennett, A. K. Enjeti, C. W. Tan, I. Goncalves, D. Hsu and R. Bird (2022). "Consensus guidelines for the management of adult immune thrombocytopenia in Australia and New Zealand." *Medical Journal of Australia* **216**(1): 43-52.

⁸ Janssens, A., D. Selleslag, J. Depaus, Y. Beguin and C. Lambert (2022). "Primary immune thrombocytopenia in adults: Belgian recommendations for diagnosis and treatment anno 2021 made by the Belgian Hematology Society." *Acta Clinica Belgica* **77**(2): 470-483.

- 3.13 Initial concerns about increasing bone marrow reticulin formation with romiplostim and eltrombopag have reduced, based on follow-up studies.
- 3.14 PBS data showed that since 2016, the number of patients who have had splenectomy has decreased by more than one-third while that of non-splenectomised patients accessing TPO-RAs has increased.

Use in children

- 3.15 ITP manifests differently in children in that ITP in children is likely to resolve after a short period of time and not require TPO-RAs.
- 3.16 However, in a small number of children the disease has a chronic course. Expert advice puts the numbers at about 10 children per year, nationally. Clinical practice guidelines suggest the use of TPO-RAs (rather than rituximab) in these children (Neunert, Terrell et al. 2019)⁹.
- 3.17 The current PBS restrictions limit subsidy to patients 18 years or older.
- 3.18 The Therapeutic Goods Administration (TGA) indications allow for paediatric use:

romiplostim

Nplate[®] is indicated for treatment of thrombocytopenia in paediatric patients aged 1 year and older with primary immune thrombocytopenia ITP for at least 6 months who are:

- non-splenectomised and have had an insufficient response, or are intolerant, to corticosteroids and immunoglobulins;
- splenectomised and have had an inadequate response to splenectomy.

eltrombopag

Revolade[®] is indicated for the treatment of:

- paediatric patients with chronic immune thrombocytopaenia (ITP) who have failed other treatments and either (a) need an increased platelet concentration for a planned procedure or (b) are at a high risk of bleeding.

Platelet counts for commencing and continuing treatment

- 3.19 The current restriction includes the following criteria for commencing romiplostim/eltrombopag subsidy:
- Intolerance to treatment with corticosteroid and immunoglobulin therapy developed during the relevant period of use, which was of a severity to necessitate permanent treatment withdrawal, details of the degree of this toxicity must be provided at the time of application.
 - Inadequate response with corticosteroid and immunoglobulin therapy; (a) a platelet count of less than or equal to 20,000 million per L; OR, (b) a platelet

⁹ Neunert, C., D. R. Terrell, D. M. Arnold, G. Buchanan, D. B. Cines, N. Cooper, A. Cuker, J. M. Despotovic, J. N. George and R. F. Grace (2019). "American Society of Hematology 2019 guidelines for immune thrombocytopenia." *Blood advances* 3(23): 3829-3866.

count of 20,000 million to 30,000 million per L, where the patient is experiencing significant bleeding or has a history of significant bleeding in this platelet range.

- 3.20 The current restriction includes the following criteria for continuing romiplostim/eltrombopag subsidy:
- For the purpose of this restriction, a continuing response to treatment with drug is defined as: (a) use of rescue medication (corticosteroids or immunoglobulins) on no more than one occasion during the most recent 24 week period of PBS-subsidised treatment with this drug, AND either of the following: (b) a platelet count greater than or equal to 50,000 million per L, OR, (c) a platelet count greater than 30,000 million per L and which is double the baseline platelet count.
 - The platelet count must be no more than 4 weeks old at the time of application.
- 3.21 Some patients may have chronically low platelet counts and require continuous TPO-RA use. Other patients may have a relapsing and remitting course, allowing treatment breaks. In some patients the disease may resolve completely and require no further treatment.

Lifespans of the medicines

- 3.22 The earliest date on which eltrombopag (Revolade) can be expected to face competition in Australia is 17 July 2025, being the day following the expiry of Novartis Pharmaceuticals Australia Pty Limited's (Novartis) compound patent on eltrombopag.
- 3.23 There is a related pharmaceutical composition patent for Revolade that expires on 1 August 2027. This patent may prevent generics from entering the market in 2025 if the particular formulation described and claimed in this patent is, in essence, the only practical delivery system for eltrombopag.
- 3.24 The compound patent for romiplostim (Nplate) is due to expire on 8 August 2023. A number of related Australian patents have lapsed (i.e. abandoned) for this drug.
- 3.25 A biosimilar of romiplostim gained marketing approval in India in 2021. However, no biosimilar romiplostim is currently under evaluation by the EMA, which typically is the first regulatory agency in a high-income country to evaluate a new biosimilar¹⁰.

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

4 Financial implications

- 4.1 The expenditure for eltrombopag and romiplostim in 2021 was \$ [REDACTED] based on PBS data at the effective pricing. Table 1 and Table 2 present estimated cost to Government if the current listing continues and the estimated costs if the listing were expanded to include use in children and remove the requirement for splenectomy unless contraindicated.

¹⁰ <https://www.greyb.com/biologics-patents-expiring-2022-2023-2024-2025-2026-2027/>

Table 1: Estimated usage and financial expenditure using effective price

Net cost to PBS/RPBS	2022	2023	2024	2025	2026	2027
Current listing						
Changed listing (Adults)						
Changed listing (Children)						
Changed listing (Total)						
Additional cost to PBS/RPBS						

Source: Romiplostim and Eltrombopag model.XLSX – prepared by the Department
 PBS = Pharmaceutical Benefits Scheme, RPBS = Repatriation Pharmaceutical Benefits Scheme

Table 2: Estimated usage and financial expenditure using published price

Net cost to PBS/RPBS	2022	2023	2024	2025	2026	2027
Current listing	\$ ¹	\$ ¹	\$ ²	\$ ²	\$ ²	\$ ³
Changed listing (Adults)	\$ ¹	\$ ²	\$ ²	\$ ²	\$ ²	\$ ³
Changed listing (Children)	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴
Changed listing (Total)	\$ ¹	\$ ²	\$ ²	\$ ²	\$ ³	\$ ³
Additional cost to PBS/RPBS	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴	\$ ⁴

Source: Romiplostim and Eltrombopag model.XLSX – prepared by the Department
 PBS = Pharmaceutical Benefits Scheme, RPBS = Repatriation Pharmaceutical Benefits Scheme

The redacted values correspond to the following ranges:

¹\$20 million to < \$30 million

²\$30 million to < \$40 million

³\$40 million to < \$50 million

⁴\$0 to < \$10 million

4.2 The estimated cost to Government over the forward estimates for the proposed restriction changes was \$█.

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

5 PBAC Outcome

5.1 The PBAC recommended the proposed extension to the restrictions of romiplostim and eltrombopag. The PBAC noted that since the listing of TPO-RAs on the PBS a decade ago, the safety and efficacy of these medicines is now better understood, with TPO-RAs now being the standard of care for refractory ITP as per International and Australian Guidelines. The PBAC acknowledged that the existing restrictions were not consistent with current clinical practice and recommended that:

- switching between eltrombopag and romiplostim be allowed at any time and wording in the restriction about switching within 24 weeks be removed to mitigate any confusion among prescribers
- the wording in the restriction regarding prior splenectomy or contraindication to splenectomy use be removed
- children be included in the updated restrictions by removing age limits
- restrictions on platelet count specified in continuing treatment be removed, and treatment be allowed to continue if the patient can maintain platelet count sufficient to prevent clinically significant bleeding
- the requirement to stipulate toxicity to corticosteroid and immunoglobulin be removed.

5.2 The PBAC noted that switching between eltrombopag and romiplostim may occur for multiple clinically valid reasons including lack of efficacy, adverse events, intolerance to dietary restrictions with eltrombopag; or other reasons such as ease of use while

travelling. Given that TPO-RAs are specialised drugs with a restricted group of specialist prescribers (haematologists) as well as different tolerability and efficacy among individual patients, the PBAC recommended that switching between eltrombopag and romiplostim be allowed at any time and wording in the restriction about switching within 24 weeks be removed to mitigate any confusion among prescribers.

- 5.3 The PBAC noted that the Australian guidelines position TPO-RA as a second line treatment, with splenectomy and rituximab as alternatives. The PBAC considered that patients in consultation with their haematologist will select the second line treatment that best fits with their preferences and values. The PBAC therefore recommended that the wording in the restriction regarding prior splenectomy or contraindication to splenectomy use be removed.
- 5.4 The PBAC noted that while paediatric ITP differs from adult ITP, around 10 children (aged < 18 years) require treatment with TPO-RAs for ITP each year. While the TGA marketing approval includes the use of TPO-RAs among the paediatric population as a second line treatment after corticosteroids and immunoglobulin therapies, the current PBS restrictions do not include children and the funding and availability of these treatments vary among hospitals, creating barriers to treatment of children. The PBAC recommended that children be included in the updated restrictions by removing age limits.
- 5.5 The PBAC noted that there is a subgroup of patients who have improvement in platelet counts from baseline but do not necessarily meet the continuing criteria, for example, patients who have platelet counts increased to 20,000 million per L after treatment. To allow treatment access for patients who have a clear and clinically meaningful improvement despite their chronically low platelet count, the PBAC recommended that the restrictions on platelet count specified in the continuing treatment restriction be removed. The PBAC recommended that treatment be allowed to continue if the patient can maintain a platelet count sufficient to prevent clinically significant bleeding. The PBAC noted that scoring systems such as ITP-BAT scores for grading bleeding are cumbersome in defining clinically significant bleeding. The PBAC considered that clinical judgement of specialist haematologists, to whom prescribing of TPO-Ras is restricted, can be relied on for assessing the clinical significance of bleeding for the purposes of continuation.
- 5.6 The PBAC noted that, when applying for authority to prescribe PBS subsidised initial treatment, the current restriction requires that prescribers provide information on toxicity to corticosteroid and immunoglobulin therapy where intolerance to these treatments developed, necessitating treatment withdrawal, in order to justify moving to the next line of treatment, i.e. TPO-RAs. The PBAC considered that the requirement to stipulate toxicity to corticosteroid and immunoglobulin therapy was not necessary and recommended that it be removed.
- 5.7 The PBAC recommended continuing the written authority approval or HPOS upload for initial treatment and telephone or electronic approval for first continuing treatment. The PBAC noted that the maximum quantity authorised for romiplostim is for one week's supply without any repeat while the maximum quantity for eltrombopag lasts up to 6 months for each patient. To reconcile this difference in

supply amount between the TPO-RAs, the PBAC recommended that the maximum quantity for romiplostim be increased to cover up to 6 months of treatment.

Outcome:

Recommended

6 Recommended listing

6.1 The recommended listing for romiplostim 250 mcg injection and romiplostim 500 mcg injection is described below. The changes to the PBS listings for romiplostim are to be flowed on to eltrombopag 50 mg (5826P, 5828R) and eltrombopag 25 mg (5825N, 5827Q).

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	Ne.of Rpts	Available brands
ROMIPLOSTIM					
romiplostim 250 microgram injection, 1 vial	9696H (HB)	1	1	5	Nplate
romiplostim 250 microgram injection, 1 vial	9697J (HS)	1	1	5	Nplate
romiplostim 500 microgram injection, 1 vial	9698K (HB)	1	1	5	Nplate
romiplostim 500 microgram injection, 1 vial	9699L (HS)	1	1	5	Nplate
<p>Category / Program: Section 100 – Highly Specialised Drugs Program</p> <p>Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners</p> <p>Restriction type: <input checked="" type="checkbox"/> Authority Required – (in writing only via post/HPOS upload)</p> <p>Indication: Severe thrombocytopenia</p> <p>Treatment Phase: Initial treatment – New patient</p> <p>Clinical criteria:</p> <p>The condition must be severe chronic immune (idiopathic) thrombocytopenic purpura (ITP)</p> <p>AND</p> <p>Clinical criteria:</p> <p>Patient must have failed to achieve an adequate response to, or be intolerant to, corticosteroid therapy for at least 4-6 weeks</p> <p>AND</p> <p>Clinical criteria:</p> <p>Patient must have failed to achieve an adequate response to, or be intolerant to, immunoglobulin therapy</p> <p>AND</p> <p>Clinical criteria:</p> <p>The treatment must be the sole PBS-subsidised thrombopoietin receptor agonist (TRA) for this condition</p> <p>Prescribing Instructions: The following criteria indicate failure to achieve an adequate response to corticosteroid and/or immunoglobulin therapy and must be demonstrated at the time of initial application:, (a) a platelet count of less than or equal to 20,000 million per L; OR, (b) a platelet count of 20,000 million to 30,000 million per L, where the patient is experiencing significant bleeding or has a history of significant bleeding in this platelet range.</p> <p>Prescribing Instructions: The medical practitioner should request 1 vial of the appropriate strength, to titrate therapy based on the weight of the patient. A maximum of 5 repeats will be authorised.</p> <p>Prescribing Instructions: Once a patient's dose has been stable for a period of 4 weeks, authority approvals for sufficient vials of appropriate strength based on the weight of the patient and dose (microgram/kg/week) for up to 4 weeks of treatment. may be requested under the Balance of Supply listing. The total period of treatment authorised under this restriction must not exceed 24 weeks.</p>					

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	Where a patient has started initial treatment with either of romiplostim or eltrombopag, change of therapy to the alternative agent may be authorised under the Balance of supply or change of therapy restriction to complete up to 24 weeks initial treatment.
	Prescribing Instructions: Authority approval will not be given for doses higher than 10 micrograms/kg/week
	Prescribing Instructions: The authority application must be made in writing and must include; (1) a completed authority prescription form, (2) a completed Idiopathic Thrombocytopenic Purpura Initial PBS Authority Application - Supporting Information Form, (3) details of a platelet count supporting the diagnosis of ITP, and, (4) details of the reason of medical contraindication for surgery and date of assessment.
	Prescribing Instructions: The platelet count must be no more than 4 weeks old at the time of application.
	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au , Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos , Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001
	Category / Program: Section 100 – Highly Specialised Drugs Program
	Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners
	Restriction type: <input checked="" type="checkbox"/> Authority Required – (Telephone /electronic)
	Indication: Severe thrombocytopenia
	Treatment Phase: First Continuing treatment or Re-initiation of interrupted continuing treatment
	Clinical criteria:
	The condition must be severe chronic immune (idiopathic) thrombocytopenic purpura (ITP)
	AND
	Clinical criteria:
	Patient must have demonstrated a sustained platelet response to PBS-subsidised treatment with this drug for this condition under the Initial treatment restriction if the patient has not had a treatment break; or
	Patient must have demonstrated a sustained platelet response to the most recent PBS-subsidised treatment with this drug for this condition prior to interrupted treatment
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised thrombopoietin receptor agonist (TRA) for this condition
	Prescribing Instructions: For the purposes of this restriction, a sustained response is defined as the patient having the ability to maintain a platelet count sufficient to prevent clinically significant bleeding based on clinical assessment.
	Prescribing Instructions: The medical practitioner should request sufficient number of vials of appropriate strength based on the weight of the patient and dose (microgram/kg/week) to provide 4 weeks of treatment. Up to a maximum of 5 repeats may be authorised.
	Prescribing Instructions: Authority approval will not be given for doses higher than 10 micrograms/kg/week
	Prescribing Instructions: The platelet count must be conducted no later than 4 weeks from the date of completion of the most recent PBS-subsidised course of treatment with this drug
	Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
	Category / Program: Section 100 – Highly Specialised Drugs Program
	Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners
	Restriction type: <input checked="" type="checkbox"/> Authority Required –(Telephone/electronic)
	Indication: Severe thrombocytopenia
	Treatment Phase: Second or Subsequent Continuing treatment
	Clinical criteria:
	The condition must be severe chronic immune (idiopathic) thrombocytopenic purpura (ITP)
	AND
	Clinical criteria:

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	Patient must have previously received PBS-subsidised treatment with this drug for this condition under first continuing or re-initiation of interrupted continuing treatment restriction
	AND
	Clinical criteria:
	Patient must have demonstrated a continuing response to PBS-subsidised treatment with this drug
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised thrombopoietin receptor agonist (TRA) for this condition
	Prescribing Instructions: The platelet count must be no more than 4 weeks old at the time of application.
	Prescribing Instructions: The medical practitioner should request sufficient number of vials of appropriate strength based on the weight of the patient and dose (microgram/kg/week) to provide 4 weeks of treatment. Up to a maximum of 5 repeats may be authorised.
	Prescribing Instructions: Authority approval will not be given for doses higher than 10 micrograms/kg/week
	Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).
	Category / Program: Section 100 – Highly Specialised Drugs Program (Public Hospitals)
	Prescriber type: <input checked="" type="checkbox"/> Medical Practitioners
	Restriction type: <input checked="" type="checkbox"/> Authority Required –(Telephone/electronic)
	Indication: Severe thrombocytopenia
	Treatment Phase: Balance of supply or change of therapy
	Clinical criteria:
	The condition must be severe chronic immune (idiopathic) thrombocytopenic purpura (ITP)
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised thrombopoietin receptor agonist (TRA) for this condition
	AND
	Clinical criteria:
	Patient must have received insufficient therapy with this drug for this condition under the Initial 1-restriction-OR
	Patient must have received insufficient therapy with this drug for this condition under the Initial 2-restriction
	Patient must have received insufficient therapy with this drug for this condition under the First Continuing treatment or Re-initiation of interrupted continuing treatment restriction OR
	Patient must have received insufficient therapy with this drug for this condition under the Second and subsequent Continuing treatment restriction OR
	Patient must be swapping therapy from eltrombopag to this drug for this condition
	AND
	Prescribing Instructions: Where a patient has started initial treatment with either of romiplostim or eltrombopag, change of therapy to the alternative agent may be authorised under this restriction to complete up to 24 weeks initial treatment.
	For initial treatment, once a patient's dose has been stable for a period of 4 weeks, authority approvals may be granted for sufficient vials of appropriate strength based on the weight of the patient and dose (microgram/kg/week) for up to 4 weeks supply with sufficient repeats for the balance of up to 24 weeks treatment.
	Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).

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

Amgen Australia Pty Limited welcomes the PBAC's recommendation to extend the restrictions of the TPO-RA class for immune thrombocytopaenia (ITP), improving access to these important therapies for Australian patients and clinicians.

Novartis had no comment.