Authorized Medicines and Regulatory Considerations

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Disclosures

None

CURRENTLY AUTHORIZED DRUGS FOR ITP IN CANADA

Drug (Brand Name)	Mechanism	Authorized ITP Indication	Authorization Date	Market Date
Romiplostim (Nplate)	TPO-RA (injectable)	Adults with chronic ITP with inadequate response to corticosteroids, immunoglobulins, or splenectomy	February 19, 2009	April 15, 2009
Eltrombopag (Revolade)	TPO-RA (oral)	Adult and pediatric patients with chronic ITP with insufficient response to corticosteroids or immunoglobulins	January 12, 2011	April 1, 2011
Fostamatinib (Tavalisse)	SYK inhibitor	Adults with chronic ITP with insufficient response to other treatments	November 17, 2020	December 10, 2020
Avatrombopag (Doptelet)	TPO-RA (oral)	Adults with chronic ITP with insufficient response to a previous treatment	November 3, 2023	January 22, 2024

REGULATORY CONSIDERATIONS

Authorizations supported by

- RCTs
- Single arm studies

Endpoints

- Platelet response (≥ 50 x 10^9/L)
  - Durable platelet response
- Bleeding events, reduction in rescue therapy

Romiplostim

Eltrombopag

Fostamatinib

Avatrombopag

- Platelet count - primary endpoint
- Bleeding reduction not evaluated as an endpoint across all trials
- Some trials included clinically meaningful outcomes alongside platelet response

REGULATORY CONSIDERATIONS

Bleeding events

- Not always powered for statistical significance
- In some trials, captured as adverse events rather than formal endpoint
- Low event rates and small sample sizes limit interpretability

REGULATORY CONSIDERATIONS

Labelling considerations

- Variability in platelet thresholds for dose modification across products
- Resulting challenges in achieving consistent labelling

REGULATORY CONSIDERATIONS

Limited clinical outcome data

- Inconsistent bleeding endpoints
- QoL measurements limited

Heterogeneity in endpoint definitions

- Durable response definitions vary

Trial design

- Placebo controlled vs active comparator