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## Cost-Consequence Model Comparing Eltrombopag and Romiplostim for Pediatric Patients with Previously-Treated Immune Thrombocytopenia

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### BACKGROUND

- Immune thrombocytopenia (ITP) is an auto-immune disorder characterized by enhanced platelet destruction, decreased platelet production, and, subsequently, increased bleeding risk. It is a chronic condition (ITP) in many patients (1).
- In pediatric patients, severe thrombocytopenia may limit activities and those patients who have an insufficient response to therapy may be at risk of severe, potentially life-threatening bleeding complications (2).
- Approximately 40% of children treated with ITP are children younger than 10 years (3).
- Mortality rates vary. For instance, approximately 70%, ITP is a self-limiting disease that resolves naturally within 6 months (2, 4, 5). The disease becomes chronic in 20-30% of pediatric patients, for whom spontaneous remission is unlikely (2, 6).
- In the United States, the average estimate of the incidence of ITP is 5 children per 100,000 per year (7).
- To help prevent bleeding episodes, ITP therapies increase platelet counts. Many first-line therapies curb immune system-mediated platelet destruction (corticosteroids, immunoglobulins).
- Thrombopoietin receptor (TPO) agonists (eltrombopag and romiplostim) have recently emerged as promising therapies for ITP patients who are refractory to other treatments (8).
- The efficacy of eltrombopag (EPAG) in pediatric patients is demonstrated in the randomized, double-blind, multi-center, Phase 2 and 3 trials PETIT and PETIT-2. In these trials, patients treated with EPAG had significantly higher platelet response rates (PETIT) and sustained platelet response rates (PETIT-2) than placebo-treated patients (9, 10).
- While EPAG is the only TPO-agonist FDA approved for use in pediatric ITP, romiplostim (ROMI) has been used in Phase 3 clinical trials.
- To date, no head-to-head trials have compared EPAG and ROMI and few indirect treatment comparisons have assessed their relative efficacy and safety (11).

### OBJECTIVES

- This study’s objective was to develop an economic model to compare the costs and benefits of EPAG and ROMI in pediatric ITP in the US.

### METHODS

- A cost-consequence model was developed using a decision tree approach to evaluate the costs of EPAG and ROMI relative to treatment success in previously-treated pediatric patients.
- Data on platelet count response rate, bleeding events, and adverse events were derived from all relevant identified phase III and IIT clinical trials; efficacy data from the randomized, double-blind, multi-center Phase 2 and 3 trials PETIT and PETIT-2 were used (9, 10).
- ROMI efficacy data was adjusted so that the ROMI placebo group would match the placebo group of the EPAG trial.
- Costs incorporated in the model included drugs and administration, routine care, rescue medications, bleeding-related adverse events, other adverse events, and mortality costs (Table 1).

### RESULTS

#### EFFICACY

- Summary of the efficacy results is presented in Figure 3. (a) Regarding outcomes, EPAG demonstrated a 22.1% incremental benefit over ROMI when assessing severe bleeding events.
- (b) Moderate bleeding (WHO 3) was also evaluated, with EPAG having a 15.7% incremental benefit over ROMI. EPAG again showed a benefit over ROMI, with an incremental benefit of 2.3% over ROMI when platelet response was assessed.

#### COSTS

- A summary of the cost results is presented in Figure 2 and Table 2. The overall estimated cost of EPAG per patient was $65,600, compared to $101,000 for ROMI.
- EPAG’s lower cost compared to ROMI was largely due to lower drug costs ($62,200 vs. $84,400), administration costs ($50 vs. $1,960), and significantly lower costs due to severe bleeding ($594 vs. $10,250).

### LIMITATIONS

- This analysis, while robust, is accompanied by some minor limitations.
- Trial endpoints sometimes varied within the literature, direct matching and data selection were therefore challenging.
- Retrospectively and prospectively, two common treatments for ITP could not be included as comparators due to the lack of available data.
- The time horizon used in this model was relatively short but allowed modeling of within-trial endpoints without the need for extrapolation techniques.

### PROBABILISTIC SENSITIVITY ANALYSIS

- When considering cost per severe bleeding event avoided, EPAG was dominant over ROMI (less expensive and more effective).
- EPAG was considered dominant over ROMI when assessing the cost per responder and per bleeding event (any grade).
- Probabilistic sensitivity analysis was consistent with the base case findings.

### CONCLUSIONS

- TPO-agonist agents are favourable options for the treatment of pediatric patients with ITP who have an insufficient response to corticosteroids or immunoglobulins. We showed that EPAG may be the preferred TPO-agonist to treat ITP when indirectly compared to ROMI, largely driven by its favorable severe bleeding outcomes and lower drug and administration costs.

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**Figure 1:** Indirect Treatment Comparison Network

**Figure 2:** The relative efficacy of eltrombopag and romiplostim

**Figure 3:** The relative efficacy of eltrombopag and romiplostim

**Table 1:** Costs incorporated in model

**Table 2:** Costs of treatment with eltrombopag or romiplostim