Effectiveness and safety of avatrombopag for the treatment of adults with newly diagnosed, persistent, or chronic immune thrombocytopenia: Interim results from the phase 4 ADOPT study

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CONCLUSIONS

- In this interim analysis of real-world data from the ADOPT study, avatrombopag was shown to be effective among adult patients with newly diagnosed, persistent, and chronic ITP treated in clinical practice
- No new safety concerns have been identified to date
- These findings suggest that the benefits of avatrombopag treatment among patients with ITP are similar across disease phases

BACKGROUND

- Avatrombopag is a thrombopoietin receptor agonist (TPO-RA) approved for the treatment of chronic immune thrombocytopenia (ITP) in adults with insufficient response to a previous treatment[1]
- The efficacy and safety of avatrombopag have been established in phase 3 clinical trials[2,3]; however, data on real-world usage are limited
- ADOPT (NCT04943042) is an ongoing phase 4, multicenter, observational study designed to examine real-world outcomes with avatrombopag in clinical practice

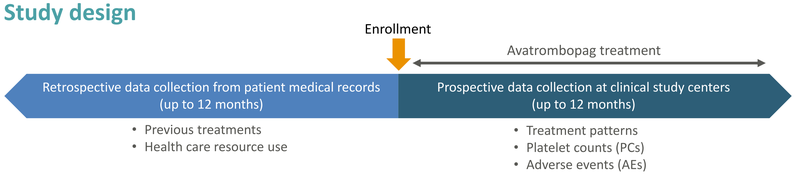
AIMS

- To examine interim efficacy and safety results among patients in ADOPT stratified by ITP disease phase

METHODS

- **Setting:** 60 clinical study centers across 9 European countries
- **Patients:** Adults (aged ≥18 years) with an established ITP diagnosis who were initiating or already being treated with avatrombopag
  - Patients with ITP secondary to other conditions were excluded
- **Statistical analysis:** Outcomes were summarized descriptively and stratified by ITP disease phase, based on the time from ITP diagnosis to first avatrombopag treatment

Study design



Patient subgroups

- **Newly diagnosed:** <3 months from ITP diagnosis
- **Persistent:** 3 to 12 months from ITP diagnosis
- **Chronic:** >12 months from ITP diagnosis

Study endpoints

Primary endpoint: Cumulative weeks with PC ≥30×10^9/L

Key secondary endpoints

- Cumulative weeks with PC ≥50×10^9/L
- PC ≥30×10^9/L for ≥8 consecutive weeks
- PC ≥50×10^9/L for ≥8 consecutive weeks
- Rescue medication use

Safety endpoints

- Serious AEs (SAEs)
- AEs of special interest (AESIs; thromboembolic events, bleeding events of WHO grade ≥3)
- AEs leading to avatrombopag discontinuation

RESULTS

- As of November 12, 2024, 200 patients were enrolled and 51 (25.5%) had completed the study (**Figure 1**)
- More than two-thirds of patients in the persistent and chronic groups and less than one-third in the newly diagnosed group had been previously treated with a TPO-RA (**Figure 2**)
- The median cumulative number of weeks with PC ≥30×10^9/L or PC ≥50×10^9/L among patients with 12 weeks of follow-up was high across groups (**Figure 3**), and 100% of patients with ≥8 weeks of follow-up had ≥8 consecutive weeks with PC ≥50×10^9/L
- Use of rescue therapy among all enrolled patients ranged from 5.3% in the newly diagnosed group to 21.1% in the persistent group (**Figure 4**)
- AEs were comparably prevalent across groups; 3 patients in the chronic group had AEs resulting in avatrombopag discontinuation and 10 patients had AESIs, including embolism, atheroembolism, deep vein thrombosis, thrombosis, pulmonary embolism, and cerebral venous thrombosis (**Table 1**)
- Three deaths were reported, none of which were avatrombopag related (**Table 1**)

Figure 1: Patient disposition as of November 12, 2024

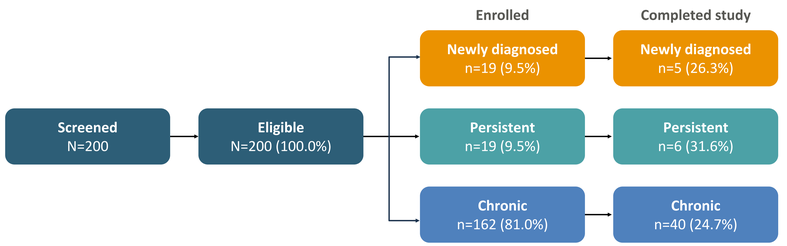
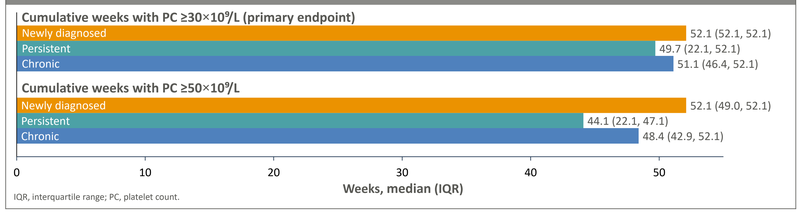


Figure 2: Baseline patient demographic and clinical characteristics

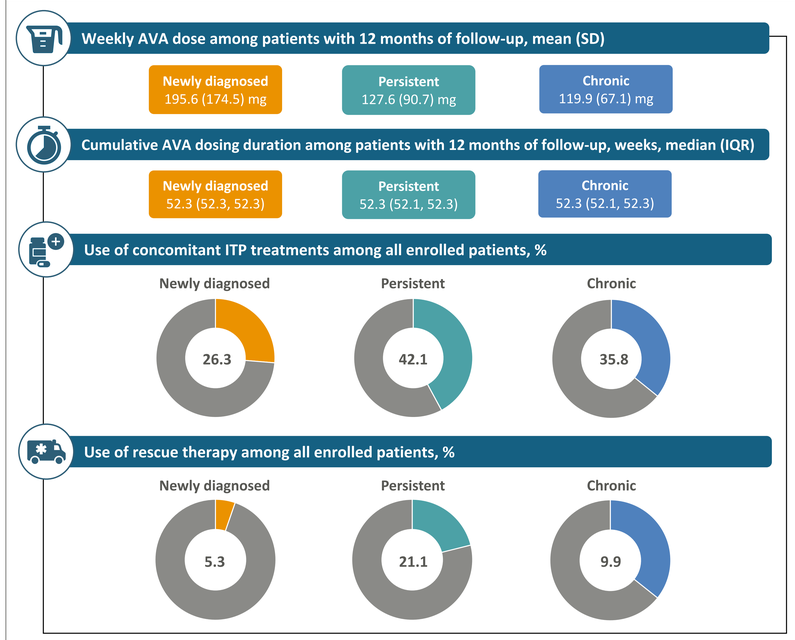
AVA, avatrombopag; IQR, interquartile range; SD, standard deviation; TPO-RA, thrombopoietin receptor agonist.

Figure 3: Interim effectiveness outcomes among patients with 12 months of follow-up



IQR, interquartile range; PC, platelet count.

Figure 4: Interim treatment characteristics from enrollment to 12 months



AVA, avatrombopag; IQR, interquartile range; ITP, immune thrombocytopenia; SD, standard deviation.

Table 1: Interim safety outcomes

	Newly diagnosed (n=19)	Persistent (n=19)	Chronic (n=162)
Patients with AEs, n (%)	3 (15.8)	3 (15.8)	28 (17.3)
Treatment-related AEs	0	0	11 (6.8)
AEs resulting in discontinuation	0	0	3 (1.9)
Patients with SAEs, n (%)	3 (15.8)	3 (15.8)	16 (9.9)
Deaths[a]	2 (10.5)	0	1 (0.6)
Patients with AESIs, n (%)	0	2 (10.5)[b]	8 (4.9)[c]

AE, adverse event; AESI, adverse event of special interest (thromboembolic events, bleeding events of WHO grade ≥3); SAE, serious adverse event.
[a]No deaths were avatrombopag related. [b]Includes 1 report of embolism and 1 uncoded event. [c]Includes 1 report each of atheroembolism, deep vein thrombosis, thrombosis, pulmonary embolism, and cerebral venous thrombosis; 3 patients had uncoded events.

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3. Mei H, et al. *Res Pract Thromb Haemost*. 2023;7:e102158.

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