

A European retrospective study to describe real-world outcomes in patients with primary immune thrombocytopenia treated with avatrombopag: Interim results

Poster 02

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CONCLUSIONS

- This interim analysis demonstrates real-world clinical profile of avatrombopag in patients with primary immune thrombocytopenia in Europe and supports data from clinical trials and other post-marketing evidence.
- 68% of the patients had a complete response by week 12, 71% by week 26, and 81% by week 52 after avatrombopag initiation.
- Platelet counts reached the thresholds of $\geq 50 \times 10^9/L$ and $\geq 100 \times 10^9/L$ in a median of 8 (95% confidence intervals: 7-21) and 21 (9-196) days, respectively, from avatrombopag initiation.
- Of patients who reached to $\geq 50 \times 10^9/L$ threshold, around 60% maintained the response at 2 year in overall population and in TPO-RA subgroups.

INTRODUCTION

- Primary immune thrombocytopenia (ITP) is an autoimmune disorder characterised by isolated thrombocytopenia (peripheral blood platelet count (PC) $< 100 \times 10^9/L$) in the absence of other causes or disorders that may be associated with thrombocytopenia.^{1,2}
- Avatrombopag (AVA), an oral thrombopoietin-receptor agonist (TPO-RA), is a safe and efficacious treatment approved for patients with ITP (2021 by European Medicines Agency).³
- There is limited real-world evidence on AVA's clinical effectiveness in European population treated in routine clinical practice.⁵

OBJECTIVES

- This study aims to report real-world evidence on the clinical outcomes of patients with primary ITP treated with AVA in Europe

METHODS

Study design

- A non-interventional, retrospective, multi-centre chart in six European countries (Belgium, Czech Republic, Germany, Italy, Spain, and United Kingdom)

Study population

- Adult patients with primary ITP treated with AVA

Inclusion criteria:

- Confirmed diagnosis of primary ITP documented in medical records
- Receiving AVA for the treatment of primary ITP within routine clinical practice
- Medical records available for ≥ 12 weeks prior to and from AVA initiation
- Aged ≥ 18 years at the start of first TPO-RA treatment
- Providing consent to access patient's medical records in line with country regulations

Exclusion criteria:

- Participating in any form of interventional study during follow-up

Study definition

- Index date:** Date of initiation of AVA

- Baseline period:** 26 weeks prior to AVA initiation

- Post-index period (follow-up):** Period after the index date until date of death or date of most recent follow-up visit or PC assessment (whichever was earliest)

- Platelet response:** Defined as achieving a meaningful PC threshold (i.e., $\geq 30 \times 10^9/L$ or $\geq 50 \times 10^9/L$) while they were on AVA in the absence of rescue therapy (date first achieved was date of first response)

- Complete platelet response:** Defined as achieving a PC $\geq 100 \times 10^9/L$ (date first achieved was date of first response) in the absence of rescue therapy

Primary endpoints

- Proportion of patients achieving meaningful PC thresholds and complete response by week 12, 26, 52 and at any time while on AVA post-index
- Proportion of patients with a $\geq 50\%$, $\geq 75\%$, and 100% increase in PC from baseline by week 12, 26, and 52 among patients who respond to AVA
- Time from index date to an initial platelet response
- Duration of response

Statistical methods

- Quantitative variables were summarised using mean, standard deviation (SD), medians, and interquartile range (IQR).
- Categorical variables were described with frequencies and percentages. Point estimates for proportions were presented along with associated 95% confidence intervals (CI).
- Time-to-event analyses (based on Kaplan-Meier) were used to estimate the median time to response and duration of response.
- PC counts obtained during rescue therapy was excluded from the analysis.

RESULTS

Patients' baseline characteristics

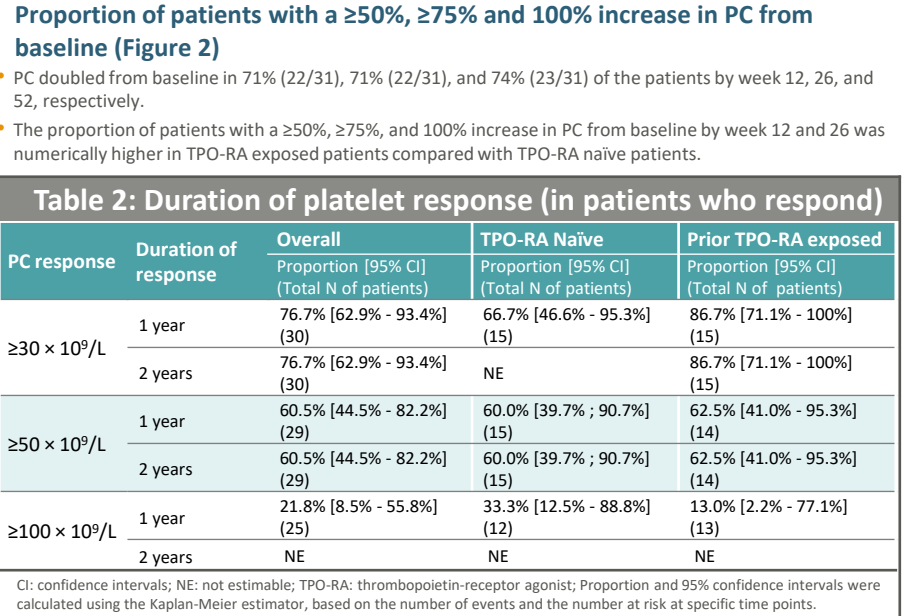
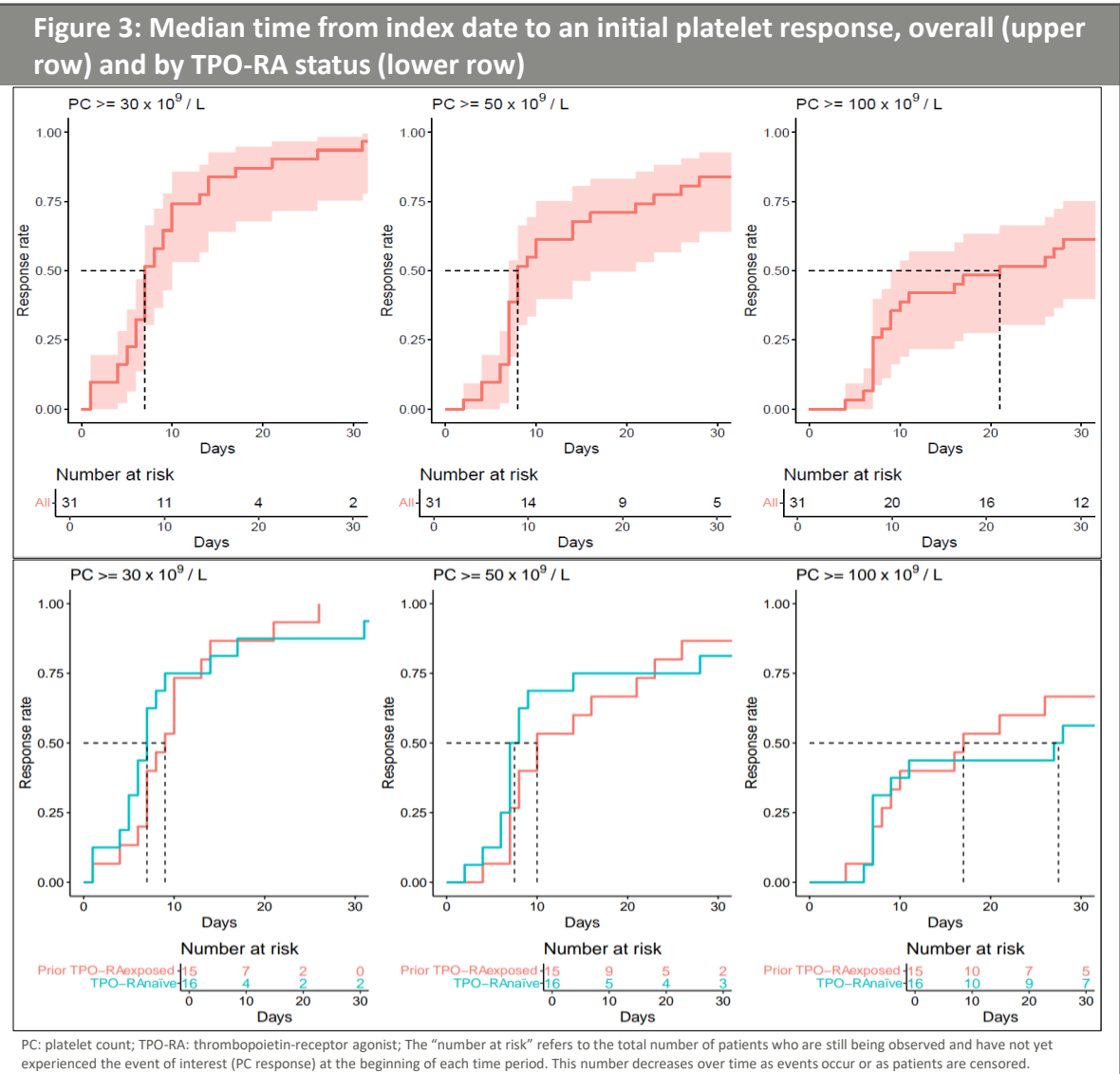
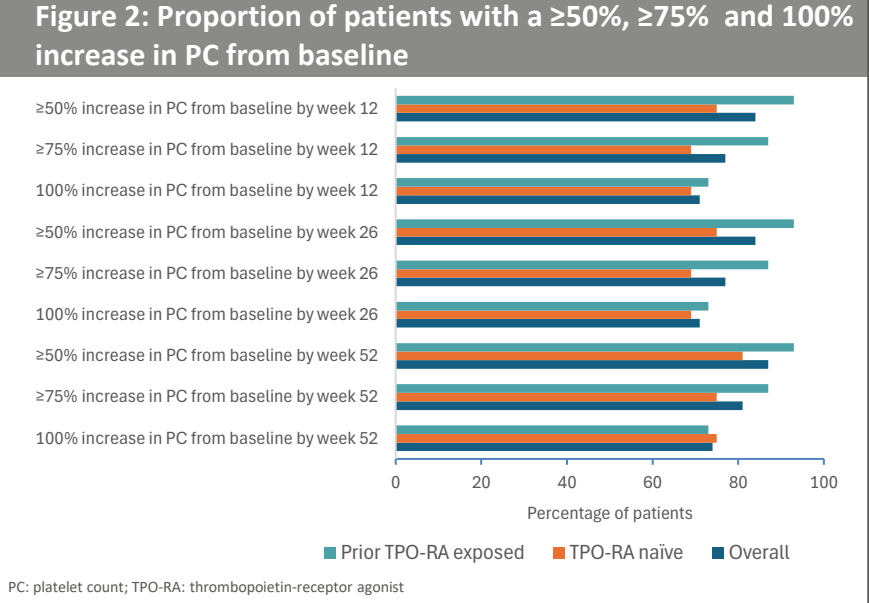
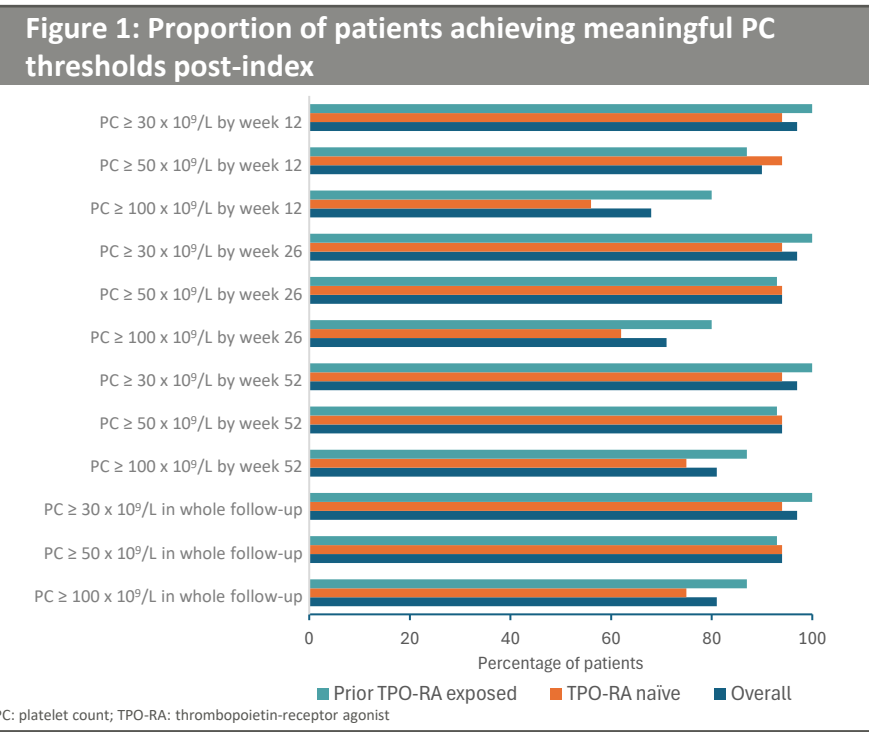
- By July 2024, data relating to 31 patients had been collected: 16 TPO-RA naïve and 15 prior TPO-RA exposed (60% eltrombopag, 13.3% romiplostim, 26.7% both) (**Table 1**).
- Mean (SD) age of patients at data collection was 58.8 (21.1) years and 52% of patients were female.
- Median (IQR) time from ITP diagnosis until AVA initiation was 5 (0.6-12.1) years and patients were followed for a median time of 37 (25.6-53.4) weeks.
- Median baseline PC was $42.5 \times 10^9/L$ which is higher than the threshold for initiating treatment ($< 30 \times 10^9/L$).

Table 1: Patients' baseline characteristics			
	Overall, N=31	TPO-RA Naïve, N=16	Prior TPO-RA exposed, N=15
Age at data collection, years, Mean (SD)	58.8 (21.1)	56.1 (23.6)	61.6 (18.4)
Female sex, N (%)	16 (52%)	8 (50%)	8 (53%)
Median (IQR) baseline platelet count, $\times 10^9/L$	42.5 (21.5-83.5)	53.5 (23.8-99.8)	42 (14-63.5)
History of splenectomy, N (%)	2 (6%)	0	2 (13%)
Number of prior TPO-RA treatments, N (%)			
0	16 (52%)	16 (100%)	NR
1	11 (35%)	NR	11 (73%)
2	4 (13%)	NR	4 (27%)
Number of prior steroid treatments, N (%)			
0	10 (32%)	4 (25%)	6 (40%)
1	18 (58%)	11 (69%)	7 (47%)
2	3 (10%)	1 (6%)	2 (13%)
Time from diagnosis to index, years, Mean (SD)	10.2 (14)	4 (4.7)	17.4 (17.5)
Median (IQR)	5 (0.6-12.1)	1.6 (0.1-7.9)	11.1 (4.5-26.7)
Follow-up, weeks, Mean (SD)	51.1 (38.5)	41.1 (33.7)	61.7 (41.4)
Median (IQR)	37 (25.6-53.4)	32.2 (22.1-44.9)	47.3 (32.4-80.5)

IQR: interquartile range; NR: not relevant; SD: standard deviation; TPO-RA: thrombopoietin-receptor agonist

Proportion of patients achieving meaningful PC thresholds post-index (Figure 1)

- By week 12, 97% (30 out of 31 patients) and 90% (28/31) of the patients achieved PC threshold of $30 \times 10^9/L$ and $50 \times 10^9/L$, respectively.
- By week 26, 97% (30/31) and 94% (29/31) of the patients achieved PC threshold of $30 \times 10^9/L$ and $50 \times 10^9/L$, respectively.
- 68% (21/31) of the patients had a complete response ($\geq 100 \times 10^9/L$) by week 12, 71% (22/31) by week 26, and 81% (25/31) by week 52 post-index.
- 81% (25/31) of the patients (75% [12/16] for TPO-RA naïve patients, 87% [13/15] for TPO-RA exposed patients) had a record of complete response anytime post-index.



Time from index date to an initial platelet response (Figure 3)

- The median (95% CI) time to achieve a PC count of $\geq 50 \times 10^9/L$ and $\geq 100 \times 10^9/L$ was 8 (7-21) days and 21 (9-196) days, respectively, from index date.
- The median time to achieve a complete response was numerically longer in naïve TPO-RA patients compared to those previously exposed to TPO-RA.

Duration of response (Table 2)

- Of patients who reached to $\geq 50 \times 10^9/L$ and $\geq 100 \times 10^9/L$ threshold, 60.5% (95% CI: 44.5%-82.2%) and 21.8% (8.5%-55.8%) maintained the response at 1 year, respectively.
- Of patients who reached to $\geq 50 \times 10^9/L$ threshold, 60.5% (44.5%-82.2%), 60.0% (39.7%-90.7%), and 62.5% (41.0%-95.3%) maintained the response at 2 year in overall population, TPO-RA naïve, and prior TPO-RA exposed group, respectively.

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Disclosures

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