Real-World Outcomes of Avatrombopag Treatment in Primary Immune Thrombocytopenia Stratified by Prior TPO-RA Exposure: Results of the REAL-AVA 2.0 Study

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- Authors SN, ML, and SC are consultants of Sobi, Inc.
- Authors SK, AO, CB, and MV are employees of Sobi, Inc.
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Background

Primary immune thrombocytopenia (pITP) is an autoimmune disorder characterized by low platelet counts (PCs) and heightened risk of serious bleeding episodes.[1]

Thrombopoietin receptor agonists (TPO-RAs) are often used in ITP patients who have not responded adequately to earlier treatments (e.g., corticosteroids or intravenous immunoglobulin [IVIG]).[2]

TPO-RAs* currently approved for the treatment of ITP in adults include eltrombopag (ELT), romiplostim (ROMI), and **avatrombopag (AVA)**.[3-5]

In clinical trials, >90% of AVA-treated patients achieved platelet counts ≥50k/µL. However, **real-world evidence** remains limited.[6]

The **REAL AVA 2.0 study** evaluated patient outcomes following AVA treatment, including among those with and without prior TPO-RA exposure.

*Approval and availability of TPO-RAs varies by country.

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Methods

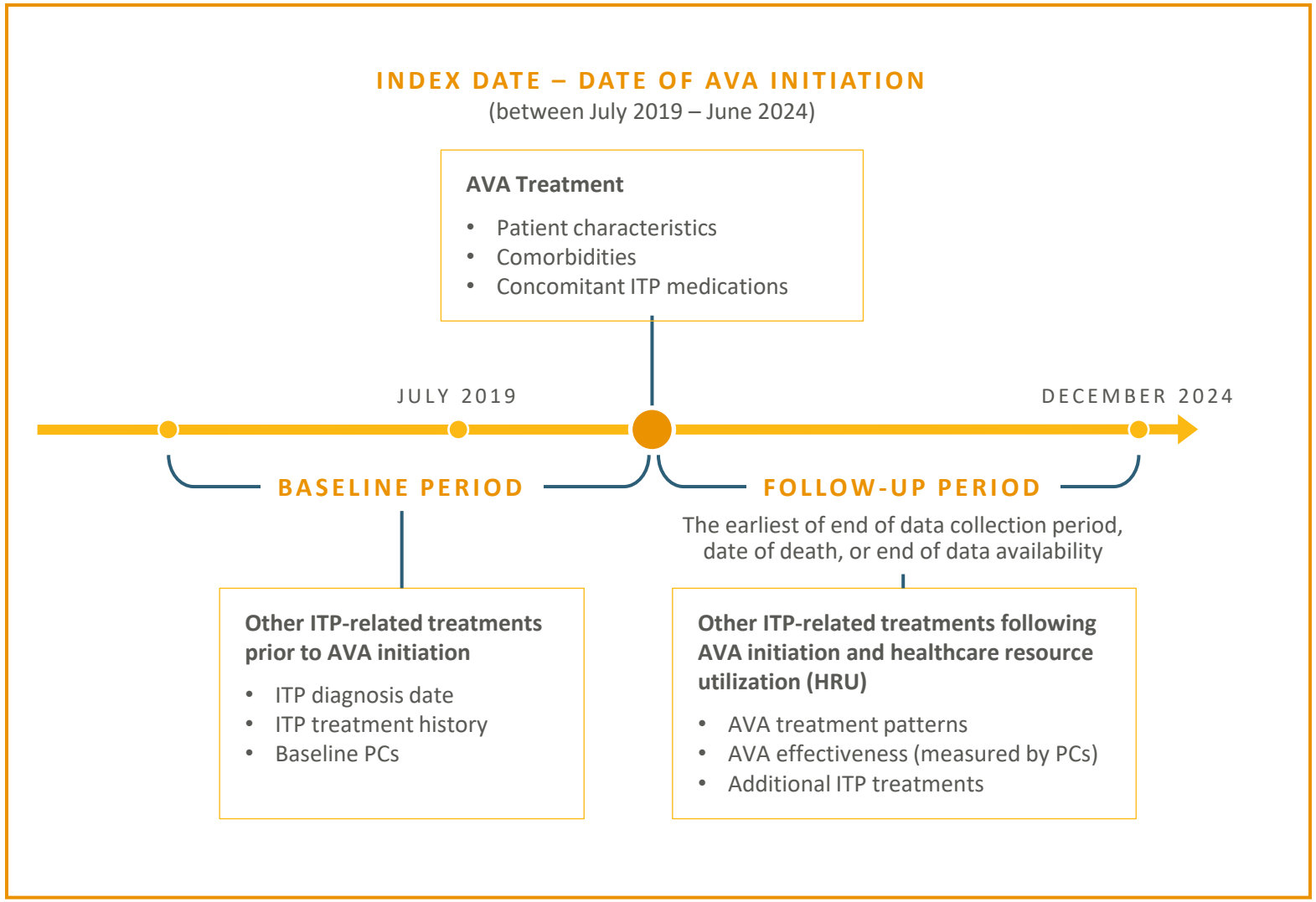
Study design and population

REAL-AVA 2.0: retrospective multi-center chart review of adult patients treated with AVA for primary ITP in the US

Sites included academic medical centers and community-based practices across all geographic regions of the US

Additional study inclusion criteria:

- ≥1 platelet count (PC) observation in the 3 months prior to AVA initiation (baseline period)
- No prior participation in AVA clinical trials
- Complete medical records during baseline and ≥6 months post-index unless the patient died



Methods

Study outcomes and statistical analysis

Response to AVA	Duration of response to AVA	Durability of response	Changes in use of concomitant steroids and immunosuppressants
At least one PC above the given threshold (i.e., ≥30k, ≥50k, and ≥100k/μL) during AVA treatment • PCs observations obtained during or immediately following rescue therapy use* were not included in the response analysis	Total number of days during AVA treatment with PC above each response threshold prior to loss of response, calculated among responders • Loss of response was defined as ≥2 consecutive PC values ≥1 week apart under the response threshold	Proportion of time on AVA with response level above each PC threshold, calculated among responders	Among patients with steroid use on the index date, proportion who reduced steroid dose or discontinued use during AVA treatment Among patients with immunosuppressant use on the index date, proportion who discontinued use during AVA treatment

All outcomes were assessed among the overall population and stratified by TPO-RA exposure status prior to the index date

*PCs were disregarded if they fell within the following time periods after the rescue therapy: Platelet transfusions: 1 week; IVIG or anti-D immunoglobulin: 4 weeks; and immunosuppressants or steroids: 8 weeks

Patient characteristics

Two-third (66%) of patients used a TPO-RA prior to initiating AVA

Nearly two-fifths of patients had acute/persistent ITP at index (38%)

Median ITP disease duration was 2.2 years overall and was longer among patients with prior TPO-RA exposure than without exposure (2.8 vs. 0.4 years)

On average, patients had used 3.1 ITP treatments prior to AVA initiation

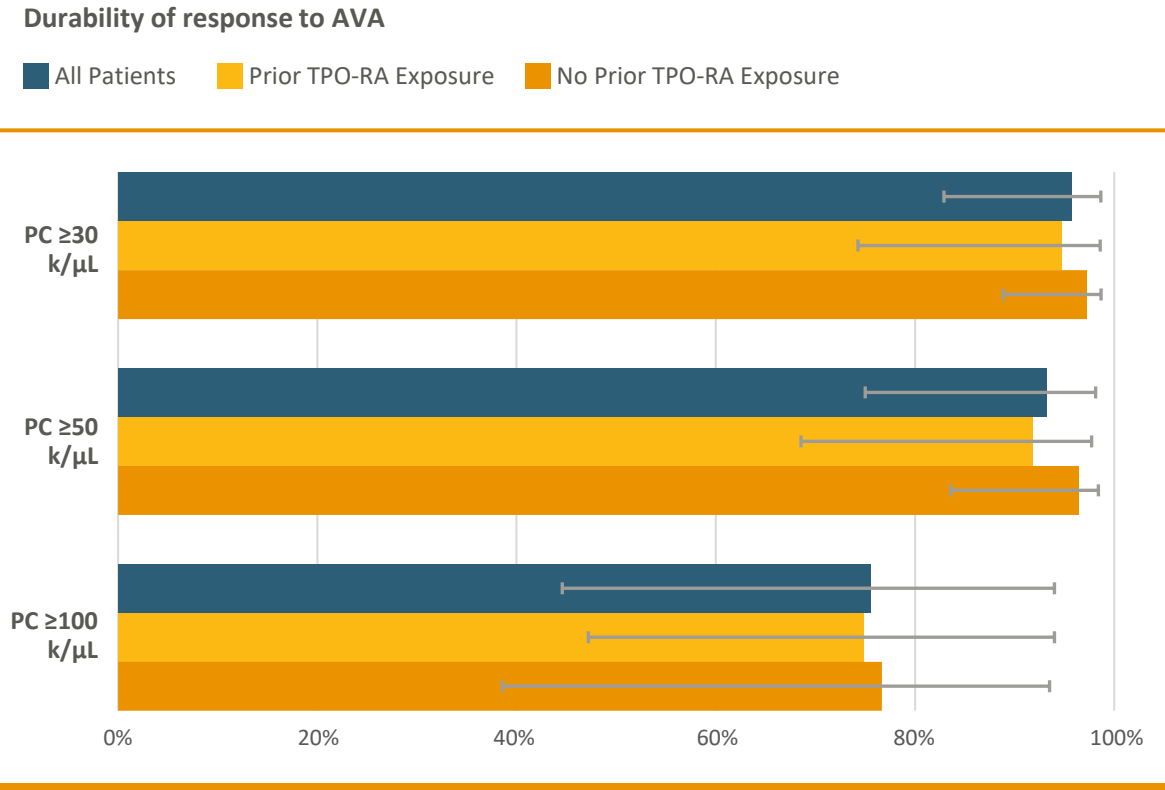
	All Patients N = 177	PRIOR TPO-RA TREATMENT: Prior TPO-RA N = 117	PRIOR TPO-RA TREATMENT: No Prior TPO-RA N = 60	P-value
DEMOGRAPHIC CHARACTERISTICS				
Age at index date, mean ± SD years	56.4 ± 18.9	58.4 ± 19.0	52.4 ± 8.3	< 0.05 *
Female, n (%)	96 (54.2%)	67 (57.3%)	29 (48.3%)	0.332
White, n (%)	132 (74.6%)	93 (79.5%)	39 (65.0%)	0.056
CLINICAL CHARACTERISTICS				
ITP disease duration, median [IQR] years	2.2 [0.3, 6.8]	2.8 [0.8, 8.5]	0.4 [0.2, 2.2]	< 0.001 *
Acute/persistent ITP+, n (%)	68 (38.4%)	30 (25.6%)	38 (63.3%)	< 0.001 *
Baseline PC, median [IQR] k/µL	52.0 [31.0, 89.0]	52.0 [32.5, 107.0]	51.0 [29.5, 74.0]	0.459
PRIOR ITP TREATMENTS				
Prior ITP treatments used, mean ± SD number	3.1 ± 1.8	3.8 ± 1.6	1.7 ± 1.0	< 0.001 *
Any prior use of eltrombopag, n (%)	90 (50.8%)	90 (76.9%)	0 (0.0%)	< 0.001 *
Any prior use of romiplostim, n (%)	66 (37.3%)	66 (56.4%)	0 (0.0%)	< 0.001 *
Rescue therapy use during baseline, n (%)	55 (31.1%)	33 (28.2%)	22 (36.7%)	0.327
Steroid use during baseline, n (%)	86 (48.6%)	44 (37.6%)	42 (70.0%)	< 0.001 *
Steroid use on index, n (%)	43 (24.3%)	24 (20.5%)	19 (31.7%)	0.146
Immunosuppressant use during baseline, n (%)	14 (7.9%)	13 (11.1%)	1 (1.7%)	< 0.05
Immunosuppressant use on index, n (%)	10 (5.6%)	9 (7.7%)	1 (1.7%)	0.168
IVIG use during baseline, n (%)	102 (57.6%)	71 (60.7%)	31 (51.7%)	0.323

*Indicates a statistically significant comparison; +Acute/persistent ITP is defined as an ITP disease duration < 12 months.

Abbreviations: ITP, immune thrombocytopenia; IQR, interquartile range; IVIG, intravenous immunoglobulin; PC, platelet count; TPO-RA, thrombopoietin receptor agonists; SD, standard deviation.

Durability of PC response to AVA

AVA response rates ranged from 76%-90% and durability ranged from 76%-96% in the overall population



Durability of response (%) = $\frac{\text{Time on AVA with PC response}}{\text{Total time on AVA}}$

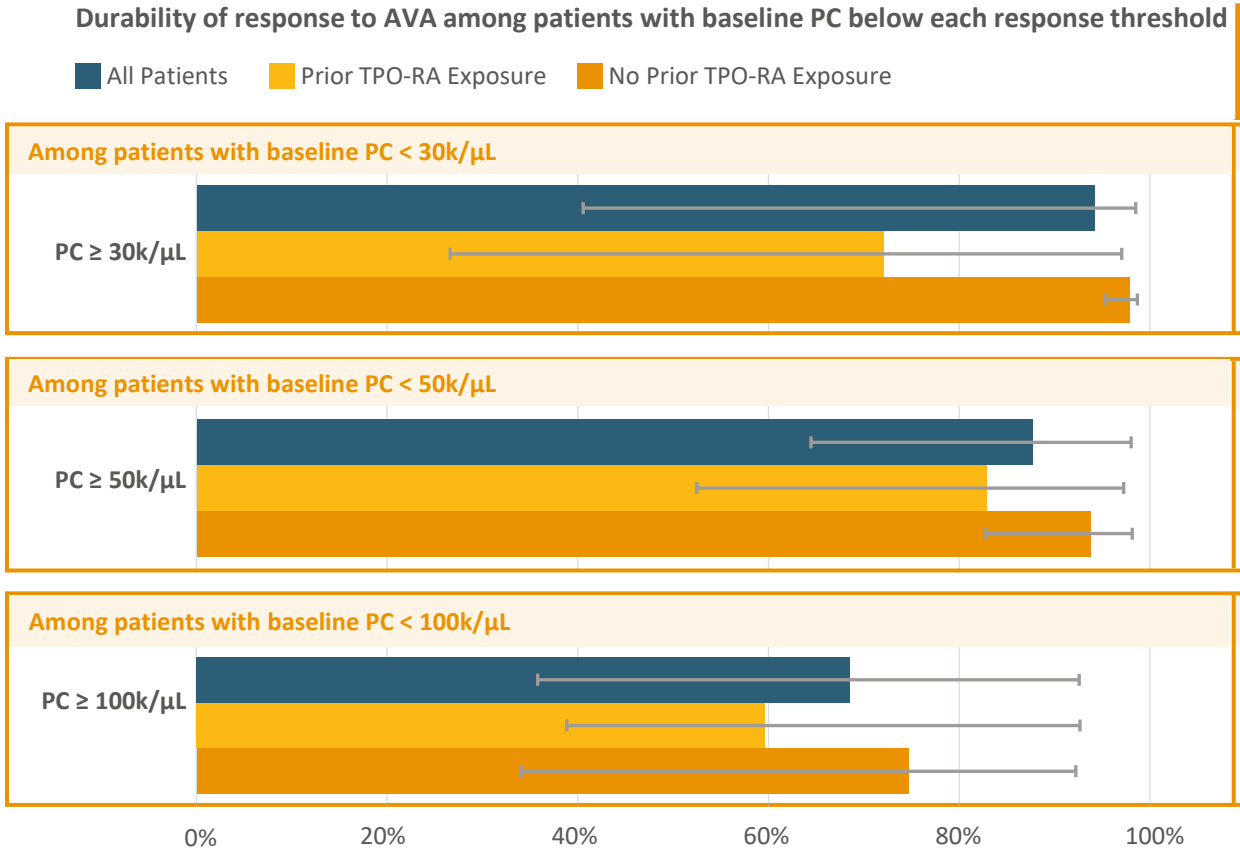
	All patients N = 177	Prior TPO-RA N = 117	No Prior TPO-RA N = 60
Duration of follow-up, median [IQR] months	19.9 [11.0, 34.3]	22.5 [12.8-36.5]	18.1 [9.5-23.3]
Duration of AVA treatment, median [IQR] months	12.8 [5.9, 23.2]	12.9 [5.9-28.4]	12.7 [6.1-22.4]
ACHIEVED OR MAINTAINED PC RESPONSE			
Response at PC ≥ 30k/µL	160 (90.4%)	104 (88.9%)	56 (93.3%)
Duration, months	12.0 (5.2, 22.7)	11.8 (5.1, 23.1)	12.1 (6.0, 22.4)
Durability, median (IQR) % of time	95.7 (82.9, 98.7)	94.8 (74.3, 98.6)	97.3 (88.9, 98.7)
Response at PC ≥ 50k/µL	153 (86.4%)	100 (85.5%)	53 (88.3%)
Duration, median (IQR) months	12.1 (5.2, 22.4)	11.9 (4.8, 22.3)	12.4 (7.0, 22.4)
Durability, median (IQR) % of time	93.2 (75.0, 98.1)	91.8 (68.6, 97.7)	96.5 (83.6, 98.4)
Response at PC ≥ 100k/µL	134 (75.7%)	82 (70.1%)	52 (86.7%)
Duration, median (IQR) months	9.7 (4.3, 18.8)	11.3 (4.1, 19.3)	8.6 (4.7, 17.7)
Durability, median (IQR) % of time	75.5 (44.6, 94.0)	74.9 (47.2, 94.0)	76.7 (38.6, 93.5)

Abbreviations: IQR, interquartile range; PC, platelet count; TPO-RA, thrombopoietin receptor agonists; k/µL, thousand per microliter.

Durability of PC response to AVA among patients with baseline PC below each response threshold

Durability of response remained high when response was assessed among patients with baseline PC below each response threshold

Durability of response to AVA among patients with baseline PC below each response threshold



	All patients	Prior TPO-RA	No Prior TPO-RA
Baseline PC < 30k/µL	**N = 40**	**N = 25**	**N = 15**
Response at PC ≥ 30k/µL	34 (85.0%)	21 (84.0%)	13 (86.7%)
Duration, median (IQR) months	8.3 (3.5, 22.3)	6.1 (1.9, 21.4)	19.0 (6.2, 22.6)
Durability, median (IQR) % of time	94.2 (40.5, 98.5)	72.1 (26.6, 97.1)	97.9 (95.3, 98.7)
Baseline PC < 50k/µL	**N = 84**	**N = 55**	**N = 29**
Response at PC ≥ 50k/µL	67 (79.8%)	43 (78.2%)	24 (82.8%)
Duration, median (IQR) months	15.2 (4.9, 22.6)	10.1 (3.5, 28.9)	18.4 (7.8, 22.5)
Durability, median (IQR) % of time	87.8 (64.4, 98.1)	82.9 (52.5, 97.3)	93.8 (82.8, 98.2)
Baseline PC < 100k/µL	**N = 137**	**N = 86**	**N = 51**
Response at PC ≥ 100k/µL	99 (72.3%)	54 (62.8%)	45 (88.2%)
Duration, median (IQR) months	8.0 (3.4, 18.7)	8.2 (3.5, 19.3)	8.0 (3.4, 17.4)
Durability, median (IQR) % of time	68.6 (35.8, 92.7)	59.7 (38.9, 92.7)	74.7 (34.1, 92.3)

Abbreviations: IQR, interquartile range; PC, platelet count; TPO-RA, thrombopoietin receptor agonists; k/µL, thousand per microliter.

Changes in concomitant medications

Nearly all patients with steroid use at index reduced or discontinued steroid use after AVA initiation

	All Patients (N=177)	Prior TPO-RA (N=117)	No prior TPO-RA (N=60)
Steroid use at index*	N = 43 24,3%	N = 24 20,5%	N = 19 31,7%
Steroid use not reduced or discontinued post-index	2.3% N = 1	4.2% N = 1	0.0% N = 0
Reduction in steroid use	98% reduction	96% reduction	100% reduction

4 out of 10 patients using immunosuppressants at index discontinued their use following AVA initiation.

Prior TPO-RA: **3/9**

No prior TPO-RA: **1/1**

*Index date is defined as the date of AVA initiation.

Limitations

- Patients included in this study were treated at the 11 participating medical centers; findings may not fully represent the broader US ITP population treated with AVA
- Six months of follow-up was required, except in cases when patients died within that time, mitigating immortal time and survivorship bias; however, patients lost to follow-up may differ from those who continued their care at the same medical center for 6 months or longer
- PCs were collected as part of routine medical care and not at standardized time intervals, which may lead to misestimation of response duration and durability
- Patient's medical history, especially prior to being under the participating clinician's care, may not have been well-documented
- There is potential for abstractor errors; this was mitigated by standardized CRF training and thorough data quality checks
- Observational nature of the study limits the ability to infer causal relationships

Conclusions

In this real-world study, the majority of patients who initiated AVA for primary ITP achieved or maintained response at clinically meaningful PC thresholds, irrespective of prior TPO-RA use.

Almost all patients with concomitant steroid use were able to reduce or discontinue their steroid use while treated with AVA.

These results support AVA as an effective treatment option for primary ITP in clinically diverse patient populations.

Acknowledgements

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