Clinically Meaningful Response to Avatrombopag (AVA) for the Treatment of Pediatric Immune Thrombocytopenia (ITP): Results from the Phase 3b Multicenter, Randomized, Double-Blind, Placebo (PBO)-controlled, Parallel-group Trial

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Poster #PF1251

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CONCLUSION

A significant and rapid response to avatrombopag (AVA) was seen in this heavily pretreated pediatric ITP population, regardless of how response was measured.

BACKGROUND

- Current guidelines recommend the use of thrombopoietin receptor agonists (TPO-RAs) for children and adolescents with ITP who do not respond to first-line treatment[1].
- Avatrombopag (AVA), an oral TPO-RA, could be a desirable option for pediatric patients as it does not require an injection in a physician's office, is taken with meals, and does not carry food-type or timing restrictions.
- Top-line results of the phase 3b, multicenter, randomized, double-blind, placebo-controlled, parallel-group trial to evaluate the efficacy and safety of AVA for the treatment of pediatric patients with immune thrombocytopenia were previously reported[2].
  - The primary endpoint of platelet response (≥2 consecutive platelet counts (PC) ≥50×10^9/L without rescue therapy) was met by 81.5% for AVA versus 0% for placebo (p<0.0001) in a population where 55/75 (73.3%) had failed to respond to a previous TPO-RA.
- The aim of the current analyses is to expand the evaluation of platelet response (R) to AVA in pediatric ITP and evaluate the clinically meaningful response (CMR), a platelet response threshold often used in real world pediatric practice.

METHODS

- The phase 3b, multicenter, randomized, double-blind, placebo-controlled, parallel-group trial evaluated the efficacy and safety of AVA for the treatment of pediatric patients with ITP for ≥6 months (NCT04516967) (**Figure 1**).
- These post-hoc analyses evaluate achieving a R [PC ≥50×10^9/L] and CMR [PC ≥30×10^9/L], in the absence of rescue therapy, to better characterize the onset of action and durability of response.

RESULTS

- Overall, 75 patients aged 1 to 17 years were enrolled; 54 were randomized to AVA and 21 to PBO (**Table 1**).

Table 1: Patient Baseline Characteristics

	AVA (N=54)	PBO (N=21)
Female, n (%)	24 (44.4)	12 (57.1)
Age, years (mean ± SD)	8.9 ± 4.4	9.9 ± 4.1
Race, n (%) White Asian	 48 (88.9) 3 (5.6)	 15 (71.4) 1 (4.8)
Platelet count ≤15 × 10^9/L, n (%)	45 (83.3)	17 (81.0)
Platelet count (mean ± SD)	12.0 ± 6.8	11.2 ± 6.6
Bruising or bleeding, n (%)	39 (72.2)	16 (76.2)
WHO bleeding scale for the 7 days prior to baseline, n (%) Grade 1 Grade 2	 36 (66.7) 3 (5.6)	 14 (66.7) 2 (9.5)
Time from primary ITP diagnosis to first dose, weeks (mean ± SD)	202 ± 164	225 ± 181
≥3 previous ITP medications received since diagnosis, n (%)	37 (68.5)	14 (66.7)
Prior TPO-RA use, n (%)	40 (74.1)	15 (71.4)
Prior TPO-RA response, n (%)	17 (42.5)	3 (20.0)
Splenectomy, n (%)	2 (3.7)	2 (9.5)

Figure 1: Phase 3b Study Design

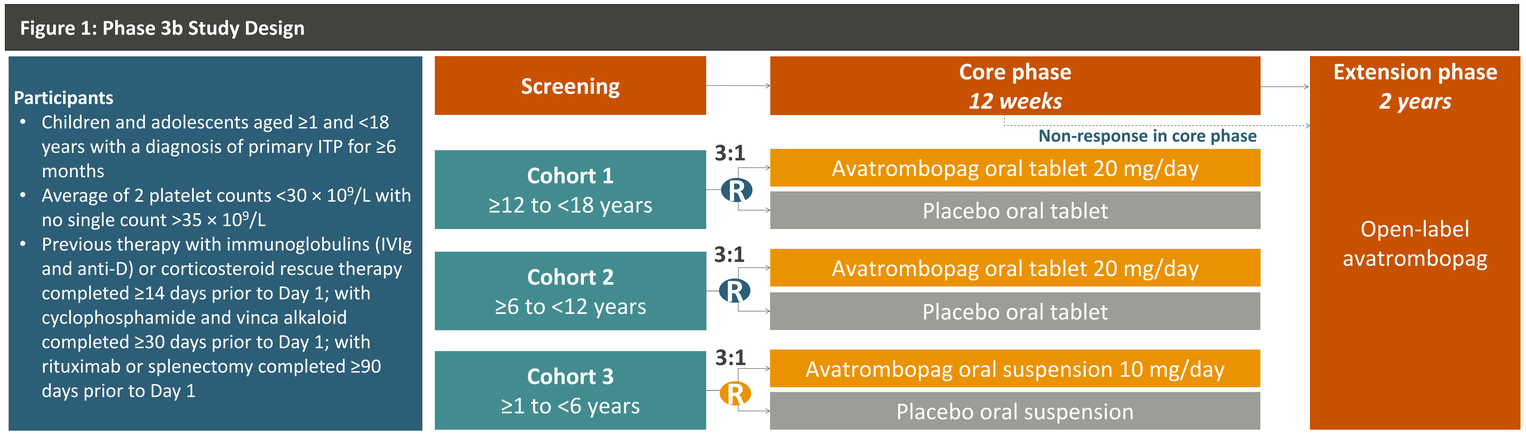


Figure 2: Proportion of Patients Achieving Clinically Meaningful Response (CMR) [≥30 × 10^9/L]

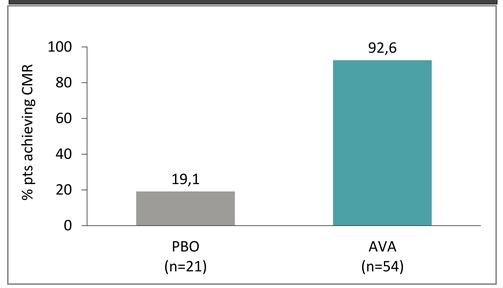


Figure 3: Proportion of Patients Achieving Response (R) [≥50 × 10^9/L]

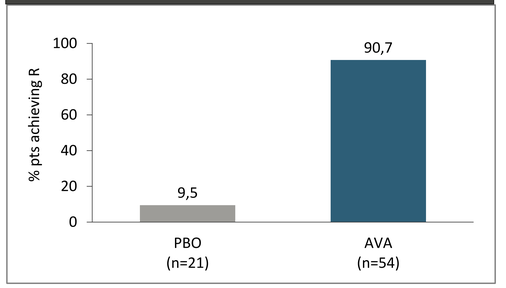


Figure 4: Proportion of Patients Achieving CMR by Number of Consecutive Platelet Measures

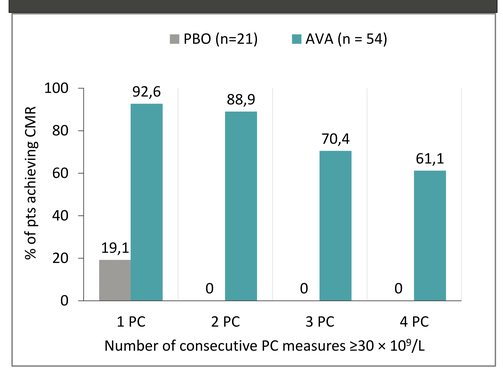


Figure 5: Proportion of Patients Achieving R by Number of Consecutive Platelet Measures

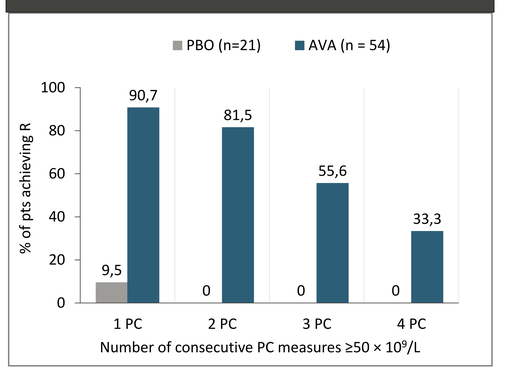
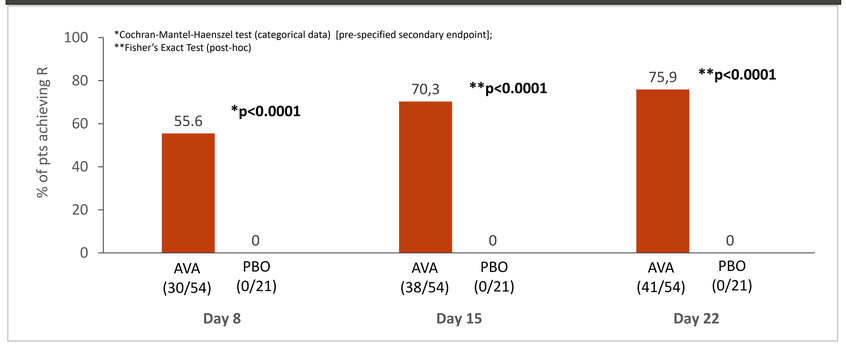


Figure 6: Proportion of Patients Achieving R (≥50 × 10^9/L) by Day 8, Day 15, and Day 22



DISCLOSURES

Study was funded by Sobi, Inc.

REFERENCES

1. Neunert C, et al. *Blood Adv.* 2019;3:3829–66.
2. Grace R, et al. European Hematological Association 2024 Hybrid Congress; Madrid, Spain; June 13–16, 2024