# Analysis of Baseline Influences on Platelet Response to Avatrombopag (AVA) from a Phase 3b Multicenter, Randomized, Double-Blind, Placebo (PBO)-controlled, Parallel-group Trial to Evaluate the Efficacy and Safety of AVA for the Treatment of Pediatric Patients with Immune Thrombocytopenia (ITP)

Rachael F. Grace[1], Göksel Leblebisatan[2], Yesim Aydinok[3], Şule Ünal[4], John Grainger[5], Amanda Grimes[6], Michele Lambert[7], Jessica Zhang[8], Brian Jamieson[8], Michael Vredenburg[8], Scott Kolodny[8]

[1]Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School, Boston, USA, [2]Department of Pediatric Hematology, Çukurova University Medical Faculty, Adana, Turkey, [3]Department of Pediatric Hematology and Oncology, Ege University School of Medicine, Izmir, Turkey, [4]Hacettepe University Faculty of Medicine, Department of Pediatric Hematology, Ankara, Turkey, [5]Department of Haematology, Royal Manchester Children's Hospital, Manchester, United Kingdom, [6]Texas Children's Hospital, Baylor College of Medicine, Houston, USA, [7]Children's Hospital of Philadelphia, Philadelphia, USA [8]Sobi Inc., Morrisville, USA

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## CONCLUSIONS

**A platelet response to AVA was noted across patients with a variety of baseline characteristics. High response rates were noted despite 77% of patients receiving multiple TPO-RAs prior to study entry and 73% with no response to prior TPO-RAs.**

## BACKGROUND

- After failure of first-line therapies (e.g. corticosteroids or immunoglobulin) in pediatric immune thrombocytopenia (ITP), treatment options for children include immunosuppressants and thrombopoietin receptor agonists (TPO-RAs).
- The oral TPO-RA AVA could be a desirable option for pediatric patients as AVA 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[1].
  - The primary endpoint of platelet response (≥2 consecutive platelet counts ≥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 thrombopoietin receptor agonist (TPO-RA).
- The aim of this analysis was to evaluate the correlation of baseline characteristics with platelet response to AVA in pediatric ITP.

## 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 the proportion of patients randomized to AVA with a platelet response based on baseline characteristics [sex, presence of WHO-defined bleeding (Grades ≥1), ITP duration, number of prior ITP treatments, prior treatment with TPO-RA, type of prior TPO-RA treatment, response to prior TPO-RA, low weight for age (Low Weight: <55kg in Cohort 1, <33kg in Cohort 2, <18kg in Cohort 3)].

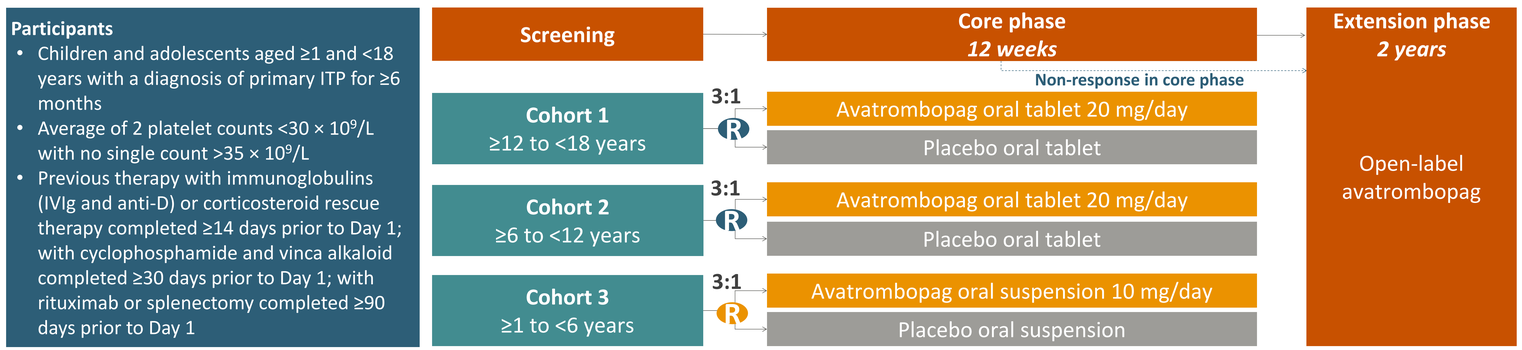
## RESULTS

- 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 (%)<br>White<br>Asian | <br>48 (88.9)<br>3 (5.6) | <br>15 (71.4)<br>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 (%)<br>Grade 1<br>Grade 2 | <br>36 (66.7)<br>3 (5.6) | <br>14 (66.7)<br>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) |

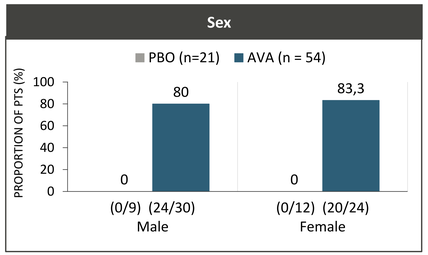
**Figure 1: Phase 3b Study Design**

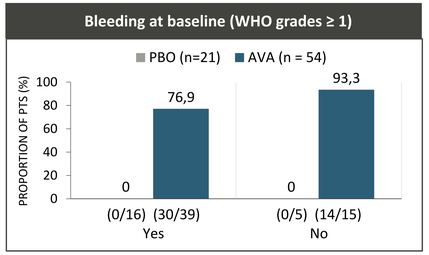


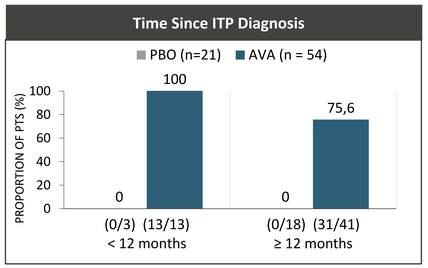

**Baseline Characteristic Correlates of Platelet Response* to AVA**

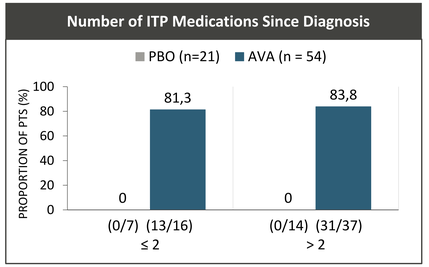
*achieving at least 2 consecutive PC ≥ 50×$10^9$/L without rescue therapy during the 12-week core phase

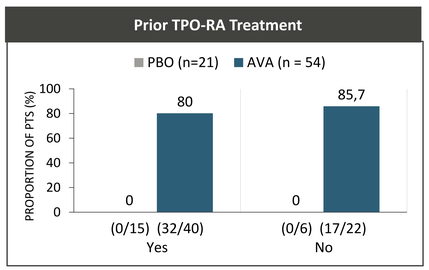
- The baseline characteristics correlating with platelet response to AVA include female sex, no bleeding at enrollment, duration of ITP <12 months, no prior TPO-RA treatment, a response to prior TPO-RA treatment, and low baseline weight

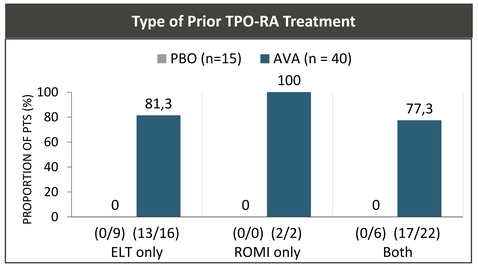


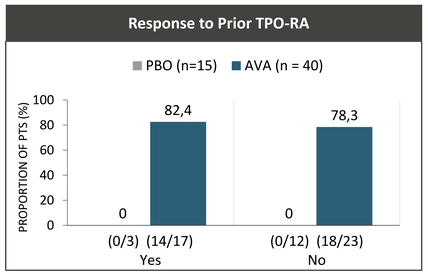



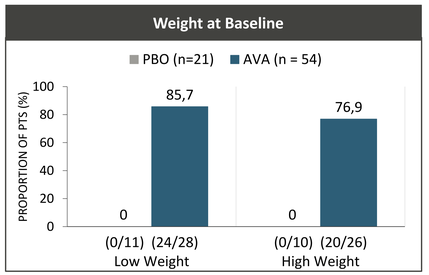




ELT: eltrombopag; ROMI: romiplostim




## REFERENCES

1. Grace R, et al. European Hematological Association 2024 Hybrid Congress; Madrid, Spain; June 13–16, 2024

## DISCLOSURES

Study was funded by Sobi, Inc.