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Effect of Ianalumab Plus Eltrombopag on Patient-Reported Outcomes in Primary Immune Thrombocytopenia: Results From the VAYHIT2 Phase 3 Trial

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Key Findings and Conclusions

- Improvements in ITP patient–relevant PROs across the Symptoms, Fatigue, Bother, and Activity Scales were observed in all groups, with particularly consistent improvements observed in the ianalumab 9 mg/kg group
- Improvements in ITP–specific PROs were maintained after eltrombopag tapering, suggesting sustained benefit
- When the relationship between changes in platelet count and ITP–specific PROs was evaluated across all treatment groups, a weak positive association was observed, most notably for the Symptoms Scale
- Fatigue improvements were weakly associated with increased platelet count, suggesting platelet response alone may not fully explain improvement in patient-reported fatigue

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Introduction

- Immune thrombocytopenia (ITP) is an acquired disease characterized by a low platelet count (<100 × 10^9/L) and increased bleeding risk[1]
- ITP considerably affects patients' health-related quality of life (HRQoL). In the ITP World Impact Survey 2.0, challenges commonly reported by patients included daily disruption (34%) and decreased sleep quality (57%)[2,3]
- Ianalumab plus eltrombopag demonstrated significantly prolonged time to treatment failure and improved stable response rates at 6 months vs placebo plus eltrombopag in patients with primary ITP previously treated with corticosteroids in VAYHIT2 (NCT05653219).[4] In addition, improvement in fatigue was reported using the Patient-Reported Outcomes Measurement Information System Short Form version 1.0 Fatigue 13a (PROMIS-Fatigue) scale[4]
- Here we describe the effect of ianalumab plus eltrombopag on HRQoL with ~1-year follow-up using the ITP Patient Assessment Questionnaire (ITP-PAQ), which is a validated, disease-specific instrument. We also describe potential associations between ITP-PAQ and PROMIS-Fatigue scores with platelet counts

Methods

- Full study design was published previously[4]
- Patients were randomized (1:1:1) to receive ianalumab 9 or 3 mg/kg or placebo as 4 monthly intravenous infusions, plus daily eltrombopag for 16 weeks. Eligible patients then initiated tapering of eltrombopag (from day 1 of week 17) until discontinuation by day 1 of week 25[4]
- PROs were collected at the following timepoints, regardless of whether patients had experienced treatment failure, received new ITP therapies or continued eltrombopag:
  - Screening/baseline (day 1 of week 1)
  - On-treatment period (day 1 of weeks 5, 9, 13, and 15)
  - Eltrombopag tapering to discontinuation period (day 1 of weeks 17, 21, and 25)
  - End of treatment
  - Post-treatment follow-up (day 1 of week 33 and every 12 weeks thereafter*)
- Patient-reported outcomes (PROs) were secondary end points in the VAYHIT2 study and were assessed using multiple measures including the ITP-PAQ and the PROMIS-Fatigue Scale. Scores were summarized descriptively
  - ITP-PAQ is a disease-specific, 44-item,10-scale instrument for HRQoL. Each scale is scored from 0 to 100, with higher scores indicating better HRQoL. Scores were analyzed using repeated linear mixed models, with least-square mean changes from baseline calculated for each treatment group
  - The PROMIS-Fatigue scale assesses the experience and impact of fatigue over the past 7 days. Items are rated on a 5-point Likert scale, then totaled and converted to a standardized T score; higher T scores indicate greater fatigue[4]
- This analysis included patients with an evaluable ITP-PAQ score at baseline and ≥1 postbaseline visits, and it focused on the ITP-PAQ Scales of Symptoms, Fatigue, Bother, and Activity
- Post hoc scatter plots with linear regression lines were used to explore associations between platelet count and PRO end points

*Patients who tapered off eltrombopag and maintained a platelet count of ≥30 × 10^9 /L without rescue/new ITP therapy entered the safety and efficacy follow-up; PROs were collected at weeks 33, 45, and every 12 weeks thereafter until week 141, and at relapse or end of study. Patients who met the criteria for treatment failure entered the safety follow-up only; PROs were collected at week 33, every 12 weeks between weeks 45 and 117, and at the end of study.

RESULTS

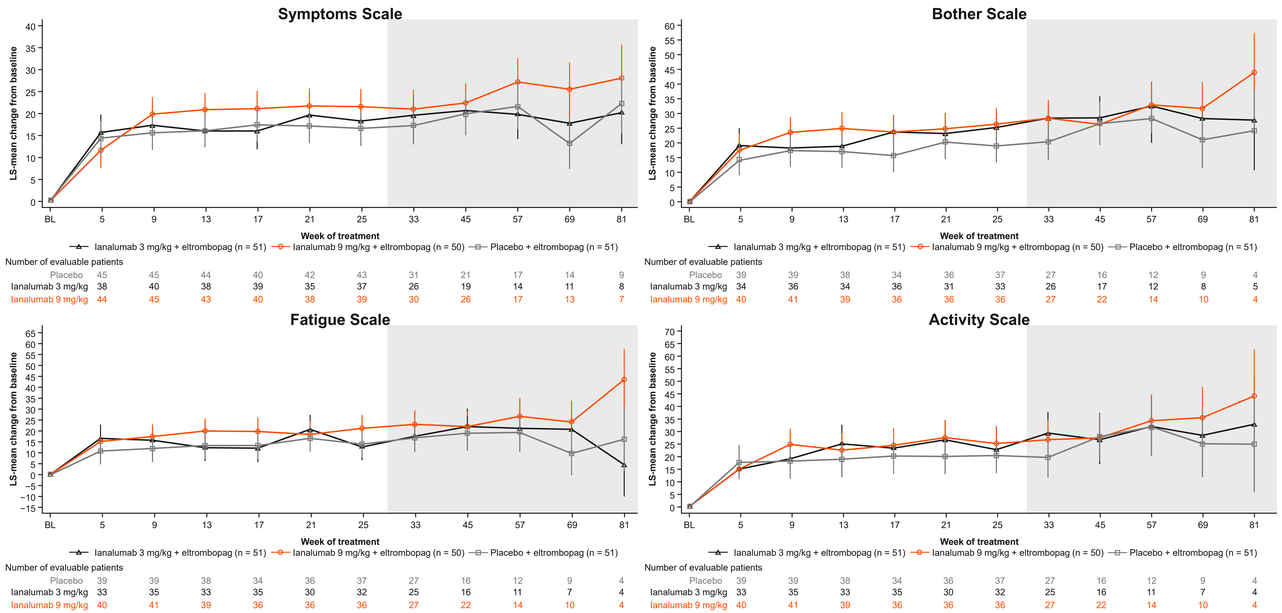
- At data cutoff (June 20, 2025), the median duration of follow-up was 12.9, 13.6, and 11.6 months with ianalumab 9 mg/kg plus eltrombopag (hereafter referred to as ianalumab 9 mg/kg), ianalumab 3 mg/kg plus eltrombopag (hereafter referred to as ianalumab 3 mg/kg), and placebo plus eltrombopag (hereafter referred to placebo), respectively
- Baseline scores for ITP-PAQ Symptoms, Bother, and Activity Scales were comparable across all 3 treatment groups **(Table 1)**
- Across the ITP-PAQ Scales of Symptoms, Fatigue, Bother, and Activity, improvements were observed in all treatment groups beginning early and extending through eltrombopag tapering (day 1 of week 17) and discontinuation (day 1 of week 25), up to week 81; the greatest and most consistent improvements were generally observed with ianalumab 9 mg/kg at key timepoints (**Figure 1**)
- The extended follow-up for VAYHIT2 is ongoing

Table 1. Baseline Measures for ITP-PAQ Domains

Parameter	Ianalumab 9 mg/kg + eltrombopag N = 50	Ianalumab 3 mg/kg + eltrombopag N = 51	Placebo + eltrombopag N = 51
Symptoms Scale			
n/M (%)	46/50 (92.0)	43/51 (84.3)	46/51 (90.2)
Median (IQR)	64.6 (45.8-75.0)	66.7 (54.2-83.3)	70.8 (58.3-79.2)
Fatigue Scale			
n/M (%)	42/50 (84.0)	38/51 (74.5)	40/51 (78.4)
Median (IQR)	56.3 (37.5-75.0)	57.3 (43.8-68.8)	62.5 (46.9-78.1)
Bother Scale			
n/M (%)	42/50 (84.0)	39/51 (76.5)	40/51 (78.4)
Median (IQR)	61.1 (41.7-80.6)	58.3 (41.7-77.8)	68.1 (45.8-80.6)
Activity Scale			
n/M (%)	42/50 (84.0)	38/51 (74.5)	40/51 (78.4)
Median (IQR)	56.3 (25.0-100.0)	56.3 (25.0-87.5)	50.0 (25.0-75.0)

IQR, interquartile range; ITP-PAQ, Immune Thrombocytopenic Purpura Patient Assessment Questionnaire; n, number of patients who completed questionnaire; M, number of patients still on study.

Figure 1. Changes From Baseline for ITP-PAQ Scales of Symptoms, Fatigue, Bother, and Activity



BL, baseline; ITP-PAQ, Immune Thrombocytopenic Purpura Patient Assessment Questionnaire; LS, least-square.
The symbols at each timepoint indicate LS means. The vertical lines indicate 95% CIs. Changes over time were estimated using a mixed-effect repeated measurement analysis of changes from baseline. The gray-shaded areas indicate the time period during which more than 40% of patients in the full analysis set did not have both an evaluable baseline score and an evaluable postbaseline score, as ongoing patients have not yet reached these timepoints.

Figure 2. Association Between Changes From Baseline in Platelet Count and ITP-PAQ Scales at Week 25

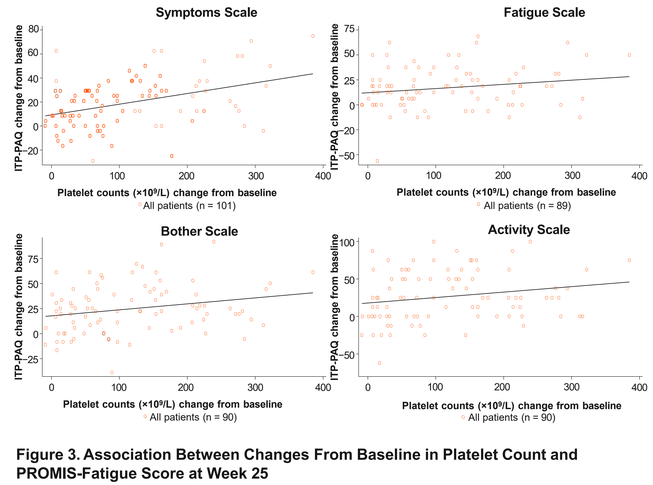
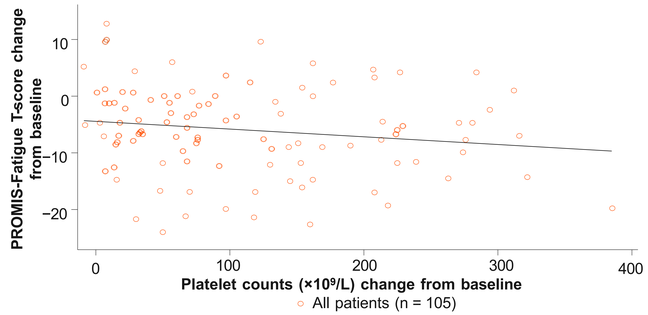


Figure 3. Association Between Changes From Baseline in Platelet Count and PROMIS-Fatigue Score at Week 25



- In a scatter plot analysis of all treatment groups, greater increases in platelet count were weakly associated with improvements in ITP-PAQ Symptoms, Fatigue, Bother, and Activity Scales at week 25; the association appeared most apparent for the Symptoms Scale (**Figure 2**)
- Consistent with the primary analysis results showing early improvement in PROMIS-Fatigue scores across all treatment arms,[3] a weak inverse relationship between platelet count increase and PROMIS-Fatigue T-score reduction was observed (**Figure 3**); however, the weak association suggests that platelet response was not consistently associated with subjective fatigue improvement at week 25

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