Abstract: P1611

Title: EFFICACY AND SAFETY OF AVATROMBOPAG FOR THE TREATMENT OF CHRONIC IMMUNE THROMBOCYTOPENIA IN A CHINESE ADULT POPULATION: A MULTICENTER, RANDOMIZED PHASE III TRIAL

Abstract Type: Poster Presentation

Session Title: Platelet disorders

Background:

Immune thrombocytopenia (ITP) is an autoimmune disorder resulting in decreased platelet counts and increased bleeding risk. Avatrombopag, a second-generation thrombopoietin-receptor agonist, has been approved for the treatment of chronic ITP in the US and Europe.

Aims:

This study aimed to demonstrate the efficacy and safety of avatrombopag versus placebo in Chinese adults with chronic ITP.

Methods:

This multicenter, randomized (2:1), double-blind, parallel-group Phase 3 study enrolled Chinese adults with ITP of ≥12 months and a platelet count $<30\times10^9$/L to once-daily oral avatrombopag (initial dose 20 mg) or matching placebo. After a 6-week double-blind core treatment phase, eligible subjects entered the open-label extension phase and received avatrombopag treatment for up to 30 weeks. The primary endpoint was the proportion of responders with a platelet count of ≥ 50 × 10^9/L at week 6 of core treatment phase in absence of rescue treatment. Subjects with missing platelet count measurement at Week 6 or treated with rescue treatment before Week 6 were considered as non-responders. All randomized subjects were included in the primary endpoint analysis.

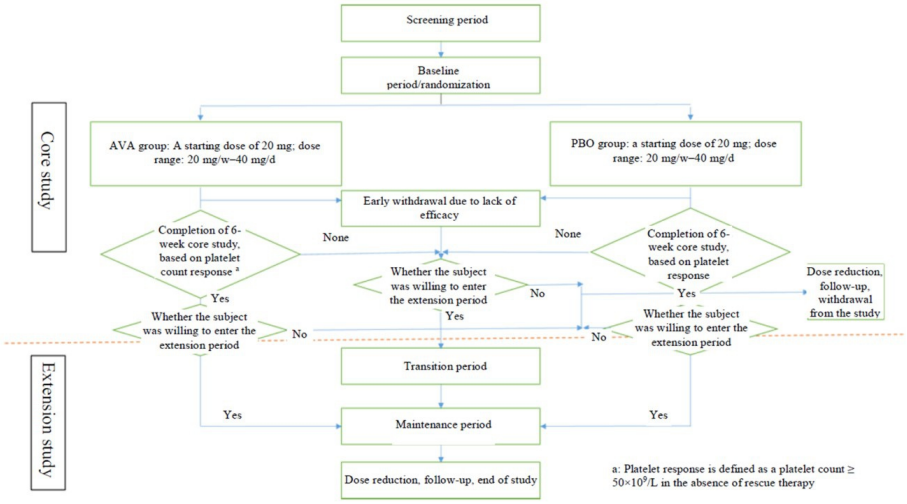
Results:

A total of 74 subjects were randomized into the study, 48 into the avatrombopag group and 26 into the placebo group. At Week 6, 77.08% (95% CI:62.69%, 87.97%) of subjects treated with avatrombopag achieved a platelet count of ≥ 50 × 10^9/L without receiving rescue treatment, vs 7.69% (95% CI:0.95%, 25.13%) in the placebo group, with a treatment difference of 69.39% (95% CI:56.15%, 86.26%, $p<0.0001$). The primary efficacy endpoint was met with statistically significant results favoring avatrombopag compared with placebo. In the core treatment phase, the proportion of responders at Day 8 in the avatrombopag group was 72.92% (95%CI: 58.15%, 84.72%), compared with 3.85% (95%CI: 0.10%, 19.64%) in the placebo group. In the avatrombopag group, the median cumulative number of weeks of platelet response was numerically higher than placebo (4.1 vs 0 weeks, respectively). Across the core and extension phases (whole study), the median cumulative number of weeks of platelet response in avatrombopag-treated subjects was 17.6 weeks. In the core treatment phase, avatrombopag-treated subjects had a lower incidence of ITP-related bleeding symptoms than placebo-treated subjects (70.83% vs. 88.46%). Treatment-emergent adverse events (TEAEs) were reported in 85.4% and 76.9% subjects treated with avatrombopag and placebo, respectively; exposure adjusted incidence of TEAEs was similar between two groups; no treatment-related SAEs were reported. In the whole study, the exposure-adjusted incidence of TEAEs in the avatrombopag group was consistent with that of the core treatment phase; 98.6% and 19.4% of subjects treated with avatrombopag reported TEAEs and SAEs, respectively.

Summary/Conclusion:

Avatrombopag was well-tolerated and efficacious for treatment of adult Chinese ITP patients.

Figure 1. Study Design



Keywords: Clinical outcome, Phase III, Chronic ITP