

## Efficacy and Safety of IQYMUNE® , a Ten Percent Intravenous Immunoglobulin in Adult Patients With Chronic, Primary Immune Thrombocytopenia

Francesco Rodeghiero, Dariusz Woszczyk, Borhane Slama, Anait Melikyan, Jean-Francois Viillard, Rabye Ouaja, Ousmane Alfa Cisse, Alain Sadoun, Abdulgabar Salama

### Abstract

**Background:** Intravenous immunoglobulin (IVIg) IQYMUNE® is a highly purified 10% IVIg that was assessed using the new stringent definition of response described in the revised guideline on the clinical investigation of IVIg. The efficacy and the safety of IQYMUNE® were investigated in adult patients with chronic primary immune thrombocytopenia (ITP).

**Methods:** In this phase III multinational, multicentre, prospective, uncontrolled, open-label, single-arm study, adult patients with a baseline platelet count  $< 30 \times 10^9/L$  were treated with IVIg 10% at a dose of 2 g/kg body weight administered over 2 consecutive days. The primary endpoint was Response over the study period and was defined according to the recent and most stringent European Medicines Agency guidelines (platelet count  $\geq 30 \times 10^9/L$  and a  $\geq 2$ -fold increase from baseline, no new bleeding, and no concomitant treatment with drugs that affect platelet count and/or induce bleeding cessation).

**Results:** Thirty-eight patients were enrolled; 73 infusions were administered (38 on Day 1 and 35 on Day 2). Response was reached by 24 patients corresponding to 63.2% of patients in the full analysis set (95% CI: 46.0; 78.2) and 68.6% of patients in the per-protocol set (95% CI: 50.7; 83.1). The median time to Response was 1 day. The median duration of Response was 13.5 days. Reasons for non-response were failure to reach the required platelet count ( $n = 12$ ), a new bleeding event ( $n = 1$ ), and forbidden medication use ( $n = 1$ ). Among the 23 patients with a baseline platelet count  $\leq 20 \times 10^9/L$ , 19 patients (82.6%) achieved a platelet count  $\geq 50 \times 10^9/L$  at least once before Day 5 (previous European Medicines Agency definition of response). Treatment was well tolerated even in patients with a high flow rate ( $\geq 6$  mL/kg/h in 40% of patients). Headache (34.2%), pyrexia (15.8%), and creatinine renal clearance decrease, including one case of decrease in glomerular filtration rate (10.5%) were the most frequently reported drug-related adverse events.

**Conclusions:** Administration of IQYMUNE® for 2 consecutive days at a dose of 2 g/kg was safe and efficacious. These results support the treatment of adult patients with chronic ITP with IQYMUNE®.

---

Link all'articolo: <https://pubmed.ncbi.nlm.nih.gov/32300420/>