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

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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-armed study, adult patients with a baseline platelet count < 30 × 10⁹/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 ≥ 30 × 10⁹/L and a ≥ 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 ≤ 20 × 10⁹/L, 19 patients (82.6%) achieved a platelet count ≥ 50 × 10⁹/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 (≥ 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/