EFFICACY AND SAFETY OF PRIVIGEN® IN PATIENTS WITH CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY: THE PRIMA TRIAL

Presenter: Patty Riley (CLS Behring)

Jean Marc Leger[1], Jan De Bleeker[2], Claudia Sommer[3], Wim Robberecht[4], Mika Saarela[5], Jerzy Kamienowski[6], Zbigniew Stelmasiak[7], Othmar Zenker[8], Orell Mielke[8], Artur Bauhofer[8], Björn Tackenberg[9], Ingemar S. J. Merkies[10]

[1] Reference Center for Rare Neuromuscular Diseases, Hôpital Pitié-Salpêtrière and University Paris VI, Paris, France; [2] AZ St-Lucas, Gent, Belgium; [3] Universitätsklinikum Würzburg, Würzburg, Germany; [4] UZ Leuven, Leuven, Belgium; [5] Department of Neurology, Helsinki University Central Hospital, Helsinki, Finland; [6] Dolnośląski Szpital Specjalistyczny, Wroclaw, Poland; [7] Samodzielny Publiczny Szpital Kliniczny, Lublin, Poland; [8] CSL Behring GmbH, Marburg, Germany; [9] Department of Neurology, Philipps University, Marburg, Germany; [10] Spaarne Hospital, Hoofddorp and Maastricht University Medical Centre, Maastricht, The Netherlands

Abstract

The Privigen Impact on Mobility and Autonomy (PRIMA) trial was a multicenter, open-label, single-arm study, assessing the efficacy and safety of intravenous immunoglobulin (IVIG) (Privigen, CSL Behring) in IVIG-untreated and IVIG-pre-treated patients with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP). Eligibility of IVIG-pre-treated patients was determined by the degree of disease deterioration in a wash-out period (up to 10 weeks) in which IVIG treatment was withdrawn; patients with an increase of ≥1 adjusted Inflammatory Neuropathy Cause and Treatment scale (INCAT) disability score point were enrolled in the study. Patients received an induction dose of 2 g/kg body weight (bw) administered over 2–5 days, followed by seven infusions of 1 g/kg bw at 3-week intervals (treatment duration 22 weeks). Final assessments were performed 3 weeks after the last dose. Response was defined as a decrease in adjusted INCAT disability score of ≥1 point (clinically meaningful improvement). The primary efficacy endpoint was the response rate (i.e., percentage of responders). The predefined threshold for success was a lower limit of the 95% confidence interval (CI) of response rate higher than 35%. Of the 31 patients screened, 28 were enrolled in the study (15 IVIG-untreated; 13 IVIG-pretreated) and comprised the intention-to-treat (ITT) population. Seventeen patients in the ITT population were responders; the response rate was 61%, 95% CI 42.4–76.4. Therefore, the primary endpoint of the study was achieved. The response rates were 47% and 77% among IVIG-untreated and IVIG-pre-treated patients, respectively. The mean INCAT score improved from 3.7 at baseline to 2.3 at completion. Four patients experienced one serious adverse event (SAE) each, of which two cases of hemolysis were considered at least possibly related to study medication. Both patients presented clinical and laboratory signs of hemolysis, but recovered completely without requiring blood transfusion or other treatment. Hemolysis has been reported as a rare adverse effect of IVIG; risk factors include high cumulative dose of IVIG, non-O blood type, and underlying inflammatory state. The other SAEs (mild diverticulitis; moderate CIDP deterioration) were considered not related to study medication. In conclusion, Privigen treatment resulted in a clinically meaningful improvement in patients with CIDP.

Potential conflicting interests and financial disclosure:

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