INTRODUCTION

The phase 3 BELIEVE trial is evaluating the efficacy and safety of luspatercept in adults with β-thalassemia or hemoglobin E/β-thalassemia. Baseline characteristics of patients enrolled in the BELIEVE trial (NCT00204438) were previously reported on behalf of the trial investigators.4

METHODS

Study Design

The initial randomized, double-blind, placebo-controlled, multicenter trial enrolled patients with β-thalassemia (homozygous (HbSS) or compound HbS) or hemoglobin E/β-thalassemia. A total of 224 patients were randomized (112 to the luspatercept arm and 112 to the placebo arm) to receive luspatercept or placebo s.c. every 21 days plus best supportive care (BSC) for up to 96 weeks.4

RESULTS

Among the 80 (87.9%) luspatercept responders, mean changes (95% confidence interval [CI]) in serum ferritin in the 24 weeks prior to randomization were -665.94 (-761.12, -570.76) mg/L for luspatercept-treated patients, compared with -301.7 (-395.9, -207.5) mg/L for placebo-treated patients.5

Results of the subgroup analyses are shown in Table 2. Among the 130 (78.0%) luspatercept responders and 36 (57.0%) placebo responders, mean (95% CI) ferritin changes in the 24 weeks prior to randomization were -500.00 (-582.0, -418.0) and -207.5 (−295.0, -119.9) mg/L, respectively (least square mean difference: −301.7 mg, respectively)

CONCLUSIONS

Luspatercept treatment resulted in clinically meaningful and maintained reductions in serum ferritin levels.

REFERENCES


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DISCLOSURES

J.B.P. is a consultant for Janssen Biotech, a subsidiary of Johnson & Johnson, and consultant and/or speaker for Protagonist Therapeutics, Celgene Corporation, Genesis Pharmaceuticals, and Protagonist Therapeutics Inc. A.T.T. is an employee of Novartis and has ownership interests in Protagonist Therapeutics and Protagonist Therapeutics Inc.