Deferasirox Continues to Reduce Iron Overload in Non-transfusion-dependent Thalassemia: A One-year, Open-label Extension, Double-blind, Placebo-controlled, Study (THALASSA)

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BACKGROUND

In patients with non-transfusion-dependent thalassemia (NTDT) such as Cohen’s syndrome or the beta-thalassemias, iron overload can result in dyspnea and cardiovascular and endocrine complications.

METHODS

Study Design

This was a multicenter, prospective, randomized, double-blind, placebo-controlled Phase II extension study conducted by 27 centers across six countries (China/Hong Kong/Taiwan/HKMSI). Study design/demographics criteria have been previously described.8

Patient Population

Patients randomized to deferasirox during the core study (n=110) who received deferasirox at either 5, 10, or 15 mg/kg/day during the core extension (n=56) were included in this analysis. The main exclusion criteria of the core studies were MacFarlane criteria for decompensation or bleeding, and/or lack of compliance. Patients in the extension phase could receive an extension core dose adjustment to 10 mg/kg/day.

RESULTS

Iron Concentration (LIC)

The percentage of patients achieving LIC ≤20 mg/kg/day was 80.4% and 74.4% for the placebo/deferasirox group (n=56) and the deferasirox extension group (n=130), respectively.

Safety

Most patients (≥77%) reported one or more treatment-emergent adverse events (TEAEs), with the most common being gastrointestinal (GI) events. For patients in the placebo/deferasirox group (n=56), 21.4% of patients interrupted the study due to TEAEs (n=12). For patients in the deferasirox extension group (n=130), the interruption rate was 19.2% (n=25). The most common reasons for study interruption were TEAEs of the GI system (n=11) or skin (n=9) and wound healing disorder (n=8). One patient in the deferasirox extension group had urinary protein/creatinine ratio increase (n=3).

DISCUSSION AND CONCLUSIONS

This study shows that NTDT patients receiving deferasirox for up to 2 years continue to experience decreases in LIC, and this reduction in iron overload is associated with an improvement in the safety profile. The overall tolerability and effectiveness of deferasirox as a chelating agent for managing iron overload in NTDT patients continues to be demonstrated.

REFERENCES


Acknowledgments

This study was sponsored by Novartis Pharmaceuticals, Inc. All authors contributed to and approved the final manuscript. The authors acknowledge the contributions of the THALASSA Investigators who conducted this study.

Presented at ASH, Atlanta, Georgia, December 8–11, 2011

This study was sponsored by Novartis Pharma AG.