

P1485 A MULTICENTER, RETROSPECTIVE STUDY ON REAL-WORLD EXPERIENCE OF PATIENTS WITH SICKLE CELL DISEASE TREATED WITH VOXELOTOR

Topic: 26. Sickle cell disease

Biree Andemariam¹, Modupe Idowu², Nirmish Shah³, Richard Drachtman⁴, Archana Sharma⁴, Alexander Glaros⁵, Maureen Achebe⁶, Alecia Nero⁷, Susanna Curtis⁸, Caterina Minnitti⁸

¹ New England Sickle Cell Institute, University of Connecticut Health, Farmington, United States;² University of Texas Health, Houston, United States;³ Duke University School of Medicine, Durham, United States;⁴ Rutgers Robert Wood Johnson Medical School, New Brunswick, United States;⁵ Division of Pediatric Hematology/Oncology, Central Michigan University, Detroit, United States;⁶ Brigham and Women's Hospital, Boston, United States;⁷ University of Texas Southwestern Medical Center, Dallas, United States;⁸ Albert Einstein College of Medicine, Bronx, United States

Background: Sickle cell disease (SCD) is an inherited systemic disorder, with pathology driven by polymerization of sickle hemoglobin (HbS). Voxelotor, a HbS polymerization inhibitor, is approved in the United States for treatment of SCD in adults and pediatric patients aged ≥ 4 years and in the European Union for the treatment of hemolytic anemia due to SCD in adult and pediatric patients ≥ 12 years of age as monotherapy or in combination with hydroxycarbamide. Efficacy and safety data from the randomized, placebo-controlled HOPE trial demonstrated the effectiveness of voxelotor in increasing hemoglobin (Hb) levels and reducing markers of hemolysis. Real-world studies complement and expand upon information gathered in randomized clinical trials by providing evidence of treatment safety and efficacy in clinical practice.

Aims: The Retrospective Study to Evaluate Outcomes in Patients With Sickle Cell Disease Treated With Oxbryta (RETRO) aims to characterize real-world safety and effectiveness of voxelotor in adults and adolescents (aged ≥ 12 years) with SCD treated with voxelotor as part of their usual care.

Methods: RETRO is a multicenter, post-marketing, retrospective study that collected laboratory and clinical data from patients' medical records 1 year before and 1 year or more after initiation of voxelotor treatment. Patients with documented SCD who received voxelotor for ≥ 2 consecutive weeks were included in this analysis.

Results: Data from 216 patients across 9 US sites were collected and analyzed. The mean (SD) patient age was 33.5 (14.2) years, and the mean (SD) duration of voxelotor treatment was 51.1 (25.6) weeks. Reasons for voxelotor prescription (n, %) included reducing the following: anemia (151, 69.9%), pain (51, 23.6%), frequency of vaso-occlusive crises (45, 20.8%), and the need for blood transfusions (17, 7.9%); multiple reasons may have been selected. Most patients were prescribed an initial voxelotor dose of 1500 mg (n=187, 86.6%), and 68.1% (n=147) of patients used hydroxyurea concomitantly. A total of 25.0% (n=54) of patients had a dosage interruption or adjustment. Reasons for dosage change (n, %) included adverse event (AE; 37, 17.1%), other (22, 10.2%), pill burden (2, 0.9%), and lack of efficacy (1, 0.5%); multiple reasons may have been selected.

A total of 198 patients had recorded baseline and post-treatment Hb values. In these patients, the mean (SD) peak observed post-treatment Hb level increased from baseline by 1.4 (1.6) g/dL, from 7.8 (1.5) g/dL to 9.2 (2.0) g/dL (**Figure**). In patients with recorded baseline and post-treatment indirect bilirubin levels (n=80) and reticulocyte percentages (n=178), the mean (SD) minimum observed post-treatment value for indirect bilirubin decreased from baseline by 1.1 (1.9) mg/dL, from 3.1 (2.0) mg/dL to 1.9 (1.9) mg/dL, and reticulocyte percentage decreased from baseline by 3.8% (5.8%), from 11.6% (6.8%) to 7.7% (5.1%). The safety and tolerability of voxelotor in the real-world setting will be presented. The most common non-SCD-related treatment-emergent AEs were diarrhea, headache, and rash; 37.0% (n=80) of patients reported ≥ 1 non-SCD-related AE, and most AEs were mild in severity.

Image:

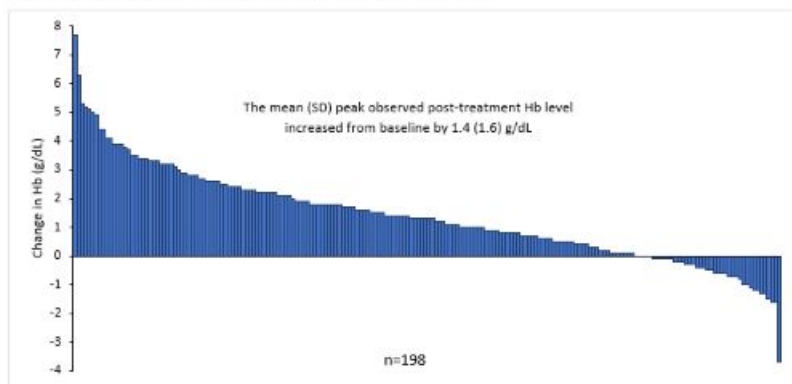
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Figure: Per-Patient Peak Hemoglobin Change From Baseline



Hb, hemoglobin.

Summary/Conclusion: RETRO is the first multicenter study to collect and analyze retrospective data from patients with SCD treated with voxelotor in a real-world setting. These interim results are consistent with the HOPE trial, showing that voxelotor treatment was associated with increased Hb levels and decreased hemolytic markers. The safety data are also consistent with those from the HOPE trial.

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