

**Title: Pediatric Patient Reported Outcomes in Patients Receiving Voxelotor for Sickle Cell Disease**

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**Background:** Sickle cell disease (SCD) describes a group of chronic, inherited blood disorders resulting from mutations that reduce or abolish normal hemoglobin production. The most common clinical manifestations of SCD are related to hemolytic anemia and vaso-occlusion, which can cause acute and chronic pain and end-organ damage. Voxelotor is a first-in-class sickle hemoglobin–polymerization inhibitor, FDA approved for patients with SCD aged ≥12 years, that modulates hemoglobin oxygen-affinity, prevents sickling of red blood cells, and possibly interrupts the molecular pathogenesis of the disease. It is currently being investigated in patients aged 4-11 years. Incorporating the patient voice into drug development is increasingly important to regulators; thus, collecting qualitative data to gain patient and caregiver insights alongside clinical data can aid in understanding treatment benefits in this patient population.

**Aims:** The purpose of this study is to collect qualitative data to understand patient experiences with voxelotor, as reported by pediatric patients and their caregivers. Specifically, the study aims to understand how the treatment affects symptoms and related impacts, and overall health-related quality of life (HRQOL).

**Methods:** This qualitative study will involve interviewing children with SCD (aged 4-11 years) and their caregivers to be recruited from multiple sites in the US. Eligible patients will have been taking voxelotor for ≥4 weeks at the time of the interview. Concept elicitation interviews are to be conducted via telephone by experienced qualitative researchers using a semi-structured interview guide. Interview transcripts will be analyzed with MAXQDA software. This will allow theme identification using an induction-abduction approach, enabling the analysis to remain grounded in the data. Inter-coder reliability will be established early in the coding process by having two analysts independently code the same transcript.

**Results:** The interviews are ongoing. Changes in symptoms, effects on daily life, and other impacts to HRQOL reported by patients and their caregivers will be presented.

**Conclusion:** This will be the first study to report qualitative experiences of pediatric patients treated with voxelotor.

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