A Qualitative Study of the Experiences of Pediatric Patients Receiving Voxelotor

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OBJECTIVE

To collect qualitative data to understand the experiences of pediatric patients with sickle cell disease (SCD) receiving voxelotor, with a focus on how the treatment affects symptoms and related impacts, as well as overall health-related quality of life.

BACKGROUND

- SCD is the most common inherited blood disorder in the United States, affecting approximately 70,000 to 100,000 Americans.[1,2]
- The most common clinical manifestations of SCD are related to hemolytic anemia and vaso-occlusion, which can lead to acute and chronic pain and end-organ damage.[3]
- Voxelotor is a first-in-class oral therapy that targets the molecular pathogenesis of SCD by modulating hemoglobin's affinity for oxygen to inhibit sickle hemoglobin polymerization.[4,5]
  - It is FDA approved for patients aged ≥12 years and currently under priority review in patients aged 4 to 11 years.[6,7]
- Collecting qualitative data directly from patient and caregiver insights can aid in gaining a better understanding of meaningful treatment benefits in this patient population and help to incorporate the patient voice into the development of voxelotor.

METHODS

- This was a qualitative study involving one-time interviews conducted with patient-caregiver dyads in order to elicit the perspectives of both children receiving voxelotor and their primary caregivers.
- Patients were all participating in a clinical trial at a single academic site in the United States.
- Key inclusion criteria:
  - Patient is aged 4 to 11 years old with a diagnosis of SCD
  - Patient is currently taking voxelotor for treatment of SCD and had been taking it for ≥4 weeks at the time of the interview
- Interviews were conducted by experienced qualitative interviewers using a semi-structured interview guide via telephone and a web conferencing platform (WebEx).
- Thematic analysis, a rigorous and transparent method that is well suited to qualitative research,[8] was undertaken on interview data within MAXQDA.

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DISCLOSURES

Clark Brown: Consultant: Global Blood Therapeutics, Imara, Novo Nordisk; research support: Forma Therapeutics, Global Blood Therapeutics, Imara, Novartis. **Anne Beaubrun:** Employee, equity ownership: Global Blood Therapeutics. **Kelly Lipman:** Employee, ICON plc. **Olga Moshkovich:** Employee, ICON plc. **Ryan Murphy:** Employee, ICON plc. **M. Alex Bellenger:** Employee, ICON plc. **Irene Agodoa:** Employee, equity ownership: Global Blood Therapeutics.

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RESULTS

DEMOGRAPHICS AND CLINICAL CHARACTERISTSICS

Interviews were carried out with 10 patient-caregiver dyads (Tables 1 and 2).

Current SCD clinical severity was rated by physicians as "mild" for all patients.

Physicians reported improvement in 8 (80%) patients using the Clinical Global Impression of Change scale.

Hemoglobin levels improved in 9 (90%) patients, from a mean of 9.0 g/dL (range 7.6-11.1) before initiation of voxelotor to 9.6 g/dL (8.2-12.2), based on the most recent steady-state measure at the time of study screening.

Table 1
Patient Demographics (N=10)

Age, mean (range), years	8.9 (6-11)
Sex, n (%)	
Female	7 (70)
Male	3 (30)
Black/African American, n (%)	10 (100)
Time on voxelotor, mean (range), months	18 (3-36)

Table 2
Caregiver Demographics (N=10)

Age, mean (range), years	32.3 (30-38)
Sex, n (%)	
Female	7 (70)
Male	3 (30)
Black/African American, n (%)	10 (100)
Highest level of education, n (%)	
High school/General Educational Development	8 (80)
Bachelor's degree	1 (10)
Master's degree	1 (10)
Employment, n (%)	
Full-time	9 (90)
Unemployed	1 (10)

CHANGE IN SYMPTOMS

Of the participants with responses, most reported improvement in the severity and/or frequency of pain crises (n=5/8; 63%), fatigue (n=4/5; 80%), and jaundice (n=4/5; 80%) (Figure 1).[a]

Caregivers and patients also reported observing positive changes with respect to:

- Chronic/breakthrough pain (other than pain crises) (n=2)
- Ability to focus (n=1)
- Nail strength (n=1)
- Appetite (n=1)

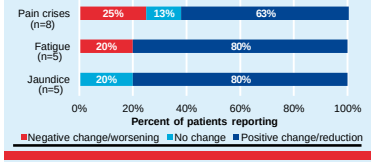
[a]Of the 10 patients interviewed, spontaneous responses were not reported for 2 patients for pain crises, 5 patients for fatigue, and 5 patients for jaundice.

> *I'd say we've had less pain crises since we started, and I'd say that her hemoglobin has raised a full gram.* –Caregiver

> *She's not tired anymore…even the fatigue is not like what it was before. I mean…we can see the difference with the new med. We really can see it with the new med.* –Caregiver

> *…since she started off [taking voxelotor] – I've not known her to have any yellowing of the eyes [anymore].* –Caregiver

Figure 1
Most Frequent Spontaneously Reported Symptom Changes Experienced After Starting Voxelotor Treatment



IMPACT ON DAILY LIFE

Most of the sample (n=8; 80%) reported improvement in health-related quality of life, specifically citing a general feeling of improved quality of life after starting voxelotor treatment.

> *Well, I like that, you know, [it] improved their quality of their health, I like that.* –Caregiver

Both caregivers and patients reported improvements in the patient's ability to engage in activities, such as swimming and other play (n=5; 50%).

> *She learned to ride her bike, she, she could go swimming, she could, she could hang with her friends all day long. We noticed that almost immediately, and that was the most remarkable change…* –Caregiver

Caregivers (n=3; 30%) reported improvements in the school/academic environment for their child, related to increased focus, reduced absences from school, and increased participation in the classroom and other school-related activities.

> *I think the medication has been very, very helpful in getting [name] to where she is now, to where school is not such a hassle, and she doesn't mind turning on her camera [for virtual school] if she needs to. She doesn't mind answering questions, you know, and sometimes before she wouldn't want to answer even if she knew the answer…and now she's actually helping other students in her class.* –Caregiver

CONCLUSIONS

This study, the first study to report on the qualitative experiences of pediatric patients treated with voxelotor, highlights the utility and potential benefit of voxelotor in the pediatric SCD population.

Almost all patients experienced improvements in their health-related quality of life after initiating therapy with voxelotor; improvements across a variety of symptoms and a positive impact on daily life were also reported.

These findings should aid in the design of post-marketing surveillance trials that require SCD-specific patient-reported outcome measures.