Real-World Experience of Voxelotor for the Management of Complications in Sickle Cell Disease

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INTRODUCTION

This study sought to assess the real-world impacts of voxelotor treatment on the rates and management of sickle cell disease (SCD)-related complications.

SCD is an inherited systemic disorder that is driven by sickle hemoglobin (HbS) polymerization and red blood cell sickling, which leads to a chronic cycle of hemolysis, anemia, and vaso-occlusion.[1,2]

Voxelotor is a HbS polymerization inhibitor that is indicated for treatment of SCD in adults and adolescents aged ≥12 years.[3]

Emerging evidence shows that voxelotor, an oral, once-daily tablet, may improve the clinical symptoms of SCD and reduce vaso-occlusive crisis (VOC) rates, as well as the need for transfusions.

METHODS

- Medical and pharmacy claims data for patients aged ≥12 years with SCD who initiated voxelotor therapy between November 2019 and June 2021 were procured from the Symphony Health claims database.
- Patients with ≥1 year's data before the index date (date of the first voxelotor claim for each patient) were included in the analyses.
  Baseline demographic and clinical characteristics were summarized using descriptive and inferential statistics.
- Annualized rates per patient-year (PPY) for transfusions, VOCs, and VOC-related and all-cause hospitalizations were compared for the 3 months before voxelotor initiation versus the period after voxelotor initiation.
  - Before: **Pre-index period**
  - After: **Post-index period**
- Hemoglobin (Hb) responses were evaluated for a subset of patients for whom Hb lab data were available (at least 1 Hb value measured in both the pre-index and post-index periods).

REFERENCES

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2. Cintron-Garcia J, Ajebo G, Kota V, et al. Mortality trends in sickle cell patients. *Am J Blood Res.* 2020;10(5):190-197.
3. Oxbryta. Prescribing information. Global Blood Therapeutics; January 2021.

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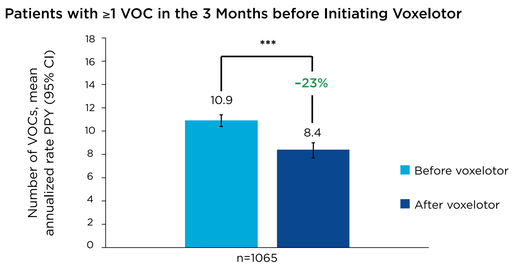
RESULTS

Patient Demographics

	Voxelotor patients (N=3128)
Sex, %	
Male	40.1
Female	59.9
Age, mean, years (SD)	34.7 (14.6)
Patients with a VOC in 3 months before initiating voxelotor, %	34

Data presented are based on an interim data cut (June 2021).
VOC, vaso-occlusive crisis.

Patients with Recent VOCs Receiving Voxelotor Experienced Fewer VOCs during the Post-Index Period



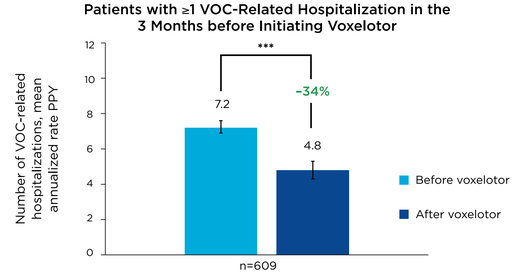
***P<0.001.
Data presented are based on an interim data cut (June 2021).
Error bars are 95% CIs. 95% CIs were obtained from bootstrapping.
"Before" refers to the 3-month period before the first voxelotor administration. "After" refers to the period from the index date to the end of follow-up.
PPY, per patient-year; VOC, vaso-occlusive crisis.

Patients Receiving Voxelotor Had an Increase in Hb during the Post-Index Period

	Voxelotor patients (n=74)
Hb, mean, g/dL (95% CI)	
Pre-index value	7.8 (7.5, 8.2)
Post-index value	8.9 (8.5, 9.4)
Patients who had a change in Hb >1 g/dL at any point during follow-up, %	60.8

Data presented are based on an interim data cut (June 2021).
95% CIs were obtained from bootstrapping.
Hb, hemoglobin.

Patients with Recent VOC-Related Hospitalizations Receiving Voxelotor Had Fewer VOC-Related Hospitalizations during the Post-Index Period



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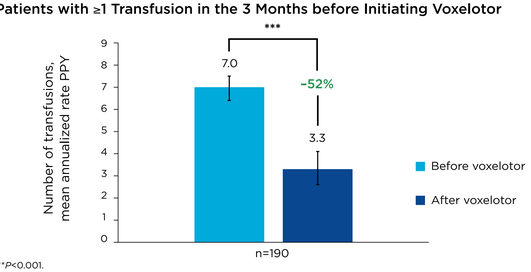
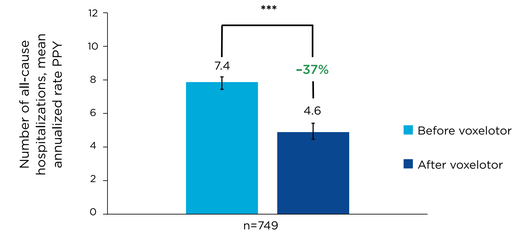
PPY, per patient-year; VOC, vaso-occlusive crisis

Patients with Recent Transfusions Receiving Voxelotor Had Fewer Transfusions during the Post-Index Period



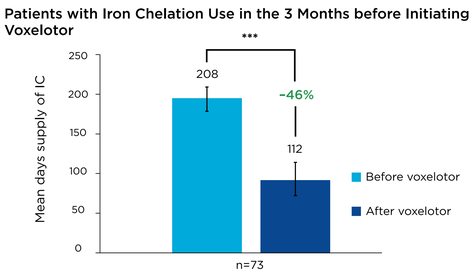
***P<0.001.
Data presented are based on an interim data cut (June 2021).
Error bars are 95% CIs. 95% CIs were obtained from bootstrapping.
"Before" refers to the 3-month period before the first voxelotor administration. "After" refers to the period from the index date to the end of follow-up.
PPY, per patient-year.

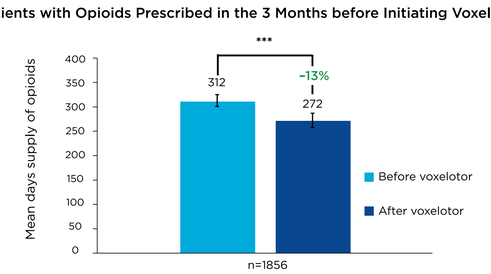
The Number of All-Cause Hospitalizations Decreased in Recently Hospitalized Patients Receiving Voxelotor



***P<0.001.
Data presented are based on an interim data cut (June 2021).
Error bars are 95% CIs. 95% CIs were obtained from bootstrapping.
"Before" refers to the 3-month period before the first voxelotor administration. "After" refers to the period from the index date to the end of follow-up.
PPY, per patient-year.

Patients Receiving Voxelotor Had Reduced Iron Chelation Use and Fewer Opioids Prescribed during the Post-Index Period





- Of the 73 patients with iron chelation use in the pre-index period, 43 (56%) did not have any IC usage in the post-index period (*P*<0.001).
- In the 81 patients with any ESA use in the 3 months pre-index, 17 (21%) had no ESA use after starting voxelotor (*P*<0.001).

***P<0.001.
Data presented are based on an interim data cut (June 2021).
Error bars are 95% CIs. 95% CIs were obtained from bootstrapping. "Before" refers to the 3-month period before the first voxelotor administration.
"After" refers to the period from the index date to the end of follow-up. ESA, erythropoiesis stimulating agent; IC, iron chelation; PPY, per patient-year.

CONCLUSIONS

In real-world practice, voxelotor increased Hb, consistent with results from the HOPE trial.

This real-world evidence provides additional support for the use of voxelotor in the treatment of SCD and the management of its associated complications.

Data indicate statistically significant reductions in transfusions, VOCs, and all-cause and VOC-related hospitalizations after voxelotor use.

Limitations of this analysis include the possibility of secular trend biases or general regression to the mean.

DISCLOSURES

Nimish Shah
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- Speaker: Alexion

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- Nothing to disclose

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- Research funding: Global Blood Therapeutics, Novartis

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