Real-World Effectiveness of Voxelotor for Treating Sickle Cell Disease in the US

Ahmar U. Zaidi, MD[1]; Thokozeni Lipato, MD[2]; Ofelia Alvarez, MD[3]; Alexander Lonshteyn, PhD[4]; Derek Weycker, PhD[4]; Nhat Pham[4]; Thomas E. Delea, MSIA[4]; Irene Agodoa, MD[5]; Ze Cong, PhD[5]; **Nirmish Shah, MD[6]**

[1]Children's Hospital of Michigan, Central Michigan University, College of Medicine, Detroit, MI, USA; [2]VCU Health, Richmond, VA, USA; [3]University of Miami, Miami, FL, USA; [4]Policy Analysis Inc. (PAI), Brookline, MA, USA; [5]Global Blood Therapeutics, South San Francisco, CA, USA; [6]Duke University School of Medicine, Durham, NC, USA

ASH 2020

Disclosures

Ahmar U. Zaidi
- Speaker, research funding, consultant: Global Blood Therapeutics
- Consultancy: bluebird bio, Cyclerion, Emmaus, Imara, Novartis

Thokozeni Lipato
- Speaker: Global Blood Therapeutics

Ofelia Alvarez
- Membership on entity's advisory committees: Forma Therapeutics, Novartis

Alexander Lonshteyn
- Current employment: Policy Analysis Inc.
- Research funding: Global Blood Therapeutics, Novartis

Derek Weycker
- Current employment and equity holder: Policy Analysis Inc.
- Research funding: Global Blood Therapeutics, Novartis

Nhat Pham
- Current employment: Policy Analysis Inc.
- Research funding: Global Blood Therapeutics, Novartis

Thomas E. Delea
- Current employment and equity holder: Policy Analysis Inc.
- Research funding: Global Blood Therapeutics, Novartis

Irene Agodoa
- Current employment and current equity holder in a publicly traded company: Global Blood Therapeutics

Ze Cong
- Former employee and equity holder in a publicly traded company: Global Blood Therapeutics

Nirmish Shah (presenting author)
- Membership on entity's speaker bureau: Global Blood Therapeutics, Novartis, Alexion
- Research funding: Global Blood Therapeutics, Novartis
- Consultancy: Global Blood Therapeutics, bluebird bio, CSL Behring

Voxelotor Is an Oral, Once-Daily HbS Polymerization Inhibitor

Until late 2019, few treatments had been approved by the FDA for treating SCD.

Voxelotor (Oxbryta®) was approved by the FDA in November 2019 for the treatment of SCD in patients ≥12 years of age under an accelerated approval based on results of the pivotal HOPE study.[1]

In the HOPE study,[a] **voxelotor was associated with an average Hb increase of 1.1 g/dL**, and 51% of patients receiving voxelotor 1500 mg achieved a >1 g/dL Hb increase at week 24.[2]

This study sought to assess the real-world effectiveness of voxelotor based on data during the first 7 months post FDA approval

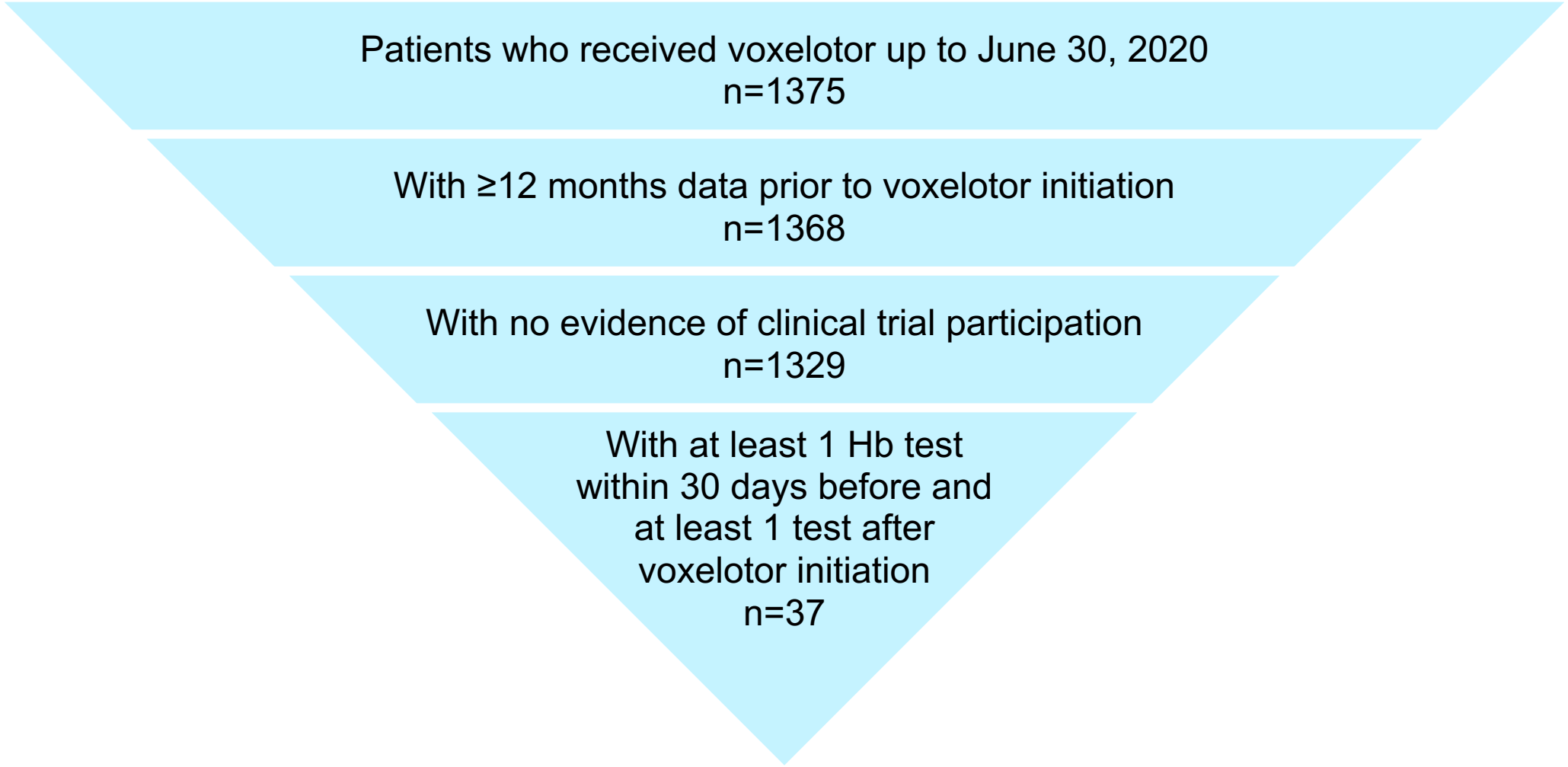
[a]Eligible patients had Hb levels between 5.5 and 10.5 g/dL, experienced 1 to 10 VOCs in the previous year, and were not transfusion dependent.
FDA, US Food and Drug Administration; Hb, hemoglobin; HbS, sickle hemoglobin; SCD, sickle cell disease; VOC, vaso-occlusive crisis.
1. Oxbryta. Prescribing information. Global Blood Therapeutics, Inc; 2019. 2. Vichinsky E, et al. *N Engl J Med*. 2019;381(6):509-519.

Methods

- Data on medical and pharmacy claims for patients who were aged ≥12 years and receiving voxelotor from December 2019 to June 2020 were obtained from the Symphony Health claims data set.
  - Patients with at least 1 year of data prior to their first voxelotor claim date were included.
- Rates of transfusions and VOCs per patient per year (PPPY) were compared for the 3-months before voxelotor initiation versus the period after voxelotor initiation (mean follow-up, 2.9 months).
- Change in Hb level and the percentage of patients achieving a >1 g/dL increase in Hb were assessed in the subset of patients with Hb laboratory data available.
  - Evaluated patients had at least 1 Hb measurement within 30 days before voxelotor initiation and at least 1 Hb measurement after voxelotor initiation.
- Confidence intervals and *P* values for changes in outcomes were based on bootstrapping.

Hb, hemoglobin; VOC, vaso-occlusive crisis.

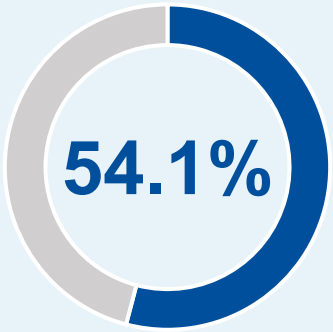
Patient Flow



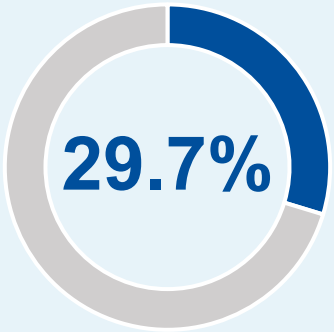
Hb, hemoglobin.

Voxelotor Resulted in Significant Improvements in Hb

		After		
	Before	Average Hb n=37	Maximum Hb n=37	Last Hb n=37
Hb, g/dL, mean (CI 95%)	7.8 (7.3, 8.3)	8.7 (8.0, 9.5)	9.3 (8.5, 10.1)	8.7 (7.9, 9.5)
Change, mean (CI 95%)	–	0.9 (0.4, 1.4)	1.5 (0.9, 2.0)	0.8 (0.2, 1.5)
P value	–	<0.001	<0.001	0.008



of patients achieved a >1 g/dL Hb increase at some time point



of patients achieved a >2 g/dL Hb increase at some time point

Hb, hemoglobin.

Voxelotor Was Associated With Significant Reductions in Rates of Transfusions and VOCs

Rate	Number of patients, n	Pre-index value, mean (95% CI)	Post-index value, mean (95% CI)	Change, mean (95% CI)	Percent reduction	*P* value
Transfusions among all patients	1329	0.12 (0.09, 0.15)	0.08 (0.06, 0.11)	−0.04 (−0.07, −0.01)	33%	0.018
≥1 transfusion in prior year	187	0.9 (0.7, 1.0)	0.5 (0.3, 0.6)	−0.4 (−0.6, −0.2)	44%	<0.001
≥8 transfusions in prior year	22	3.0 (2.4, 3.7)	1.7 (0.8, 2.7)	−1.4 (−2.4, −0.2)	47%	0.018
VOCs among all patients	1329	1.0 (0.9, 1.2)	0.7 (0.6, 0.8)	−0.3 (−0.4, −0.3)	30%	<0.001
≥1 VOC in prior year	475	2.9 (2.7, 3.1)	1.7 (1.5, 1.9)	−1.3 (−1.5, −1.1)	45%	<0.001

VOC, vaso-occlusive crisis.

Conclusions

Based on the first 7 months' data after the approval of voxelotor in the US, in real-world practice, voxelotor increases mean Hb by approximately 1 g/dL, consistent with the results of the HOPE trial.

Rates of transfusion and VOC were decreased after voxelotor initiation.

This real-world evidence provides additional support for the use of this novel therapy in the treatment of hemolytic anemia and its associated complications in the SCD population.

Further evaluation with a larger sample size and longer follow-up will help to confirm these findings.

Hb, hemoglobin; SCD, sickle cell disease; VOC, vaso-occlusive crisis.

Acknowledgments

- This study was supported by Global Blood Therapeutics.

Nelson Jen, PhD (Healthcare Consultancy Group, with funding from Global Blood Therapeutics) provided editorial assistance.