Voxelotor Treatment Is Associated With Decreased Usage of Iron Chelation, Opioids, and Erythropoietin-Stimulating Agents in Patients With Sickle Cell Disease

Nirmish Shah, MD; Thokozeni Lipato, MD; Ofelia Alvarez, MD; Thomas E. Delea, MSIA; Nhat Pham, PhD; Alexander Lonshteyn, PhD; Derek Weycker, PhD; Andy Nguyen, PhD; Anne Beaubrun, PhD

Duke University School of Medicine, Durham, NC, USA; VCU Health, Richmond, VA, USA; University of Miami Miller School of Medicine, Miami, FL, USA; Policy Analysis Inc. (PAI), Brookline, MA, USA; Global Blood Therapeutics, South San Francisco, CA, USA

OBJECTIVE
To assess healthcare resource utilization related to sickle cell disease (SCD) management, defined by use of iron chelation therapy, opioid prescription days, and erythropoietin-stimulating agent (ESA) use, in voxelotor-treated patients in the real world.

BACKGROUND
SCD, an inherited disorder associated with sickle hemoglobin polymerization, results in chronic anemia, hemolysis, and vaso-occlusive crises.1 — Management of SCD complications leads to substantial healthcare costs.

Oxbryta® (voxelotor) tablets were approved by the US Food and Drug Administration in November 2019 for treatment of patients with SCD aged 12 years based on results from the pivotal HOPE trial.2 — Voxelotor is a sickle hemoglobin-polymerization inhibitor that significantly increased hemoglobin levels and reduced markers of hemolysis in patients in the HOPE trial.3 — A prior analysis of real-world data found reduced rates of transfusions and vaso-occlusive crises in patients after voxelotor treatment.4

METHODS
Medical and pharmacy claims data for patients aged 12 years or older from voxelotor from December 2019 to December 2020 were obtained from the Symphony Health Claims database.

The rate of ESA use was assessed in patients with SCD aged 12 years on voxelotor from December 2019 to December 2020 compared for the post-index and 3-month pre-index periods.

The total daily supplies over a 3-month (90-day) period of iron chelation therapy and of opioid prescription were adjusted for follow-up and compared for the post-index and 3-month pre-index periods.

The rate of ESA use was assessed in patients with any ESA use 3 months pre-index.

RESULTS
Patients (n=5929)

Table 1: Demographic and Clinical Characteristics

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Change (P-value)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, mean</td>
<td>19 (15-25)</td>
<td>20 (16-25)</td>
<td>1 (0.001)</td>
</tr>
<tr>
<td>Male, %</td>
<td>41</td>
<td>39</td>
<td>-2 (0.07)</td>
</tr>
<tr>
<td>ESA use, %</td>
<td>75</td>
<td>50</td>
<td>-25 (0.001)</td>
</tr>
</tbody>
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ESA use all decreased after voxelotor initiation in a large population of patients receiving the therapy.

CONCLUSIONS
Based on the first 13 months of real-world practice data post-approval of voxelotor, the use of iron chelation therapy, opioid prescription days, and ESA use all decreased after voxelotor initiation in a large population of patients receiving the therapy.

Analysis limitations include the possibility of secular trend biases or general regression to the mean.

This study suggests that voxelotor treatment may reduce healthcare resource utilization and the economic burden associated with management of SCD.