Abstract: PB3216

Title: MONITORING THE REAL-WORLD IMPACTS OF VOXELOTOR TREATMENT ON BLOOD TRANSFUSION REQUIREMENTS, QUALITY OF LIFE, AND PHYSIOLOGICAL WEARABLE METRICS IN UK PATIENTS WITH SICKLE CELL DISEASE

Abstract Type: Publication Only

Topic: Sickle cell disease

Background:

Sickle cell disease (SCD) currently sees limited options for treatment, and standards of care are often limited to hydroxyurea (HU) and blood transfusion in the UK. Voxelotor, a first-in-class HbS polymerization inhibitor, is approved in the UK for the treatment of hemolytic anemia due to SCD in patients aged ≥12 years. While clinical trials have demonstrated a significant impact on hemoglobin levels, there is a need for real-world evidence around the subsequent impact on health and quality of life to aid in patient, clinician, and regulatory decision-making.

Aims:

To better understand the long-term, real-world impacts of voxelotor treatment on patients' transfusion requirements, patient-reported outcomes (PROs), and physiological metrics.

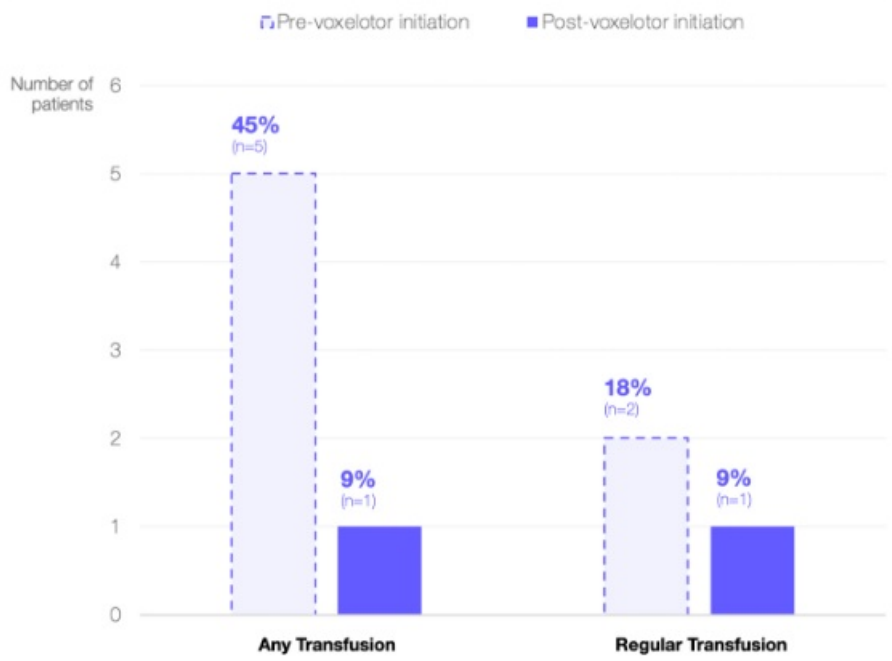
Methods:

Eleven patients receiving voxelotor were identified through a digital survey disseminated online to SCD patients, capturing their treatment dates and transfusion patterns. 6 of these patients had also provided consent for the capture of data through a wearable device, and ePRO data through a specialized mobile phone app, as part of an independent digital ecosystem (n=13,683 combined datapoints). Patient responses were assessed for changes in transfusion rate. ePRO and wearable metrics for the 6 voxelotor patients were compared to other SCD ecosystem patients matched by genotype and age range (n=471, 838,206 datapoints), and those who have reported a need for either regular or ad hoc blood transfusions (n=171, 600,077 datapoints).

Results:

Eleven patients received voxelotor for a mean±SD (range) of 526±178 (266-843) days. 7 (64%) also received HU treatment. The mean age was 34±12 (15-48) years, 6 (55%) were male, and all patients were HbSS genotype. Prior to voxelotor treatment, 5 (45%) patients had required a blood transfusion, of which 2 (18%) required regular transfusions (every <6 weeks). Following voxelotor initiation, only 1 (9%) patient required any form of transfusion.

FIGURE 1. Overview of patient-reported transfusion rates pre- vs. post-initiation with voxelotor treatment (n=11).



Both EQ-5D-5L and EQ-5D Health State scores were significantly higher in voxelotor-treated patients than in the transfusion group, at 0.820 vs. 0.768 ($p<0.001$) and 83 vs. 67 ($p<0.001$), respectively. While both scores were also higher in voxelotor-treated patients compared to matched patients, this was only significant for Health State scores (83 vs. 72, $p<0.001$). Pain (2.2) and fatigue (4.0) scores were lower in the voxelotor group than both transfused (pain=3.3, fatigue=5.2) and matched (pain=3.0, fatigue=5.0) patients ($p<0.001$).

Activity-based metrics were significantly higher in voxelotor-treated patients compared to transfusion and matched patients in terms of steps (3,301 vs. 2,846 vs. 2,895, $p<0.001$), distance (2,506 vs. 2,076 vs. 2,122 meters, $p<0.001$), elevation (31 vs. 18 vs. 19 floors, $p<0.001$), active time (20:09 vs. 15:07 vs. 16:49 minutes, $p<0.001$), and calories (178 vs. 119 vs. 117 kcal, $p<0.001$).

Summary/Conclusion

In this small cohort, patients receiving voxelotor reported a reduced need for blood transfusions after commencing treatment. Positive PRO and wearable metrics were aligned to higher EQ-5D scores, lower pain, and lower fatigue scores; these measures were higher than in matched patients and those who had received transfusions. A limitation of this study is the small number of UK voxelotor-treated patients and in contrast to the comparator groups. Future studies should include more patients to better understand voxelotor's real-world impact in the UK.

Keywords: Treatment, Patient reported outcomes, Sickle cell disease