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Real-World Effectiveness of Voxelotor for the Treatment of Sickle Cell Disease: A Chart Review Study

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Disclosures

Global Blood Therapeutics
employee and stockholder

Background

- Voxelotor (Oxbryta®) is a sickle hemoglobin–polymerization inhibitor approved by the FDA in November 2019 for the treatment of SCD in adults and adolescents ≥12 years of age under accelerated approval based on results from the HOPE trial, an international, randomized controlled study.[1]

- In the HOPE trial, treatment with voxelotor 1500 mg increased average Hb by 1.1 g/dL over baseline at week 24 in patients with baseline Hb levels ranging between 5.5 g/dL and 10.5 g/dL.[2]

FDA, US Food and Drug Administration; Hb, hemoglobin; SCD, sickle cell disease.

1. US Food and Drug Administration, FDA approves voxelotor for sickle cell disease. Accessed October 14, 2020. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-approves-voxelotor-sickle-cell-disease
2. Vichinsky E, et al. *N Engl J Med*. 2019;381(6):509-519

Objective

To assess the real-world effectiveness of voxelotor in treating SCD based on data during the first several months post FDA approval

FDA, US Food and Drug Administration; SCD, sickle cell disease.

Methods

- An online, retrospective chart review conducted by Ipsos' Syndicated Sickle Cell Disease Therapy
  - 77 practicing US physicians
  - first half of 2020
  - all had least 5 patients with SCD ≥12 years of age
  - each performed retrospective reviews of 5 to 12 patient records

- Indirect bilirubin, LDH and percent reticulocytes were collected at
  - treatment initiation
  - first and last measurements after initiation

- Changes in symptoms and other quality-of-life aspects assessed by physicians if patients started voxelotor before the most recent visit as part of their current SCD treatment

LDH, lactate dehydrogenase; SCD, sickle cell disease.

Patient Characteristics

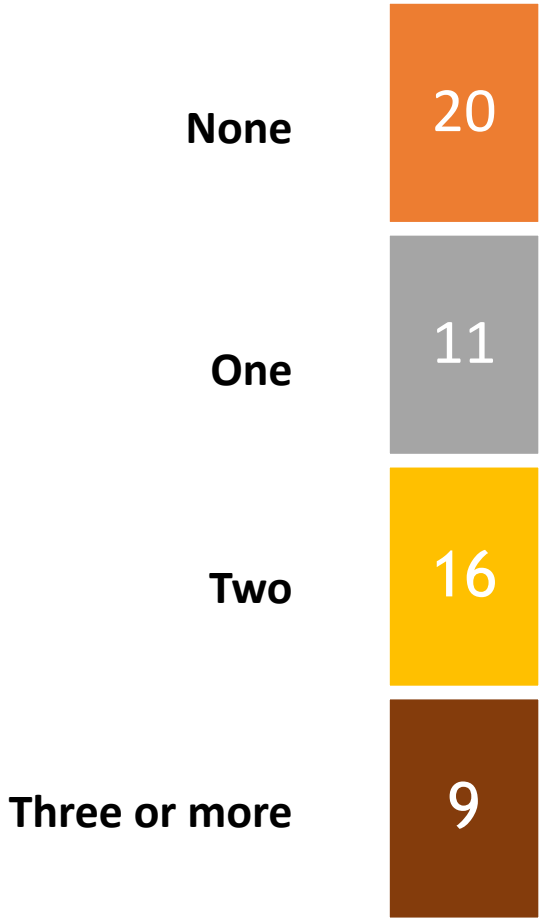
	Voxelotor N=56
Mean age, years	37
12 to <18, n (%)	7 (13)
18 to 30, n (%)	17 (30)
31 to 50, n (%)	16 (29)
≥51, n (%)	16 (29)
Male, n (%)	41 (73)
Genotype, n (%)	
HbSS	23 (41)
HbSC	19 (34)
HbSβ^0	10 (18)
Mean baseline Hb, g/dL	8.4
<5.5, n (%)	7 (13)
5.5 to 10.5, n (%)	37 (66)
>10.5, n (%)	12 (21)

	Voxelotor N=56
Comorbidities, n (%)	
Vaso-occlusive crises	15 (27)
Fatigue	14 (25)
Hypertension	8 (14)
Hyperlipidemia	6 (11)
Leg ulcers	5 (9)
Cognitive impairment	5 (9)
Depression/anxiety	4 (7)
Obesity (BMI ≥30)	4 (7)
Acute chest syndrome	3 (5)
Chronic obstructive pulmonary disease	3 (5)
Ophthalmic complications	2 (4)
None of the above	22 (39)

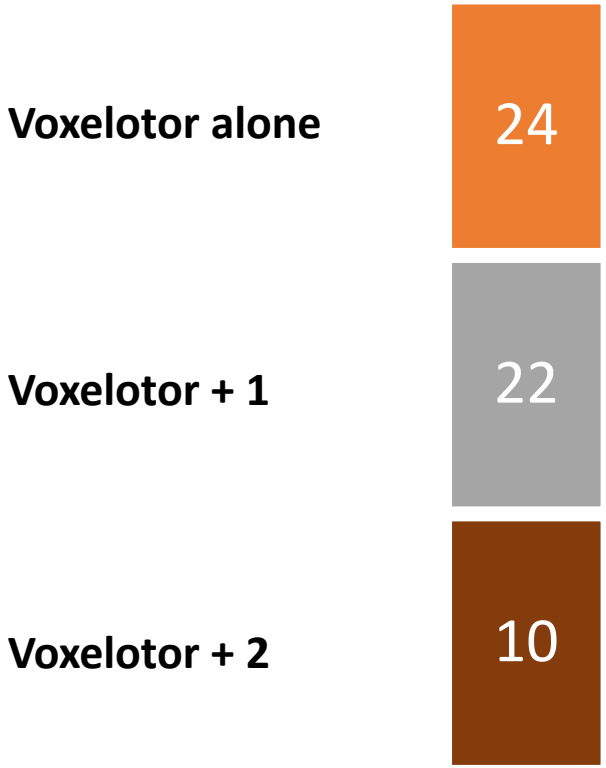
BMI, body mass index; Hb, hemoglobin; HbSβ^0, sickle beta zero thalassemia; HbSC, heterozygous for sickle cell disease; HbSS, homozygous for sickle cell disease.

Current and Historic SCD Treatments

Previous SCD Treatments in 56 Patients

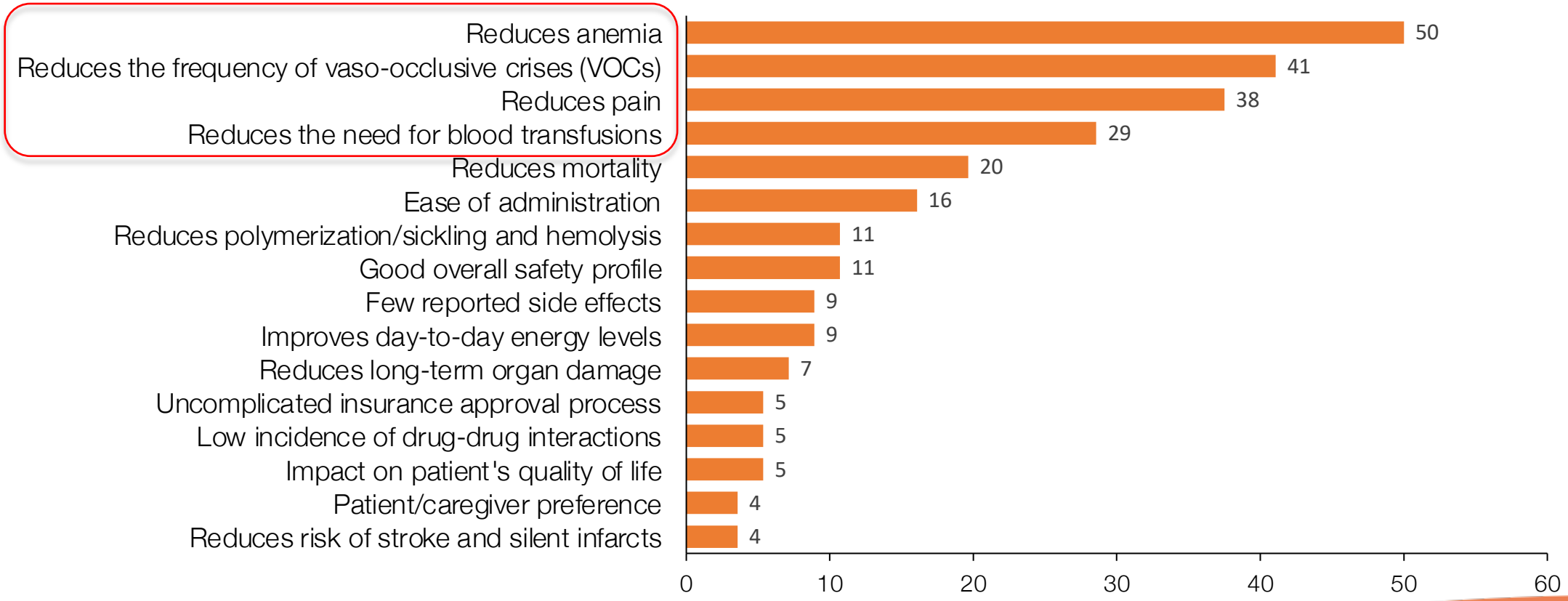


Treatment Combinations in 56 Patients On Voxelotor

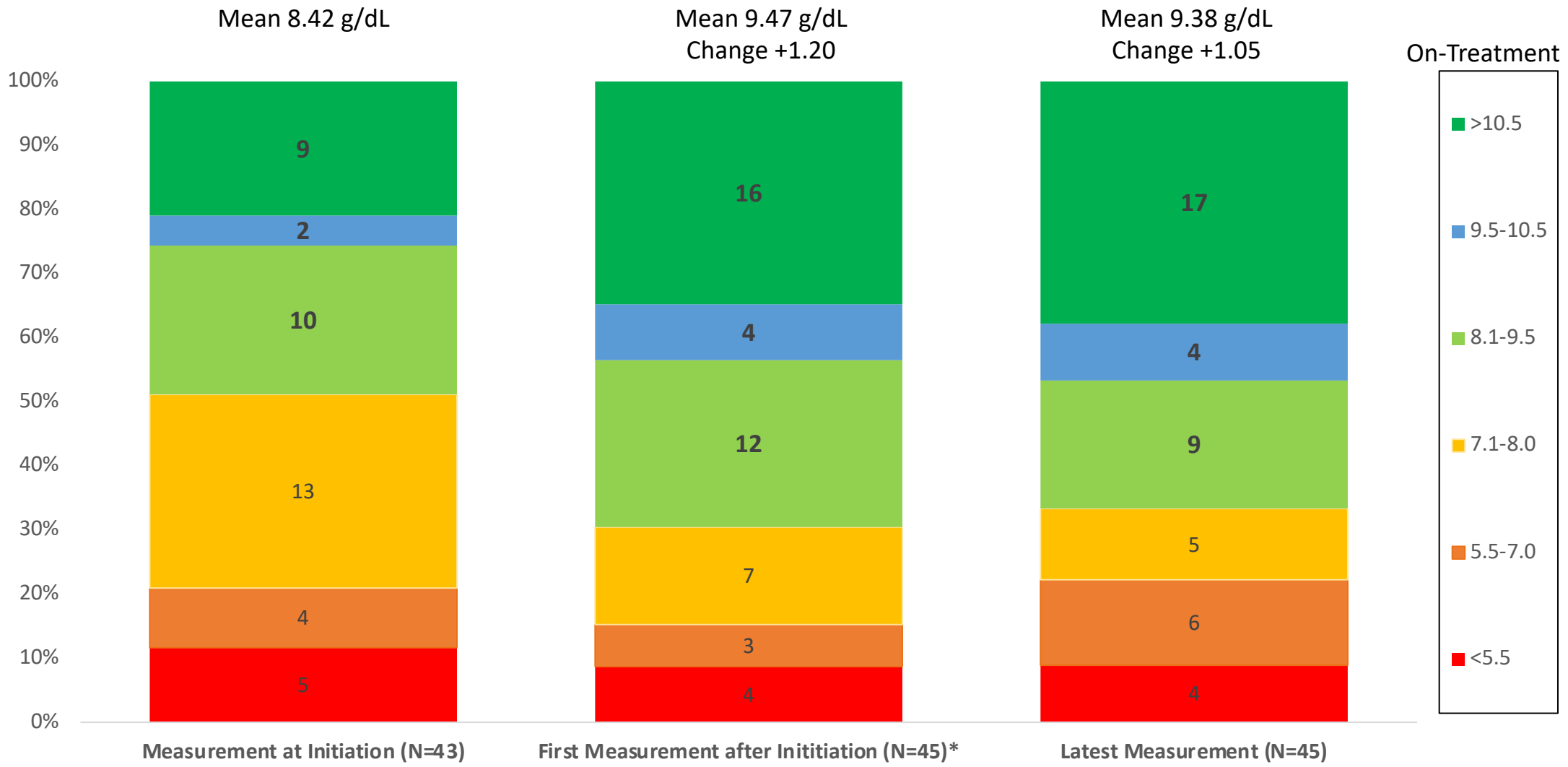


SCD, sickle cell disease.

Reasons for Prescribing Voxelotor



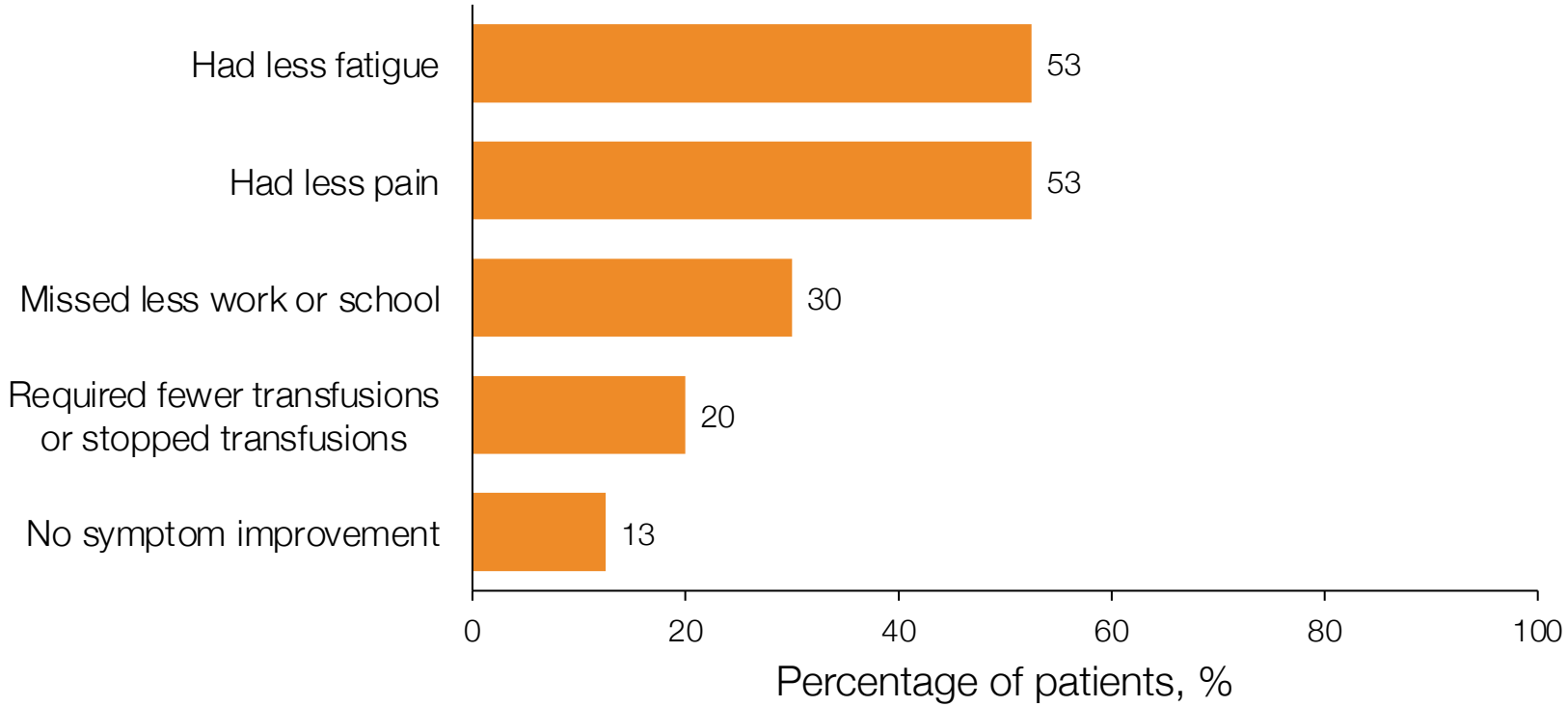
Hemoglobin Change Among Patients Receiving Voxelotor



*Occurred on average 4 weeks after treatment initiation

Hb change calculated based on paired patient-level comparisons at each time point

Physician-Reported Symptoms and Quality-of-Life Changes Among Patients Receiving Voxelotor



Conclusions

- In this real-world evidence study, voxelotor increased Hb by over 1 g/dL on average and decreased hemolysis markers among patients with SCD tested for Hb in this sample, to a degree consistent with the randomized, controlled HOPE trial results.
- Evidence also suggests that voxelotor treatment was associated with improvement in important symptoms of SCD and other aspects of quality of life.
- Further evaluation with a larger sample size and longer follow-up will help confirm
  these findings.

Hb, hemoglobin; SCD, sickle cell disease.

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Thank you

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