P1491 REAL-WORLD INCIDENCE OF VASO-OCCCLUSIVE CRISES IN PATIENTS WITH SICKLE CELL DISEASE (SCD) AND A HIGH BASELINE DISEASE BURDEN TREATED WITH CRIZANLIZUMAB: RESULTS FROM A MANAGED ACCESS PROGRAM (MAP)

Topic: 26. Sickle cell disease

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Background:

Vaso-occlusive crises (VOCs) are the hallmark of SCD and can lead to serious complications and organ damage. P-selectin is a cell adhesion protein that plays a key role in the multicellular interactions that can lead to VOCs. Crizanlizumab, a first-in-class humanized monoclonal antibody that blocks P-selectin, is approved in several regions to prevent/reduce VOCs for SCD patients (pts) aged ≥16 years. Since June 2018, pts in some countries have received crizanlizumab before health authority approval via a MAP (NCT03720626).

Aims:

To describe the rate of VOCs and use of opioids for VOC-related pain relief 12 months before crizanlizumab initiation and after ≥12 months of crizanlizumab treatment in SCD pts participating in the MAP, in countries where publication of these data is allowed.

Methods:

The MAP was designed to provide access to crizanlizumab for pts with a serious or life-threatening disease (SCD) for which no comparable or satisfactory alternative to crizanlizumab was available in their country. Other eligibility criteria included: aged 16–70 years (18–70 years in Italy); history of VOCs as determined by the treating physician (including recurrent VOCs while taking preventative therapies eg hydroxyurea [HU]); and ineligibility for a crizanlizumab clinical trial. Pts’ disease burden in the 12 months prior to crizanlizumab initiation (baseline period) was provided by the treating physician. This analysis reports VOC and opioid use frequency after ≥12 months of crizanlizumab treatment, overall and stratified by SCD genotype and history of HU use, compared with baseline.

Results:

As of Feb 2022, 188 pts have received crizanlizumab in the MAP, of whom 87 have been treated for ≥12 months (Brazil, n=79 [91%]; Italy, n=5 [6%]; Spain, n=2 [2%]; Israel, n=1 [1%]). Median (interquartile range [IQR]) age of the 87 pts was 33 (25–40) years, 57% were female, 45% were African American, 8% Caucasian, 20% Hispanic and 28% of ‘other’ ethnicity, and 82% had an HbSS genotype. History of HU use was reported for 41/56 (73%) pts with available data. During the baseline period, 85% (n=74/87) of pts were hospitalized for a total of 220 SCD-related complications.

At baseline, 100% (n=87) and 93% (n=81) of pts had ≥1 home- and ≥1 healthcare-managed VOC, respectively, vs 79% and 63% of pts after ≥12 months of crizanlizumab treatment. Overall, crizanlizumab led to a median (IQR)

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absolute reduction from baseline of −3.0 (−6.0 to −1.0) home-managed and −2.0 (−4.0 to 0) healthcare-managed VOCs after ≥12 months of treatment. Similar reductions in VOC rates post- vs pre-crizanlizumab were observed when stratifying the data by SCD genotype/prior HU use (Figure), although some groups only contain a small number of pts.

Opioids were taken for VOC-related pain relief by 95% of pts (n=83/87) at baseline and by 69% (n=60/87) in the 12 months after crizanlizumab treatment; the most common was morphine (48% [n=40/83] and 40% [n=24/60], respectively).

Adverse events were consistent with those reported in other crizanlizumab studies.

**Summary/Conclusion:**

Pts in the crizanlizumab MAP had significant disease burden at baseline, as evidenced by the high rate of home- and healthcare-managed VOCs, proportion of patients with SCD-related complications and use of opioids for VOC-related pain relief, despite many pts reporting prior HU use. Crizanlizumab treatment led to clinically relevant reductions from baseline in the median annualized rates of home- and healthcare-managed VOCs and use of opioids in this real-world setting, consistent with results from SUSTAIN.