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P1441 REAL-WORLD OUTCOMES AND DIGITALLY MONITORED QUALITY OF LIFE IN CRIZANLIZUMAB-TREATED PATIENTS WITH SICKLE CELL DISEASE.

Topic: 26. Sickle cell disease

Kim Summers*[1], Orlando Agrippa[1], Kofi Anie[2], Paul Telfer[3], Sanne Lugthart[4]

[1]Sanius Health, London, United Kingdom; [2]London North West University Healthcare Nhs Trust, London, United Kingdom; [3]Queen Mary University Of London, London, United Kingdom; [4]University Hospitals Bristol And Weston, United Kingdom

Background:

Recently, novel disease-modifying treatments for Sickle Cell Disease (SCD) patients have emerged. One therapy is crizanlizumab, which received NICE managed access agreement in 2021. It is currently undergoing an MHRA review of the results from the completed phase-3 STAND trial. With indications of no significant difference in annual veno-occlusive crisis (VOC) rates compared to placebo, there is a critical need for real-world data to provide deeper understanding of the treatment's impact on patient lives.

Aims:

This work sought to characterise the SCD patient experience following initiation of crizanlizumab, analysing side effects, symptoms, healthcare utilisation, and quality of life (QoL) through the EQ-5D-5L (EQ-5D) instrument.

Methods:

An online survey captured feedback on crizanlizumab experiences for 30 SCD patients, 22 of whom were enrolled within a digital data capture ecosystem. This ecosystem included a patient-reported outcomes (PRO) portal for daily EQ-5D scores. Data for a final cohort of 10 patients with EQ-5D scores pre- and post- crizanlizumab initiation were analysed. This was also compared to scores for past (n = 17) and present (n = 63) hydroxyurea (HU)-treated patients, in addition to a general SCD patient cohort (n = 117) within the ecosystem.

Results:

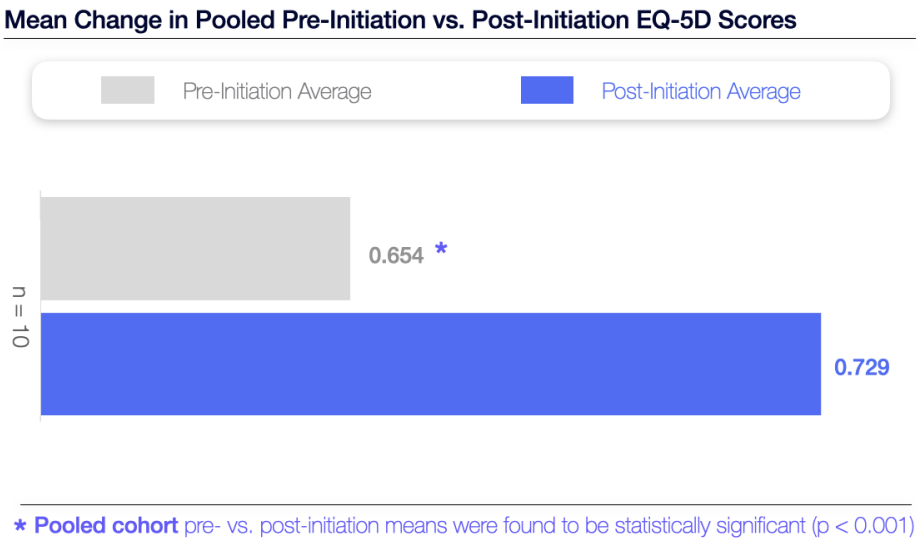
At the point of the survey, the 30 patients had received crizanlizumab for a mean of 6 months (SD 12.7 months), 25 (83%) of whom were co-treated with hydroxyurea, and 1 (3%) with voxelotor. The mean age was 31 (range, 18-60) years, with 23 (77%) females. 24 (80%) patients were of the HbSS genotype. Acute chest syndrome was the most common self-reported comorbidity (30%), followed by leg ulcers (10%), and hypertension (10%).

19 (63%) patients reported side effects, the most being pain (27%), migraine/headache (23%), and fatigue/exhaustion (17%). While 27% of patients saw an improvement in pain, 10% reported a worsening, and 10% either had no change or mixed responses. Fatigue was reported to improve in 10% of patients, worsen in 10%, and remain similar/mixed in 10% of patients. Shortness of breath worsened in 10% of patients.

17 (57%) of patients required admission and 17 (57%) attended A&E since beginning treatment. Although 21 (70%) experienced a VOC since treatment initiation, 18 (60%) patients reported an improvement through reduced VOC frequency (27%), lower severity/intensity (23%), prevented need for hospital care (23%), and reduced VOC duration (7%).

Pooled pre- and post-crizanlizumab EQ-5D scores in the 10 patients showed a significant increase ($p<0.001$). A mean individual improvement of 28% (range, -58-269%) was reported, and 60% of patients demonstrated an increase in EQ-5D post-initiation. Comparison of post-initiation EQ-5D scores with current HU, past HU, and all

other SCD patients in the ecosystem found no significant differences.



Summary/Conclusion: Our data provide the first published snapshot of longitudinal monitoring for EQ-5D in SCD patients treated with crizanlizumab. An overall improvement in patient-reported experiences with VOCs was found following treatment, with a paralleled improvement in QoL scores. While post-treatment initiation EQ-5D scores did not display a significant difference in comparison with other treatments, most patients recorded an improvement from their individual pre-treatment levels through day-to-day remote tracking.

The mixed responses in reported improvements to VOC frequency, severity, and QoL highlight a need to further investigate which specific subgroups may benefit most from crizanlizumab.