ELSEVIER

Blood 142 (2023) 7328–7329

The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

904.OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Unlocking Insights: A Single Centre Patient-Reported Outcome Experience in Patients with Severe Hemophilia a without Inhibitors on Emicizumab

Ingrid Blydt-Hansen, MD[1], *Michelle Bech, NP*[2], *Zainab Al-Housni*[3], *Federico Germini*[3], *Mark W Skinner, JD*[4], *Shannon Jackson, MD FRCPC*[5]

[1] University of British Columbia, Vancouver, Canada
[2] St. Paul's Hospital, Vancouver, Canada
[3] McMaster University, Hamilton, Canada
[4] Institute for Policy Advancement, Washington, DC
[5] University of British Columbia, Vancouver, CAN

Introduction:
Emicizumab (Hemlibra®) prophylaxis has been demonstrated to be more effective at decreasing bleeding episodes than the factor VIII infusions that had previously been used as bleeding prophylaxis. Emicizumab has been available in Canada for persons with hemophilia A with baseline FVIII <1% without inhibitors as a form of prophylaxis since October 2021. Previous work by our team used the Patient-Reported Outcomes, Burdens and Experiences (PROBE) survey to assess outcomes following the initiation of Emicizumab in our local population, which demonstrated a positive overall trend in composite scores generated by the survey following treatment initiation. The PROBE instrument includes the EQ5D5L instrument as a generic quality of life assessment, which consists of 5 dimensions.

Aims:
The explicit aim of this study was to assess changes in EuroQuol-5D-5L (EQ5D5L) index utility scores and dimensions as well as Visual Analog Scores (VAS) scores at 3 and 6 months following Emicizumab introduction to gain insight into the clinical impact beyond bleeding rates.

Methods:
In partnership with the PROBE Investigators, individualized non-identifying survey links were generated by the clinic and provided to all transitioning patients who then consented within the PROBE link to complete the survey. Responses were shared with the clinic and decoded to allow identification. Previously, composite scores for the relevant patients were identified at 0, 3 and 6 months to assess the patient-reported clinical trajectory following Emicizumab introduction. Here, the EQ5D5L scores were analyzed using descriptive statistics and the minimal clinically important difference (MCID) was calculated for each EQ5D5L dimensions (MCID is a change between 2-4) and the VAS (MCID is a change of 10).

Results:
65/85 (76%) of eligible patients ≥ 18 years of age in British Columbia transitioned as of June 1, 2023. 54 patients (mean age 39.3, median 36 with a total age range of 19-59) completed a baseline PROBE survey as part of usual clinical care prior to the Emicizumab transition. 26 patients completed PROBE surveys at 0 and 3 months and 11 patients completed PROBE surveys at 0-, 3- and 6-month intervals. Mean baseline PROBE (/1), EQ5D5L (/1) and VAS (/100) scores were 0.779 (SD 0.15), 0.789 (SD 0.15) and 72.01 (SD 18.65), respectively.

EQ5D5L domains are each scored /5 where higher numbers corresponded to worse outcomes; the 5 domains include mobility, self-care, usual activities, pain/discomfort, and anxiety/depression. At 3 months, the MCID for EQ5D5L domains was met by 1 person in both *usual activities* and *anxiety/depression*. At 6 months, the MCID was met by 1 person in each of the subsections of the EQ5D5L and by 2 individuals in the *usual activities* dimension.

Higher VAS scores correspond to better health. At 3 months, the MCID for VAS was met by 42% (11/26) with the majority (7/11) observed to have a higher VAS score than at baseline. At 6 months, the MCID was met by 45% (5/11) with the majority (4/5) having a higher VAS score than at baseline.

Conclusion:
Within our small cohort, EQ5D5L index utility scores consistently improved at 3 and 6 months from the time of Emicizumab initiation. Of the EQ5D5L domains, the MCID was met at 3 months in both usual activities and anxiety/depression by 1 person

and in all parameters at 6 months by at least 1 person. Analysis of the VAS scores indicated a consistent perceived improvement by 42% at 3 months and in the majority participants at 6 months. Using the EQ5D5L was feasible to better understand the patient experience for those with Hemophilia A without inhibitors following Emicizumab introduction and provides insight into the specific patient-reported outcomes that are impacted by this treatment transition.

Disclosures Germini: *McMaster University:* Current Employment, Ended employment in the past 24 months; *Canadian Venous Thromboembolism and Outcomes Research (CanVECTOR); - Hamilton Health Sciences (HHS):* Research Funding; *Novo Nordisk, Roche, Takeda, Bayer, Pfizer, BioMarin:* Other: Research funding as a co-investigator: institution received research funds from:; *Canadian Association of Emergency Physicians:* Honoraria.

https://doi.org/10.1182/blood-2023-188221