

Health-Related Quality of Life Among Patients with Hemophilia A Receiving Prophylactic Therapy- Patient Reported Outcomes from the Adelphi Hemophilia A Disease Specific Programme™

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Summary

Hemophilia A is caused by a variation in the Factor 8 (F8) gene which is inherited in an X-linked manner.



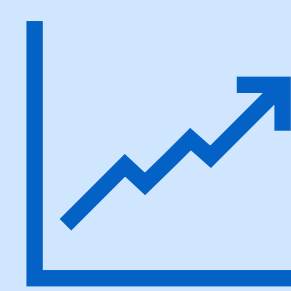
Data were captured via the Adelphi Hemophilia A Disease Specific Programme™ through physician-completed and patient-completed questionnaires



110 Patients were included who were on prophylaxis treatment



This real-world survey is one of the first to examine the changes in PROs dependent on prophylaxis treatment type



Patients receiving emicizumab were associated with more favorable EQ5D utility scores compared to those on other prophylactic treatments, whereas CATCH scores did not differ significantly



Background

- Patient reported outcomes (PROs) are tools used to assess the effect of treatments on the patient's perceived quality of life and their ability to function during different activities¹
- The EQ-5D is a PRO tool where a descriptive system is a preference-based health related quality of life (HRQoL) measure with one question for each of the five dimensions that include mobility, self-care, usual activities, pain/discomfort, and anxiety/depression.² The EQ5D can be converted to a utility score where 0 = death and 1 = full health
- The Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH) is a PRO tool that allows for treatment burden to be presented in domain scores, where 100 is the highest possible and 0 the least possible burden to the patient
- This study aims to use EQ-5D and the CATCH PRO instruments to describe real-world differences in HRQoL among PwHA receiving prophylactic therapy

Methods

- This study utilized data from the Adelphi Hemophilia A Disease Specific Programme™, a point-in-time survey of patients with hemophilia A (PwHA) collected in the United States of America from February 2020 - May 2021. Only those receiving prophylactic treatment at the time of the survey have been included in this analysis.
- The methods of this survey have been previously published and a full description has been validated³
- Participants submitted information using an online survey or pen and paper format
 - This included data on sociodemographic and clinical characteristics, hemophilia A treatments, and quality of life
- The study sample included PwHA who were receiving emicizumab or another form of prophylaxis, such as factor VIII replacement, at the time of data collection
- Bivariate statistics (chi-squared and paired t-tests) were used to assess for unadjusted differences by treatment
- Multivariable regression models were used to assess for differences by treatment type while adjusting for respondent age, disease severity, and inhibitor status

Results

- A total of 110 PwHA who were receiving prophylactic treatment participated in this survey; 75 (68.2%) had severe and 35 (31.8%) had mild or moderate disease. 19 (17.3%) of these patients had active inhibitors at the time of the study. (**Table 1**)
- Forty-seven (42.7%) of patients were receiving emicizumab and 63 (57.3%) were receiving other prophylaxis (**Table 1**)
- Nine (19.6%) of the patients in the emicizumab group had active inhibitors and 10 (15.9%) of the other prophylaxis group also had active inhibitors. There were 33 (70%) patients with severe disease in the emicizumab group and 42 (67%) in the other prophylaxis group (**Table 1**)

Table 1. Physician-Reported Patient Characteristics

	PwHA (n=110 unless stated)	Emicizumab Group (n=47)	Other Prophylaxis Group (n=63 unless stated)
Age at time of study completion, years, mean (SD)	35.4 (12.3)	36.1 (11.0)	34.9 (13.1)
Ethnicity, n (%)			
White/ Caucasian	71 (64.6)	33 (70.2)	38 (60.3)
HA Clinical Severity, n (%)			
Mild/Moderate (≥1% baseline FVIII activity)	35 (31.8)	14 (29.8)	21 (33.3)
Severe (<1% baseline FVIII activity)	75 (68.2)	33 (70.2)	42 (66.7)
Treatment Type, n (%)			
Emicizumab	47 (42.7)	47 (100)	0 (0)
Other Prophylaxis	63 (57.3)	0 (0)	63 (100)
Active inhibitors* n (%)	19 (17.4)	9 (19.6)	10 (15.9)
Employment Status, n (%)			
Working full time	62 (60.2)	28 (63.6)	34 (57.6)
Patient activities**, n (%)			
Swimming	32 (29)	11 (23)	21 (33)
Hiking	29 (26)	15 (32)	14 (22)
Weight lifting	25 (23)	11 (23)	14 (22)
Riding a normal bicycle	23 (21)	10 (21)	13 (21)
Stationary bicycle	22 (20)	12 (26)	10 (16)
Fishing	20 (18)	5 (11)	15 (24)
Yoga	20 (18)	10 (21)	10 (16)

*Active inhibitors: inhibitors which are present at the time of data collection
 **Patient-reported activities that are completed on a regular basis

Results

- The EQ-5D utility score (mean (SD)), was 0.77 (0.23) in the emicizumab group compared to 0.68 (0.27) in the other group, (p = 0.07) (**Figure 1**) Adjusted analyses suggested that patients receiving emicizumab treatment were more likely to have a better EQ-5D score compared to those in the other prophylaxis group (Coefficient of change in EQ-5D 0.10, 95% CI: 0.00 to 0.20, p = 0.05). (**Figure 1**)
- A study found that minimally important difference in EQ-5D scores is 0.074⁴, so although unadjusted analyses did not reach statistical significance, the minimally important difference was met.
- Self-reported EQ-5D Visual Analogue Scores (VAS) were similar for both groups. (**Figure 2**)

Figure 1. Mean EQ5D Utility Score

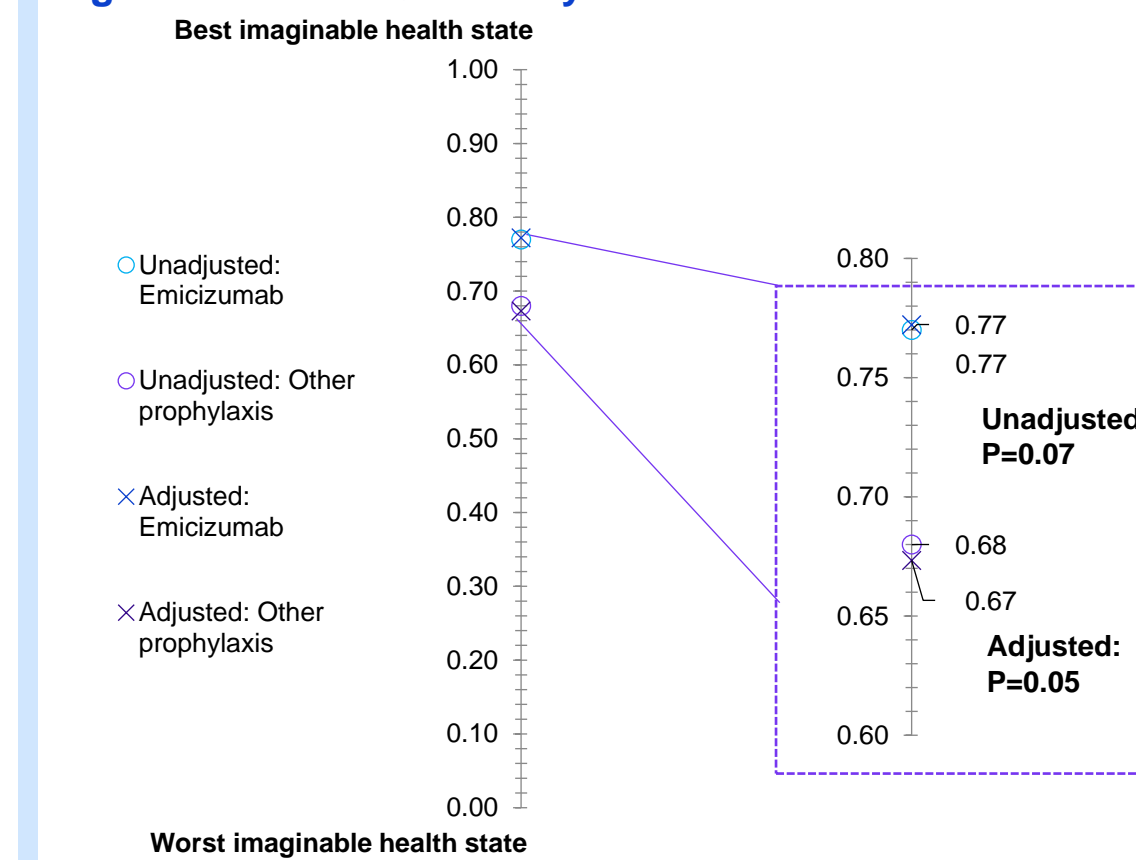
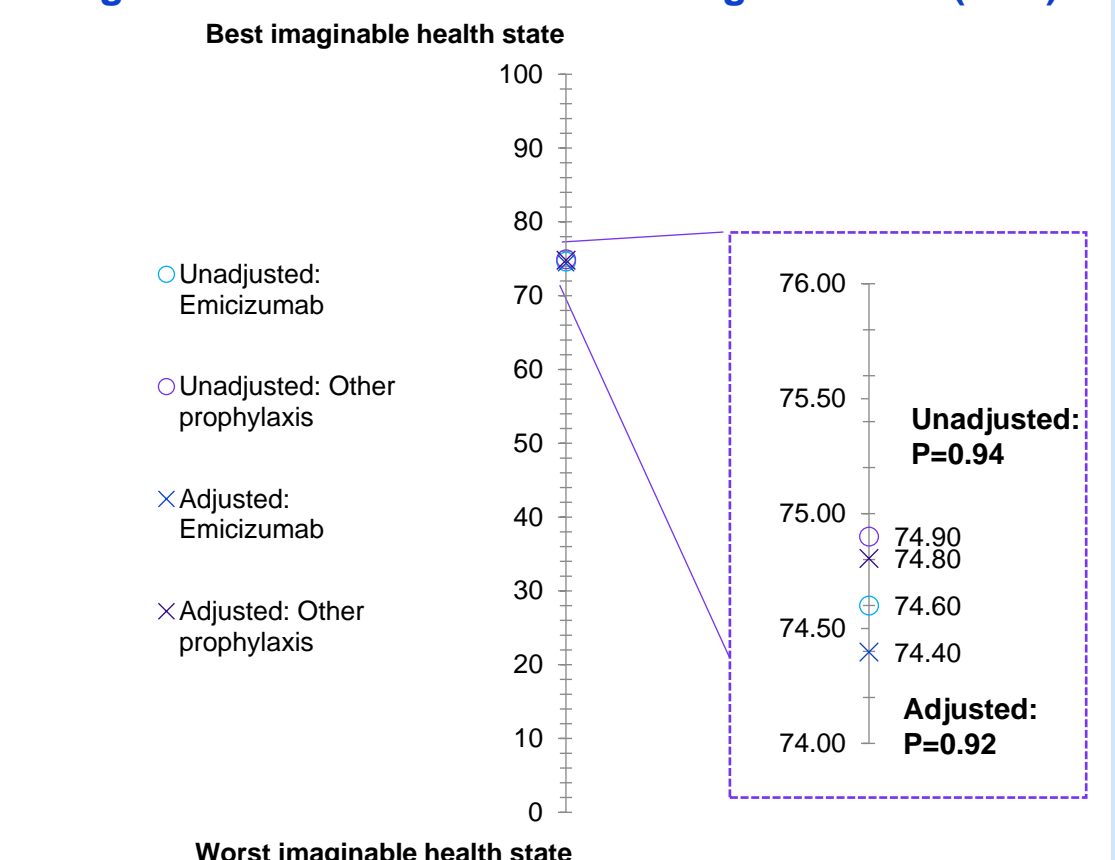


Figure 2. Mean EQ5D Visual Analogue Scores (VAS)

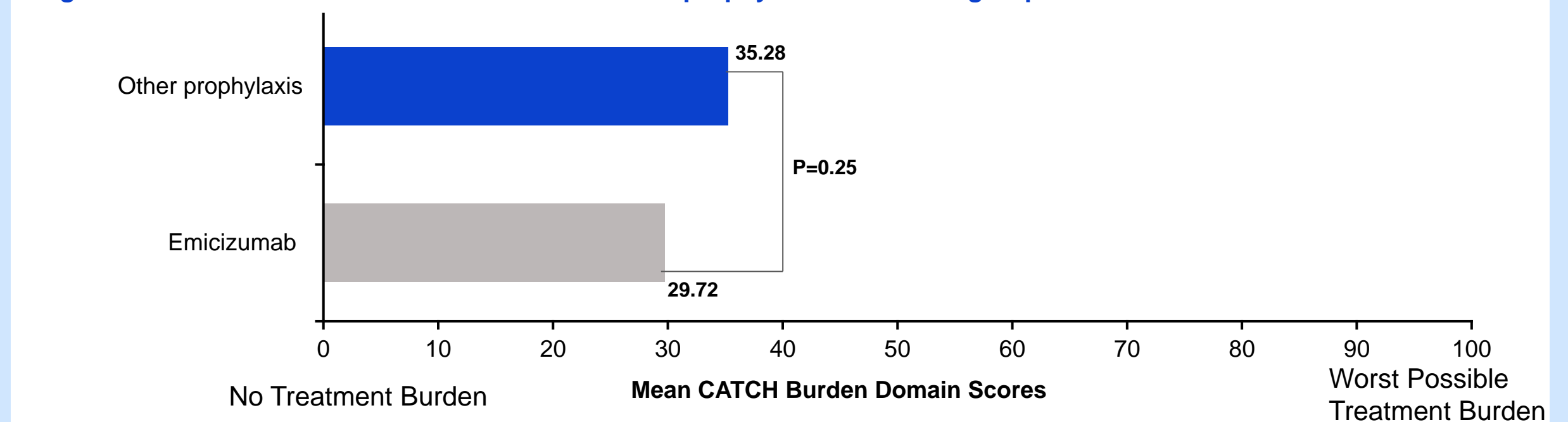


*Adjusted scores controlled for respondent age, disease severity, and inhibitor status

Results

- CATCH treatment burden domain scores (mean (SD)) were 29.72 (22.48) in the emicizumab group, compared to 35.28 (26.89) in the other prophylaxis group, (p = 0.25) (**Figure 3**), however this was not statistically significant
- Adjusted analyses showed that differences in treatment burden scores between PwHA receiving emicizumab and those receiving other prophylactic therapies were not statistically significant (Coefficient of change in CATCH score -6.21 95%CI: -15.46 to 3.05, p = 0.19)

Figure 3. Mean CATCH Burden Domains for different prophylactic treatment groups



Conclusions

- This study describes cross-sectional differences and similarities in the HRQoL of PwHA receiving differing prophylactic therapies
- The adjusted EQ5D utility score of patients who received emicizumab was more favorable when compared to other prophylaxis options, suggesting a score closer to 1 (full health) than patients on other prophylactic treatments. However on the self-reported VAS element, no significant difference was seen
- Although the mean CATCH domains for patients on emicizumab treatment was lower than patients on other prophylactic treatments (closer to 0 which is the least possible burden to the patient), this result was not significant when adjusted analysis was conducted
- Future studies should survey PwHA at multiple time points to assess for changes in each patient's outcomes over time
- It is important to note the disability paradox, where PwHA may have higher than expected EQ5D scores making differences in QoL difficult to ascertain

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Acknowledgments

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