Early patient experiences with emicizumab in the United States: a qualitative study

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Summary

Hemophilia A (HA) can cause serious impairments in quality of life, even with standard factor (F) VIII treatment.[1] Regular intravenous infusions for FVIII prophylaxis can be burdensome and reduce treatment compliance.[2]

Emicizumab is a treatment for HA that works differently from FVIII replacements and is administered subcutaneously.

Researchers interviewed persons and caregivers of persons with HA (PwHA) using emicizumab to better understand their experience, and assessed the usability of the Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH) questionnaire in this sample.

CATCH appears to be a suitable tool for measuring treatment impact in HA. Also, in qualitative interviews, PwHA and caregivers of PwHA treated with emicizumab reported improvements in quality of life versus previous HA treatment.

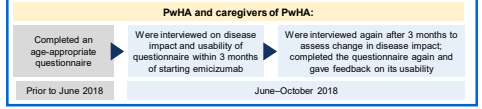
Introduction

- CATCH is a new questionnaire developed to assess the impact of hemophilia and its treatment on PwHA and their families.[3] There are three versions of CATCH: adult, pediatric, and caregiver.
- Adult and pediatric versions of CATCH measure the impact of hemophilia on activities (daily, recreational, and social), work/school, pain, preoccupation, and treatment burden. The caregiver version assesses only preoccupation and treatment burden.
- Emicizumab is a subcutaneously-administered, non-factor VIII treatment for HA. Emicizumab was recently approved for prevention of bleeding (prophylaxis) in PwHA with or without FVIII inhibitors.
- We interviewed PwHA and caregivers with the aim of better understanding the patient/caregiver experience with emicizumab versus previous HA treatment.
- The suitability of CATCH as a measure of hemophilia disease impact and change after starting emicizumab was also assessed.

Methods

- Participants completed either the adult (PwHA aged ≥18 years), pediatric (8–<18 years) or caregiver (caregivers of pediatric PwHA) version of an online questionnaire.
- PwHA and their caregivers were then interviewed between June and October 2018, within 3 months of starting emicizumab and then again 3 months later **(Figure 1).**
- The study was approved by New England Independent Review Board; all participants completed consent or assent prior to participation.

Figure 1. Assessment of experiences with emicizumab and utility of the CATCH.



Results

In total, 15 participants (Table 1) (5 children/teenagers, 4 adults and 6 caregivers) provided responses. FVIII inhibitors were present in 93% of PwHA.

Table 1: Participant baseline characteristics.

Variable	Adult (*n* = 4)	Pediatric (*n* = 5)	Caregiver*(*n* = 6)
Age			
Mean (range)	33 (24–41)	13.4 (10–17)	8.8 (8–10)†
Gender, *n* (%)			
Male	4 (100)	5 (100)	6 (100)
Hemophilia severity, *n* (%)			
Moderate	3 (75)	0 (0)	0 (0)
Severe	1 (25)	5 (100)	6 (100)
FVIII inhibitors, *n* (%)	4 (100)	5 (100)	5 (83)
Bleeds in the past 30 days			
Mean (range)	0.75 (0–2)	0.60 (0–2)	0.67 (0–3)
Bleeds in the past year			
Mean (range)	3.67 (2–5)	3.60 (1–6)	5.00 (0–12)
Joint problems, *n* (%)	2 (50)	4 (80)	3 (50)
Treatment at HTC, *n* (%)	2 (50)	5 (100)	5 (83)

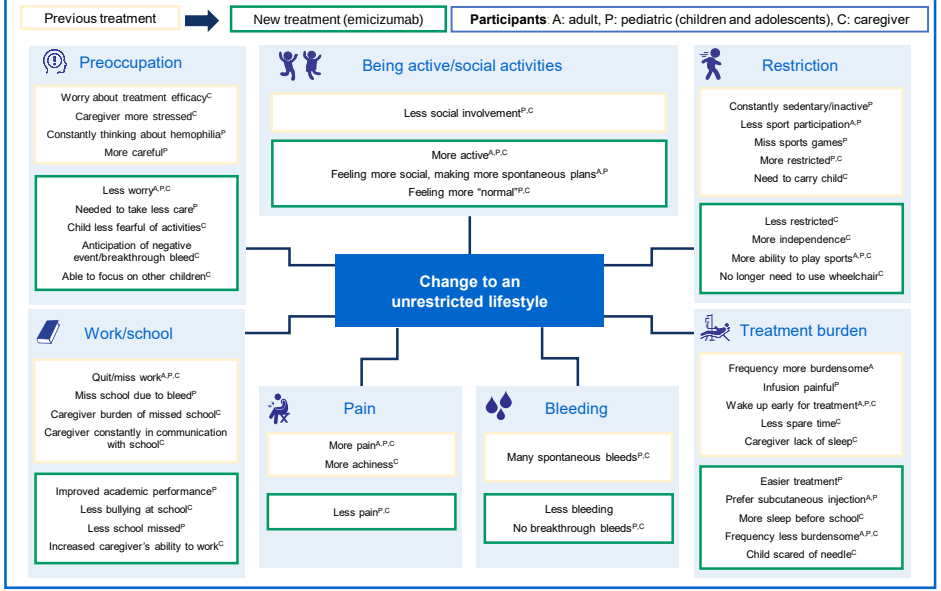
*Characteristics relate to the children of the caregivers. †*n* = 5; data missing for one child.
HTC, hemophilia treatment center; SD, standard deviation.

- Overall, participants gave positive feedback on the survey, finding it understandable and reflective of the changes in their lives after starting emicizumab.
- Participants provided minor suggestions for improvements to the questionnaire, such as clarification and rewording.

Switching to emicizumab treatment was associated with reductions in treatment burden, bleeding, and pain; less restriction and preoccupation; increased physical activity; and better performance at work or school.

- PwHA described "good days" and "bad days" differently when asked about the pre- and post-emicizumab periods.
- Themes associated with switching to emicizumab are shown in **Figure 2**.

Figure 2. Themes associated with switching to emicizumab for treatment of HA, based on qualitative data.



Results

During interviews, participants described previous experiences with "good days" and "bad days" before and after treatment with emicizumab differently.

"Good days"

- Based on experiences with previous HA treatment, most participants described their good days as days without negative events.
- After switching to emicizumab, participants characterized good days by describing positive activities/experiences.

"Bad days"

- Based on experience with previous HA treatment, bad days were mostly associated with the inability to move or leave the house due to a bleed or pain.
- After switching to emicizumab, participants reported that they either no longer experienced bad days, or that the bad days were less bad or not associated with HA.

Conclusions

- Adults and children with HA and caregivers reported overall decreased disease impact and treatment burden, and improvements in several aspects of health-related quality of life when taking emicizumab compared to previous treatment.
- In interviews, PwHA reported less bleeding and pain, increased activity and a greater feeling of living a "normal life" after switching to emicizumab.
- CATCH is a relevant tool to assess the benefit of a new therapy such as emicizumab for HA treatment.

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References

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Disclosures

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