Self-Reported Prophylaxis Treatment Experience Among Persons Living With Hemophilia A in the US: A Cross-Sectional Analysis of Survey Results Combined With Medical Records

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BACKGROUND

- Hemophilia A is characterized by deficiency of coagulation factor VIII, which may lead to spontaneous or excessive trauma-induced bleeding episodes.[1]
- Prophylactic treatment is essential to prevent bleeding, joint damage, and other hemophilia-related comorbidities.[1]
- Standard of care prophylactic treatments include standard half-life (SHL), extended half-life (EHL), and the more recent non-factor therapies (NFTs).[1,2]
- With the expansion of NFTs, there is limited evidence on patient experience and satisfaction, which are crucial to identify contemporary care gaps and facilitate shared decision making when selecting prophylaxis.

OBJECTIVES

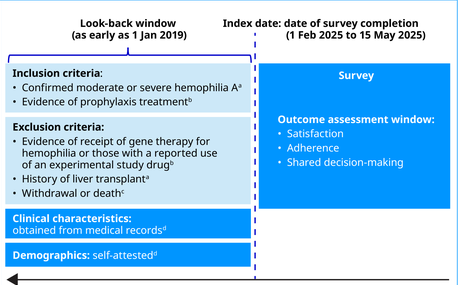
- **Primary:** To describe satisfaction with the current or most recent prophylactic treatment and perceptions of shared decision-making among people with hemophilia A (PwHA).
- **Secondary:** To describe the demographic and clinical characteristics of the study population, and self-reported adherence to treatment.

METHODS

Study Design

- This was a non-interventional, retrospective study consisting of real-world data from longitudinal medical records in the United States retrieved by PicnicHealth and a one-time cross-sectional survey.
- The medical records were produced during routine clinical care of individuals across sites of care and clinical providers (eg, hospitals, outpatient clinical centers, emergency rooms, lab testing sites, and imaging facilities).
- The Hemophilia Patient Experience Survey (HPES) was collected from PwHA between February and May 2025 via the PicnicHealth research platform.
- The index date was defined as the HPES response date (**Figure 1**).

Figure 1: Study design



[a] At any point in patient medical history.
[b] On or after January 1, 2019.
[c] Prior to February 1, 2025
[d] Covariate assessment windows were defined by index dates. (1) At any time in patient history to index date: sex, race, ethnicity, age at diagnosis, time between diagnosis and index, hemophilia A severity, prevalent factor VIII inhibitor disorder status, prevalent arthropathy. (2) At index date: age at index. (3) Within 1 year prior to index date: geography of care sites, presence of chronic pain. The assessment timeframe for baseline annualized bleed rate was in the year prior to the hematology visit that occurred closest to the survey response.

Participant Population

- The study included people with a confirmed diagnosis of moderate or severe hereditary hemophilia A in the medical records and who had evidence of hemophilia prophylaxis treatment on or after January 1, 2019.
- Only the subset of participants who completed the HPES were included in the study.
- Exclusion criteria included evidence of receipt of gene therapy for hemophilia or those with a reported use of a study drug from a clinical trial on or after January 1, 2019, history of liver transplant at any time, and withdrawal or death prior to February 1, 2025.

Study Assessments

- The HPES included 9 questions on satisfaction with various aspects of current/most recent prophylactic treatment: effectiveness, side effects, cost, ease of preparation and administration, frequency of administration, pain upon administration, interference with daily activities, anxiety related to administration, and overall satisfaction, each measured on 5-point Likert scales (from "Very Dissatisfied" to "Very Satisfied").
- In addition, the HPES included questions on the perceptions of shared patient/physician decision-making and patients' self-reported adherence to current/most recent prophylactic treatment.
- Demographic characteristics were self-attested, whereas clinical characteristics were abstracted from medical records.
- Data were summarized descriptively.

RESULTS

Baseline Demographics and Clinical Characteristics

- 334 individuals with moderate or severe hemophilia A who had taken prophylaxis treatment since January 1, 2019, were identified on the PicnicHealth research platform.
  - Of those, 305 (91%) met the full eligibility criteria, and 193 (63%) responded to the HPES and constitutes the study cohort.
- Baseline demographics and clinical characteristics of participants in the study and in subsets according to treatment class are shown in **Table 1**.
- The majority (92%) of participants responded they were currently on prophylaxis treatment and had initiated their current or most recent prophylaxis treatment a median (range) of 36 (0–359) months prior to the HPES survey (60 [0–358] months SHL; 20 [0–301] months EHL; 46 [0–359] months NFT).
- Currently or most recently prior to index date, 35 (18%) in the cohort received SHL, 52 (27%) EHL, and 106 (55%) NFT.
- In the study cohort, 86% had severe hemophilia A (86% SHL; 81% EHL; 89% NFT).
- The 151 participants in the study cohort with ≥180 days of continuous prophylaxis use in the 1 year prior to the index date had a mean (SD) annualized bleeding rate of 0.82 (1.66), with comparable values observed across treatment classes.

Satisfaction With Current/Most Recent Prophylactic Treatment

- The results of the satisfaction survey are shown in **Figure 2**.
- When asked about overall satisfaction with prophylaxis, 82% of the study cohort, 77% of SHL, 79% of EHL, and 86% of NFT participants reported being satisfied/very satisfied.
- In the study cohort, 83% of PwHA were satisfied/very satisfied with the effectiveness of their prophylactic treatment in preventing bleeding whereas 16% were dissatisfied/very dissatisfied with the anxiety related to administering prophylaxis, 15% with cost, and 10% with pain upon prophylaxis administration.
- In the SHL cohort, 80% were satisfied/very satisfied with prophylactic effectiveness, whereas 29% were dissatisfied/very dissatisfied with anxiety related to prophylaxis administration, and 23% with both administration frequency and cost.
- In the EHL cohort, 87% were satisfied/very satisfied with side effects whereas 15% were dissatisfied/very dissatisfied with both cost and treatment interfering with work, school, travel, and social activities, and 12% with anxiety related to prophylaxis administration.
- In the NFT cohort, 85% were satisfied/very satisfied with effectiveness whereas 13% were dissatisfied/very dissatisfied with administration anxiety, 12% with pain, and 11% with cost.

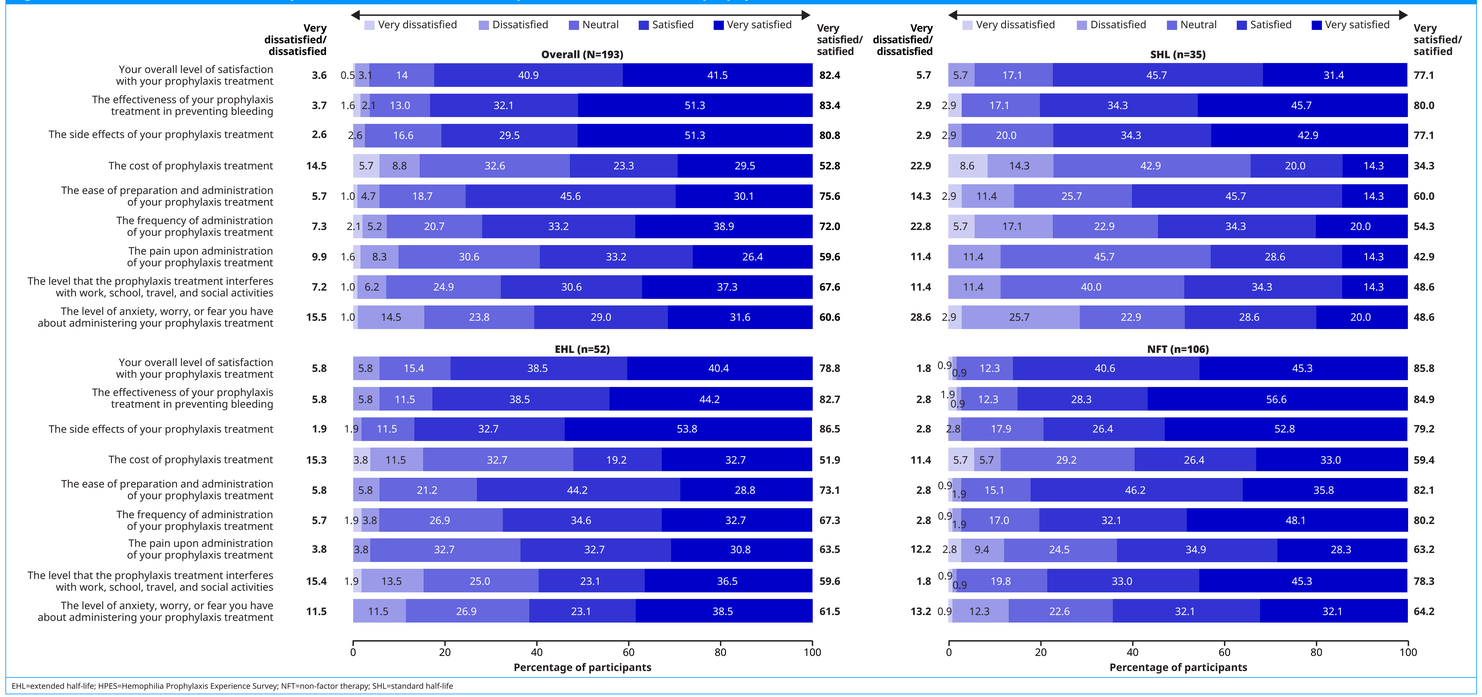
Perceptions of Shared Decision-Making

- Of HPES respondents, 71% had changed prophylaxis ≥1x prior to the index date.
- Decision-making in choosing/changing treatment was reported as doctor-led in 16% of cases, patient-led in 29%, and shared equally in 55% in the study cohort (**Table 2**).
- Shared decision-making was least frequently reported among the SHL group (40%) and most frequently reported in the NFT group (60%).

Adherence to Prophylactic Treatment

- Among respondents, 67% in the study cohort, 54% in the SHL, 73% in the EHL, and 69% in the NFT cohorts reported rarely or never/almost never missing or delaying prophylaxis (**Figure 3**).
- Often/very often missing or delaying prophylaxis was reported by 7% in the study cohort, 17% in the SHL, 2% in the EHL, and 7% in the NFT cohorts.

Figure 2: HPES satisfaction: Please rate your (current) satisfaction with your current/most recent prophylaxis treatment



EHL=extended half-life; HPES=Hemophilia Prophylaxis Experience Survey; NFT=non-factor therapy; SHL=standard half-life

Table 1: Baseline demographics and clinical characteristics

Characteristic	Overall N=193	SHL n=35	EHL n=52	NFT n=106
Age[a], n (%)				
<12 y	21 (11)	2 (6)	2 (4)	17 (16)
12–17 y	12 (6)	3 (9)	2 (4)	7 (7)
≥18 y	160 (83)	30 (86)	48 (92)	82 (77)
Mean age (SD)[a], y	33 (15)	34 (14)	36 (13)	32 (16)
Median age (range)[a], y	35 (4–80)	35 (10–71)	36 (11–68)	35 (4–80)
Sex, n (%)				
Female	1 (1)	1 (3)	0	0
Male	192 (99)	34 (97)	52 (100)	106 (100)
Race, n (%)				
American Indian or Alaska Native	3 (2)	1 (3)	0	2 (2)
Asian	4 (2)	1 (3)	2 (4)	1 (1)
Black or African American	18 (9)	2 (6)	7 (13)	9 (8)
White	141 (73)	26 (74)	36 (69)	79 (75)
Other/unknown	27 (14)	5 (14)	7 (13)	15 (14)
Ethnicity, n (%)				
Hispanic or Latino	35 (18)	8 (23)	9 (17)	18 (17)
Not Hispanic or Latino	145 (75)	23 (66)	43 (83)	79 (75)
Unknown	13 (7)	4 (11)	0	9 (8)
Severity of disease, n (%)				
Moderate	27 (14)	5 (14)	10 (19)	12 (11)
Severe	166 (86)	30 (86)	42 (81)	94 (89)
Factor VIII inhibitor disorder, n (%)	48 (25)	8 (23)	6 (12)	34 (32)
Resolved	34 (71)	8 (100)	6 (100)	20 (59)

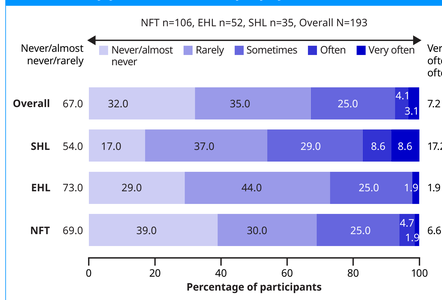
[a] At index date.
EHL=extended half-life; NFT=non-factor therapy; SHL=standard half-life

Table 2: HPES shared decision-making: Please describe how you and your doctor chose your current prophylaxis treatment/most recent prophylaxis treatment

Shared decision-making, n (%)	Overall N=193	SHL n=35	EHL n=52	NFT n=106
Doctor-led	30 (16)	6 (17)	2 (4)	22 (21)
Equal input doctor and patient	107 (55)	14 (40)	29 (56)	64 (60)
Patient-led	56 (29)	15 (43)	21 (40)	20 (19)

EHL=extended half-life; HPES=Hemophilia Patient Experience Survey; NFT=non-factor therapy; SHL=standard half-life

Figure 3: HPES adherence to treatment: How often did you miss or delay your current/last prophylaxis treatment?



EHL=extended half-life; HPES=Hemophilia Prophylaxis Experience Survey; NFT=non-factor therapy; SHL=standard half-life

CONCLUSIONS

- In this study, PwHA were overall satisfied with their current prophylactic treatment.
- However, PwHA were less satisfied with the anxiety, cost, and pain associated with prophylactic administration.
- Evaluating the gaps in treatment satisfaction for PwHA may facilitate meaningful shared decision-making with healthcare providers when selecting prophylaxis treatments.

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Abstract
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