HFXD in America

Predictors of Single Factor Replacement (SFR) Therapy Utilization Among Patients with Hereditary Factor X Deficiency (HFXD): Insights from the HFXD in America Survey

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INTRODUCTION

Hereditary Factor X Deficiency (HFXD) is a rare genetic coagulation disorder leading to delayed hemostasis and potentially life-threatening bleeding symptoms[1,2]

Management of HFXD has historically included the use of prothrombin complex concentrates (PCCs) and fresh frozen plasma (FFP)

Single-factor replacement (SFR), which uses human plasma-derived factor X concentrate, has emerged as a guideline-recommended treatment option for management of HFXD[3]

Despite its clinical advantages, little is known about the real-world factors that influence whether patients with HFXD receive SFR therapy

AIM

The aim of this analysis is to identify predictive factors for SFR therapy in patients with HFXD, based on data from the HFXD in America Survey[4]

1. Herrmann FH, et al. Haemophilia. 2006;12:479-489.
2. Brown DL, et al. Haemophilia. 2008;14:1176-1182.
3. National Bleeding Disorders Foundation Medical and Scientific Advisory Council (MASAC). MASAC Document 290. National Bleeding Disorders Foundation. 2024
4. Branchford B, et al. Blood Coagul Fibrinolysis. 2024;35(3):73-81.

METHODS

The HFXD in America Survey was a prospective, cross-sectional, online survey of patients with HFXD, patient proxies and caregivers conducted from October 2021 to June 2022.

The survey contained questions around patient demographics, diagnosis, disease burden, quality of life, and treatment patterns.

Data from the patient survey were analyzed to identify variables strongly associated with current use of SFR.

To analyze associations with current use of SFR, Pearson's chi-square tests and univariate regressions were conducted to identify significant variables, which were subsequently included in the binary multivariate logistic regression model (Figure 1).

Due to the small sample size, a parsimonious multivariate logistic regression model was developed. Variables were chosen for the multivariate binary logistic regression model if they approached statistical significance ($p<0.05$) from the univariate regression analysis along with baseline patient demographics, age, gender, and race.

Figure 1. Analytical Steps for Identifying Predictors of SFR Use

Data Collection and Initial Analysis
Collect survey data and analyze each question's association with currently receiving SFR

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Correlation and Regression Analysis
Use Pearson's chi-square tests and univariable binary logistic models to identify variables strongly associated with SFR use

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Variable Selection for Modeling
Key variables were chosen if they approached statistical significance ($p<0.05$) in the univariate regression analysis; baseline patient demographics including age, gender, and race were also included in the multivariate binary logistic regression model

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Statistical Assessment and Validation
Assess odds ratios, confidence intervals, and p-values to determine significance

Demographics

- Patients currently receiving SFR were predominantly older in age, female, and non-White than those not currently receiving SFR.
- Those currently receiving SFR were more likely to have ever received 3-4 or 5-6 HFXD treatments, while those not receiving SFR were more likely to have received only 1-2 treatments.
- Among individuals currently receiving SFR, the majority identified bruising/hematoma as one of their most common types of bleeding.

Table 1. Patient-Reported Demographics of those currently receiving SFR vs. no SFR

	Currently receiving SFR (n=16)	Not currently receiving SFR (n=14)
Age, mean (SD)		
Years in age	29.2 (19.1)	19.6 (6.5)
Gender, N (%)		
Female	6 (37.5)	6 (42.9)
Male	10 (62.5)	8 (57.1)
Race, N (%)		
Non-White	11 (68.8)	5 (35.7)
White	5 (31.3)	9 (64.3)
Number of HFXD treatments ever received, N (%)		
1-2	3 (18.8)	7 (50.0)
3-4	8 (50.0)	6 (42.9)
5-6	5 (31.3)	1 (7.1)
Most common bleeding type[a], N (%)		
Bruising or hematoma	9 (56.3)	2 (14.3)
Bleeding type ever experienced[b], N (%)		
Bruising or hematoma	15 (93.8)	10 (71.4)
Heavy menstrual bleeds	8 (50.0)	3 (21.4)
Bleeding within the digestive tract	5 (31.3)	1 (7.1)

[a]The survey question was a "select all that apply" format: "Which types of bleeding events are the most common?"
[b]The survey question was a "select all that apply" format: "What type of bleeding events has your person with a bleeding disorder ever experienced?" The survey question response was included in the univariable analysis but excluded from the multivariable analysis due to its high level of insignificance
Abbreviations: **HFXD** – Hereditary Factor X Deficiency; **SFR** – Single-factor replacement.

RESULTS

Predictors of SFR Use: Univariate Logistic Regression Analysis

Overall , the use of 3-4 different medications compared to those who used only 1–2 medications used in the past year was the only significant variable related to current SFR treatment

Table 2. Predictors of SFR use in HFXD Patients through Univariate Logistic Regression

Survey Question	Variable	OR [95%CI]	P-value
Are you currently receiving conservative management (rest, ice, compression, elevation)?	Conservative Management or Not Currently Using Conservative Management	7.80 [0.80, 75.64]	0.076
On what type of schedule are you currently receiving treatments?	Prior to Surgery or Not Prior to Surgery	5.91 [0.60, 58.48]	0.129
Have you ever experienced bruising/hematoma?	Bruising/Hematoma or No Bruising/Hematoma	6.00 [0.58, 61.84]	0.132
Have you ever experienced heavy menstrual bleeds?	Heavy Menstrual Bleeds or No Heavy Menstrual Bleeds	3.67 [0.73, 18.33]	0.114
Have you ever experienced bleeding within the digestive tract?	Bleeding in the Digestive Tract or No Bleeding in the Digestive Tract	5.91 [0.60, 58.48]	0.129
In the past 12 months, how many different medications do you take for any condition?	3-4 Medications or 1-2 Medications	0.09 [0.01, 0.87]	0.038

Abbreviations: **CI** – Confidence Interval; **HFXD** - Hereditary Factor X Deficiency; **OR** – Odds ratio; **SFR** – Single-factor replacement.

Predictors of the current use of SFR therapy: Multivariate Logistic Regression Analysis

- After adjusting for these variables in the multivariate logistic regression model, the number of HFXD treatments ever received was a significant predictor of current use of SFR therapy, indicating that the odds of receiving SFR therapy increased 3.2 times for each additional HFXD treatment received over a lifetime (Table 3).
- Patients who reported bruising or hematoma as their most common bleeding event had a greater odds to be on SFR compared to those who did not, although this difference was not statistically significant.
- Age, gender, and race did not significantly influence whether a patient received SFR.

Table 3. Predictors of SFR use in Patients with HFXD through Multivariate Logistic Regression

Survey Question	Variable Type[a]	OR [95% CI]	P-value
What is your age in years?	Years	1.00 [0.90, 1.10]	0.98
Which gender do you identify as?	Female or Male	0.44 [0.03, 6.23]	0.55
What is your race?	Non-White or White	9.72 [0.87, 109.0]	0.07
How many HFXD treatments have you ever received?	Each Additional HFXD Treatment Ever Received (Range: 1-6)	3.24 [1.10, 9.52]	0.03
What is your most common type of bleeding event?	Bruising/Hematoma or No Bruising/ Hematoma	6.26 [0.58, 67.99]	0.13

[a]Reference groups based on survey questions: age (years), gender (male), race (white), HFXD treatments ever received (number of HFXD treatments ever received), and most common bleeding type (no bruising/hematoma)
Abbreviations: **CI** – Confidence Interval; **HFXD** – Hereditary Factor X Deficiency; **OR** – Odds ratio; **SFR** – Single-factor replacement.

CONCLUSION

This analysis showed that SFR treatments are more likely to be prescribed after other HFXD treatments with the likelihood increasing with each additional treatment used. Additional insights into this result could help healthcare professionals understand HFXD treatment patterns and outcomes

Further research with larger sample sizes is needed to determine if race or bleeding type are related to treatment choice

Addressing these factors could help healthcare providers enhance HFXD treatment outcomes and improve overall disease management

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