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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Disclosure Statement

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Introduction

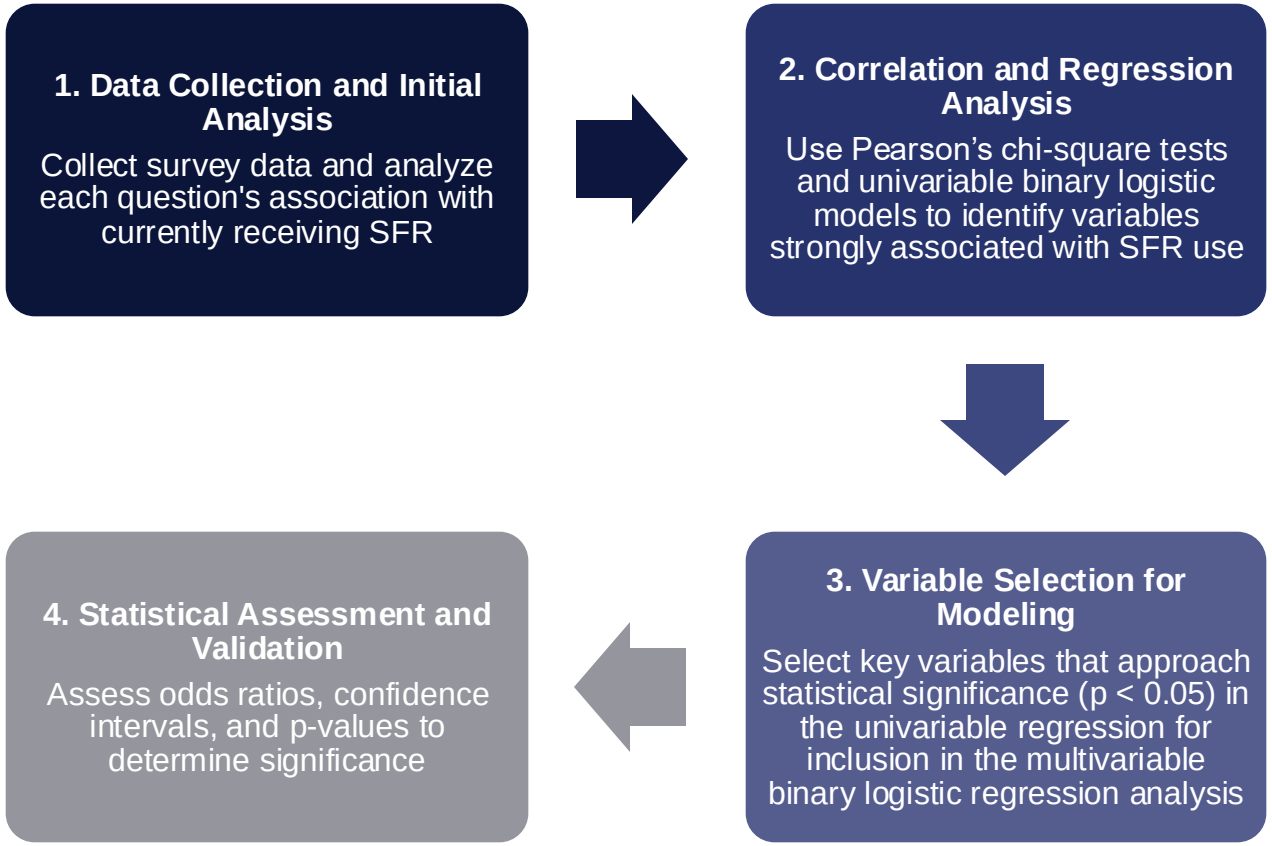
- Hereditary Factor X Deficiency (HFXD) is a rare genetic coagulation disorder leading to delayed hemostasis and potentially life-threatening bleeding symptoms[1,2]
- Treatment options for HFXD include prothrombin complex concentrates (PCCs) and fresh frozen plasma (FFP)
- Single-factor replacement (SFR) is another treatment option recommended for management of HFXD[3]
- Little is known about factors that influence whether patients receive SFR
- 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 and their patient proxies (caregivers) conducted from October 2021 to June 2022[1]
- 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

Analytical Steps for Identifying Predictors of being on SFR



1. Branchford B, et al. *Blood Coagul Fibrinolysis.* 2024;35(3):73-81
HFXD: Hereditary Factor X Deficiency; SFR: Single-factor replacement

Baseline Demographics – Currently receiving SFR vs no SFR

- 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

	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 (%)		
Male	6 (37.5)	6 (42.9)
Female	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
HFXD: Hereditary Factor X Deficiency; SFR: Single-factor replacement

The number of HFXD treatments ever received was a significant predictor of current use of SFR therapy

HFXD in America

Multivariable Logistic Regression Analysis Results

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)

- The odds of receiving SFR therapy increase 3.2 times for each additional HFXD treatment received over a lifetime
- Age, gender, and race did not significantly influence whether a patient received SFR

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

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Conclusion

- This analysis showed that SFR treatments are more likely to be prescribed after other HFXD treatments. Additional insights into this result could help healthcare professionals understand HFXD treatment patterns and outcomes
- Further research with larger sample sizes is needed to identify barriers to SFR treatment including perspectives of both healthcare professionals and patients
- Addressing these factors could help healthcare providers enhance HFXD treatment outcomes and improve overall disease management

HFXD: Hereditary Factor X Deficiency; SFR: Single-factor replacement

Questions?