HFXD in America

Welcome to the HFXD in America survey. Thank you for taking the time to complete this survey that will help assess the patient and caregiver experience with hereditary factor X deficiency. *Please complete this survey only once.*

This survey will take approximately 8-10 minutes to complete.

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* 1. Are you a primary caregiver of a person diagnosed with hereditary factor X deficiency?

- Yes
- No

TITLE: Hereditary Factor X Deficiency (HFXD) in America: Quality of Life and Treatment Experience in Patients with Hereditary Factor X Deficiency (HFXD)

PROTOCOL NO: P21.004.BPL
IRB Protocol # 20214176

SPONSOR: Bio Products Laboratory

STUDY-RELATED PHONE NUMBER: 919-602-0283

INFORMED CONSENT

You are invited to participate in a research study being conducted by Bio Products Laboratory (BPL). The purpose of the study is to assess the patient and caregiver experience with hereditary factor X deficiency (HFXD), including the path to diagnosis and treatment, specific burdens of disease management, and unmet needs. If you choose to participate in this study, you will be asked to complete an online survey about your experience, as a patient or as a caregiver, about your experience with HFXD. This online survey should take approximately 10-15 minutes to complete. When you finish the survey your participation in this research is complete.

PARTICIPATION

Your participation in this survey is voluntary. You may refuse to take part in the research or exit the survey at any time without penalty or loss of benefits to which you are otherwise entitled. You may refuse to answer any question for any reason. This is not a treatment study. Your alternative is to not participate in this research study.

BENEFITS & RISKS

You are not expected to benefit from this research study. Your responses may help us learn more about this bleeding disorder that can be used to more fully understand its impact on the lives of patients and family members. There are no foreseeable risks involved in participating in this study other than those encountered in day-to-day life. However:

- The possible risks or discomforts of the study are minimal, though you may feel uncomfortable answering some personal or health-related questions.
- Some of the survey questions may be distressing to you as you think about your experiences with this bleeding disorder.
- There is also the possible loss of confidentiality.

You will receive, upon completion of the survey, a digital reward that will be made available at the end of the survey.

CONFIDENTIALITY

The survey will be completed anonymously, no personal identifiable information (PII) will be collected or stored during the survey. Your survey answers will be stored initially with SurveyMonkey.com in a password protected electronic format. Your IP address attached to your Wi-Fi network will only be collected at time of the survey, however it will not be stored once the survey is completed and will not be linked to the final survey data. Only the deidentified data related to the survey answers will be downloaded, along with that from many other research subjects, into an anonymous dataset for analysis.

At the completion of the survey, your email address will be collected for compensation purposes only. It will not be linked to the final survey data and will be stored on a different server not accessible by the study team.

COMPENSATION

You will receive $15 for completing the Caregiver survey.

Once you have completed the survey you will be asked if you want to redeem your reward. The redeem button will take you to another website where you will be able to provide an email address where a unique reward link will be sent. Using this unique reward link, you can redeem and download your gift card to a list of unique retailers or donate to a list of approved charities. This can only be done at the end of the survey and must be done at that time, or you will lose your opportunity for compensation. No names will be collected in this step, and you will not be contacted in any way following redemption of the gift card. There is no cost to your participation.

CONTACT

If you have questions, concerns, or complaints, or think this research has hurt you or made you sick, talk to the research team at the phone number listed above on the first page.

If you have further questions or concerns about your rights as a participant in this study, contact HFXDinAmerica@aesara.com.

This research is being overseen by WCG IRB. An IRB is a group of people who perform independent review of research studies. You may talk to them at 855-818-2289 or researchquestions@wcgirb.com if:

- You have questions, concerns, or complaints that are not being answered by the research team.
- You are not getting answers from the research team.
- You cannot reach the research team.
- You want to talk to someone else about the research.
- You have questions about your rights as a research subject.

* 2. **ELECTRONIC CONSENT:** Please select your choice below. You may print a copy of this consent form for your records. Clicking on the “Agree” button indicates that

· You have read the above information
· You voluntarily agree to participate

- ◯ Agree
- ◯ Disagree

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3. How did you hear about this survey?

- ◯ From a family member or friend
- ◯ From a doctor
- ◯ From a treatment center
- ◯ From a specialty pharmacy
- ◯ From social media
- ◯ From a patient advocacy group (such as the National Hemophilia Foundation)
- ◯ Other (please specify)

4. Are you the caregiver for multiple people with hereditary factor X deficiency? If “Yes”, please specify how many people.

- ◯ No, I am only the caregiver for one person with hereditary factor X deficiency
- ◯ Two
- ◯ Three
- ◯ Four
- ◯ Five or more

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The focus of this survey is on your person's hereditary factor X deficiency, which will be referred to as their bleeding disorder. If you are the caregiver for multiple people with hereditary factor X deficiency: when completing the survey, please concentrate on the person you spend the most time providing care.

5. What is the age (in years) of your person with a bleeding disorder?

6. What is your person with a bleeding disorder's disease severity?

- ◯ Mild
- ◯ Moderate
- ◯ Severe
- ◯ Not Sure

7. What is your relationship to your person with a bleeding disorder?

- ◯ Parent/Guardian
- ◯ Spouse/Partner
- ◯ Sibling
- ◯ Other relative (aunt, uncle, grandparents, etc.)
- ◯ Other (please specify)

8. How long have you been a primary caregiver to your person with a bleeding disorder?

- ◯ Less than 1 year
- ◯ Between 1 to 2 years
- ◯ Between 3 to 5 years
- ◯ Greater than 5 years

9. How would you describe the process of getting your person with a bleeding disorder accurately diagnosed originally (such as going to doctor appointments, receiving tests or labs, etc.)?

- ◯ Very easy
- ◯ Somewhat easy
- ◯ Neither easy nor difficult
- ◯ Somewhat difficult
- ◯ Very difficult
- ◯ I do not recall
- ◯ I was not involved during the diagnosis process

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10. Please describe your person with a bleeding disorder's overall diagnosis journey including experiences with their health care provider.

11. What treatments has the person with a bleeding disorder **ever** received for hereditary factor X deficiency?

- [ ] Blood transfusions
- [ ] Fresh frozen plasma (FFP)
- [ ] Prothrombin complex concentrate or PCCs (Kcentra TM, Beriplex®, Octaplex®, Bebulin®, Profilnine®, FEIBA)
- [ ] Single-factor replacement (Coagadex®)
- [ ] Tranexamic acid (Cyklokapron®, Lysteda®, Novaplus)
- [ ] Aminocaproic acid (Amicar®)
- [ ] Oral contraceptives
- [ ] Conservative management (rest, ice, compression, elevation)
- [ ] Do not recall
- [ ] Other (please specify)
- [ ] None

12. Which are your person with a bleeding disorder currently receiving?

- ◯ Blood transfusions
- ◯ Fresh frozen plasma (FFP)
- ◯ Prothrombin complex concentrate or PCCs (Kcentra TM, Beriplex®, Octaplex®, Bebulin®, Profilnine®, FEIBA)
- ◯ Single-factor replacement (Coagadex®)
- ◯ Tranexamic acid (Cyklokapron®, Lysteda®, Novaplus)
- ◯ Aminocaproic acid (Amicar®)
- ◯ Oral contraceptives
- ◯ Conservative management (rest, ice, compression, elevation)
- ◯ [Insert text from Other]
- ◯ Do not recall
- ◯ None of the above

13. What was the reason for not receiving any treatments after diagnosis?

- ○ The health care provider has not prescribed any treatments
- ○ The health insurance does not cover the treatments
- ○ They did not feel like they need treatment at this time
- ○ They did not have time to receive treatments
- ○ Do not recall
- ○ Other (please specify)

14. On what type of schedule is your person with a bleeding disorder currently receiving treatments?

- ☐ Episodic or on-demand (as needed treatment for acute/active bleeds)
- ☐ Intermittent (periodic) prophylaxis (as needed treatment to prevent bleeds, such as before a sports game)
- ☐ Regular prophylaxis (routine treatment to prevent bleeds, such as monthly, weekly, twice weekly, etc.)
- ☐ Prior to surgery (as needed prior to any invasive surgery)
- ☐ Other (please specify)

15. Please describe how often your person with a bleeding disorder receives regular prophylaxis (routine treatment to prevent bleeds, such as monthly, weekly, twice weekly, etc.).

16. What was the health care provider’s reasoning for starting regular prophylaxis? (routine treatment to prevent bleeds, such as monthly, weekly, twice weekly, etc.)

17. What type of bleeding events has your person with a bleeding disorder **ever** experienced?

- [ ] Bruising/hematoma
- [ ] Nose bleeds
- [ ] Gum bleeds
- [ ] Blood in the urine
- [ ] Heavy menstrual bleeds
- [ ] Bleeding in the joints
- [ ] Bleeding in the muscles (soft tissue bleeds)
- [ ] Bleeding within the skull or brain
- [ ] Bleeding within the digestive tract
- [ ] Other (please specify)

  _____

- [ ] None

18. Which bleeding even is the **most common**?

- ◯ Bruising/hematoma
- ◯ Nose bleeds
- ◯ Gum bleeds
- ◯ Blood in the urine
- ◯ Heavy menstrual bleeds
- ◯ Bleeding in the joints
- ◯ Bleeding in the muscles (soft tissue bleeds)
- ◯ Bleeding within the skull or brain
- ◯ Bleeding within the digestive tract
- ◯ [Insert text from Other]

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19. How would you best describe your experience with your person with a bleeding disorder's **treatments** for hereditary factor X deficiency? Please rate the following statements from ”All of the time” to “None of the time”.

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
I feel like the treatments help control bleeding in my person with a bleeding disorder.	○	○	○	○	○	○
I am satisfied with how the treatments control bleeding in my person with a bleeding disorder	○	○	○	○	○	○
I feel like the treatments have reduced the overall burden of being the primary caregiver	○	○	○	○	○	○

20. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Feel burdened by ordering supplies and preparing/administering bleeding-related medication to your person with a bleeding disorder?	○	○	○	○	○	○
Feel overwhelmed by having to accompany your person with a bleeding disorder to medical appointments?	○	○	○	○	○	○
Feel strained by traveling to and from the hospital?	○	○	○	○	○	○

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21. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Witness your person with a bleeding disorder's pain?	○	○	○	○	○	○
Worry about your person with a bleeding disorder having pain during infusions?	○	○	○	○	○	○
Worry about your person with a bleeding disorder complaining of bleed pain?	○	○	○	○	○	○
Suffer when you see your person with a bleeding disorder in pain and can't do anything about it?	○	○	○	○	○	○
Feel distressed that your person with a bleeding disorder has breakthrough bleeding*?	○	○	○	○	○	○

*any bleeding occurring while being treated

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22. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Feel that you don't have enough time for yourself because of your involvement with your person with a bleeding disorder's bleeding-related care?	○	○	○	○	○	○
Feel stressed between trying to give to your person with a bleeding disorder's bleeding-related care as well as to other family responsibilities, job, etc.?	○	○	○	○	○	○
Give up exercising or going to the gym because of the bleeding disorder caregiving responsibilities?	○	○	○	○	○	○
Feel that your family gives up things because of your person with a bleeding disorder's bleeding-related care?	○	○	○	○	○	○

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23. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Worry about the impact a bleeding disorder has on your other family members?	○	○	○	○	○	○
Feel that you don't have much time left over for other family members after caring for your person with a bleeding disorder?	○	○	○	○	○	○
Feel stressed as a family because of caregiving demands?	○	○	○	○	○	○
Feel strained with your spouse/partner because of caregiving demands?	○	○	○	○	○	○

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24. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Experience fatigue because of your person with a bleeding disorder's bleeding-related care?	○	○	○	○	○	○
Have sleepless nights because of your person with a bleeding disorder's bleeding-related care?	○	○	○	○	○	○
Feel tired emotionally and physically because of caring for a bleeding disorder, like a big heavy blanket was on you?	○	○	○	○	○	○
Experience changes in your appetite due to bleeding-related care?	○	○	○	○	○	○
Feel your health suffered because of your involvement with your person with a bleeding disorder's bleeding-related care?	○	○	○	○	○	○

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25. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Feel like you live on a roller coaster following the ups and downs of your person with a bleeding disorder's health?	○	○	○	○	○	○
Feel you have lost control of your life since your person with a bleeding disorder's diagnosis of hereditary factor X deficiency?	○	○	○	○	○	○
Feel like you are always on edge due to the presence of a bleeding disorder in your life?	○	○	○	○	○	○
Feel that the stress due to a bleeding disorder's bleeding-related care is overwhelming?	○	○	○	○	○	○
Feel a sense of impending doom or like you were waiting for the other shoe to drop?	○	○	○	○	○	○

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26. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Feel the bleeding disorder is causing financial burdens for the family?	○	○	○	○	○	○
Feel that your person with a bleeding disorder's bleeding-related care interfered with your job or daily activities?	○	○	○	○	○	○
Lose time from work because of medical appointments?	○	○	○	○	○	○
Have to take turns going to work with your spouse/partner?	○	○	○	○	○	○
Cut down the hours you work to care for your person with a bleeding disorder?	○	○	○	○	○	○

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27. During the past six months, how often did you…

	All of the time	Most of the time	Some of the time	A little of the time	None of the time	N/A or prefer not to answer
Feel that learning to manage your person with a bleeding disorder has made you feel better about yourself?	○	○	○	○	○	○
Feel that your family has become more compassionate and understanding of others due to your bleeding disorder experience?	○	○	○	○	○	○
Feel that you have become a stronger person after going through this bleeding-related care experience with your person with a bleeding disorder?	○	○	○	○	○	○
Feel that you have developed an inner strength?	○	○	○	○	○	○
Feel that your different sense of perspective makes you better able to solve problems now?	○	○	○	○	○	○

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These following questions are asking about your person with a bleeding disorder:

28. With which gender does your person with a bleeding disorder identify?

- ◯ Female
- ◯ Male
- ◯ Transgender Female
- ◯ Transgender Male
- ◯ Non-Binary
- ◯ Prefer to self-describe: [free-text]
- ◯ Other
- ◯ Prefer not to say

29. What is your person with a bleeding disorder's ethnicity?

- ◯ Hispanic or Latino
- ◯ Not Hispanic or Latino
- ◯ Prefer not to answer

30. What is your person with a bleeding disorder's race?

- ☐ White
- ☐ Black or African American
- ☐ Asian
- ☐ Native Hawaiian or Other Pacific Islander
- ☐ American Indian or Alaskan Native
- ☐ Prefer not to answer

31. Is your person with a bleeding disorder of Mediterranean or Middle Eastern descent? While hereditary factor X deficiency can occur in individuals of any ethnic group, people with Mediterranean or Middle Eastern descent have a higher risk of inheriting factor X deficiency

- ◯ Mediterranean
- ◯ Middle Eastern
- ◯ Neither
- ◯ Prefer not to answer

32. What is your person with a bleeding disorder's employment status?

- ◯ Student
- ◯ Full-Time Employed
- ◯ Part-Time Employed
- ◯ Unemployed
- ◯ Unable to work due to disability
- ◯ Other: [free text]
- ◯ Prefer not to answer

33. What is your person with a bleeding disorder's annual income level?

- ◯ Under $20,000
- ◯ $20,001 - $40,000
- ◯ $40,001 - $60,000
- ◯ $60,001 - $80,000
- ◯ $80,001 - $100,000
- ◯ More than $100,000
- ◯ Prefer not to answer
- ◯ Not Applicable

HFXD in America

These following questions are asking about you, a primary caregiver:

34. What is your age (in years)?

35. With which gender do you identify?

- ◯ Female
- ◯ Male
- ◯ Transgender Female
- ◯ Transgender Male
- ◯ Non-Binary
- ◯ Other
- ◯ Prefer not to say
- ◯ Prefer to self-describe:

36. What is your ethnicity?

- ◯ Hispanic or Latino
- ◯ Not Hispanic or Latino
- ◯ Prefer not to answer

37. What is your race?

- ☐ White
- ☐ Black or African American
- ☐ Asian
- ☐ Native Hawaiian or Other Pacific Islander
- ☐ American Indian or Alaskan Native
- ☐ Prefer not to answer

38. Are you of Mediterranean or Middle Eastern descent? While hereditary factor X deficiency can occur in individuals of any ethnic group, people with Mediterranean or Middle Eastern descent have a higher risk of inheriting factor X deficiency.

- ◯ Mediterranean
- ◯ Middle Eastern
- ◯ Neither
- ◯ Prefer not to answer

39. What is your employment status?

- ◯ Student
- ◯ Full-Time Employed
- ◯ Part-Time Employed
- ◯ Unemployed
- ◯ Unable to work due to disability
- ◯ Prefer not to answer
- ◯ Other (please specify)

40. What is your annual income level

- ◯ Under $20,000
- ◯ $20,001 - $40,000
- ◯ $40,001 - $60,000
- ◯ $60,001 - $80,000
- ◯ $80,001 - $100,000
- ◯ More than $100,000
- ◯ Prefer not to answer

Thank you for completing this survey!

If your person(s) with a bleeding disorder has not yet completed the patient survey, please direct them to our study website to complete this survey for an additional reward.

If your person(s) with a bleeding disorder is not able to complete the patient survey independently, you may assist them or you may complete the survey as a proxy. You can access the patient proxy survey through the same patient survey link.