Which characteristics can predict the well-being of people with hereditary factor X deficiency?

This plain language summary reflects the content of the following scientific abstract:

Predictors of hemophilia well-being index scores among patients with hereditary factor X deficiency (HFXD): insights from the HFXD in America survey

View poster based on the scientific abstract

How do you say the medical terms used in this summary?

Deficiency
<deh-FIH-shun-see>

Factor X
<FAK-ter-ten>

Hemophilia
<HEE-moh-FIH-lee-uh>

Hereditary
<heh-REH-dih-tayr-ee>

Prophylaxis
<proh-fuh-LACK-suhss>

Glossary

- **Clotting factors:** Proteins in the blood that help to form clots and stop bleeding. Each clotting factor is numbered from 1 to 13, written as Roman numerals (I–XIII).
- **Factor X:** A clotting factor (protein) in the blood that is needed for the blood to clot properly. 'X' is pronounced 'ten'.
- **Gene:** A section of deoxyribonucleic acid (DNA for short) arranged in bundles called chromosomes. DNA is the genetic material inside cells that is passed from parents to their biological children. Chromosomes are found within each cell of the body. They contain instructions for how every cell in the body develops and functions.
- **Hemophilia:** A rare inherited condition where the blood clots more slowly than usual.
- **Hemophilia Well-being Index (HWBI for short):** A questionnaire used by researchers to understand the well-being of people with hemophilia. Responses are summarized as a score to help researchers understand how the condition impacts well-being.
- **Hereditary factor X deficiency (HFXD for short):** An ultra-rare inherited condition where the blood clots more slowly than usual. It can be categorized as mild, moderate, or severe.
- **Ultra-rare condition:** A condition affecting fewer than 1 in 50,000 people. In the USA, the Orphan Drug Act defines a rare disease as a condition affecting fewer than 200,000 people in the USA.
- **Well-being:** How a person feels about themselves and their life.

Date of summary: March 2025 Survey start date: October 2021 Survey end date: June 2022

What are the key takeaways of the research?

- Researchers assessed online survey responses from 30 people with **hereditary factor X deficiency (HFXD for short)** in the USA. They wanted to find characteristics that could predict people's **well-being**.
- Researchers found that some types of bleeding symptoms people experienced were related to having a lower quality of life.
  - Having frequent severe bleeding or a family member with a history of bleeding symptoms was related to having a lower quality of life.

What is HFXD?

- **HFXD** is an **ultra-rare** blood clotting disorder.
  - It is caused by a change to a **gene** that is passed down in families, from biological parents to children. This is why it is called a hereditary condition.
  - It affects between 1 in 500,000 and 1 in a million people worldwide.
  - Diagnosing **HFXD** can be challenging, especially if a person has mild symptoms.
- **Factor X** is the 10th **clotting factor**. It is either missing or reduced in people with **HFXD**.
  - People with **HFXD** may bleed more often or for longer than most other people. This bleeding can be life-threatening.
- Having **HFXD** can affect a person's quality of life. For example, they may feel anxious or worried about bleeding. They may need to avoid taking part in risky activities like contact sports.
  - People with **HFXD** might have to miss school or work due to their condition or treatment.

How is HFXD treated?

- The types of treatment, and how often people with **HFXD** need to have treatment, will depend on how severe the condition is.
  - If a person has mild symptoms that aren't causing problems, they may not need any treatment.
  - People with mild or moderate **HFXD** often receive treatment as needed (known as on demand) if bleeding starts.
    - They may also receive treatment routinely to help prevent bleeding (known as prophylaxis).
    - They may also receive treatment before an operation or participating in certain sports.
  - People with severe **HFXD** usually need regular prophylaxis treatment to replace the missing or reduced **factor X** in their blood.
- Treatments for **HFXD** aim to replace the missing or reduced **factor X** in a person's blood.
  - Most treatment options are made from donated human blood – specifically from plasma, the straw-colored fluid that blood cells are carried in. Plasma contains **clotting factors**.
    - These blood products are treated during the manufacturing process to eliminate any known virus such as hepatitis or HIV.

How is well-being assessed in people with HFXD?

- Researchers can look at the **well-being** of people with **HFXD** using a questionnaire called the **Hemophilia Well-being Index (HWBI for short)**.
  - **Hemophilia** is another rare inherited blood clotting disorder.
  - There is no specific questionnaire designed for people with **HFXD** because it is so rare. Therefore, researchers adapted this questionnaire for people with **HFXD**.
- The **HWBI** is a questionnaire that looks at a person's **well-being** in 8 categories:

- Each category is scored from 1 to 4, with a total summary score of 32.
  - A higher **HWBI** score suggests better **well-being**.

What was the aim of the research?

- Researchers wanted to find characteristics that can help predict the **well-being** of people with **HFXD** in the USA.
  - Understanding people's **well-being** can help researchers and healthcare professionals to understand how effective treatments for **HFXD** are, and how **HFXD** affects the lives of people with the condition and their caregivers.
- Researchers looked at information collected in the '**HFXD** in America' survey. This was an online survey of people with **HFXD** and caregivers of people with HFXD.
  - The survey took place between October 2021 and June 2022.
  - It was developed by researchers to better understand the impact of **HFXD** on the lives of people living with the condition and their caregivers.
  - It included questions about different aspects of the condition, including its impact on people's quality of life and which treatments they received.

- This summary describes an analysis of information that the survey collected about the following information:

Age

Gender identity

Race

Frequently having severe bleeding symptoms

Having close family members who have experienced bleeding symptoms

- The researchers looked at the relationship between characteristics and a person's **HWBI** score for **well-being**.

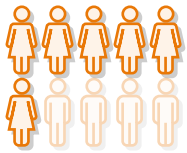
Who took part in the research?

30 people with HFXD

completed the **HFXD** in America survey

27 out of 30 people with **HFXD** completed the **HWBI**

People were aged between 2 and 65 years old (average of **25 years old**)



6 in 10 (60%) identified as **female**

5 in 10 (45%) identified as **White**

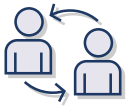
What were the results of the research?

- Researchers found that having a close family member with a history of bleeding symptoms was related to a lower **HWBI** score, suggesting a lower quality of life.
- People with a family history of bleeding symptoms were predicted to score 8 points lower on the **HWBI** scale than those who did not.

- Frequently having severe bleeding was also related to a lower **HWBI** score, suggesting a lower quality of life.
- People who described their most common bleeding symptoms as severe were predicted to score 9 points lower on the **HWBI** scale than those who did not.

Characteristics **predicting** a lower **well-being** score

Frequently having severe **bleeding symptoms**



Having **close family members** who have experienced unusual bleeding

Characteristics **not predicting** a lower **well-being** score

Age (years)

Gender identity (male vs female)

Race (non-White vs White)

What were the main conclusions reported by researchers?

- Researchers found that frequent severe bleeding symptoms and having a family member with a history of bleeding symptoms may relate to how well people cope with **HFXD**.
- Controlling bleeding symptoms is an important way to reduce the impact of **HFXD** on a person's quality of life.

Who sponsored the research and the scientific abstract?

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The scientific abstract was sponsored by Kedrion Biopharma, Inc.

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Where can you find more information?

More information can be found in the poster based on the scientific abstract, which you can access for free here:

View poster

Disclaimers

The purpose of this plain language summary is to help you to understand the findings from recent research. This summary reports the results of a single piece of research. The results of the research may differ from those of other pieces of research. Health professionals should make treatment decisions based on all available evidence, not on the results of a single piece of research.

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