Which characteristics can predict whether people with hereditary factor X deficiency receive single factor replacement therapy?

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

Predictors of single factor replacement (SFR) therapy utilization 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>

▶ **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.
- **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.
- **Single factor replacement (SFR for short) therapy:** A medicine that is a concentrate of factor X.
- **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.
- **US Food and Drug Administration (FDA for short):** A US government agency responsible for protecting public health by ensuring the safety of products and treatments.

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. Additional analysis was done to understand characteristics that could predict how likely someone was to be receiving a treatment called **single factor replacement (SFR for short) therapy**.
- Researchers found that people were more likely to receive **SFR therapy** after they have received other treatments compared to those not currently receiving SFR therapy.

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 different aspects of 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.

What is SFR therapy?

- **SFR therapy** is a type of treatment for **HFXD**.
  - It is a concentrate of **factor X**, made from the donated plasma of healthy people.
  - It is given by an injection through a needle into a vein.
  - The **US Food and Drug Administration (FDA for short)** approved **SFR therapy** for treating adults and children with **HFXD** in 2015. Approval varies by country; please check with your local provider for more details.
- When and how often a person receives **SFR therapy** can vary. It can be given:
  - On demand.
  - As prophylaxis.
  - Before an operation or participating in certain sports.

What was the aim of the research?

- Researchers wanted to find characteristics that can help predict whether people with **HFXD** in the USA are receiving **SFR therapy**.
  - While **SFR therapy** is available as a treatment option, little is known about the characteristics that influence whether people with **HFXD** receive this medicine.
  - Understanding characteristics related to treatment may be beneficial to people with **HFXD** and their caregivers know about treatments for the condition.
- 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

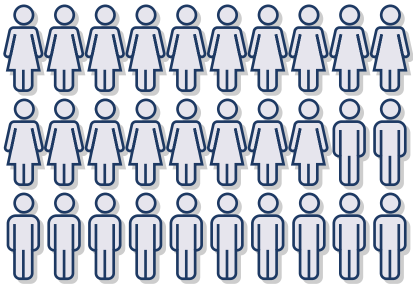
Most common bleeding symptoms

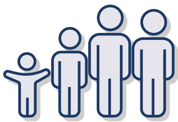
Number of treatments received for **HFXD**

- The researchers looked for characteristics related to a person's likelihood of currently receiving **SFR therapy**.

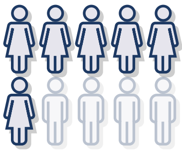
Who took part in the research?

30 people
with **HFXD**
completed the **HFXD**
in America survey

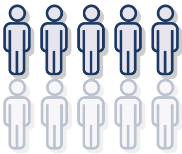




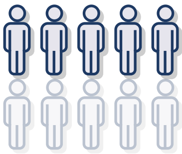
People were aged between 2 and 65 years old (average of **25 years old**)



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



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



5 in 10
(53%) had received **SFR therapy**

What were the results of the research?

- Researchers found that the number of **HFXD** treatments a person had previously received could predict whether they were currently receiving **SFR therapy**.
  - For every extra treatment for **HFXD** that people have received in the past, their chance of needing **SFR therapy** roughly triples.
    - This means that someone who's had multiple treatments in the past for **HFXD** is much more likely to get **SFR therapy** than someone who's only had one or no treatment.
- The other characteristics that the researchers assessed did not predict whether a person was receiving **SFR therapy**.
  - A person's age or gender did not influence whether they received **SFR therapy**.
  - **SFR therapy** was less likely to be prescribed to White people than non-White people, and more likely to be prescribed to people whose most common bleeding symptom was bruising. However, the researchers found that these results were not statistically different. So, this evidence is not strong enough to establish whether or not there is a link between currently receiving **SFR therapy** and a person's race or history of bruising symptoms.

Characteristics **predicting** a person's current use of **SFR therapy**

Number of treatments received for **HFXD** (Between 1 and 6 previous treatments)

Characteristics **not predicting** a person's current use of **SFR therapy**

Most common bleeding symptoms
(bruising vs not bruising)

Age
(years)

Gender identity
(male vs female)

Race
(non-White vs White)

What were the main conclusions reported by researchers?

- Researchers found that people with **HFXD** are more likely to receive **SFR therapy** after other treatments have been tried.
- Larger studies are needed to better understand the barriers that people with **HFXD** face in accessing **SFR therapy** and their views on this treatment.

Who sponsored the research and the scientific abstract?

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Kedrion Biopharma Inc.

Parker Plaza, 400 Kelby Street, 11th Floor, Fort Lee, NJ 07024, USA

Phone (USA): +1-201-242-8900

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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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