

Does marstacimab improve joint health in people with hemophilia A or hemophilia B without inhibitors?

The full title of this abstract is: Joint health in participants with hemophilia A and hemophilia B without inhibitors treated with marstacimab from the phase 3 BASIS trial

SUMMARY DETAILS

Please note this summary only contains information from the scientific abstract

Study number: NCT03938792

Date of summary:
September 2024

Study start date: March 2020
Study end date: April 2023

For more information on this study, go to: <https://clinicaltrials.gov/study/NCT03938792>

KEY TAKEAWAY

What are the key takeaways from this study?

- Participants with hemophilia A or hemophilia B without inhibitors took part in this study.
- Researchers looked at the health and bleeding in the joints for up to 16 months of marstacimab treatment compared with the factor replacement treatment the participants had before marstacimab.

After taking marstacimab for 1 year (12 months):

- There was a small improvement in the participant joint health scores.
- Participants had fewer joint bleeds.
- Participants had fewer bleeds in target joints.
- Participants that had previously been treated on-demand had fewer target joints.
- Participants who took marstacimab for up to 16 months continued to have reduced bleeding events.

PHONETICS

Find out how to say medical terms used in this summary

- Hemophilia** <HEE-moh-FIH-lee-uh>
- Marstacimab** <mar-STAY-see-mab >
- Inhibitor** <In-HIH-bih-ter>
- Prophylaxis** <PROH-fih-LAK-sis>

GLOSSARY

Hemophilia: A medical condition where the blood does not clot properly. Clotting is when liquid blood becomes gel-like, to help to control bleeding. People with hemophilia can bruise easily and will bleed for longer than those without hemophilia.

Clotting factor: Proteins that help the blood bind together into a clot to stop bleeding.

Antibody: Proteins made by the body to fight and destroy infections.

Inhibitor: Types of antibodies produced in the body. Sometimes the body thinks the clotting factor used to treat hemophilia is harmful and produces antibodies (called inhibitors) to destroy it. The inhibitors stop or slow down the clotting factors from working to form a blood clot.

On-demand: Treatment that is only taken when it is needed, such as to stop severe cases of bleeding that are already happening.

Prophylaxis: Treatment or action that is taken regularly to help prevent a disease or condition. In hemophilia, routine prophylaxis treatment is taken to stop bleeds from happening in the first place.

Target joint: A joint where there have been 3 or more bleeds in a 6-month time span.

Tissue Factor Pathway Inhibitor (TFPI): A protein that naturally slows down the process of blood clotting in the body. TFPI works separately from clotting factors.

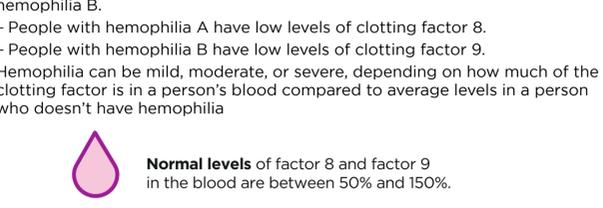
INTRODUCTION

What is hemophilia?

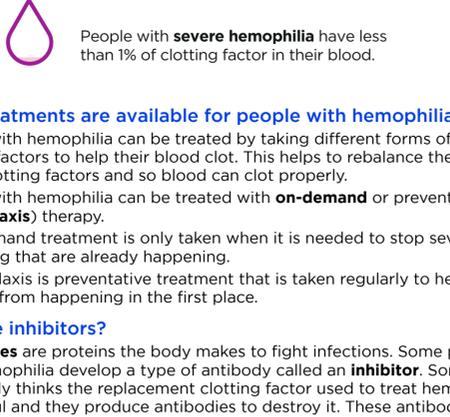
- **Hemophilia** is a medical condition that affects the blood's ability to clot properly to stop bleeding.
 - People with hemophilia can bruise easily and bleed for longer if they cut themselves than people without hemophilia.
 - Bleeding can happen inside the body as well.
 - Bleeding in the joints and muscles is the most common type of bleeding with hemophilia. This bleeding causes swelling and pain, making it difficult to move the joints. If bleeding is not controlled, joints can become damaged over time. Overall, this can lower the quality of life for a person with hemophilia.
- Hemophilia is passed on from biological parents to children through their genes. This means it is an "inherited" condition.
- Hemophilia mainly affects boys and men but there are some cases of women and girls severely affected by hemophilia.
- Traditionally, people with hemophilia have been treated by being given man-made replacement clotting factor to prevent and treat prolonged bleeding.

What are clotting factors?

- **Clotting factors** are proteins that help the blood bind together to form a clot and stop any bleeding. There is a balance between different types of clotting factors. Some clotting factors help the blood to clot. Some clotting factors help the blood to flow freely in vessels. Vessels are tubes in the body that transport blood.
- The balance of these clotting factors helps the blood to clot when it is needed.
 - When the amount of the clotting factors that help the blood to clot is low, the balance of blood clotting and flowing freely changes so blood does not clot properly.
- People with hemophilia have low amounts of certain pro-clotting factors in their blood which means their blood does not clot properly.



- The two most common forms of hemophilia are hemophilia A and hemophilia B.
 - People with hemophilia A have low levels of clotting factor 8.
 - People with hemophilia B have low levels of clotting factor 9.
- Hemophilia can be mild, moderate, or severe, depending on how much of the clotting factor is in a person's blood compared to average levels in a person who doesn't have hemophilia



What treatments are available for people with hemophilia?

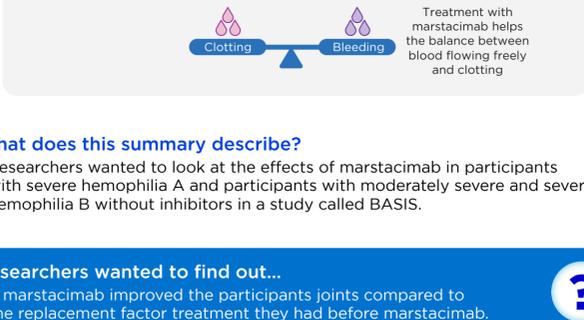
- People with hemophilia can be treated by taking different forms of replacement clotting factors to help their blood clot. This helps to rebalance the amount of blood clotting factors and so blood can clot properly.
- People with hemophilia can be treated with **on-demand** or preventative (**prophylaxis**) therapy.
 - On-demand treatment is only taken when it is needed to stop severe cases of bleeding that are already happening.
 - Prophylaxis is preventative treatment that is taken regularly to help stop bleeds from happening in the first place.

What are inhibitors?

- **Antibodies** are proteins the body makes to fight infections. Some people with hemophilia develop a type of antibody called an **inhibitor**. Sometimes their body thinks the replacement clotting factor used to treat hemophilia is harmful and they produce antibodies to destroy it. These antibodies are called **inhibitors**. The *inhibitors* stop or slow down the replacement clotting factors from working to form a blood clot. People who develop inhibitors need different treatment options.

What is marstacimab?

- **Marstacimab** is an medicine being studied by researchers as a treatment for people with hemophilia A or B.
- Marstacimab is an antibody that works by attaching to a protein in the blood called **Tissue Factor Pathway Inhibitor (TFPI)** for short.
- TFPI is a protein that naturally slows down the process of blood clotting in the body. TFPI works separately from clotting factors. Marstacimab attaches to TFPI to stop it from slowing down blood clotting. This helps allow clotting when needed.
- Because TFPI works separately from clotting factors, marstacimab may help blood to clot in people with hemophilia whether or not they have inhibitors to clotting factors.
- Marstacimab is given once a week as an injection under the skin.



What does this summary describe?

- Researchers wanted to look at the effects of marstacimab in participants with severe hemophilia A and participants with moderately severe and severe hemophilia B without inhibitors in a study called BASIS.

Researchers wanted to find out...

- If marstacimab improved the participants joint compared to the replacement treatment they had before marstacimab.



STUDY DETAILS

Who took part in this study?

Men and boys 12 to 74 years old with severe hemophilia A or moderately severe to severe hemophilia B without inhibitors



The participants continued taking their regular treatment for the first 6 months - either **on-demand** or **prophylaxis**

Previously treated on-demand



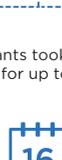
Previously on prophylaxis



Some participants stopped the study early before they started taking marstacimab



The participants then took a single dose of marstacimab 300 mg, then once-weekly injections of marstacimab 150 mg for 12 months



Some participants took marstacimab in the study for up to 16 months



RESULTS

What were the results of this study?

In the participants who previously had on-demand treatment:



The number of bleeding events in joints decreased by 91% (33 bleeds before marstacimab compared to 3 bleeds with marstacimab)



The number of bleeding events in target joints decreased by 92% (23 bleeds before marstacimab compared to 2 bleeds with marstacimab)



Participants had fewer target joints

In the participants who previously had prophylaxis:

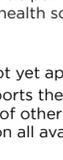


The number of bleeding events in joints decreased by 27% (6 bleeds before marstacimab compared to 4 bleeds with marstacimab)

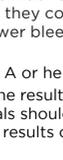


The number of bleeding events in target joints decreased by 25% (3 bleeds before marstacimab compared to 2 bleeds with marstacimab)

In both groups of participants:



There was a small improvement in participants' joint health scores



When the participants continued taking marstacimab for up to 16 months, they continued to have lower bleeding rates

- Marstacimab is not yet approved to treat hemophilia A or hemophilia B.
- This summary reports the results of a single study. The results of this study may differ from those of other studies. Health professionals should make treatment decisions based on all available evidence, not on the results of a single study.

CONCLUSIONS

What were the main conclusions of this study?

- Researchers looked at how well up to 16 months of treatment with marstacimab worked to reduce joint bleeds.
- After 1 year of treatment with marstacimab:
 - Men had fewer bleeding events in their joints than with their previous treatment - a 91% decrease in the on-demand group and a 27% decrease in the prophylaxis group.
 - Men had fewer bleeding events in target joints than with their previous treatment - a 92% decrease in the on-demand group and a 25% decrease in the prophylaxis group.
 - There was a small improvement in joint health scores.
- Men who continued to take marstacimab for up to 16 months still experienced reduced bleeding events.

MORE INFORMATION

Who sponsored this study?

This study was sponsored by Pfizer Inc.

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Pfizer would like to thank everyone who took part in this study.

Where can I find more information?

For more information on this study, please visit: <https://clinicaltrials.gov/study/NCT03938792>

For more information on clinical trials in general, please visit: <https://www.clinicaltrials.gov/ct2/about-studies/learn>

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