



Clinical Trial Results

This summary reports the results of only one study. Researchers must look at the results of many types of studies to understand if a study medicine works, how it works, and if it is safe to prescribe to participants. The results of this study might be different than the results of other studies that the researchers review.

Sponsor: Pfizer Inc.

Medicine(s) Studied: Marstacimab

Protocol Number: B7841003

Dates of Trial: 30 May 2018 to 05 August 2020

Title of this Trial: Study to Evaluate the Long-Term Safety, Tolerability and Efficacy of Subcutaneous Marstacimab in Subjects With Severe Hemophilia

[A Multicenter, Open-Label Study to Evaluate the Long-Term Safety, Tolerability and Efficacy of Subcutaneous PF-06741086 in Subjects With Severe Hemophilia]

Date(s) of this Report: 04 March 2022

— Thank You —

Pfizer, the Sponsor, would like to thank you for participating in this clinical trial and provide you a summary of results representing everyone who participated. If you have any questions about the study or results, please contact the doctor or staff at your study site.

Why was this study done?

Hemophilia is a disease that is “inherited”, which means it runs in families, and while girls and women can have it too, it mainly affects boys and men. People with hemophilia have problems with their blood not clotting. After an injury, someone with hemophilia will bruise more easily and, if cut, will bleed for longer, but not faster. Sometimes bleeding can happen inside the body and this “internal bleeding” can lead to serious problems.

People with hemophilia have low amounts of one of two proteins called factor VIII and factor IX. When that protein that is essential to clotting is either missing or doesn't work properly, they are diagnosed with hemophilia. People with low factor VIII have hemophilia A, while people with low factor IX have hemophilia B. Doctors prescribe medicines called “factor replacement therapy” to help the blood clot in people with hemophilia. These treatments work by replacing clotting factors VIII or IX. People with hemophilia A have the option of using Hemlibra, which is treatment for factor VIII replacement. Some people cannot be treated with factor replacement therapy for long amounts of time as the body can develop “inhibitors” that stop the treatment from working. Thus other types of treatments are needed to help people with hemophilia.

Another way hemophilia may be treated is to stop the protein called “tissue factor pathway inhibitor” (TFPI). TFPI acts like a brake and slows the clotting of blood. Marstacimab (PF-06741086) is a new type of treatment known as a monoclonal antibody. Monoclonal antibodies are copies of certain proteins found in the body that can help treat different conditions. Marstacimab works by attaching to TFPI, which removes the brake and lets the blood clot in people with hemophilia A and B with or without inhibitors.

Marstacimab is given as a subcutaneous injection with a needle just underneath the skin. The medicine eventually enters the bloodstream and is carried around the body where it may help to prevent or reduce the number of bleeding episodes.

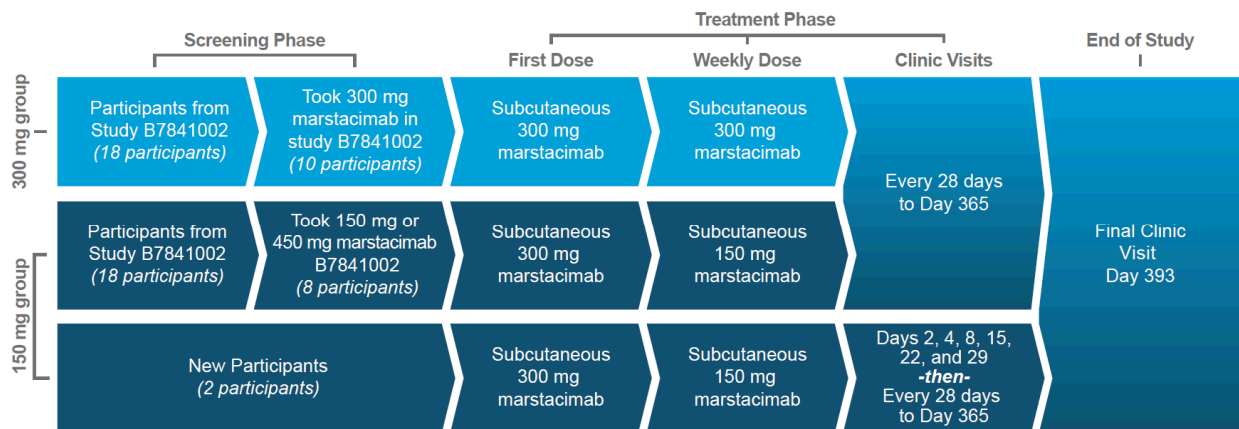
The purpose of this study was to learn more about the safety of Marstacimab at different doses in participants with severe hemophilia.

What happened during the study?

This study evaluated two groups of participants given weekly injections of Marstacimab at different doses. This was done to see if participants had any medical problems while taking Marstacimab and to see if this treatment could prevent bleeding episodes.

The study was open to male participants, between 18 and 75 years old, with a diagnosis of severe hemophilia type A or B, with or without inhibitors. Twenty (20) participants enrolled in the study: 18 of these participants had participated in a previous Marstacimab study (B7841002) and the other 2 participants, both without inhibitors, were taking Marstacimab for the first time.

This was an open-label study, so everyone, including the participants, knew what treatment was given to each group.



While participants were only in the study for 1 year, the entire study took 26 months to complete. The Sponsor ran this study at 11 locations in 9 countries in Africa, Europe, North America and South America. It began 30 May 2018 and ended 05 August 2020. Twenty (20) men and no women participated. All participants were between the ages of 19 and 57 years old.

Participants were to be treated until the end of their 1 year study period. Of the 20 participants who started the study, 18 finished the study. Two (2) participants left before the study was over by their choice or a doctor decided it was best for a participant to stop being in the study.

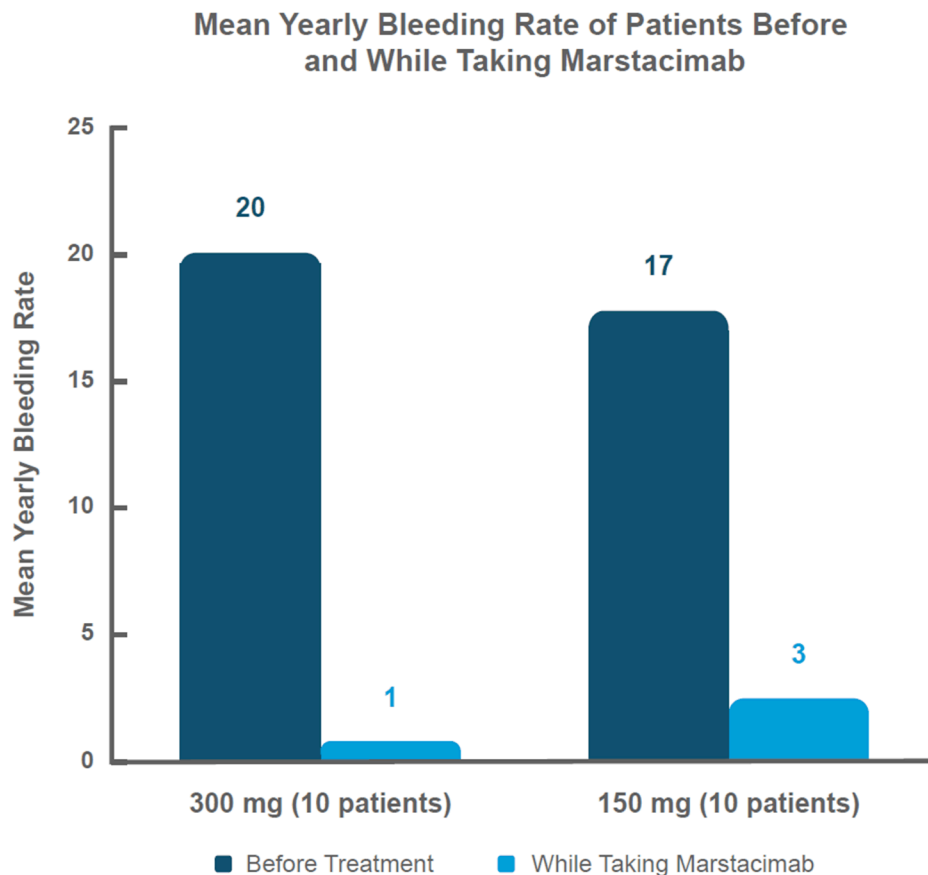
When the study ended in August 2020, the Sponsor began reviewing the information collected. The Sponsor then created a report of the results. This is a summary of that report.

What were the results of the study?

Did participants taking Marstacimab have any medical problems?

To learn more about safety, the study doctors did a number of tests and exams, including lab tests, heart tracings, physical exams, vital signs (blood pressure, breathing rate, temperature, and heart rate) and heart-related medical problems. There were no meaningful changes found on these tests.

Researchers also collected information on the number of bleeding events each participant had during the study. On average, participants had less frequent bleeding over the course of the year with Marstacimab treatment.





This does not mean that everyone in this study had these results. Other studies may produce different results, as well. These are just some of the main findings of the study, and more information may be available at the websites listed at the end of this summary.

What medical problems did participants have during the study?

The researchers recorded any medical problems the participants had during the study. Participants could have had medical problems for reasons not related to the study (for example, caused by an underlying disease or by chance). Or, medical problems could also have been caused by a study treatment or by another medicine the participant was taking. Sometimes the cause of a medical problem is unknown. By comparing medical problems across many treatment groups in many studies, doctors try to understand what the side effects of an experimental drug might be.

Fourteen (14) out of 20 participants in this study had at least 1 medical problem. No participants left the study because of medical problems. The most common medical problems are listed below.

Most Common Medical Problems (Reported by More than 1 Participant)		
Medical Problem	300 mg Marstacimab (10 Participants Treated)	150 mg Marstacimab (10 Participants Treated)
Bleeding into a joint	2 (20%)	1 (10%)
Internal bleeding in a small area	0	2 (20%)

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Were there any serious medical problems?

A medical problem is considered “serious” when it is life-threatening, needs hospital care, or causes lasting problems.

No participants died during the study. One (1) participant in the 300 mg Marstacimab group had 2 serious medical problems: bleeding in the brain and seizure. The researchers did not consider these events to be related to the study drug.

Where can I learn more about this study?

If you have questions about the results of your study, please speak with the doctor or staff at your study site.

For more details on your study protocol, please visit:

www.clinicaltrials.gov

Use the study identifier **NCT03363321**

www.clinicaltrialsregister.eu

Use the study identifier **2017-001255-31**

Please remember that researchers look at the results of many studies to find out which medicines can work and are safe for participants.

Again, thank you for volunteering.
**We do research to try to find the
best ways to help participants, and you
helped us to do that!**