Clinical Study 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 medication works, how it works, and if it is safe to prescribe to patients. The results of this study might be different than the results of other studies that the researchers review.

Sponsor: Pfizer Inc.

Medicine(s) Studied: PF-06755347

Protocol Number: B7801001

Dates of Study: 17 July 2017 to 06 January 2023

Title of this Study: A Study of PF-06755347 in Healthy Men

[A Phase 1, Randomized, Double-Blind, Sponsor-Open, Placebo-Controlled, First-in-Human Trial to Evaluate the Safety, Tolerability, and Pharmacokinetics of PF-06755347 After Single Ascending Intravenous and Subcutaneous Dosing in Healthy Adult Male Participants and Open-Label After Single Subcutaneous Dosing in Male and Female Participants With Persistent or Chronic Primary Immune Thrombocytopenia]

Date(s) of this Report: 30 November 2023

– Thank You –

If you participated in this study, Pfizer, the Sponsor, would like to thank you for your participation.

This summary will describe the study results. If you have any questions about the study or the results, please contact the doctor or staff at your study site.

Why was this study done?

What is primary immune thrombocytopenia?

Primary immune thrombocytopenia (ITP) is a disease where there is very low number of platelets in the blood. Platelets help with blood clotting, which is the body's way of stopping a wound from bleeding too much.

In primary ITP, the body's own immune system attacks the platelets in the blood by mistake. This makes people with this condition have very low number of platelets and bleed or bruise easily.

What is PF-06755347?

PF-06755347 is an injectable medicine that was tested in this study. It is made of parts of proteins called immune globulins that are designed to work with the body's immune system in fighting off diseases. Researchers think that PF-06755347 can help people with primary ITP.

In this study, PF-06755347 was given in 2 injection types:

- into a vein, also known as "intravenously" (IV)
- under the skin, also known as "subcutaneously" (SC)

What was the purpose of this study?

The main purpose of this study was to see if a dose of PF-06755347 IV or SC was safe when given to healthy adult male participants. Researchers also planned to study PF-06755347 in adults with primary ITP. But, researchers were not able to start this part of the study as the Sponsor's plans for the study medication changed.

Researchers wanted to know:

- **Were there safety concerns seen in participants after getting a dose of PF-06755347 IV or SC?**
- **What medical problems did participants have during the study?**

What happened during the study?

How was the study done?

Researchers wanted to see if a dose of PF-06755347 IV or SC was safe when given to healthy adult male participants. This was done by slowly raising the dose of PF-06755347 IV or SC and testing them one by one in different groups of participants.

PF-06755347 was first tested at very low doses:

- 0.01 mg/kg PF-06755347 IV in one group of participants
- 25 mg PF-06755347 SC in another group of participants

Each participant in the IV or SC group received a single dose of their assigned study medication one at a time on Day 1. Researchers checked on the health of the participants after the study medication was given. Before another participant in the same group was given a study medication, those who got PF-06755347 via IV were watched for at least 96 hours, and those who got PF-06755347 via SC were watched for at least 120 hours.

The study began with participants receiving PF-06755347 via IV. The next higher dose level of PF-06755347 was given to the next group of participants if there were no safety concerns seen. The dose could be

lowered in between previous doses for safety concerns. The Sponsor redesigned the study to include dosing via SC when the highest IV dose of PF-06755347 that did not lead to severe medical problems (0.3 mg/kg) did not produce enough PF-06755347 in the blood. In the study:

- there were 6 dose levels tested in the IV group.
- there were 5 dose levels tested in the SC group.

Participants had different tests done and stayed at the study site for about 8 days. Study staff also checked on the participants' health during this period. Participants returned to the study site for up to 7 times until Day 71 for checkup.

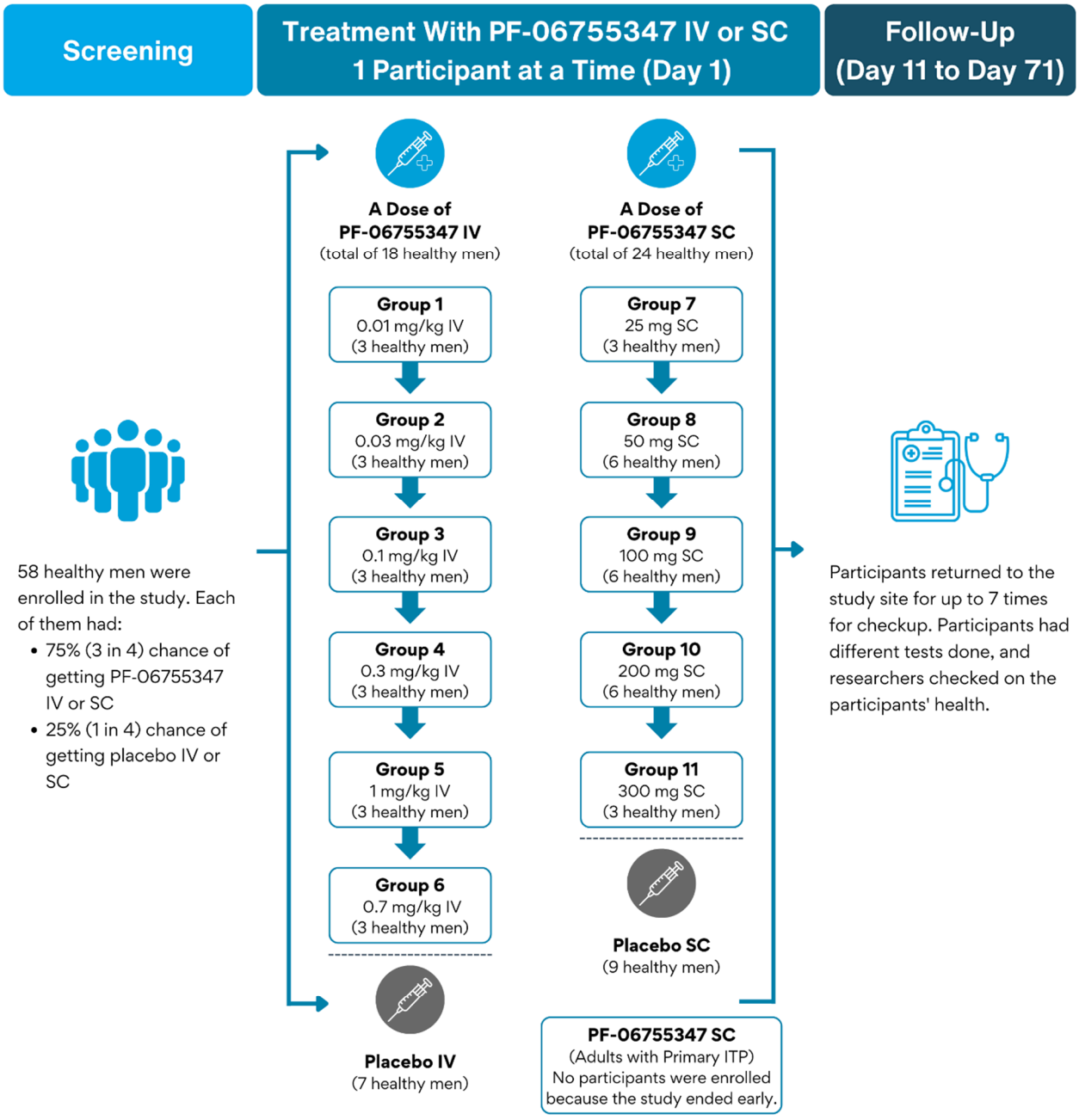
Researchers then compared the results of those who got PF-06755347 via IV or SC with the results of those who got placebo via IV or SC. A placebo does not have any medicine in it, but it looks just like PF-06755347.

Participants and researchers did not know who got PF-06755347 or placebo. This is known as a "blinded" study. Each participant had 75% (3 in 4) chance of getting PF-06755347 IV or SC and 25% (1 in 4) chance of getting placebo IV or SC.

Researchers took samples of blood from participants throughout the study and measured the level of PF-06755347 in them. Researchers also checked the participants' health throughout the study and asked them how they were feeling.

Figure 1 shows what happened during the study.

Figure 1. Overall study design



Where did this study take place?

The Sponsor ran this study at 4 locations in 4 countries (Belgium, New Zealand, United Kingdom, and United States). The Sponsor decided to stop the study in the United States. No participants were dosed at that location.

When did this study take place?

It began 17 July 2017 and ended 06 January 2023.

Who participated in this study?

The study included 58 healthy adult male participants. All participants were between the ages of 18 and 54 years.

- 54 participants finished the study.
- 4 participants left before the study was over by their choice, due to a medical problem, or due to other reasons.

How long did the study last?

Study participants were in the study for up to 71 days. The entire study took about 5 and a half years to complete.

The Sponsor ended the study before participants with ITP could enroll in the study. The study was stopped earlier than planned because of a change in the Sponsor's plans for the study medication. It was not due to safety concerns with PF-06755347.

When the study ended in January 2023, 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?

Were there safety concerns seen in participants after getting a dose of PF-06755347 IV or SC?

To answer this question, researchers reviewed the results of participants' blood tests, vital signs (blood pressure, heart rate, and body temperature), and electrocardiogram (ECG). An ECG is used to see if the heart is beating in a healthy way.

Researchers found a few abnormal results in blood tests, vital signs, or ECG. But, the overall results did not raise any safety concerns when participants were given only one dose of PF-06755347:

- below 0.7 mg/kg in the IV group.
- up to 300 mg in the SC group.

This does not mean that everyone in this study had these results. This is a summary of just some of the main results of this study. Other studies may have different results.

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 unknown 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 effects a study medication might have on a participant.

Overall, 50 out of 58 participants (86%) had at least 1 medical problem.

In the IV group, at least 1 medical problem was seen in:

- 17 out of 18 participants (94%) who got PF-06755347.
- 4 out of 7 participants (57%) who got placebo.

One (1) out of 18 participants (6%) who got PF-06755347 via IV left the study because of a medical problem.

Table 1 shows the most common medical problems seen in 3 or more participants in either IV group:

Below are instructions on how to read Table 1.

Instructions for Understanding Table 1.

- The **1st** column of Table 1 lists medical problems that were commonly seen in 3 or more participants who were given a study medication via IV.
- The **2nd** column tells how many of the 18 participants who got a dose of PF-06755347 via IV (regardless of dose level) reported each medical problem overall. Next to this number is the overall percentage of the 18 participants who got a dose of PF-06755347 via IV (regardless of dose level) who reported the medical problem.
- The **3rd** column tells how many of the 7 participants who got placebo via IV reported each medical problem. Next to this number is the percentage of the 7 participants who got placebo via IV who reported the medical problem.

- Using these instructions, you can see that 6 out of 18 participants (33%) who got PF-06755347 via IV reported headache. Zero (0) out of 7 participants (0%) who got placebo via IV reported headache.

Table 1. Commonly reported medical problems by participants (IV group)

Medical Problem	PF-06755347 IV Overall (18 Participants)	Placebo IV (7 Participants)
Headache	6 out of 18 participants (33%)	0 out of 7 participants (0%)
Queasy feeling or nausea	4 out of 18 participants (22%)	0 out of 7 participants (0%)
Fever, often seen after getting a drug that is designed to affect the immune system, sometimes with lower blood pressure	4 out of 18 participants (22%)	0 out of 7 participants (0%)
Back pain	4 out of 18 participants (22%)	0 out of 7 participants (0%)

Table 1. Commonly reported medical problems by participants (IV group)		
Medical Problem	**PF-06755347 IV Overall (18 Participants)**	**Placebo IV (7 Participants)**
Feeling cold	3 out of 18 participants (17%)	0 out of 7 participants (0%)

In the SC group, at least 1 medical problem was seen in:

- 23 out of 24 participants (96%) who got PF-06755347.
- 6 out of 9 participants (67%) who got placebo.

No participants who got PF-06755347 or placebo via SC left the study because of a medical problem.

Table 2 shows the most common medical problems seen in 3 or more participants in either SC group:

Below are instructions on how to read Table 2.

Instructions for Understanding Table 2.

- The **1st** column of Table 2 lists medical problems that were commonly seen in 3 or more participants who were given a study medication via SC.
- The **2nd** column tells how many of the 24 participants who got a dose of PF-06755347 via SC (regardless of dose level) reported each medical problem overall. Next to this number is the overall percentage of the 24 participants who got a dose of

PF-06755347 via SC (regardless of dose level) who reported the medical problem.

- The **3rd** column tells how many of the 9 participants who got placebo via SC reported each medical problem. Next to this number is the percentage of the 9 participants who got placebo via SC who reported the medical problem.
- Using these instructions, you can see that 11 out of 24 participants (46%) who got PF-06755347 via SC reported redness at the injection site. Zero (0) out of 9 participants (0%) who got placebo via SC reported redness at the injection site.

Table 2. Commonly reported medical problems by participants (SC group)

Medical Problem	PF-06755347 SC Overall (24 Participants)	Placebo SC (9 Participants)
Redness at the injection site	11 out of 24 participants (46%)	0 out of 9 participants (0%)
Pain at the injection site	5 out of 24 participants (21%)	0 out of 9 participants (0%)
Feeling tired	4 out of 24 participants (17%)	2 out of 9 participants (22%)

Table 2. Commonly reported medical problems by participants (SC group)		
Medical Problem	**PF-06755347 SC Overall (24 Participants)**	**Placebo SC (9 Participants)**
Back pain	4 out of 24 participants (17%)	1 out of 9 participants (11%)
Headache	4 out of 24 participants (17%)	0 out of 9 participants (0%)
Infection of the nose and throat (cold)	3 out of 24 participants (13%)	0 out of 9 participants (0%)

Did study participants have any serious medical problems?

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

Overall, 1 out of 58 participants (2%) had a serious medical problem. The participant had a fever with very low blood pressure commonly seen after getting a drug that is designed to affect the immune system about 1 hour and 15 minutes after getting PF-06755347 via IV. The participant was given treatment medications right away and recovered on the next day. Researchers believe that this serious medical problem was related to PF-06755347. No participants died during the study.

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.pfizer.com/research/research_clinical_trials/trial_results	Use the protocol number **B7801001**

The full scientific report of this study is available online at:

www.clinicaltrials.gov	Use the study identifier **NCT03275740**
www.clinicaltrialsregister.eu	Use the study identifier **2018-003315-21**

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

Again, if you participated in this study, **thank you** for volunteering. We do research to try to find the best ways to help patients, and you helped us to do that!