CLINICAL TRIAL RESULTS

This summary reports the results of one study investigating whether the study medicine is safe to prescribe to patients. As of the date of this report, this study and its primary study are the only studies to investigate this study medicine in Duchenne Muscular Dystrophy patients. The results reported here might differ from additional studies that may be conducted with the same medicine in the future.

Sponsor: Pfizer, Inc.

Medicine(s) Studied: Domagrozumab

Protocol Number: B5161004

Dates of Trial: 13 October 2016 to 22 November 2018

Title of this Trial: An Open-label Extension Study To Evaluate Safety Of PF-06252616 in Boys With Duchenne Muscular Dystrophy

[A Multicenter, Open-Label Extension Study to Evaluate the Long Term Safety of PF-06252616 in Boys With Duchenne Muscular Dystrophy]

Date of this Report: 9 January 2020

– Thank You –

Pfizer, the Sponsor, would like to thank you and your son 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 son's study site.

WHY WAS THIS STUDY DONE?

Duchenne muscular dystrophy (DMD) is a disease that damages muscles, causing muscle weakness and loss of the ability to walk. DMD is most common in boys.

Domagrozumab is an experimental medicine studied for DMD. Domagrozumab has not been approved for use outside of research studies.

The main goal of this study was to learn more about the long-term safety of domagrozumab in boys with DMD. Researchers wanted to answer this question:

- What medical problems did boys have during the study?

To answer this question, researchers looked at:

- Medical problems that caused the boy to temporarily stop taking domagrozumab, or that caused the dose of domagrozumab to be lowered
- How severe medical problems were, whether the medical problems were related to taking domagrozumab, and whether boys needed to leave the study because of the medical problems
- Lab test findings
- Findings from physical examination, x-rays, and other tests

WHAT HAPPENED DURING THE STUDY?

This study included boys with DMD who participated in a previous study (Study B5161002) with domagrozumab. Certain lab tests were done to make sure the boys were a good fit to join this study.

The previous study (Study B5161002) included 3 treatment groups, and was done in 2 parts:

Group 1:

- Part 1: Domagrozumab starting at 5 milligrams per kilogram of weight (mg/kg), then increased to 20 mg/kg, then to 40 mg/kg
- Part 2: Domagrozumab 40 mg/kg

Group 2:

- Part 1: Domagrozumab starting at 5 mg/kg, then increased to 20 mg/kg, then to 40 mg/kg
- Part 2: Placebo

Group 3:

- Part 1: Placebo
- Part 2: Domagrozumab starting at 5 mg/kg, then increased to 20 mg/kg, then to 40 mg/kg

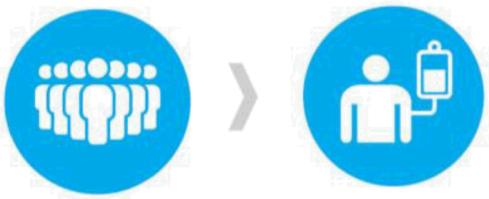
A placebo looks just like the medicine, but doesn't have any medicine in it.

This was an "open-label" study, which means that the boys, their parents, and the researchers knew what medicine and dose the boys received during the study.

All boys in this study received domagrozumab at a dose of 40 mg/kg, given once per month. Domagrozumab was given as an IV infusion, which means that a needle is placed in the vein and the study drug slowly drips into the vein.

When boys came to study visits, lab tests, x-rays, physical examinations, and other tests were done, and they were asked about any medical problems.

The figure on the following page shows what happened during this study.



59 boys from previous study joined this study

Boys received domagrozumab 40 mg/kg, once per month

This study was planned to last up to 4 years. However, the Sponsor decided to stop the study early in November 2018, because results from the previous study did not show that boys benefited from taking domagrozumab.

On average, boys were in the study for about 10 months, but the entire study lasted about 2 years. The sponsor ran this study at 22 locations in Canada, Italy, Japan, United Kingdom, and United States. It began 13 October 2016 and ended 22 November 2018. All 59 participants were boys between the ages of 7 and 11.

Boys were to be treated for up to 4 years. Of the 59 boys who entered the study, no boys (0%) completed it. 55 boys (93%) left the study when the Sponsor decided to stop it early. Additionally, 3 boys (5%) did not complete the study by their choice or their parent's choice, and 1 boy (2%) passed away for a reason not related to taking domagrozumab.

The Sponsor decided to stop the study early in November 2018, because results did not show that boys benefited from taking domagrozumab. After the study ended, the Sponsor then created a report of the results. This is a summary of that report.

WHAT MEDICAL PROBLEMS DID BOYS HAVE DURING THE STUDY?

The researchers recorded any medical problems the boys had during the study. Boys 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 boy 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.

49 out of 59 boys (83%) in this study had at least 1 medical problem. The most common medical problems are listed below.

Most Common Medical Problems (Reported in More Than 5% of Boys)				
Medical Problem	Group 1 (19 Boys treated)	Group 2 (20 Boys treated)	Group 3 (20 Boys treated)	Total (59 Boys treated)
Fall	4 (21%)	3 (15%)	6 (30%)	13 (22%)
Headache	5 (26%)	1 (5%)	5 (25%)	11 (19%)
Common cold	4 (21%)	3 (15%)	4 (20%)	11 (19%)
Vomiting	3 (16%)	2 (10%)	3 (15%)	8 (14%)
Nosebleed	3 (16%)	1 (5%)	2 (10%)	6 (10%)
Fever	2 (11%)	3 (15%)	1 (5%)	6 (10%)
Infection of the nose, throat, or upper airways	3 (16%)	0 (0%)	2 (10%)	5 (9%)
Stuffy nose	1 (5%)	1 (5%)	3 (15%)	5 (9%)
Flu	1 (5%)	4 (20%)	0 (0%)	5 (9%)
Throat pain	0 (0%)	2 (10%)	3 (15%)	5 (9%)
Back pain	2 (11%)	0 (0%)	2 (10%)	4 (7%)

Stomach pain	**2 (11%)**	**0 (0%)**	**2 (10%)**	**4 (7%)**
Diarrhea	**1 (5%)**	**0 (0%)**	**3 (15%)**	**4 (7%)**
Bruise	**1 (5%)**	**1 (5%)**	**2 (10%)**	**4 (7%)**
Cough	**0 (0%)**	**1 (5%)**	**3 (15%)**	**4 (7%)**
Skin wound	**2 (11%)**	**1 (5%)**	**0 (0%)**	**3 (5%)**
Runny nose	**0 (0%)**	**1 (5%)**	**2 (10%)**	**3 (5%)**
Broken bone in leg	**0 (0%)**	**1 (5%)**	**2 (10%)**	**3 (5%)**
Seasonal allergies	**0 (0%)**	**0 (0%)**	**3 (15%)**	**3 (5%)**

Researchers also found that:

- No boys in this study had medical problems that caused them to temporarily stop taking domagrozumab, or that caused the dose of domagrozumab to be lowered.

- Most of the medical problems that happened during this study were considered to be mild in severity. 4 boys (7%) had medical problems that were considered to be severe.

- 6 boys (10%) had medical problems that were considered to be related to taking domagrozumab. These medical problems were considered to be mild in severity.

- 1 boy (2%) left this study and passed away because of a medical problem. This medical problem was not considered to be related to taking domagrozumab.

- Findings from lab studies, physical examination, x-rays, and other tests were used to learn more about the safety of domagrozumab. In this study, no long-term safety issues were associated with taking domagrozumab.

This does not mean that everyone in this study had these results. 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.

WERE THERE ANY SERIOUS MEDICAL PROBLEMS?

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

5 boys in this study (9%) had serious medical problems. 1 boy passed away for a reason not related to taking domagrozumab. The serious medical problems are listed below.

Serious Medical Problems

Serious Medical Problem	Group 1 (19 Boys treated)	Group 2 (20 Boys treated)	Group 3 (20 Boys treated)	Total (59 Boys treated)
Chest pain caused by reduced blood flow to heart	1 (5%)	0 (0%)	0 (0%)	1 (2%)
Syndrome that occurs when fat enters the bloodstream	0 (0%)	0 (0%)	1 (5%)	1 (2%)
Lack of movement in bowels that can cause blockage	0 (0%)	1 (5%)	0 (0%)	1 (2%)

Blockage caused by twisting of the bowels	**0 (0%)**	**1 (5%)**	**0 (0%)**	**1 (2%)**
Appendicitis	**1 (5%)**	**0 (0%)**	**0 (0%)**	**1 (2%)**
Increased level of a type of protein found in muscles of heart	**1 (5%)**	**0 (0%)**	**0 (0%)**	**1 (2%)**
Seizure	**0 (0%)**	**1 (5%)**	**0 (0%)**	**1 (2%)**

WHERE CAN I LEARN MORE ABOUT THIS STUDY?

If you have questions about the results of your son's study, please speak with the doctor or staff at your son's study site. The full scientific report of this study is available online at:

www.clinicaltrials.gov	Use the study identifier **NCT02907619**
www.clinicaltrialsregister.eu	Use the study identifier **2016-001615-21**

The results reported here might differ from additional studies that may be conducted with the same medicine in the future. No additional studies with domagrozumab are currently planned.

Again, **thank you** for assisting with this research. The commitment and expertise provided by the Duchenne Muscular Dystrophy community were invaluable contributions to the clinical study.

We do research to try to find the best ways to help patients, and you and your son helped us to do that!