Plain Language Clinical Study
Summary

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 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: Fordadistrogene movaparvovec

Protocol Number: C3391008

Dates of Study: 08 August 2022 to 03 October 2025

Title of this Study: Study of Fordadistrogene Movaparvovec in Early Stage Duchenne Muscular Dystrophy

[A Phase 2, Multicenter, Single-Arm Study to Evaluate the Safety and Dystrophin Expression After Fordadistrogene Movaparvovec (PF-06939926) Administration in Male Participants With Early Stage Duchenne Muscular Dystrophy]

Date(s) of this Report: 03 June 2026

Pfizer

– Thank You –

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

This summary will describe the study results. Do you have any questions about the study or the results? If so, please contact the doctor or staff at your child's study site.

Why was this study done?

What is Duchenne muscular dystrophy?

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. It is caused by changes in a gene called dystrophin, which is important for keeping muscle cell structure. A gene carries information that decides how your body looks and works.

DMD starts in childhood and gradually gets worse with age. In early stage DMD, a child's muscles have not yet been seriously damaged.

What is fordadistrogene movaparvovec?

Fordadistrogene movaparvovec (forda-distro-geen mova-par-vo-vec) is a "gene therapy" being tested to help treat DMD. The idea of gene therapy is to give something back to the body that is missing or not working properly. In the case of DMD, this is the dystrophin gene. Fordadistrogene movaparvovec has not been approved for use outside of research studies.

Fordadistrogene movaparvovec has 3 parts:

- A "capsid" called adeno-associated virus serotype 9 (AAV9). The AAV9 capsid is a transport tool called a "vector" when it holds the gene. This is called the AAV9 vector. The vector is a virus that has been changed so that it cannot cause an infection. When the AAV9 vector is in the bloodstream, it can carry the gene into parts of the body like the heart and muscles.
- A mini-dystrophin gene that is a shorter version of the dystrophin gene that has been changed in patients with DMD.
- A switch called a "promoter" that tells the gene where to turn on. Since the AAV9 vector will get into places in the body that do not need

dystrophin, like the liver, the promoter helps to turn the mini-dystrophin genes on in muscle and the heart only.

What was the purpose of this study?

The purpose of this study was to learn about the safety of fordadistrogene movaparvovec. The researchers did this by looking at how well the boys in the study tolerated this treatment.

To find this out, the researchers looked at all the medical and health problems the participants experienced. This information was collected for 1 year after the participants were given fordadistrogene movaparvovec. This was known as the "1-year follow-up period."

Researchers wanted to know:

- **What medical or health problems did participants have during the 1-year follow-up period after they were given fordadistrogene movaparvovec?**

The researchers looked at the medical problems to see if they were mild, moderate, or severe (see below).

Mild	A mild medical problem was one that participants could easily cope with and that did not stop them from doing their usual tasks.
Moderate	A moderate medical problem was one that caused enough discomfort to bother participants and made their usual tasks harder to do.

Severe	A severe medical problem was one that stopped participants from being able to do their usual tasks.

The researchers also looked to see:

- If any problems were seen from laboratory blood tests.
- If there were any heart-related problems.
- If any problems were seen in vital signs (such as blood pressure or oxygen levels).

The researchers also recorded participants' weight and did “neurological” tests. Neurological tests are used to check how well the brain, nerves, and muscles are working, such as how a person moves, feels, or reacts.

What happened during the study?

How was the study done?

First, researchers checked each participant to make sure they were able to join the study. This is known as screening.

Participants were given a single “intravenous infusion” of fordadistrogene movaparvovec. This means that fordadistrogene movaparvovec was given to participants via a needle into their vein from a drip, for around 2 to 4 hours.

Participants stayed in the study center for at least 7 days after they had been given fordadistrogene movaparvovec.

Participants were monitored for 1 year after they had been given fordadistrogene movaparvovec as part of the treatment follow-up. Participants were then to be monitored for up to 5 years after treatment as part of the long-term follow-up. As this study was stopped early, some participants took part in Study C3391003

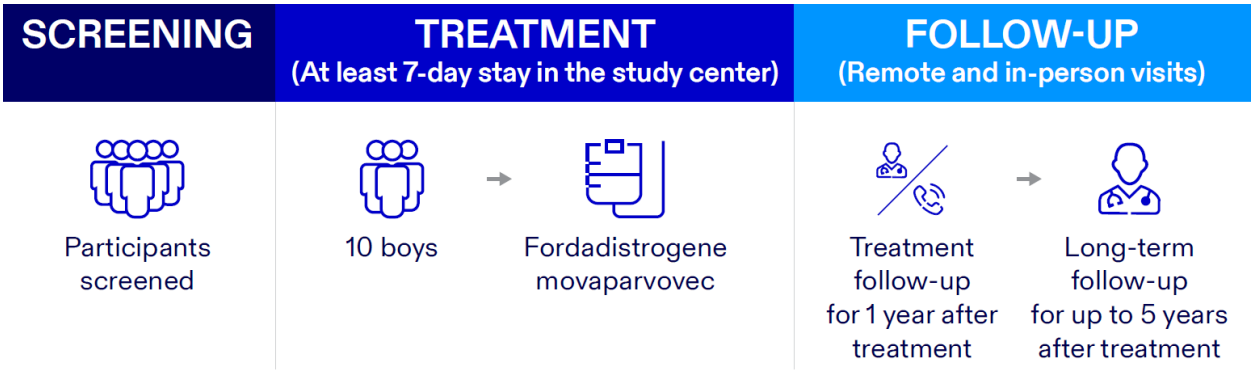
for the rest of their long-term follow-up. The reasons Study C3391008 was stopped early are explained later in this report.

Both the researchers and the participants knew the study medicine being given. This is known as an "open-label" study.

One (1) participant who was given fordadistrogene movaparvovec died due to a serious medical problem. This death is described later in this report. After this participant died, the Sponsor decided to pause giving fordadistrogene movaparvovec to participants in all studies.

Figure 1 shows what happened in this study.

Figure 1. Study design



Note: Participants were given a single intravenous infusion of fordadistrogene movaparvovec. Remote visits were only allowed during the 1-year follow-up period.

Where did this study take place?

The Sponsor screened participants at 6 locations in the United States and Australia. There were 5 locations in the United States and Australia that treated participants in this study. One (1) location in the United States screened participants, but none were suitable for the study.

When did this study take place?

The study began 08 August 2022. On 09 November 2024, the Sponsor made the decision to end the study early. The reasons for this are described later in this report. Participants were last seen by the researchers in this study on 03 October 2025.

Who participated in this study?

The study included boys with early stage DMD.

- A total of 10 boys participated.
- All participants were between the ages of 2 years and 4 years.

Of the 10 participants who started the study, 0 participants finished the study. This was because:

- The study was ended early by the Sponsor for 8 participants.
- One (1) participant had a medical problem. The participant had a cardiac arrest. This is when their heart suddenly stopped beating. The participant died due to this cardiac arrest. This death is described later in this report.
- One (1) participant left because their parent or guardian decided it was best for them to stop being in the study.

How long did the study last?

Study participants were to be in the study for up to 5 years. The study was stopped after around 3 years and 2 months.

The study was stopped early because fordadistrogene movaparvovec was not working well. The Sponsor also wanted to bring participants from multiple studies together to make it easier to monitor the participants and their results for a longer period of time.

The study was ended by the Sponsor while some participants were in long-term follow-up. These participants were invited to take part in Study C3391003 for the rest of their long-term follow-up.

When Study C3391008 ended in October 2025, 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?

The medical and health problems participants had during the study after they were given fordadistrogene movaparvovec are discussed below.

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 effects a study medicine might have on a participant.

Medical problems during the 1-year follow-up period

During the 1-year follow-up period:

- All 10 participants (100%) had at least 1 medical problem.
- No participants left the study due to a medical problem.
- No participants died.

The most common medical problems – those reported by at least 30% of participants – are described below.

Below are instructions on how to read Table 1. Table 2, Table 3, and Table 4 can be read in a similar way.

Instructions for Understanding Table 1.

- The **1st** column of Table 1 lists medical problems that were commonly reported during the study. All medical problems reported by at least 30% of participants are listed.
- The **2nd** column tells how many of the 10 participants who were given fordadistrogene movaparvovec reported each medical problem. Next to this number is the percentage of the 10 participants who were given fordadistrogene movaparvovec and reported the medical problem.
- Using these instructions for Table 1, you can see that 8 out of the 10 participants (80%) who were given fordadistrogene movaparvovec reported vomiting.

Table 1. Commonly reported medical problems by study participants during the 1-year follow-up period	
Medical Problem	**Fordadistrogene Movaparvovec (10 Participants)**
Vomiting	8 out of 10 participants (80%)
Fever	5 out of 10 participants (50%)
Rash	5 out of 10 participants (50%)
Fall	3 out of 10 participants (30%)
Not eating as much as usual	3 out of 10 participants (30%)

Were the medical problems mild, moderate, or severe?

Four (4) out of 10 participants (40%) had severe medical problems. These medical problems were:

- Two (2) out of 10 participants (20%) had vomiting.
- One (1) out of 10 participants (10%) had a breakdown of muscle tissue that releases a damaging protein into the blood.
- One (1) out of 10 participants (10%) had increased liver protein (enzyme) levels. This may be a sign of stress or damage to the liver.

All of these severe medical problems were considered to be serious. The researchers thought these medical problems were related to fordadistrogene movaparvovec.

All other medical problems were mild or moderate in severity and were not thought to be medically important.

Laboratory blood test results

The researchers checked if fordadistrogene movaparvovec affected the heart by measuring a marker in the blood called "cardiac troponin I." Cardiac troponin I is used to measure damage to heart muscle. One (1) out of 10 participants (10%) had an increase in cardiac troponin I levels after they were given fordadistrogene movaparvovec.

The researchers found the cardiac troponin I blood test results did not show any clinically meaningful changes during the 1-year follow-up period.

The researchers also looked at other laboratory blood test results. The most common abnormal laboratory blood test results – those reported by at least 60% of participants – are described below.

Table 2. Commonly reported abnormal laboratory blood test results during the 1-year follow-up period

Abnormal Laboratory Blood Test Result	Fordadistrogene Movaparvovec (10 Participants)
Increased level of a liver protein (enzyme) called "AST" in the blood	10 out of 10 participants (100%)
Increased level of a liver protein (enzyme) called "ALT" in the blood	10 out of 10 participants (100%)
Increased level of creatine kinase, an enzyme released into the blood when muscle is damaged	10 out of 10 participants (100%)

Table 2. Commonly reported abnormal laboratory blood test results during the 1-year follow-up period	
Abnormal Laboratory Blood Test Result	**Fordadistrogene Movaparvovec (10 Participants)**
Decreased level of lymphocytes, a type of white blood cell that helps fight infections	9 out of 10 participants (90%)
Increased level of monocytes, a type of white blood cell that helps fight infections	8 out of 10 participants (80%)
Increased level of potassium in the blood	6 out of 10 participants (60%)

Other test results

The researchers:

- Measured the electrical activity of participants' hearts and took detailed heart images.
- Measured vital signs which included blood pressure, heart rate, height, and oxygen levels.

The researchers had no concerns about the results for any of these.

Medical problems up to 5 years after treatment

All 10 participants (100%) in this study had at least 1 medical problem up to 5 years after treatment with fordadistrogene movaparvovec. After the 1-year follow-up period, only medical problems that were serious or thought to be related to fordadistrogene movaparvovec were to be recorded.

One (1) participant left the study due to a medical problem after the 1-year follow-up period. The participant died due to this medical problem. This is described later in this report.

The most common medical problems – those reported by at least 30% of participants – are described below.

Table 3. Commonly reported medical problems by study participants up to 5 years after treatment

Medical Problem	Fordadistrogene Movaparvovec (10 Participants)
Vomiting	8 out of 10 participants (80%)
Fever	5 out of 10 participants (50%)
Rash	5 out of 10 participants (50%)
Fall	3 out of 10 participants (30%)
Not eating as much as usual	3 out of 10 participants (30%)
Infection with a virus	3 out of 10 participants (30%)

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.

Did study participants have any serious medical problems during the 1-year follow-up period?

Seven (7) out of 10 participants (70%) had serious medical problems during the 1-year follow-up period. Some participants had more than 1 serious medical problem, and 9 serious medical problems were reported. These serious medical problems are described below.

Six (6) of the 9 serious medical problems were considered to be related to fordadistrogene movaparvovec by the researchers. The most common serious medical problems considered to be related to fordadistrogene movaparvovec were:

- Vomiting in 2 out of 10 participants (20%).
- A breakdown of muscle tissue that releases a damaging protein into the blood in 2 out of 10 participants (20%).

Table 4. Serious medical problems reported by study participants during the 1-year follow-up period

Serious Medical Problem	Fordadistrogene Movaparvovec (10 Participants)
A breakdown of muscle tissue that releases a damaging protein into the blood	2 out of 10 participants (20%)

Table 4. Serious medical problems reported by study participants during the 1-year follow-up period	
Serious Medical Problem	**Fordadistrogene Movaparvovec (10 Participants)**
Vomiting	2 out of 10 participants (20%)
A sudden life-threatening allergic reaction	1 out of 10 participants (10%)
Slow heart rate	1 out of 10 participants (10%)
Infection with a virus called enterovirus	1 out of 10 participants (10%)
Increased liver protein (enzyme) levels	1 out of 10 participants (10%)
Symptoms affecting the sexual organs and the parts of the body that make and pass urine. There is no clear physical cause for these symptoms	1 out of 10 participants (10%)

The 2 serious medical problems of vomiting, 1 serious medical problem of increased liver protein (enzyme) levels, and 1 serious medical problem of breakdown of muscle tissue that releases a damaging protein into the blood were also severe. These serious medical problems are also included in the description of severe medical problems earlier in this report.

Did study participants have any serious medical problems after the 1-year follow-up period?

One (1) further serious medical problem was reported after the 1-year follow-up period.

One (1) participant died of a cardiac arrest during the long-term follow-up period. This serious medical problem happened more than 1 year after he was given fordadistrogene movaparvovec. Before he died, this participant had a medical problem of a breakdown of muscle tissue that releases a damaging protein into the blood. This medical problem happened multiple times. During 1 of these episodes, the participant also had high blood potassium levels, which may have been a cause of the cardiac arrest. The cardiac arrest was not considered directly related to fordadistrogene movaparvovec.

After this participant died, the Sponsor decided to pause giving fordadistrogene movaparvovec to participants in all studies.

This serious medical problem was the only death during the study.

Where can I learn more about this study?

If you or your child 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 **C3391008**

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

www.clinicaltrials.gov	Use the study identifier **NCT05429372**

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 your child 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!

Pfizer