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:	PF-06939926 (fordadistrogene movaparvovec)
Protocol Number:	C3391011
Dates of Study:	13 March 2023 to 24 September 2025
Title of this Study:	A Study to Understand the Long-term Safety and Effects of an Experimental Gene Therapy for Duchenne Muscular Dystrophy [Long-Term Follow-Up Safety and Efficacy Study in Participants With Duchenne Muscular Dystrophy Who Have Received Fordadistrogene Movaparvovec in a Preceding Clinical Study]
Date(s) of this Report:	30 April 2026

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– 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.

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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.

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.

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What was the purpose of this study?

This study was a long-term follow-up study. It started 5 years after the participants had received fordadistrogene movaparvovec in a previous study, C3391001.

The purpose of this follow-up Study C3391011 was to look at the long-term safety of fordadistrogene movaparvovec for an additional 10 years (years 5 to 15). Researchers also looked at how well fordadistrogene movaparvovec worked, but that was not the main purpose of this study and is not described in this report.

Researchers wanted to know:

- **What medical problems did participants have during the 5 to 15-year-follow-up period after taking fordadistrogene movaparvovec?**

Only certain types of medical problems were recorded for this study. These included:

- Any serious medical problems or any medical problems that researchers believed to be related to fordadistrogene movaparvovec.
- Specific problems on a heart-related test.
- Any cancer. Cancer occurs when cells grow out of control.

What happened during the study?

How was the study done?

In a previous study, C3391001, fordadistrogene movaparvovec was given to participants as an “intravenous infusion”. This means that a needle is placed in the vein and the study drug slowly drips into the vein. Researchers tested

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fordadistrogene movaparvovec to find out if the study drug was tolerated and how safe it was.

This report describes the long-term follow-up in Study C3391011, which started 5 years after participants received fordadistrogene movaparvovec.

Researchers checked each participant to make sure they were able to join the study. This is known as screening. Researchers checked the participants' health during the study and asked them how they were feeling. They did this at least every 6 months.

Where did this study take place?

The Sponsor ran this study at 3 locations in the United States.

When did this study take place?

It began 13 March 2023 and was ended by the Sponsor on 24 September 2025.

This study finished early because the Sponsor wanted to bring participants from multiple studies together. This made it easier to monitor the participants and their results.

Who participated in this study?

The study included boys with DMD who participated in the previous C3391001 study where they received a dose of fordadistrogene movaparvovec.

- A total of 7 boys participated.
- All participants were between the ages of 12 years and 17 years.

Participants were to be monitored up to 15 years after they first received fordadistrogene movaparvovec treatment. Of the 7 participants who started this study, 6 participants were ongoing when the study was stopped.
Six (6) participants transferred to Study C3391003.

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One (1) participant left before the study was stopped early by their choice.

How long did the study last?

Participants were in the study for around 2.5 years. The study was ended by the Sponsor after 2.5 years, as described earlier in this report.

When the study ended in September 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 problems participants had during this study after taking fordadistrogene movaparvovec are discussed below.

What medical problems did participants have during the study?

The researchers recorded certain 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 5 to 15-year-follow-up period

A total of 4 out of 7 participants (57.1%) in this study had at least 1 medical problem. No participant left the study because of medical problems. No medical problems were believed to be related to fordadistrogene movaparvovec.

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The researchers did not have any concerns from the results of tests done during the study that tested heart function.

No participants had any new cancers.

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.

Three (3) out of 7 participants (42.9%) had at least 1 serious medical problem. None of these medical problems were believed to be related to fordadistrogene movaparvovec.

- One (1) participant had the serious medical problem of the heart not pumping blood as well as it should, called “congestive heart failure”.
- One (1) participant had the serious medical problem of a nervous system growth that is usually not a cancer tumor, called a “Schwannoma”.
- One (1) participant reported the serious medical problems of a fall and breaking their leg.

No participants died during the study.

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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 child's study site.

For more details on your study protocol, please visit:

www.pfizer.com/research/research_clinical_trials/trial_results

Use the protocol number **C3391011**

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

www.clinicaltrials.gov

Use the study identifier **NCT05689164**

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!

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