

Frequently Asked Questions

How can I or someone I know get more information about participating?

Contact your treating physician.
You can also email lelantos@fibrogen.com

Is there a cost associated with participation?

The study drug, site visits, laboratory tests, and procedures that are part of the study will be provided at no cost. You may be reimbursed for your travel expenses such as hotel and parking.

Is the study drug approved?

Pamrevlumab is an investigational drug that is not approved for use in any country. There is no guarantee that the investigational drug will be filed with or approved by any regulatory body.

How can I learn more about the LELANTOS TWO study?

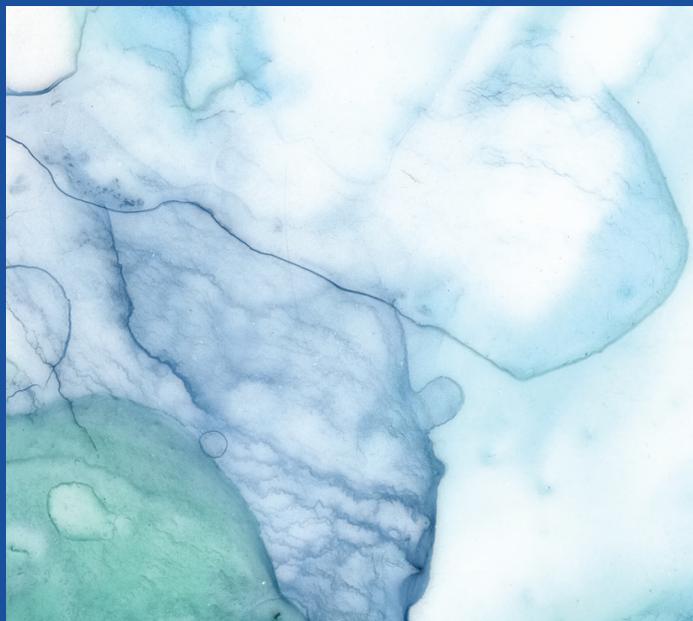
For additional information about the study, please visit ClinicalTrials.gov.



The LELANTOS TWO study is testing pamrevlumab, an investigational drug, in patients with Duchenne muscular dystrophy who are ambulatory, or able to walk without braces or assistive devices, to see if the drug can potentially improve the muscle strength of lower extremities and stabilize respiratory and heart functions.

The study will evaluate the drug's safety and potential efficacy in combination with systemic corticosteroids compared with placebo and systemic corticosteroids in patients 6 to <12 years of age.

Approximately 70 patients will participate in the study at about 50 research centers in the United States and in select European and Asia Pacific countries.



DUCHENNE MUSCULAR DYSTROPHY

LELANTOS TWO Study

You are not alone in managing Duchenne muscular dystrophy. FibroGen is studying an investigational treatment option to determine if it can help improve the condition of patients with Duchenne.



What is pamrevlumab?

Pamrevlumab is a type of protein drug called a monoclonal antibody. Monoclonal antibodies are man-made proteins designed to act like the human antibodies made by the immune system to help fight infections.

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What is Duchenne Muscular Dystrophy?

Duchenne muscular dystrophy, or Duchenne, is a genetic disorder characterized by progressive muscle degeneration and weakness affecting the whole body.

Duchenne primarily affects males but, in rare cases, can also affect females. About one of every 5,000 boys born has Duchenne, and about 20,000 babies worldwide are born with Duchenne each year.

Symptoms of Duchenne appear in early childhood, usually between ages 3 and 4, and while Duchenne progresses differently for every person, lower body external muscles are typically affected before upper external muscles. Later, heart and respiratory muscles are affected as well.



What is the purpose of the LELANTOS TWO study?

A clinical research study, or clinical trial, is designed to help medical researchers learn more about disease and improve health care for people in the future by evaluating the safety and efficacy of an investigational drug for a disease or condition. The results of clinical studies help regulatory agencies like the U.S. Food and Drug Administration (FDA) to decide if an investigational drug works and is safe.

Clinical studies offer a chance to help researchers find better treatments for others in the future.



Who can participate in the LELANTOS TWO study?

Patients may qualify to participate if they:

- Are male and at 6 to 12 years of age
- Are ambulatory, or able to walk without braces or assistive devices, at screening initiation
- Have a confirmed Duchenne muscular dystrophy diagnosis and confirmed Duchenne mutation using a validated genetic test
- Can provide written consent for themselves or from a legal guardian
- Have been on a stable dose of systemic corticosteroids for a minimum of 6 months, with no substantial change in dosage for a minimum of 3 months (except for adjustments for changes in body weight) prior to screening

Other criteria will be reviewed to determine if a patient qualifies for this study. Learn more about study inclusion and exclusion criteria on ClinicalTrials.gov.

Participation in the LELANTOS TWO study will last about 60 weeks total, which will be divided into several phases:

SCREENING PERIOD

weeks
1-4

Tests will be performed to determine if a patient is eligible to enroll in the study. It may take up to 4 weeks to determine whether the patient is able to participate in the study, based on inclusion and exclusion criteria.

STUDY TREATMENT PERIOD

weeks
5-56

The patient will be randomly assigned to treatment with either pamrevlumab (the investigational drug) or a placebo (an inactive substance).

The patient has a 50% chance of receiving the placebo.

- Treatment Group A: Pamrevlumab + systemic corticosteroid
- Treatment Group B: Placebo + systemic corticosteroid

Approximately 35 patients will be enrolled into Treatment Group A and approximately 35 patients will be enrolled into Treatment Group B. This study is “double blind,” which means that neither the patient nor study doctor will know whether he or she is receiving treatment with the investigational drug or placebo. The subject will receive infusions every 2 weeks, for 52 weeks.

FINAL FOLLOW-UP VISIT

week
60

The patient will have one last visit about 4 weeks after the Study Treatment Period ends to check on health and any potential side effects, and a safety follow-up phone call 60 days after the patient's last dose.

OPEN-LABEL EXTENSION

This study includes an optional open-label extension, with its own eligibility criteria, that will offer treatment with pamrevlumab and systemic corticosteroids for an average of two additional years for patients that complete 52 weeks of treatment and meet requirements for the extension phase.

In this open-label extension phase, pamrevlumab with systemic corticosteroids will be offered along with any other approved therapies for Duchenne at the study doctor's determination. The purpose of the open-label extension is to look at the longer-term effects of pamrevlumab given with other approved therapies.