ForPatients
by Roche

Duchenne Muscular Dystrophy (DMD)

A study to check the effect of satralizumab in children and adolescents living with Duchenne muscular dystrophy, to check if it is safe and also how it affects the different parts of the body and how it is eliminated from the body (SHIELD DMD)

A Study to Assess the Efficacy and Safety of Satralizumab in Duchenne Muscular Dystrophy (DMD)

Trial Status	Trial Runs In	Trial Identifier
Recruiting	4 Countries	NCT06450639 2024-512383-65-00 BN45398

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase II Multicenter, Open-label Study to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Satralizumab in Pediatric Patients With Duchenne Muscular Dystrophy (SHIELD DMD)

Trial Summary:

The purpose of this study is to assess the efficacy, safety, pharmacokinetics (PK) and pharmacodynamics (PD) of satralizumab, a humanized anti-interleukin-6 receptor (aIL-6R) monoclonal antibody, in ambulatory and non-ambulatory participants with DMD age # 8 to < 18 years old receiving corticosteroid therapy.

Hoffmann-La Roche	Phase 2
Sponsor	Phase

NCT06450639 2024-512383-65-00 BN45398
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Male	#8 Years & # 17 Years	No

1. Why is this study needed?

Duchenne muscular dystrophy (DMD) is a genetic disorder affecting boys that causes muscles to become weak and waste away, leading to difficulty in movement. DMD first affects the muscles that control movement and eventually muscles in the heart and respiratory muscles. DMD also makes bones weaker so people with DMD have a high risk of broken bones. Currently, certain medicines called glucocorticoids are commonly used to treat DMD, but they have many unwanted effects, including further weakening of the bones. Additionally, there are no approved treatments specifically for the bone issues in DMD.

This study is testing a medicine called satralizumab (RO5333787) to see if it can help with the muscle and bone issues of people living with DMD. Satralizumab is an experimental medicine for DMD. This means health authorities (like the U.S. Food and Drug Administration and European Medicines Agency) have not approved satralizumab for the treatment of muscle and bone issues in people with DMD.

This study aims to find out the effects of satralizumab when given along with glucocorticoids in children with DMD.

2. Who can take part in the study?

Male children who are between 8 and 17 years old with DMD, weigh between 20 to 100 kilograms (kg) and take daily corticosteroids can take part in the study. Children with and without a history of broken bones who may or may not be able to walk on their own can take part in this study.

Children cannot take part in this study if they have had major surgery within 3 months before the study or have planned a surgery or procedure during this study. Children who are receiving medications called ‘bisphosphonates’ as well as experimental drugs, and those who present other health conditions that could create a safety risk for them cannot take part in this study.

3. How does this study work?

Children will be screened to check if they are able to participate in the study. The screening will take place for up to 12 weeks before the start of the treatment.

Everyone who joins this study will be given satralizumab as an injection under the skin in the belly or thigh at Weeks 0, 2, 4, and every 4 weeks thereafter for a total of 104 weeks. The doses of satralizumab will depend on the participant’s body weight. Participants will have regular blood tests and will be checked for unwanted effects throughout the study.

This is an open-label study. This means everyone involved, including the participant and the study doctor, will know the study treatment the participant has been given.

During this study, the study doctor will see the participants 12 times during their clinic visits. These visits will first occur every two weeks during the first month, then every four weeks for the following two visits, and then approximately every 3 to 4 months for the rest of the first year. For the second year, the visits will happen every 6 months. Study doctors will see how well the treatment works and any unwanted effects participants may have. At the end, the participants will have a follow-up visit after 12 weeks of completing the study treatment, during which the study doctor will check on the participant's well-being.

Total time of participation in the study will be about 2 years. Participants have the right to stop study treatment and leave the study at any time if they wish to do so. Participants will also occasionally receive follow-up calls to check on how they are doing. If they experience any unwanted effects or injury during the study, the clinical trial doctor will explain the options and discuss a plan for further treatment.

4. What are the main results measured in this study?

The main result measured in the study is to assess if the medicine changes the bone mineral density of the bones of the lower back. This will be assessed in participants who do not have a history of any kind of broken bones. This will be measured using a dual-energy X-ray absorptiometry (DEXA) scan, from the start of the study to Week 52. DEXA is a scan that measures bone density by passing high and low-energy X-rays through the body. The amount of radiation in DEXA scans is much lower than standard X-rays.

Other key results measured in the study include:

- Number of participants with unwanted effects
- Changes in the amount of bones formed and broken down
- Average number of new broken bones during the study
- Number of participants with new broken bones during the study
- How well the body processes satralizumab
- Number of participants who had their body's defense system respond to satralizumab
- Changes in the motor function

5. Are there any risks or benefits in taking part in this study?

Taking part in the study may or may not make participants feel better. But the information collected in the study can help other people with similar health conditions in the future.

The study involves some risks to the participant. These risks are generally not greater than those related to routine medical care or the natural progression of DMD. People interested in taking part will be informed about the risks and benefits, as well as any additional procedures or tests they may need to undergo. All details of the study will be described in an informed consent document. This includes information about possible effects and other options of treatment.

Risks associated with the study drug Participants may have unwanted effects of the drug used in this study. These unwanted effects can be mild to severe, even life-threatening, and vary from person to person. During this study, participants will have regular check-ups to see if there are any unwanted effects. This medication has already been tested for other diseases and is approved for a neurological condition called Neuromyelitis Optica Spectrum Disorder.

Satralizumab
Participants will be told about the known unwanted effects of satralizumab, and possible unwanted effects based on human and laboratory studies or knowledge of similar medicines. Known unwanted effects include decreased white blood cell count, headache, joint pain, difficulty sleeping or falling asleep, hay fever, itching, and migraine.

Satralizumab will be given as an injection under the skin in the belly or thigh. Known unwanted effects of injection under the skin include irritation where the injection is given, such as swelling, rash, redness, itching, or pain.

Inclusion Criteria:

- Signed Informed Consent Form and Signed Assent Form when appropriate
- Male at birth
- A definitive diagnosis of DMD prior to screening based on documentation of clinical findings and prior confirmatory genetic testing using a clinical diagnostic genetic test
- Age # 8 and < 18 years at the time of signing Informed Consent Form
- Group 1 participants are required to meet the following criteria:
- Ambulatory (defined as able to walk independently without assistive devices) with a prior history of fractures:
- Prior history of low-trauma fracture defined as: evidence of at least one prevalent vertebral compression fracture of Genant Grade 1 or 2 (or radiographic signs of VF) or history of at least one low-trauma long-bone fracture (upper or lower extremity) OR
- Non-ambulatory, characterized as being non-ambulatory for a minimum of 6 months with onset of non-ambulatory status defined as participant- or caregiver-reported age of continuous wheelchair use approximated to the nearest month, and an North Star Ambulatory Assessment (NSAA) walk score of "0" and inability to perform the 10-Meter Walk/Run (10 MWR) at the baseline visit, with or without fractures
- Group 2 participants are required to meet the following criteria:
- Be fracture naïve, defined as: no history of prior low-trauma fractures before the baseline visit nor any radiological findings indicative of prevalent VF at the screening visit
- Be ambulatory defined as able to walk independently without assistive devices
- Age # 8 to < 12 years old at the time of screening
- Daily oral corticosteroids

Exclusion Criteria:

- Major surgery (e.g. spinal surgery) within 3 months prior to Baseline or planned surgery or procedure that would interfere with the conduct of the study for any time during this study
- Presence of any clinically significant illness

- Has serological evidence of current, chronic, or active human immunodeficiency virus (HIV), tuberculosis (TB), hepatitis C, or hepatitis B infection
- Has a symptomatic infection (e.g. upper respiratory tract infection, pneumonia, pyelonephritis, meningitis) within 4 weeks prior to baseline
- Body weight at screening <20 or > 100 kg
- Evidence of a severe vertebral fracture (VF) (defined as Grade 3), assessed by radiographic imaging at screening and quantified using the Genant semiquantitative method
- Treatment with prohibited therapies as defined by the protocol
- Has received a live or live attenuated virus vaccine within 6 weeks of the Baseline visit or expects to receive a vaccination during the first 3 months after Baseline.
- Has abnormal laboratory values considered clinically significant as defined by the protocol
- Any medical condition that might interfere with the evaluation of LS BMD, such as severe scoliosis or spinal fusion.
- Participant has previous or ongoing medical condition, medical history, physical findings or laboratory abnormalities that could affect safety, make it unlikely that treatment and follow-up will be correctly completed or impair the assessment of study results, in the opinion of the investigator
- Participant has an allergy or hypersensitivity to the study medication or to any of its constituents

Other protocol defined inclusion and exclusion criteria may apply