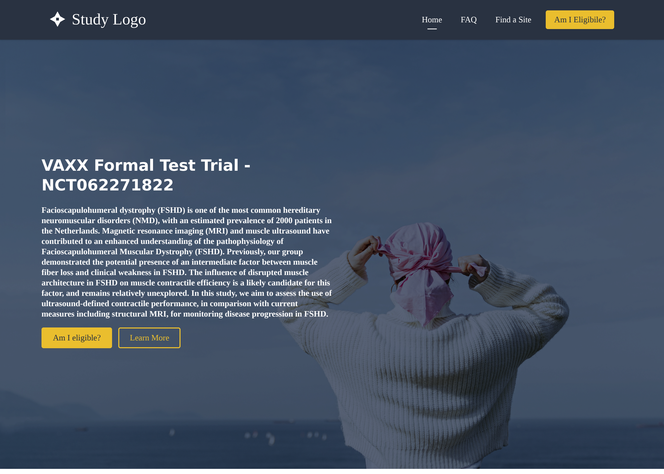
VAXX Formal Test Trial - NCT062271822



Facioscapulohumeral dystrophy (FSHD) is one of the most common hereditary neuromuscular disorders (NMD), with an estimated prevalence of 2000 patients in the Netherlands. Magnetic resonance imaging (MRI) and muscle ultrasound have contributed to an enhanced understanding of the pathophysiology of Facioscapulohumeral Muscular Dystrophy (FSHD). Previously, our group demonstrated the potential presence of an intermediate factor between muscle fiber loss and clinical weakness in FSHD. The influence of disrupted muscle architecture in FSHD on muscle contractile efficiency is a likely candidate for this factor, and remains relatively unexplored. In this study, we aim to assess the use of ultrasound-defined contractile performance, in comparison with current measures including structural MRI, for monitoring disease progression in FSHD.

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Detailed Description

Rationale: Facioscapulohumeral dystrophy (FSHD) is a slowly progressive hereditary muscle dystrophy characterized by initial asymmetrical weakness of the facial and shoulder girdle muscles, frequently followed by weakness in the trunk and leg muscles.
Previously, our research group showed the potential presence of an intermediate factor between muscle fiber loss and clinical weakness in FSHD. The influence of disrupted muscle architecture in FSHD on muscle contractile efficiency is a likely candidate for this factor. However, there is currently still a lack of studies on how the disrupted muscle architecture in muscle dystrophies influences the contractile efficiency. We might establish a baseline for muscle contractile performance with muscle ultrasound, determined by muscle strain and displacement.
Muscle imaging has previously contributed to a better understanding of the pathophysiology of various neuromuscular disorders. Both MRI and ultrasound have proven their clinical relevance in neuromuscular dystrophy. With the current development of FSHD clinical trials, the extensive need for biomarkers to follow disease progression is growing. To investigate whether muscle contractile performance can help explain the loss in strength and thereby also has the potential to act as a future biomarker, will be explored in this project.
Objective(s): We aim to assess the use of ultrasound-defined contractile performance as a biomarker for monitoring disease progression and treatment effects in patients with FSHD.
Stage I:
To establish the feasibility, optimal protocol, and repeatability of quantifying ultrasound-defined muscle contractile performance in the upper and lower limb muscles in healthy volunteers and patients with FSHD.
Stage II:

1. To determine the differences in ultrasound-defined contractile performance between healthy individuals and patients with FSHD, and compare to conventional clinical measures, ultrasound measures and MRI measures.
2. To determine the responsiveness of ultrasound-defined contractile performance to disease progression in FSHD patients after 1 year, and compare to MRI measures and other ultrasound measures.

Study design: This prospective cohort study consists of two different stages. In Stage I, the feasibility and repeatability of quantifying ultrasound-defined muscle contractile performance will be assessed in 15 healthy volunteers and 10 patients with FSHD type 1 or 2. Each participant in stage I has to visit the hospital only once. In Stage II, the ultrasound-defined contractile performance, and other ultrasound and MRI outcome measures of healthy volunteers and FSHD patients (type 1 or 2) are compared. In addition, the responsiveness of the different measurements to disease progression after 1 year will be analyzed only in FSHD patients. 50 patients with FSHD will perform these measurements of stage II during the two scheduled visits at baseline (T0) and the two scheduled visits 1-year after the first visit. In stage II, 25 Healthy volunteers will be recruited and will undergo the muscle ultrasound assessment. Only 10 out of 25 healthy volunteers will also undergo the MRI measurements. None of the healthy volunteers will perform follow-up measurements after 1 year. Therefore, 15 healthy volunteers will perform the ultrasound assessment of stage II during 1 visit and 10 healthy volunteers will perform ultrasound measurements during visit 1 and MRI measurements during visit 2 at baseline. A total of 100 individuals will participate in this study.

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Q1

- Yes
- No- Potential-Rescreen
- Maybe -exclude

Next →

Age
18 - 85

Gender
All

NCT ID
TX13295

Phase
0

Status
Recruiting Now

Medical Condition
acne

How is Plaque Psoriasis treated?

Medical Condition
A short sentence to introduce what to expect in the about condition section
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The Study
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About Clinical Trials
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Your Journey

01 Receiving the medication
You would receive etanercept (Enbrel) twice a week for 12 weeks and then once a week for 12 weeks. Etanercept, as well as study related medical care, is provided at no cost.

02 Visiting the study site
Study participation involves approximately 8 visits to your local study center over 6 to 7 months.

03 Follow-up
There would also be a follow-up telephone call 30days after completing the study. No visits are required after participation is complete.

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