

Evolution of the Functional and Muscular State of Patients With Muscular Dystrophy 2A, LGMDR1

Contact

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Recruitment Details

- Target Participants: 25
- Geographical Area: France or affiliated with French social security
- Participants Enrolled: 11

Eligibility Criteria

Inclusion Criteria

- 18+ years, both M/F
- Confirmed LGMD2A/R1 diagnosis
- Able to participate in study tests
- Signed consent form
- **Affiliate to French social security**

Exclusion Criteria

- Other interfering diseases
- Recent participation in other clinical trials
- Inability to comply with protocol
- Under legal protection
- Pregnant/breastfeeding
- Deprived of liberty
- Unable to express consent

Study Details

Lay Abstract

- **Aim** : Identify and quantify muscle strength loss and fat infiltration in upper and lower limbs over 2 years in 25 patients with LGMD 2A-R1.
- **Methods** : Non-invasive motor scales, timed tests, muscle MRI.
- **Deliverables** : Define evaluations criteria for future gene therapy clinical trials.

Overview

- **Day 1, V1 Visit:**
 1. Collect demographic data, medical history, and perform physical examination.
 2. Afternoon: Muscle MRI.
- **Day 2, V1 Visit:**
 1. Conduct NSAD and PUL functional tests.
 2. Monitor movement activity.
 3. Perform muscle assessments and complete quality-of-life questionnaires.

Follow-up Visits:

1. V2 at month 6, V3 at month 12, V4 at month 18, V5 at month 24.



CALPATeam



AP-HP.
Hôpitaux universitaires
Henri-Mondor



Gianmarco SEVERA
Neurologue



Saliha CHEROUALI
Infirmière



Christophe ALIMIM, Msc
Kinésithérapeute



Iman TAHIRI
ARC



Hussein SHOAITO, PhD
ARC

Join us in advancing the understanding and treatment of Calpain 3/LGMDR1!