

CALNATHIS



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

Contact

Edoardo MALFATTI MD, PhD

<u>edoardo.malfatti@aphp.fr</u> edoardo.malfatti@u-pec.fr

+33 01 498144 96

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

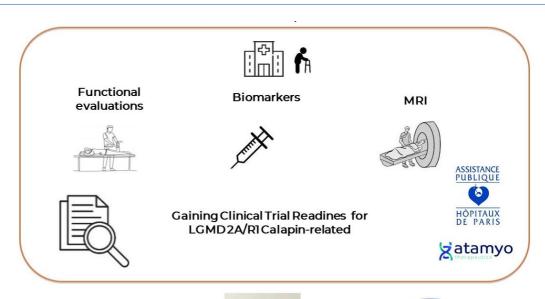
- Day 1, V1 Visit:
- 1. Collect demographic data, medical history, and perform physical examination.
- 2. Afternoon: Muscle MRI.

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.









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