

Natural History Study of Children With LAMA2-related Dystrophies

NCT06354790

Status	RECRUITING
Sponsor	Institut de Myologie, France
Enrollment	40 participants

Key Eligibility Criteria

Inclusion (9)

- Signed informed consent by the Legal Authority Responsible and/or assent by the subject (starting from 6 years old)
- Subject must be
- Supportive clinical phenotype and diagnosis of LAMA2-RD, confirmed by:
 - Two pathogenic variants in the LAMA2 gene (via a diagnostic laboratory included on an approved list of genetic testing laboratories (Annex 1)) or
 - Muscle biopsy with absence of merosin (laminin-211) and at least one pathogenic variant in the LAMA2 gene
- ... and 4 more (see full listing online)

Exclusion (5)

- Developmental quotient less than 70 and/or behavioral disorder requiring general anesthesia to perform an MRI
- Acute medical illness or hospitalization within 30 days prior to informed consent
- Participation in a previous trial of any investigational agent for LAMA2-RD, or use of any other investigational therapy within 30 days prior to informed consent, or participation in other clinical studies, within 30 days (or 5 half-lives, whichever is longer) prior to informed consent, which, in the opinion of the PI, may potentially confound results from this study
- Other significant medical condition and/or overall fragility of medical status, which in the opinion of the Investigator may confound interpretation of the clinical course of LAMA2-RD
- Pregnant or breastfeeding women

Locations (4 total)

Centre de Référence GNMH, Pédiatrie Hôpital Raymond-Poincaré, Garches, France
Service de MPR pédiatrique L'Escale - HCL, Lyon, France
Département de neuropédiatrie Pôle Femme Mère Enfant CHU de Montpellier - Hôpital Gui de Chauliac, Montpellier, France
... and 1 more locations