

# Congenital Muscle Disease Study of Patient and Family Reported Medical Information

NCT01403402

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|------------|--------------------|
| Status     | RECRUITING         |
| Sponsor    | Cure CMD           |
| Enrollment | 4,000 participants |

## Key Eligibility Criteria

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### Inclusion (1)

- Alpha 7/Alpha 9 Integrin Related Myopathy Collagen VI Related Myopathy (Ullrich through Bethlem CMD) Alpha-Dystroglycan Related Muscular Dystrophy (Dystroglycanopathy, WWS, MEB, Fukuyama, FKRP, LGMD2I, LGMD2K, LGMD2M, LGMD2N, LGMD2O) Choline Kinase B Receptor Emery-Dreifuss Muscular Dystrophy (EDMD, LGMD1B, LMNA, Emerin, FHL1, SYNE1, SYNE2, TMEM43) LAMA2 Related Muscular Dystrophy (Laminin Alpha 2 related dystrophy/MDC1A/Merosin deficient) LMNA Related Muscular Dystrophy (Laminopathy/LaminA/C, L-CMD, Emery Dreifuss muscular dystrophy) RYR1 Related Myopathy (with dystrophic presentation, including Malignant Hyperthermia, Exertional Myalgia with or without Rhabdomyolysis) SEPN1 Related Myopathy (Rigid Spine Muscular Dystrophy/RSMD1, Congenital Fiber Type Disproportion, Mallory Weiss Body Desmin, Multi-minicore Myopathy) SYNE1 (Nesprin Related Muscular Dystrophy) Telethonin Related Muscular Dystrophy (TCAP/Titin-Cap) Congenital Muscular Dystrophy Not Otherwise Specified (including Merosin Positive) Titin Related LGMD/CMD, LGMD2J Actin Aggregation Myopathy Cap Disease Central Core Disease (including Malignant Hyperthermia, Exertional Myalgia with or without Rhabdomyolysis) Centronuclear Myopathy (including Malignant Hyperthermia, Exertional Myalgia with or without Rhabdomyolysis) Congenital Fiber Type Disproportion (including Malignant Hyperthermia, Exertional Myalgia with or without Rhabdomyolysis) Core Rod Myopathy Hyaline Body Myopathy Multiminicore Myopathy Myotubular Myopathy Nemaline Myopathy Reducing Body Myopathy RYR1 Related Myopathy (including Malignant Hyperthermia, Exertional Myalgia with or without Rhabdomyolysis) Spheroid Body Myopathy Titin Related Myopathy, Titin Related Dilated Cardiomyopathy, LGMD2J Tubular Aggregate Myopathy Zebra Body Disease Myopathy Congenital Myopathy Not Otherwise Specified Congenital Myasthenic Syndrome Escobar Syndrome Myofibrillar Myopathy

### Exclusion (1)

- Charcot Marie Tooth Duchenne/Becker Muscular Dystrophy Facioscapulohumeral Dystrophy/FSHD Kennedy's Disease LGMD-1A (TTID) LGMD-1C (CAV3, Caveloin 3, Caveolinopathy, LQT9, VIP21) LGMD-1D (7q) LGMD-1E (6q23) LGMD-1F (7q32.1-q32.2) LGMD-1G (4q21) LGMD-2A (CAPN3/Calpainopathy) LGMD-2B (DYSF/Dysferlinopathy/Miyoshi Myopathy) LGMD-2C (SGCG) LGMD-2D (SGCA) LGMD-2E (SGCB) LGMD-2F (SGCD) LGMD-2L (AN05/Anoctamin 5) Lipodystrophy Myotonic Dystrophy Oculopharyngeal Muscular Dystrophy Spinal Muscular Atrophy

## Locations (1 total)

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Congenital Muscle Disease International Registry ([www.cmdir.org](http://www.cmdir.org)), Lakewood, California, United States

<https://clinicaltrials.gov/study/NCT01403402>

DISCLAIMER: This document is for informational purposes only and does not constitute medical advice. Always consult your healthcare provider before enrolling in any clinical trial. Information may not be up to date — verify details at [ClinicalTrials.gov](http://ClinicalTrials.gov). Generated by [ClinicalTrialsFinder.org](http://ClinicalTrialsFinder.org).