DMCRN-02-001: Assessing Pediatric Endpoints in DM1

NCT05224778

Status	RECRUITING
Sponsor	Virginia Commonwealth University
Enrollment	50 participants

Key Eligibility Criteria

Inclusion (3)

- Age neonate to 3 years 11 months at enrollment.
- A diagnosis of CDM, which is defined as children having symptoms of myotonic dystrophy in the newborn period (\<30 days), such as hypotonia, feeding or respiratory difficulty, requiring hospitalization to a ward or to the neonatal intensive care unit for more than 72 hours; and a genetic test confirming an expanded trinucleotide (CTG) repeat in the DMPK gene in the child or mother. An expanded CTG repeat size in the child is considered greater than 200 repeats or E1-E4 classification (E1= 200-500, E2=500-1,000, E3=1,000-1,500, E4\>1,500).
- Guardian is willing and able to sign consent and follow study procedures

Exclusion (5)

- Any other non-DM1 illness that would interfere with the ability or results of the study in the opinion of the site investigator
- Significant trauma within one month
- Internal metal or devices (exclusion for DEXA component)
- History of bleeding disorder or platelet count \<50,000
- History of reaction to local anesthetic

Locations (5 total)

University of California, Los Angeles, Los Angeles, California, United States
University of Kansas Medical Center, Fairway, Kansas, United States
University of Rochester Medical Center, Rochester, New York, United States
... and 2 more locations