DMCRN-02-001: Assessing Pediatric Endpoints in DM1

Status: RECRUITING

Eligibility Criteria

Age: Up to 59 months old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* 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 Criteria:

* 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

Conditions & Interventions

Conditions:
Congenital Myotonic Dystrophy, CDM
Keywords:
Clinical Research, Myotonic dystrophy, Congenital Myotonic Dystrophy, CDM

More Information

Contact(s): Ruby Langeslay - ruby.langeslay@vcuhealth.org
Principal Investigator: Johnson, Nicholas, E
Phase: N/A
IRB
Number: HM20023386
System ID: NCT05224778
