The Baby Duchenne Study: Characterizing Developmental and Clinical Outcomes in the First Three Years in Children With Duchenne Muscular Dystrophy

Status: RECRUITING

Eligibility Criteria

Sex: MALE
Age: 0 days to 3 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Male child between birth and 3.0 years of age at time of enrollment.
- A confirmed and documented pathogenic or likely pathogenic variant in the DMD gene.
- Ability of parent/guardian to understand and provide written informed consent (signing Parental Permission and Consent Form).
- Willingness of parent/guardian to comply with the protocol Schedule of Activities, including all study site visits.

Exclusion Criteria:

- Female
- Presence of any confirmed genetic disease, other than DMD, that could impact early development, which, in the opinion of the PI, may confound interpretation of developmental progress.
- Presence of any significant medical condition (i.e., extreme prematurity, hypoxic ischemic encephalopathy) which, in the opinion of the PI, may confound interpretation of the clinical course of DMD.
- Inability/unwillingness of parent/guardian to provide written permission (sign PPF) or to comply with the protocol Schedule of Activities.

Conditions & Interventions

Conditions:
Duchenne Muscular Dystrophy (DMD)
Keywords:
New born screening

More Information

Description:
The aim of the BABY DUCHENNE study is to evaluate the natural history and characterize the early clinical outcomes in very young children (0-3 years) with Duchenne muscular dystrophy (DMD) identified by newborn screening programs.

Contact(s): Angela Edmondson - angela.edmondson@cchmc.org
Principal Investigator:
Principal Investigator ID:
Phase:
IRB Number:
System ID: NCT07092540