Muscular Dystrophy
Registry

PROJECT SUMMARY

Objectives

- To expand our understanding of current cardiac outcomes in dystrophinopathy (DMD, BMD, carriers)
- Increase the number of patients receiving consensus-driven medical therapies (CDMT)
- Increase the number of patients receiving timely consideration of advanced therapies
- Understand the cardiac safety and potential efficacy of novel gene therapy

Inclusion

- All dystrophinopathy patients > age 10 will be enrolled
- Dystrophinopathy patients with evidence of cardiomyopathy prior to age 10 will be enrolled.*
- Patients who have received gene therapy will also be enrolled (at any age)

**** Cardiomyopathy is defined as at least one imaging study with systolic dysfunction (LVEF < 55%, FS < 28%), left ventricular dilation (defined as a z-score >+2), presence of late gadolinium enhancement, or presence of significant arrhythmia (defined as clinical diagnoses of sustained or non-sustained ventricular tachyarrhythmia, supra-ventricular tachycardias, atrial fibrillation or flutter).***

Frequently Asked Questions

Q. Does this project require ACTION consent?

- Yes, unless the patients meet one of the following criteria for waiver of consent:
  - Died prior to consent
  - Transferred care to a non-participating center
  - Lost to follow-up

Q. What data needs to be entered by our site into the MD Registry to start?

- Enter the patients info the project on the Muscular Dystrophy Demographics Form, Clinical Form, and Cardiac Testing Forms
- Enter the remainder of forms as appropriate for the patient. For example, if the patient has had an ambulatory rhythm monitor you would select that form an enter any results. If they have had multiple over the study period, you would then enter the rest as Follow-up forms.

Q. There are forms asking about things like ICD, rhythm monitoring, upper limb function and PFTs. Do I need to enter this? What if my patient hasn't had these procedures or tests?

- Fill out these forms only if these tests are available and the form applies to your patient (e.g. the patient has received a VAD).

Q. What about follow-up data entry?

- You will be notified by email 6 months after your last date of entry to provide updated information in the follow-up forms. Please fill out follow-up forms appropriate for your patient at that time. Choose unverified if you have entered the data but need to confirm it is complete.
- Choose complete if the data has been entered fully nd is ready for data analysis.

Resource Documents

Project Charter