Navigating Clinical Trials
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What to do....

This is an exciting time for Duchenne Research

• More options and choices!
• But, with that more **Responsibility**

So how do you decide what study is the right study?

• Ask the basics questions: who, what, where, when, why
• **Who**: is the PI or Pharmaceutical Company?
• **What** is the study: phase, drug, design, etc.?
• **When**: does it start, how long is it, is there an extension phase?
• **Why**: is the study being conducted?
ClinicalTrials.gov

- This is a great tool and first start
- But it needs to be tailored down for your child
  - Review Inclusion/Exclusion Criteria
    - Age
    - Ability / Strength
    - Current Medications
- See if it is manageable for your family
  - Can you travel frequently
  - Will your son be able to complete study procedures (MRI, biopsy, PFTs)
  - Will your son be able to tolerate medication (pills, injection, infusion)
- Tips:
  - Ask for a copy of the consent form
  - Ask to talk to the clinical evaluator
  - Ask questions
Consent and Assent

- Ask for a copy of the consent prior to your visit and write down any questions
  - Some can be over 20 pages long
  - Plan for at least an hour of consenting
  - Tips: bring snacks and activities

- Consent/Assent is ongoing
  - Ask questions at anytime
  - Ask the PI, CRC, or CE
  - Some questions staff will need to research as well
Sharing Data or Results

- It is important to keep research information confidential
- Research staff will share what can be shared
- Study *safety data (labs / vitals)* is reviewed *continuously*
- Study *efficacy data (strength / function)* can only be analyzed at *specific times* (ex. middle or end of trial)
- Keeping results confidential is important for FDA approval
  - To maintain validity of efficacy endpoints
  - To maintain the blind for randomized studies
Thank you