

# 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?

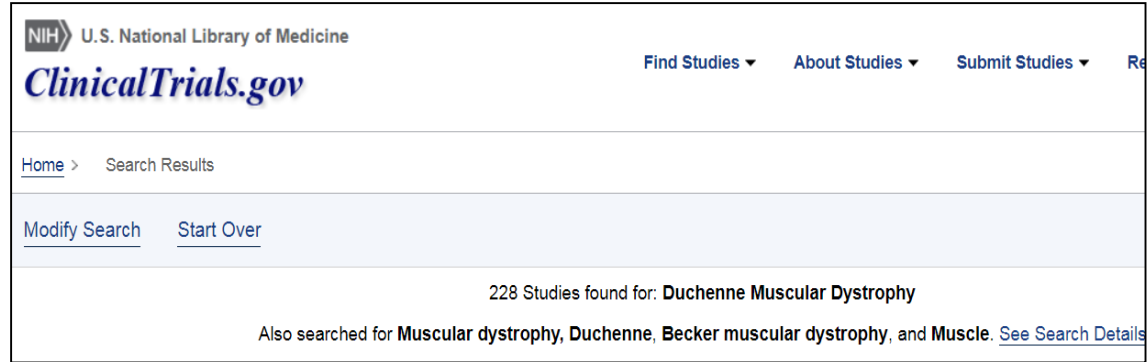
- Ask the basics questions: who, what, where, when, why
- **Who:** is the PI or Pharmaceutical Company?
- **What** is the study: phase, drug, design, etc.?
- **When:** does it start, how long is it, is there an extension phase?
- **Why:** is the study being conducted?

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# 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 **continually**
- 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



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