RESEARCH AND CLINICAL TRIALS

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Who Funds Research?

- Majority from US government (NIH, NSF, etc)
- Advocacy groups (MDA, Myositis Association)
- Private Philanthropy
- Pharmaceutical Companies
Why isn’t there more research on myositis?

- There are no restrictions on research topics at the NIH
- The only limitation is having a good idea
- Researchers need “protected time” to focus on research questions
- NIH traditionally funds “hypothesis” driven research with a high likelihood of success
- High Risk/High Reward Research or “look and see” experiments are less competitive in the current funding climate.
What is the difference between basic research and clinical research

- **Basic research** - provides the foundation of knowledge for all research

- **Clinical research** - focuses on health and illness in people. Utilizes human participants for its studies.

- **Translational research** - bridge basic science mechanism with disease pathogenesis to understand and treat human disease.
What is a Clinical Trial?

- Safety in people pharmacodynamics
- Safe in patients
- Dose finding
- Not powered for efficacy
- Efficacy in patients
- Safety in patients
Preclinical studies

- Natural history studies
- Patient reported outcomes
- Disease registries
- Genetics
- Biomarker studies
  - Serum
  - MRI/imaging
  - Pathologic
- Pharmacologic target identification
- Preclinical model development
What is a Clinical Trial?
Secrets to a successful clinical trial

- Defined natural history
- Homogenous patient population
- Clear pharmacologic biomarker
- Randomization
- Blinding
- “Hard” and “quantifiable” outcome measure
How to design a clinical trial

- Pick a “clinically meaningful” primary endpoint (secondary endpoints are allowed but do not equate with success).
  - %change in 6MWT; %change in IBMFRS
  - change in a biomarker; change in QOL

- Define enrollment criteria
  - Ambulatory?; pathologically defined?; newly diagnosed?

- Identify a pharmacologic biomarker
  - Immune marker?; muscle volume?

- Determine the duration of trial

- Determine number of patients to be enrolled

- Obtain adverse events (Data Safety Monitoring Board/DSMB)
Common Clinical Trial Design Errors

- Change of the primary endpoint to match the conclusion
- Failure to account for other variables (co-administration of another drug or blinding bias)
- Failure to prove delivery of drug/therapeutic biomarker
- Assuming linearity of disease progression (annualization of data)
- Inadequate sample size
- Loss of research subjects or removing from analysis
- Choosing the wrong endpoints
- Using natural history controls instead of a controlled trial
Patient role in clinical trial

- You are participant with “free will”
- Your doctor will treat you regardless of whether you participate

- All trials are different. How often to visit. How often for blood draws, imaging, Pre-post biopsy
- Get information upfront
Doctor’s role in clinical trials

- I am not talking about the investigator but your doctor
- Be knowledgeable about available clinical trials
- Understand the value of clinical trials
Investigators role in the clinical trial

- Communicate and honor the gift that you are giving
- Be mindful of all safety concerns
- Keep you informed of all known risks

- They will not know if you are getting drug or placebo
- They will not know if the drug is working in other patients
- They will know if other patients are having adverse events