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?

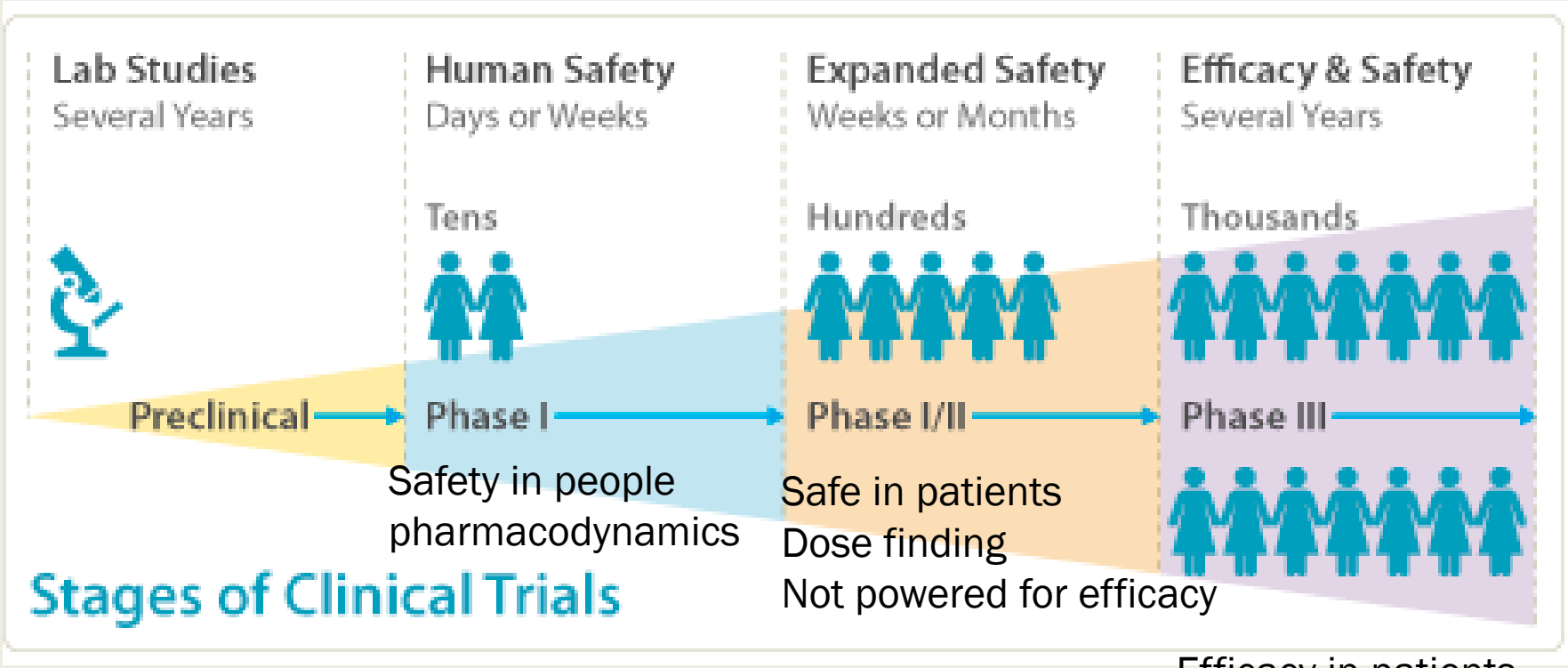
- There are no restrictions on research topics at the NIH
- The only limitation is having a good idea
- Researchers need "protected time" to focus on research questions
- NIH traditionally funds "hypothesis" driven research with a high likelihood of success
- High Risk/High Reward Research or "look and 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?



Preclinical studies

- Natural history studies
- Patient reported outcomes
- Disease registries
- Genetics
- Biomarker studies
  - *Serum*
  - *MRI/imaging*
  - *Pathologic*
- Pharmacologic target identification
- Preclinical model development

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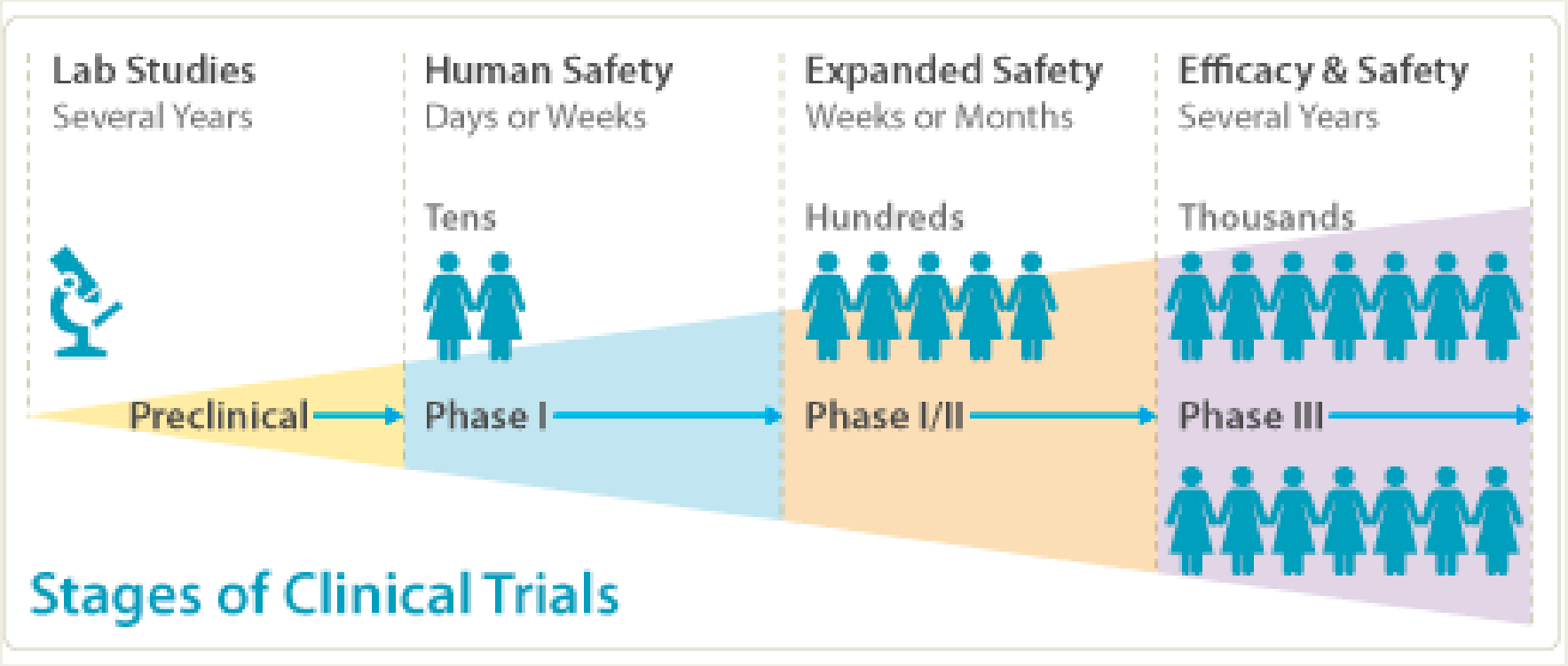

Secrets to a successful clinical trial

- 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