Drug Development and Clinical Trials

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FARA Friedreich's Ataxia Research Alliance

Objectives

- What are clinical trials?
- What are the different types or phases of trials?
- What do we need to think about related to clinical trials?
- What can you do now?

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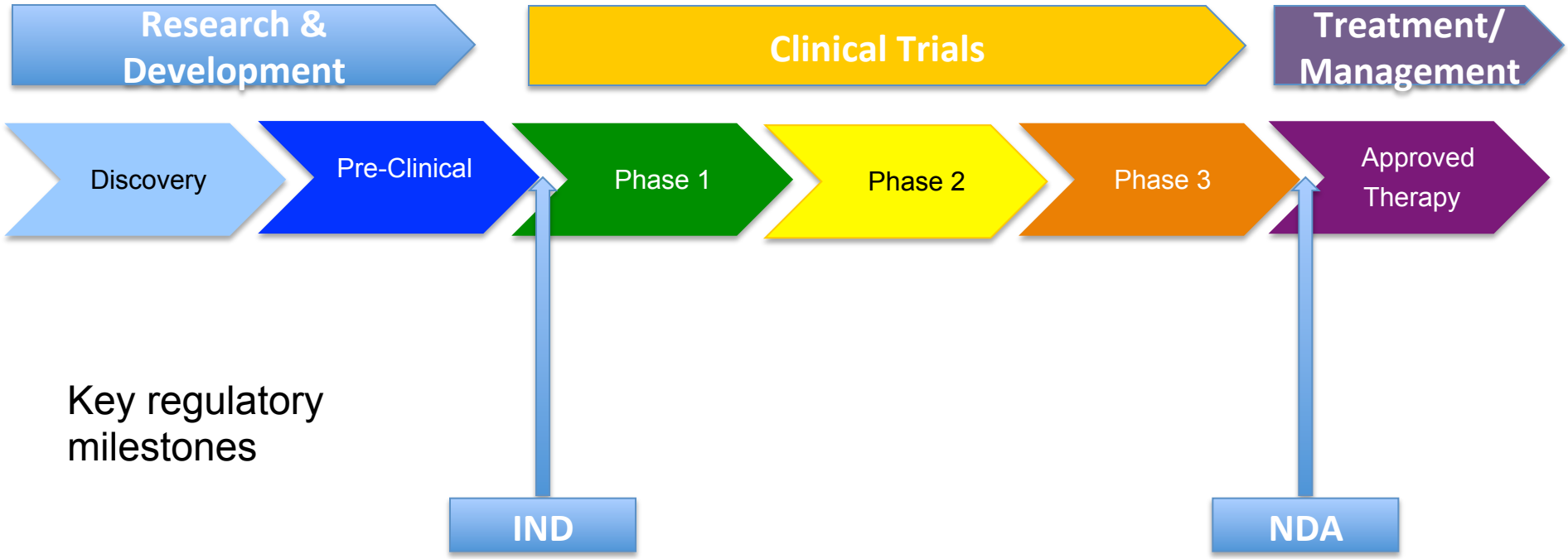
Drug Development and Clinical Trials Process



Key regulatory milestones

Not a straight line.
Time and effort at each stage is not the same.

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What is a clinical trial?

- A clinical trial is a biomedical or health-related RESEARCH studies in HUMAN beings that follow a pre-defined PROTOCOL.

- It is an EXPERIMENT; not first in line to therapy

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Clinical Trials

- What are the different types of clinical trials?
  Treatment trials test experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.
  Prevention trials look for better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. These approaches may include medicines, vaccines, vitamins, minerals, or lifestyle changes.
  Diagnostic trials are conducted to find better tests or procedures for diagnosing a particular disease or condition.
  Screening trials test the best way to detect certain diseases or health conditions.
  Quality of Life trials (or Supportive Care trials) explore ways to improve comfort and the quality of life for individuals with a chronic illness.

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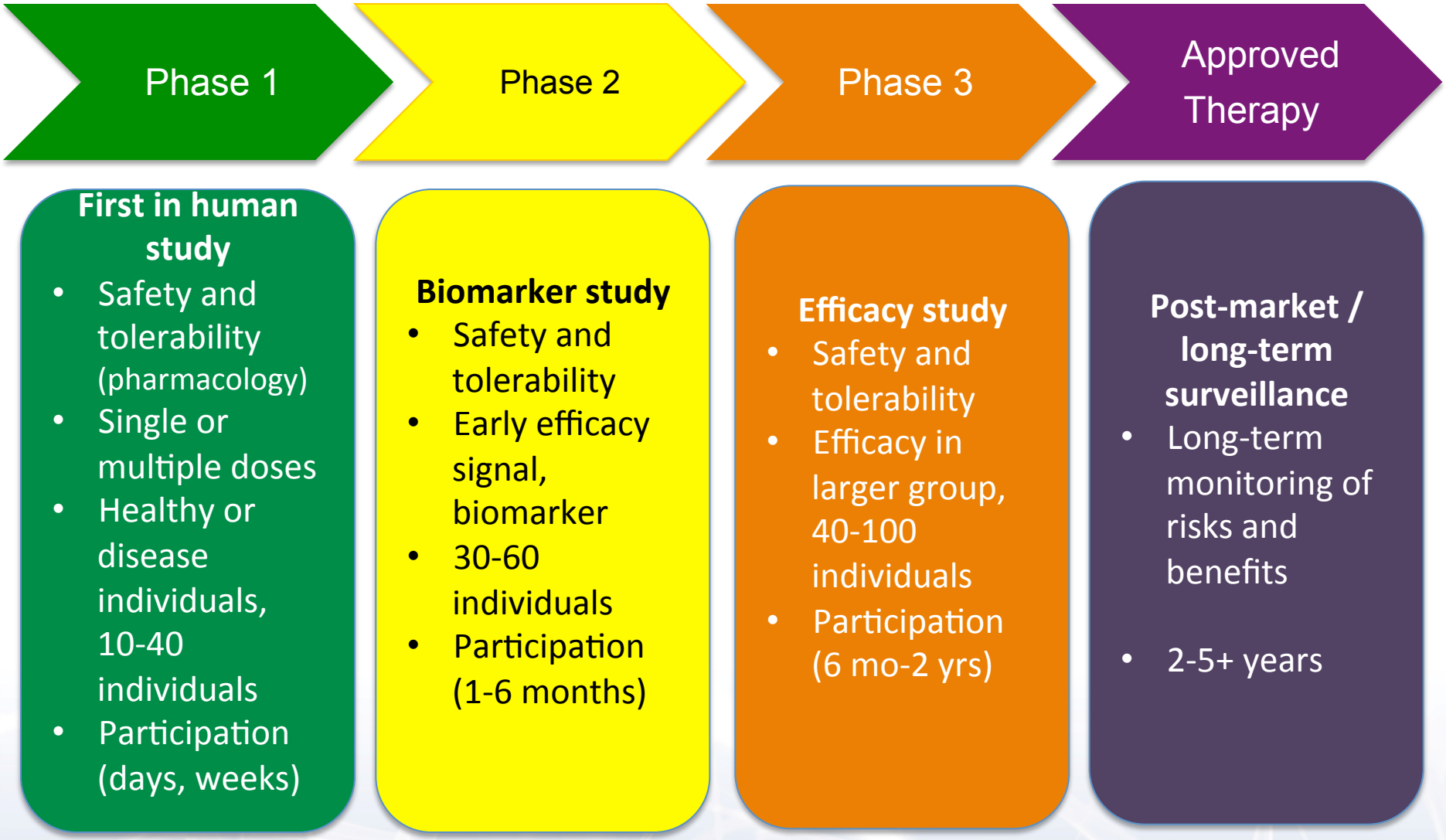
Clinical Trials



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Protocols / Study Guidelines

- How study is to be conducted
  - Purpose of study
  - Participants
  - Duration and number of visits
  - How the study will be carried out
    - how safety monitored
    - what information will be gathered about participants
    - endpoints – Biomarkers, efficacy measures
  - Stopping rules
  - Data management

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Clinical Trials – Be Informed

- How do you know if a trial is happening?
  - ClinicalTrials.gov
  - FARA patient registry – www.curefa.net/registry
- Are you a candidate for the clinical trial?
  - Inclusion and exclusion criteria
    - age, gender, the type and stage of a disease, previous treatment history, and other medical conditions
    - identify appropriate participants and keep them safe and help ensure that researchers will be able to answer the questions they plan to study
  - Before joining a clinical trial, screening step, participant must qualify for the study,

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Clinical Trials – Be Informed

- How do you decide about participation in a trial?
  - Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate. It is also a continuing process throughout the study to provide information for participants.
    - Benefits and risks
    - Procedures or tests required
    - How will the trial affect your daily life and how long will it last…. Travel???
    - Who is in charge of my care?
    - Payment, reimbursement
    - Results

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Clinical Trials – Be Informed

- Can a participant leave a clinical trial after it has begun?
  Yes. A participant can leave a clinical trial, at any time, however better to not enter the study if you have reservations, serious concerns or personal circumstances that can interfere.
  - Significant consequences to the study, especially if the number of participants is small

What if you don't qualify for a clinical trial?

- Can you still get the drug?
  - Expanded access - FDA regulations enable manufacturers of investigational new drugs to provide for "expanded access" use of the drug
    - clinical investigators are actively studying the experimental treatment in well-controlled studies, or all studies have been completed
    - there must be evidence that the drug may be an effective treatment in patients
    - the drug cannot expose patients to unreasonable risks given the severity of the disease to be treated.

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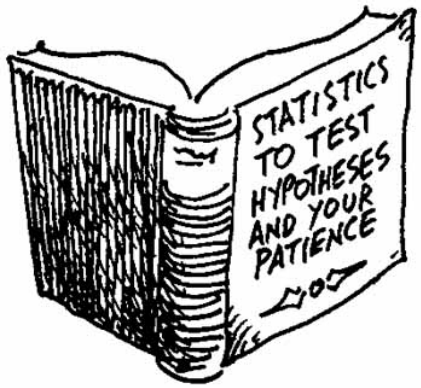
Clinical Trials – Interpreting the data/results

- Prior to the start of a clinical trial a statistical analysis plan is written
  - Deciding up front how data will be handled, specific analysis of all the measures
  - Stating what would be meaningful outcome
- However, not all studies will reach statistical significance
  - Remember they are EXPERIMENTS
  - Need to learn for the data



Clinical Trials – Being a research subject

- Be prepared, be knowledgeable
  - FARA – Patient Registry
  - Take advantage of the informed consent process – make the best decision for you!
- Follow the rules – the research protocol is for the study investigators and the subjects
  - Compliance with study visits, diets, diaries, etc…
  - Communication with the study investigators and coordinators, especially about any possible side effects
    - Report any time you visit a hospital, ER, outpatient clinic
    - You should not be determining what is side effect or not that is the responsibility of the study investigator
  - Refrain from your own experiments – this will invalidate any possible result that the study was designed to evaluate
- Confidentiality – communicating your impressions of study experiences can compromise the integrity of the data

Clinical Trial Updates

- 3 trials enrolling now
  - Phase 1 – RT001, Retrotope, Dr. Harry Saal
  - Phase 2 – MOXIe, RTA408, Reata, Dr. Ed Doherty
  - Phase 3 – STEADFAST, Actimmune, Horizon, Julie Ball

FARA Assets Across the R&D Continuum

- **Grant funding**
- **Access to academic experts & clinical network**
- **Assays**
- Mouse models
- Cell lines and biorepository
- Natural history database
- Gene expression Data

- **Patient registry / recruitment**
- **Patient engagement & access, retention**
- **Trial design advice**
- Funding support / patient costs
- Serve on DSMBs
- Endpoint, biomarker advice & development

- **FDA advocacy in post phase 2/3 meetings**
- **Serve on FDA advisory committees**
- Provide testimony at FDA hearings
- Patient engagement / education

Discovery → Pre-Clinical → Phase 1 → Phase 2/3 → FDA review & approval → Approved Therapy

- Assays
- **Animal and cell models**
- **FDA advocacy at pre-IND**
- **Prevalence data, disease burden**
- Disease characterization
- Access to academic and clinical experts
- **Validated outcome measures for clinical planning**

- **Clinical network sites for trials**
- Clinician / Site recommendations
- FDA advocacy at post phase 1 meetings
- Patient registry / recruitment
- Patient engagement and access, recruitment
- **Endpoints & Biomarkers**

- **Patient access & communications**
- Website/newsletter/blog, social media articles
- **Seminars & conferences - Co-present results**
- Payer engagement
- **Assist w/ post-market surveillance initiatives**

Adapted from the Parkinson's Disease Foundation 2013 and the Clinical Trials Transformation Initiative

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