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THE “INS AND OUTS” OF CLINICAL TRIALS

WHAT IS A CLINICAL TRIAL?

- A clinical trial, also called a clinical research study, is a carefully designed scientific evaluation of an investigational medication or treatment
- Clinical trials are conducted by doctors and researchers. They help to answer important medical questions, such as:
 - Is the investigational medication safe?
 - How does it act in the body?
 - How does it affect certain diseases or conditions?

TYPES OF CLINICAL TRIALS

- The National Institute of Health (NIH) organizes trials into 6 different categories:
 - **Treatment Trials:** test experimental treatments, new combinations of drugs, or new approaches to surgery or radiology/radiation therapy
 - **Prevention Trials:** look for better ways to prevent disease in people who have never had them or prevent them from returning
 - **Diagnostic Trials:** 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
 - **Behavioral Trials:** evaluate or compare ways to promote behavioral changes designed to improve health
 - **Quality of Life:** explore ways to improve comfort and the quality of life for individuals with chronic illness

WHO SPONSORS CLINICAL TRIALS?

- There are many different ways that clinical trials are funded and conducted. Here are some examples:

Physicians

Medical institutions

Foundations

Voluntary Groups

Pharmaceutical
Companies

Federal Agencies
(i.e., National
Health Institute)

HOW A NEW MEDICATION OR TREATMENT COMES TO BE

Before clinical research or testing can be done in human beings

they must conduct extensive pre-clinical or laboratory research

Extensive testing

Research usually involves years of experiments in animal and human cells

If this stage of testing is successful

this data will be submitted to the U.S. Food and Drug administration (FDA), requesting approval to being testing in humans

- If approved by the FDA, testing in humans begins
- This is done through a formally written and approved protocol

WHAT ARE THE PHASES OF CLINICAL TRIALS?

- Clinical trials are typically grouped into four types of phases. The clinical trial phases are:



In Phase I trials, researchers test a new drug or treatment in a small group of people for the first time to gather information on its safety, dosing and side effects

- General Trial Size: 10-80 patients

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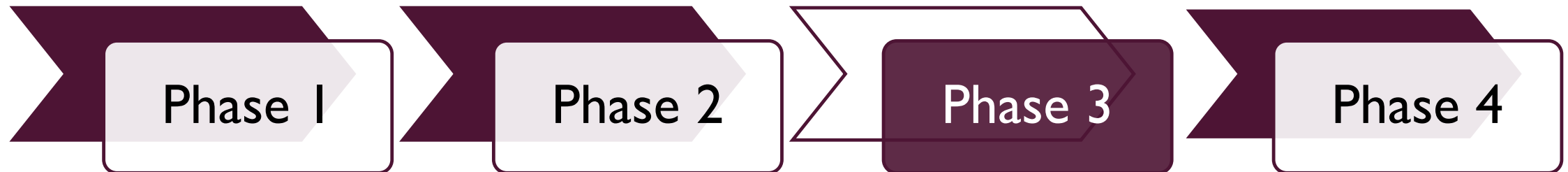


In Phase 2 trials, the drug or treatment is given to a larger group of people to see if it is effective and to better understand its safety

- General Trial Size: 100-300 patients

WHAT ARE THE PHASES OF CLINICAL TRIALS?

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In Phase 3 trials, the drug or treatment is given to large groups of people to confirm its effectiveness, compare it to commonly used treatments, and collect information that will allow the drug or treatment to be used safely.

- General Trial Size: 1000-3000 patients and beyond

WHAT ARE THE PHASES OF CLINICAL TRIALS?

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Phase 4 studies are conducted after a drug or treatment is approved for use by the regulatory authorities. They gather additional information on the drug's effects and side effects.

- Trial size varies significantly by disease area and goals of the trial

CLINICAL RESEARCH IS REVIEWED TO ENSURE ETHICAL AND SAFETY MEASURES ARE IN PLACE

- Clinical trials in the United States are approved and monitored by an Institutional Review Board (IRB) to ensure that the risks are minimal when compared with potential benefits.
- An IRB is an independent committee that consists of physicians, statisticians, and members of the community who ensure that clinical trials are ethical and that the rights of participants are protected
- An IRB has the authority to approve, require modifications in (to secure approval), or disapprove research
- This group review serves an important role in the protection of the rights and welfare of human research

WHO CAN PARTICIPATE IN CLINICAL RESEARCH?

- Each clinical trial has different requirements, also called eligibility criteria, which determine whether a person can participate
- The factors that describe who can participate in a clinical trial are called "inclusion criteria" and those that describe who cannot participate are called "exclusion criteria".
 - These criteria are based on such factors as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions.

It is important to note that inclusion and exclusion criteria are not used to reject people personally.

Instead, the criteria are used to identify appropriate participants and help keep them safe. The criteria help ensure that researchers will be able to answer the questions they plan to study.

WHAT HAPPENS WHEN SOMEONE IS THINKING ABOUT JOINING A CLINICAL TRIAL?

- A member of the clinical study team is required by law to review a detailed document about the study with participants, called the “Informed Consent Form.”
 - clinical study team includes doctors, nurses, and other health care professionals
- This document provides the participants with important information about a clinical research study and documents the agreement to participate.
- The purpose of “Informed Consent” is to make sure that:
 - Participants understand what participating in the study will involve, including the potential side effects and other risks associated with the investigational medication or the study procedures
 - Participants understand their responsibilities as a study participant
 - Any questions participants have about the study have been answered
 - Participants want to be in the study
 - Participants know that they have the right to leave the study at any time

SOMEONE AGREES TO PARTICIPATE ... WHAT HAPPENS NEXT?

- A participant will attend a screening visit and undergo tests and evaluations (as described in the Informed Consent) to determine if that person's condition is a good match for the study
- During the study, the participant will be expected to visit the study doctor regularly and undergo the clinical trial procedures (as described in the Informed Consent Form). While in the trial, the clinical study team will follow your condition closely.
- The clinical study team checks the health of the participant, give specific instructions for participation, and monitor the participant from the beginning until the end of trial.
- Some clinical trials involve more tests and doctor visits than the patient would normally have for an illness or condition

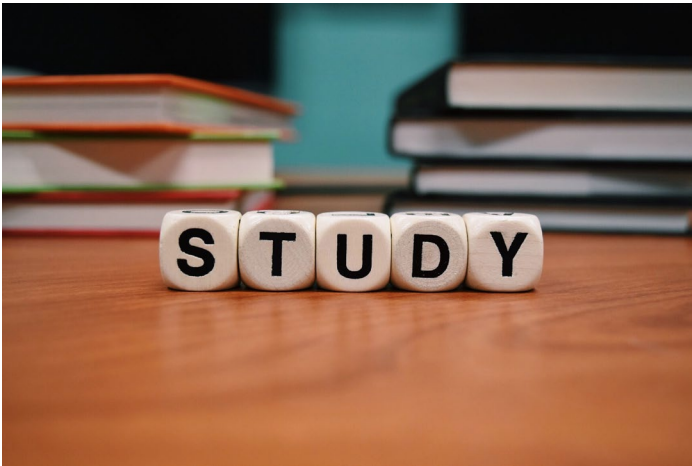
WHAT HAPPENS AFTER A CLINICAL TRIAL IS COMPLETED?

- After a clinical trial is completed, the researchers carefully examine information collected during the study before making decisions about the meaning of the findings and about the need for further testing
- Results from clinical trials are often published in peer-reviewed scientific journals.
 - **Peer review** is a process by which experts review the report before it is published to ensure that the analysis and conclusions are sound.
 - If the results are particularly important, they may be featured in the news, and discussed at scientific meetings and by patient advocacy groups before or after they are published in a scientific journal
- Once a new approach has been proven safe and effective in a clinical trial, it may become a new standard of medical practice.

PROCEDURE	SCREENING	BASELINE	VISIT 1	VISIT 2	COMPLETION
Informed Consent	X				
Eligibility Criteria	X				
Meds/Med Hx/Surg Hx	X				
Fibroscan/Ultrasound	X				
Lab Tests (screening)	X				
Study Drug Disp.		X	X	X	
Safety Evals (PE/VS)	X-Complete	X-Symptom-directed	X-Symptom-directed	X-Symptom-directed	X-Complete
Lab Tests (Hem/Chem/UA)	X	X	X	X	X
Pregnancy Tests-Serum	X				
Pregnancy Tests-Urine		X		X	X
HBV Virology	X	X	X	X	X
Patient-Reported Outcomes		X		X	X
AE/Con Meds	X	X	X	X	X

SAMPLE PROTOCOL PROCEDURES – PATIENT EXPERIENCE

GENERAL STUDY CONSIDERATIONS



- Visit schedules will vary – visits may be every week or every other week for the first couple of months, then switch to monthly and beyond as the study progresses
- Study treatment length will vary depending on disease and/or phase
 - Early Phase trials are typically shorter (1-4 weeks total)
 - Later Phase trials are longer (may be 48-52 weeks or longer)

