

Understanding Clinical Trials

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Clinical Research, Clinical Trials, Clinical Studies, Protocols

These are terms that describe the process by which we advance our understanding of disease and make medical progress in treatment.

Goals of Clinical Research

- Identify causes of disease
- Evaluate treatment options with either new or existing medications or therapies
- Compare treatments

Phases of Drug Development

Before being tested in humans, new treatments are usually evaluated in animals to evaluate basic measures of therapeutic effect and safety. Once certain safety criteria are met and the nature of the effect a drug has in animals is understood, the drug may move on to testing in human volunteers. This testing occurs in four phases:

Phase 1

- The first testing of a drug in humans
- Conducted with a small number of volunteers (20-100 participants)
- Purpose is to determine how the body handles a drug – how it is metabolized and eliminated
- Screens for adverse effects

Phase 3

- Larger number of patients with disease studied (from 200 to >3,000); Primary concern is effectiveness and safety
- About 25-30% of new treatments are eventually approved

Phase 2

- Small-scale program in up to a few hundred patients with the targeted disease
- Purpose is to evaluate efficacy and side effects and to determine ideal dosing
- About 33% of drugs studied in phase 2 go on to the next phase

Phase 4

- These studies are usually performed after a drug has been approved
 - Often mandated by the FDA and agreed to by the sponsor (drug company) as a condition of drug approval
 - Large-scale program evaluating several thousand volunteers with focus on safety
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Will I always get the new drug if I participate?

Earlier phase trials usually dispense the new medication to all patients.

Later phase trials usually involve randomly receiving a placebo or the medication being tested in one or more doses. This is called a placebo-controlled Randomized Clinical Trial (RCT) and is considered the gold standard for determining whether a medication works or not. Neither the patient or the clinician knows whether the patient is receiving a placebo or the drug.

In some late phase trials, all patients will receive the active drug; the informed consent and the personnel conducting the study will make this clear at the outset of the trial

Please see the Patient Education Sheet on “Clinical Trials – Getting Involved” for more information on clinical trials. This sheet is available on the Foundation website at <http://www.sjogrens.org/home/about-sjogrens/brochures-and-fact-sheets>.

Additional information and a list of specific trials currently available for enrollment can be viewed by visiting <http://www.sjogrens.org/news/486-sjogrens-s-clinical-trials>.