

## What is a clinical trial?

Once a candidate drug has been tested and characterized in animals, it is ready to be tested in humans and enters the clinical phase of development. A clinical trial is a health related study designed to evaluate a new drug or combination of drugs. Information gathered from clinical trials provides a basis for understanding how new therapies work, how effective they are, and if they are safe enough for use by the public. Initial clinical studies are usually performed in healthy volunteers, testing single, followed by multiple, doses of the drug. A proof-of-concept study is conducted to provide the first evidence that a candidate drug might be effective for a disease. At all stages of clinical development, the criteria for continuing to investigate the candidate drug include safety, tolerability, pharmacokinetics, duration of action, efficacy, patient acceptability, and commercial viability at the planned time of launch. If the results of a clinical trial demonstrate that the drug is beneficial and safe, the results are submitted to the appropriate regulatory agency, such as United States Food and Drug Administration (FDA) or European Medicine Agency for review. The regulatory agency uses this information to determine whether or not to approve the drug.

The different phases of clinical trials/studies are described in the table below. Phase 1, 2 and 3 clinical trials are conducted prior to marketing of the drug, while Phase 4 trials are conducted after the drug has received marketing approval.

Phase	Population type	Scale Duration	Main objective
Phase I	Healthy volunteers	Very small Days	Safety Tolerability Pharmacodynamics Pharmacokinetics
Phase II	Patients (hospitals)	Small Weeks	Safety Tolerability Proof-of-concept Dose-ranging/finding Preliminary efficacy
Phase III	Patients (hospital or general practice)	Large Months	Long-term safety and efficacy vs competition drugs
Phase IV	Patients (general practice)	Very large Months/years	Long-term safety

## **Where Can I Learn More?**

To learn more about clinical trials and what you need to consider before participating, visit [www.clinicaltrials.gov/ct2/home](http://www.clinicaltrials.gov/ct2/home). This site is an excellent resource for patients, care providers, and healthcare professionals. It provides a registry of federally and privately supported clinical trials being conducted in the United States and around the world. It also includes information about a trial's purpose, who may participate, locations, and phone numbers for more details. If you are considering participating in a clinical trial, be sure to review the resource titled "Understanding Clinical Trials" at [www.clinicaltrials.gov/ct2/info/understand](http://www.clinicaltrials.gov/ct2/info/understand).