



## Common health research terms

### A GUIDE FOR PEOPLE LIVING WITH PULMONARY FIBROSIS

As research about pulmonary fibrosis (PF) expands, many people living with PF are interested in reading about—and even participating in—clinical trials. Whether you’d like to be part of a study or just want to better understand what you’re reading about PF research, this guide helps explain common health research terms.

#### TYPES OF RESEARCH

**Basic science research** studies the biologic and chemical processes in living organisms and the molecular mechanisms of disease. Basic science research is usually conducted in a laboratory using cells or tissues grown in a lab or animals, such as mice or rats.

**Translational research** takes what’s been learned in basic science research and applies it to developing solutions to human medical problems.

**Clinical research** explores whether a medical strategy, treatment, or device is safe and effective for humans. Clinical research studies also may show which medical approaches work best for certain illnesses or groups of people.

A **patient registry** is a collection of health-related information about individuals with a specific diagnosis, disease, or condition. This information can be used by researchers to study aspects of the condition of interest.

#### TYPES OF CLINICAL TRIALS

In **interventional studies**, participants (or “study subjects”) are assigned by the investigator (researcher) to a drug, treatment, procedure, or activity (known as an “intervention”), and their outcomes are measured.

Participants in **observational studies** are observed without any study-related intervention, and their outcomes are measured and compared by investigators.

The U.S. Food and Drug Administration (FDA) allows manufacturers to provide new therapies that are still being researched to patients with serious diseases or life-threatening conditions who are unable to enroll in an ongoing clinical trial. Participants in these trials, called **expanded access studies**, have some clinical information, such as demographics or outcomes, collected as part of the study.

#### CLINICAL TRIAL PHASES

**Phase I** studies are often conducted with healthy volunteers and emphasize safety with the goal of finding out the treatment’s most frequent and serious adverse events and, if it is a medication study, how the drug is metabolized and excreted.

**Phase II** studies collect preliminary data on effectiveness (whether the intervention works in people who have a certain disease or condition). For example, participants receiving a study drug may be compared to similar participants receiving a different treatment, often an inactive substance called a placebo. Different doses of a study drug may be used and compared in this type of study. Safety continues to be evaluated and adverse events documented.

**Phase III** studies gather more definitive information about safety and effectiveness by comparing the study intervention to a placebo in larger populations. In a phase III study, the study intervention may be used in combination with other available treatments.

**Phase IV** studies occur after the FDA has approved a drug for use in patients. These include studies that are required of or agreed to by the study sponsor, and gather additional information about a drug's safety, efficacy, or real-world use.

## RESEARCH TEAM MEMBERS

**Sponsor**—An individual, company, institution, or organization that takes responsibility for the initiation, management, and/or financing of a clinical study or trial.

**Principal investigator (PI)**—The researcher, usually a doctor or other medical professional, who leads the clinical research team and, along with the other members of the research team, regularly monitors study participants' health to determine the study's safety and effectiveness. A PI is primarily responsible for the preparation, conduct, and administration of a research grant, cooperative agreement, or other sponsored project in compliance with applicable laws and regulations. A PI must also comply with policies of their own institution (such as a healthcare system or university) governing the conduct of clinical research.

**Clinical research coordinator (CRC)**—A healthcare professional who works directly with patients, or uses data from patients, to do research on health and disease and develop new treatments.

## OTHER KEY RESEARCH TERMS

**Adverse event**—An unfavorable change in the health of a participant, including abnormal laboratory findings, that happens during a clinical study or within a certain time period after the study has ended. An adverse event may or may not be caused by the intervention being studied.

**Efficacy**—The ability of a drug or treatment to produce a beneficial result.

**Eligibility criteria**—Key standards that people who want to participate in a clinical study must meet or the characteristics they must have. Common examples include age, the type and stage of a disease, previous treatment history, and other medical conditions. Studies usually have two types of eligibility criteria:

- Inclusion criteria: Characteristics a person must have to participate in the clinical study.
- Exclusion criteria: Characteristics a person must not have to participate in the clinical study.

**End points**—Targeted outcomes of a clinical trial that are statistically analyzed to help determine the efficacy and safety of the intervention being studied.

**Informed consent**—The process used by researchers to communicate with potential and enrolled participants about a clinical study, sharing important information such as the potential risks and benefits of the study and the assessments that will occur during the study period. A person must sign an informed consent document to enroll in a clinical study.

**Placebo**—An inactive pill, powder, or infusion that does not provide treatment, sometimes called a "sugar pill." A placebo may be given to some participants in a clinical trial instead of the active experimental treatment to help determine if the experimental treatment is effective. Placebos are used to prevent the "placebo effect," in which people receiving an intervention may experience improvements to their health just from believing that the intervention will help them.

**Study population**—The group of individuals who participate in a particular study or clinical trial.

**Blinded study**—A study in which the clinical trial participant does not know if they are receiving the study drug or a placebo.



### Blinded studies

Many studies include "blinding," a way of managing the information participants and/or researchers have about which participants are receiving the study drug or a placebo.

**Double-blinded study**—A study in which neither the clinical trial participant nor the researcher administering the treatment knows if the participant is receiving the study drug or a placebo.

**Unblinded study**—A study in which information about the assigned study treatment is available to all people and groups involved in the research, including researchers and trial participants.

## Still have questions?

Visit the PFF Clinical Trial Education Center at [pulmonaryfibrosis.org/clinicaltrials](https://pulmonaryfibrosis.org/clinicaltrials).