Pulmonary Fibrosis Foundation™

Participating in clinical trials

A GUIDE FOR PATIENTS WITH PULMONARY FIBROSIS

Helping build a brighter future for patients with PF

Clinical trials are research studies that explore whether a drug, treatment, device, or medical strategy is safe and effective for humans. These studies follow strict scientific standards to protect patients and produce reliable research results.

For the pulmonary fibrosis (PF) community, clinical trials offer answers to questions that could change the future of how PF is treated: How well does a new drug work or not work? Is there a better way to treat PF? Do different patients with PF benefit from different types of drugs or treatments? How do genes and people's environments—where they live, what jobs they do, how active they are—affect those living with PF?

Patients with pulmonary fibrosis may find several advantages to joining a clinical trial. Participants may obtain access to a potential new PF medication and receive valuable education about improving their health. Increased clinic visits and testing usually associated with a clinical trial may give the participant a clearer picture of the state of their disease and progression. Many PF clinical trial participants say that even if joining a trial doesn't directly benefit them, they view it as an important step on the road to helping future patients with PF.

TYPES OF CLINICAL TRIALS

Clinical trials can take several different forms:

- In **interventional studies**, participants are assigned by the investigator to a drug, treatment, or other intervention, and their outcomes are measured.
- In **observational studies**, participants are observed (without being given a drug, treatment, or other intervention) and their outcomes are measured by the investigators. The PFF Registry is one example of an observational study in which participants have agreed that their data may be used in this way.
- In **expanded access studies**, the U.S. Food and Drug Administration (FDA) allows manufacturers to provide new drugs that are still being researched to patients with serious diseases or conditions who cannot participate in a clinical trial.

Study sponsors

Clinical trials can be supported by a variety of organizations: academic medical centers, pharmaceutical companies, biotechnology companies, medical device manufacturers, nonprofits, or government agencies.

TRIAL PHASES

The FDA is responsible for carefully reviewing and approving all new drugs or treatments before they can be prescribed to patients. Interventional clinical trials involving humans have four phases:

Phase I: Researchers test a new drug or treatment in a small group of people to evaluate its safety, determine a safe dosage range, and identify side effects.

Phase II: The drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.

Phase III: The drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments (or placebos), and collect information that will allow the drug to be used safely.

Phase IV: After the drug or treatment has been approved for use, additional studies gather information on its effect in various populations and any side effects associated with long-term use.

PROTECTING YOUR SAFETY

As a clinical trial volunteer, you'll have several tools in place to protect your safety and well-being. Researchers use a process called informed consent to provide possible participants with information about the potential benefits, risks, and alternatives to participation. Before joining the study, you must sign an informed consent document to show you received and understand this information. Signing the document is not a contract. You may still withdraw from a study at any time, even if the study is not over.

Every study of a drug, biological product, or medical device regulated by the FDA must be reviewed, approved, and overseen by an institutional review board (IRB). An IRB is made up of doctors, researchers, and members of the community. Its role is to make sure the study is ethical and the rights and welfare of participants are protected. This includes reviewing the informed consent document as well as making sure research risks are as small as possible when balanced with potential benefits.

THE RESEARCH TEAM—AND YOUR PLACE ON IT

Like a sports team, a clinical research team involves people with different roles working together:

- The **principal investigator** (**PI**) is like a head coach who organizes and leads the study, records and analyzes the data, and directs the study staff. The PI creates and follows the study protocol, a detailed plan like a playbook, for how the study will be carried out.
- The **clinical research coordinator** (**CRC**), similar to an assistant coach, handles day-to-day study activities and will likely be your main point of contact.
- The organizations and people responsible for **volunteer protections**—for example, the FDA and the institutional review board—are like referees. They review the study before it starts, make sure the team follows the rules, and keep you safe and informed.
- **Clinical trial participants** are the players on the field and the most important members of the team. Without patients like you volunteering for clinical studies, new drug and treatment developments wouldn't be possible.
- **Family, friends, and support teams** are the fans in clinical research. They help you ask questions and support you during the study.

What is a blinded study?

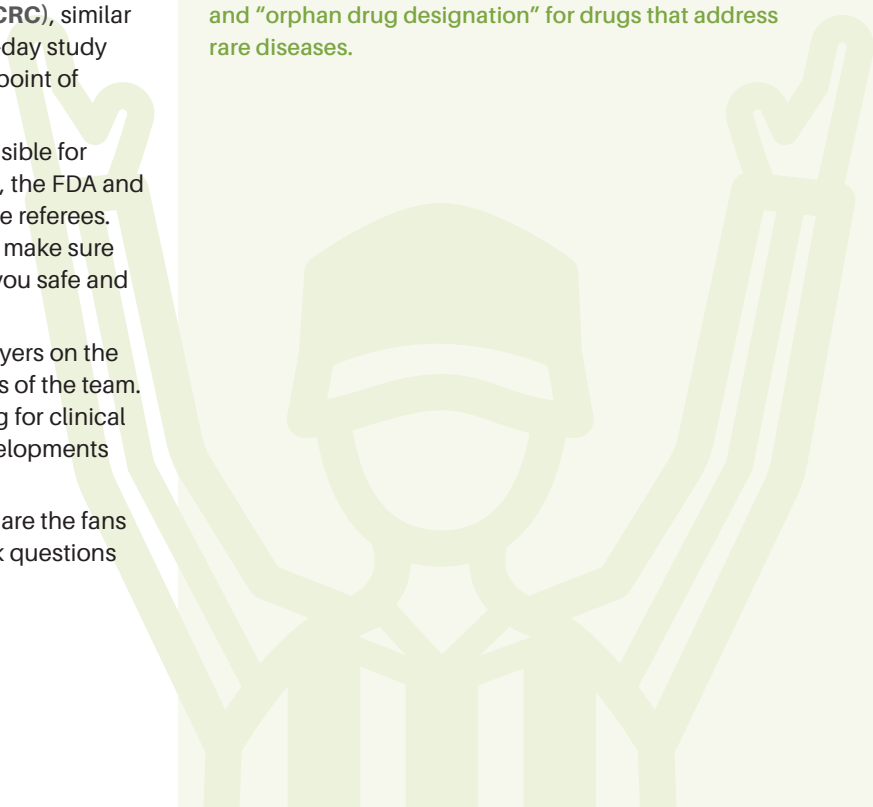
Studies using placebos are "blinded." In a single-blind study, participants don't know whether they're receiving a placebo or the drug being investigated. In a double-blind study, neither participants nor the researchers administering the treatments know which group is receiving a placebo, and in a triple-blind study, the identity of the two groups is concealed from participants, administrators, and the individuals analyzing study outcomes.The purpose of blinding is to withhold information that may influence perceptions or behaviors until the study is complete.

The power of placebos

Some studies include a "placebo," sometimes called a "sugar pill." A placebo looks like medicine but has no active ingredients in it. Having one group of volunteers take the drug being studied and another group take the placebo is an important way for researchers to measure how well the new drug will work. If you're part of a trial that uses placebos, you won't know which group you're in—but you'll be closely monitored even if you're on a placebo.

Drug approval and special designations

Sometimes clinical trials and FDA reviews are fast-tracked to help drugs or treatments more quickly reach the patients who need them. Even when trials and approval are sped up, the complex testing process makes sure these drugs or treatments are safe. The expedited "special designation" approvals most relevant to patients with PF are "breakthrough therapy" for drugs treating serious or life-threatening conditions and "orphan drug designation" for drugs that address rare diseases.



PFF clinical trials resources and tools

- The PFF website at **pulmonaryfibrosis.org/clinicaltrials** offers announcements about studies that are currently recruiting and a link to the Drug Development Pipeline, a chart showing the phases of various PF-related studies in progress.
- The PFF Clinical Trial Finder lets users search for studies, narrowing results by location, phase, study type, and more. Users can fill out a short questionnaire about their condition to automatically identify trials in North America with eligibility (inclusion/exclusion) criteria that are a preliminary match. Visit **trials.pulmonaryfibrosis.org.**

Other useful resources

- The Center for Information and Study on Clinical Research Participation (CISCRP), **ciscrp.org.**
- The National Institutes of Health's clinical trials site, **ClinicalTrials.gov.**

The PFF gratefully acknowledges the Center for Information and Study on Clinical Research Participation for providing some of the information used in this guide.

Questions to ask

As you're considering whether to participate in a study, you'll need to gather a variety of information to help you make your decision. Questions you may want to ask your doctor and the clinical trials team include:

- What's the purpose of this study?
- How long is the clinical trial anticipated to last?
- How long will my participation last?
- What will I have to do to participate?
- What are the risks?
- How often and how many times will I have to visit the clinic?
- Who will pay for my participation? (Some trials—not all—will pay volunteers for their commitment or travel time.)
- If there is medical treatment involved as part of the study, who will pay for it?
- Can I maintain all my current treatments and medications while participating?
- Who should I contact for different issues like symptoms, side effects, and scheduling routine appointments?
- Will the study results be provided to me?
- If the study results are favorable, will I have an opportunity to receive the drug or treatment later?

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