PCD FOUNDATION™

PRIMARY CILIARY DYSKINESIA

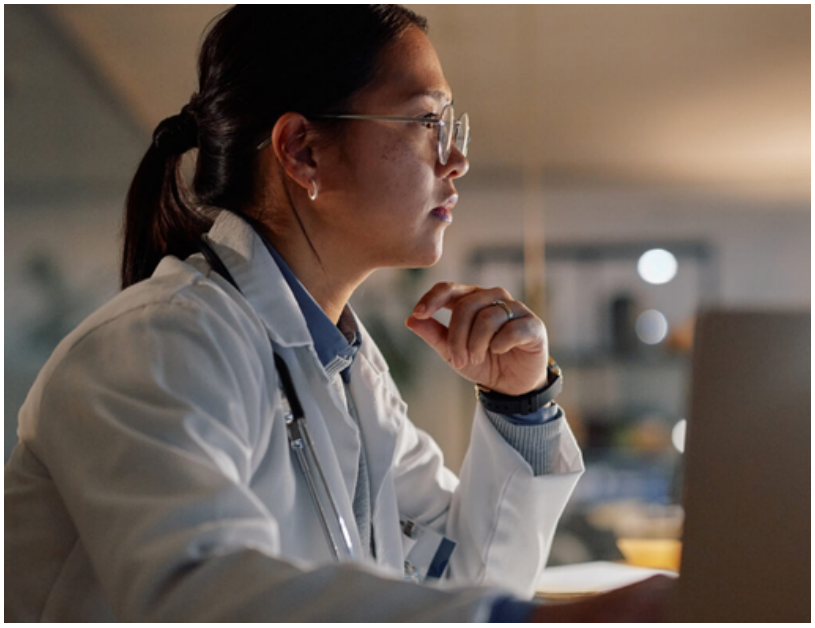
A Guide to Medical Research

Primary Ciliary Dyskinesia

- What is Medical Research?
- Why Participate in Research?
- Clinical Research in PCD
- Informed Consent Guidance

What is Medical Research?

Research is a careful and organized process where people ask questions, gather information, and analyze it to learn new things, prove ideas, or solve problems. In the medical context, it specifically involves collecting and studying data in a structured way to understand something better or add to what we already know. For example, the U.S. Department of Health and Human Services (HHS) says that research is “a planned investigation to develop or add to general knowledge.”



Types of Research

Research can be divided into two main types:

1. Basic Research

This research focuses on gaining knowledge for the sake of understanding something better. It's not always meant to solve immediate problems, but to explore ideas or theories in fields like physics, biology, or psychology.

2. Clinical Research

Sometimes referred to as clinical trials, this research includes human participants and may include intervention (like in a drug effectiveness study) or observation (like the PCD Foundation Research Registry).

Why Participate in Research?

1

Improving Patient Care & Public Health

Research can lead to better treatments and healthcare policies. Studies can help us learn more about diseases, making it easier to detect them early and treat them better.

2

Personal Benefits for Participants

It's important to note that there may be no personal benefit to participation in research and that its primary benefit is to add to general knowledge. Sometimes, however, people who join research studies might get access to new treatments before they're widely available. They may also receive extra medical attention, and they may be compensated for their time commitment and/or reimbursed for certain expenses involved in the research.

3

Advancing Medical Knowledge & Innovation

Research helps discover new treatments and medicines. Clinical trials test how safe and effective new drugs or devices are, which can improve healthcare. All new treatments and medicines need to be researched in human participants – without their participation, these outcomes are unachievable.

4

Addressing Health Disparities

Research helps ensure that everyone, no matter their condition or background, benefits from medical advancements. Studies can focus on specific groups to better meet their health needs.

5

Ensuring Scientific Integrity & Ethics

Medical research – both basic and clinical – is held to a high ethical standard to help ensure it is fair and safe for participants. Organizations like the Food and Drug Administration (FDA) and National Institutes of Heath (NIH) make sure research programs follow strict rules to protect everyone involved. The PCD Foundation complies with all regulatory requirements.

Centers for Disease Control and Prevention. (2022). Health disparities and research participation. https://www.cdc.gov/
National Institutes of Health. (2023). Why participate in clinical research? https://www.nih.gov/

Clinical Research in PCD

Living with primary ciliary dyskinesia (PCD) isn't easy. Every day, researchers, doctors, and patients are working together to find new treatments and improve care. Clinical studies are important for understanding human health and disease, and to identify potential targets for treatment. Interventional clinical trials are also essential for testing new treatments and to determine whether or not they work.

Most clinical trials need a minimum number of patients to see if a treatment really works and ensure there is adequate data behind it. Even if one person feels better during a trial, the treatment has to help the study group as a whole for it to move forward in the process to become approved. Since no treatments are approved for PCD yet, clinical trials are important to identify and secure future treatments.

Clinical trials usually happen in phases:

Phase 1: Checks if the treatment is safe and what dose is best

Phase 2: Checks how well the treatment works and if it continues to be safe

Phase 3: Tests the treatment on more people and compares it to current treatments

There are also studies that track symptoms without giving any treatment. These clinical studies help us learn more about the disease. Our **PCD Foundation Research Registry** is one example of a clinical study. We collect important health information and basic facts about people with PCD at routine appointments, so there is no extra burden on patients. The information collected helps doctors and care teams give better care. Researchers can also use this information to study PCD, look at how treatments work, understand quality of life and impact of disease on wellbeing, and plan new clinical trials. By helping to quantify the number of people diagnosed with PCD, the PCD Foundation Research Registry also helps researchers and the broader medical community see the sizable impact that further research can have.

PCD research requires patients with PCD. Because PCD is a rare disease, all PCD patients need to consider participating in clinical trials to enable the development of new treatments. If you want to learn more, talk to your doctor or visit the PCD Foundation website. By joining, you could help yourself and have a significant impact on people living with PCD in the future.

How do Clinical Trials Work?

Ever wonder what it looks like to participate in a clinical trial? Typically, patients who enroll in a clinical trial take the following steps:

Steps to Join a Clinical Trial

1. **Find a clinical trial** – You or your doctor can look for a clinical trial that you might want to join.

2. **Contact the trial team** – Usually, your doctor will share your information with the study team so they can reach out to you.

3. **Talk with the study coordinator** – A study coordinator will contact you to explain the trial and ask you some questions. They will tell you about the possible risks and benefits. They may also need time to look at your medical information to see if you can join the trial.

4. **If you are eligible** – If you are able to join, the study coordinator will set up your first study visit. At that visit, you will go over the details and sign a consent form to officially join the trial. The study coordinator will also explain the schedule for your study visits and what information you need to share with them and your doctor.

5. **Communicate openly and stay on schedule** – It's important to share all of your trial experience with both the study team and your healthcare team during the trial. You must also make sure to go to all your study visits and regular clinic visits. If the study involves medication, be sure to take the study medicine on time and complete any blood tests or other checks the study asks for.

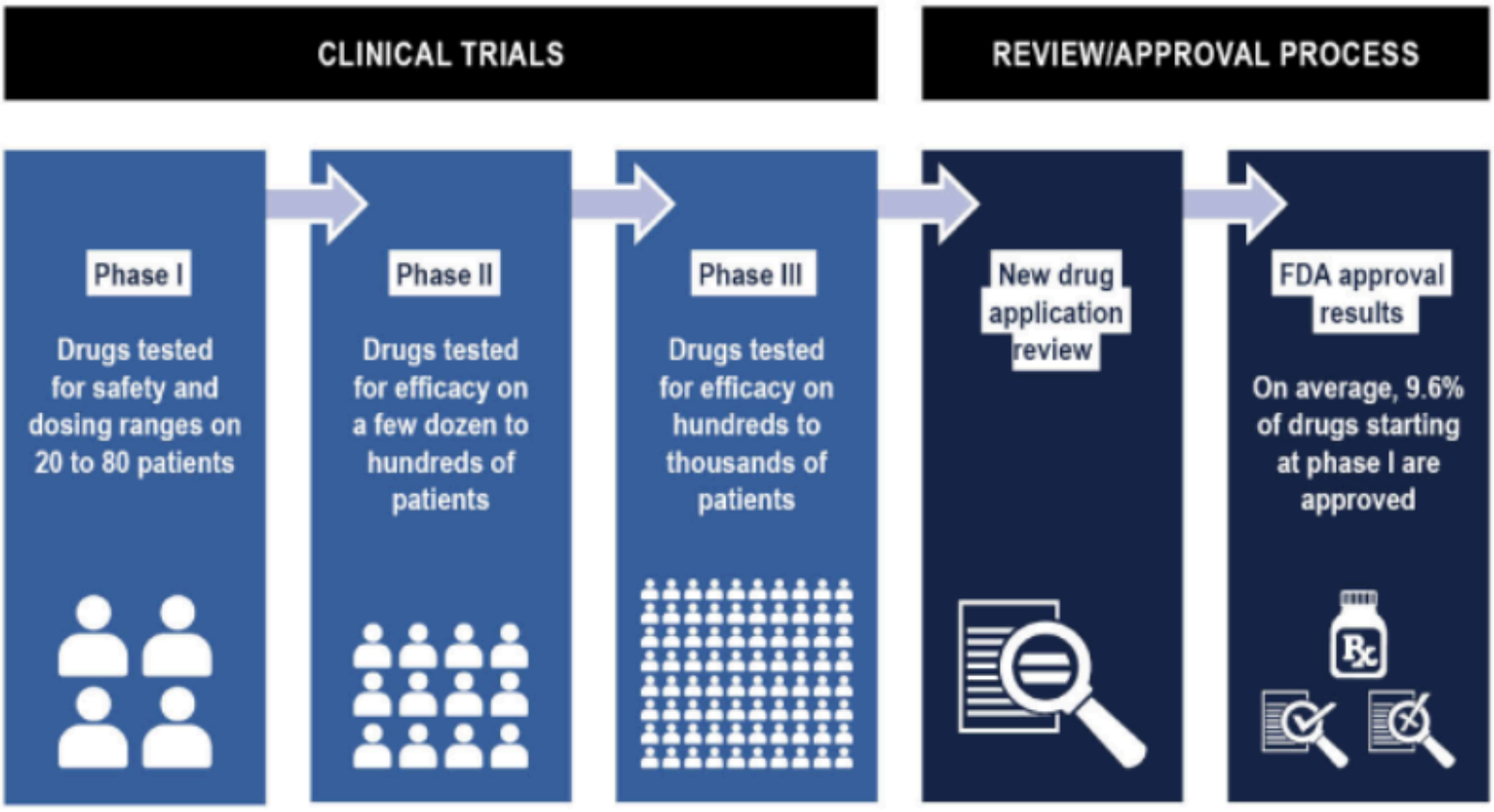
6. **When the trial ends** – The study coordinator will meet with you to finish your time in the study. Most of the time, for medication studies, you won't be told right away if the medicine worked or if it will go on for more testing or FDA approval. Sometimes, if the medicine helps a lot, the study may keep going so you can still get the medicine until it is approved. People who didn't join the trial might not be able to get the medicine until it gets approved, which could take months or even years after the trial ends.

What if I'm Not Elgible for a Clinical Trial?

It can be disappointing to learn that you can't join a clinical trial you were really hoping to participate in. Remember, people are often chosen for trials to give the best chance of seeing if the treatment works. If you are very healthy or very sick, you might not qualify. Don't worry — if the trial is successful and the medicine gets approved, there's a high likelihood that you will be able to get the medicine later, after it has been approved.

Steps to Getting A Medication Approved by the FDA

This explains how a medicine goes from being studied in a lab to becoming available for people to use. In the picture below, you can see the number of people tested at each step. For rare diseases like PCD, the number of people needed is smaller. This picture shows why it's so important for people to join trials so new medicines can get approved.



Sourced from: https://www.gao.gov/products/gao-17-564 (accessed June 30, 2025)

Informed Consent Guidance

Informed consent is a voluntary agreement that means you choose to take part in research after learning all the important facts about the trial — like its purpose, what will occur during the trial, the risks, the benefits, and other choices you might have.

Importantly, while you may sign an informed consent agreement, informed consent is NOT just a document. It is a process by which potential trial participants are fully informed of all aspects of the trial, and their rights and responsibilities should they choose to participate. You are entitled to be fully informed about all aspects of a trial before agreeing to participate.

Adults who can make their own medical decisions can sign their own informed consent. For children, a parent must give informed consent on their behalf. If an adult is unable to make their own medical decisions, a health care proxy (someone chosen to speak for them) must give informed consent.

Who is considered a “child” in research?

A child is someone who hasn’t reached the legal age to give consent for research. In the US, this means anyone under 18 years old.

How does parental permission work?

Since children can’t give informed consent themselves, a parent or legal guardian must give permission for them to take part in research. Based on the clinical trial in question, the Institutional Review Board (IRB), a group that reviews research for safety, will decide if both legal parents or guardians have to sign the consent. Usually, both parents must give permission if the study has more than a small risk and may not directly help the child.

What is assent?

Even though children under age 18 typically cannot give consent to participate in clinical trials, they are asked to give “assent,” meaning they agree that they are willing to participate. Age and other requirements for assent vary slightly by state and/or by institution, but most require that children who are able to comprehend what they will be asked to do and who can communicate be given the opportunity to assent.

To find current clinical trials for PCD, visit **clinicaltrials.gov**.

In the search bar for condition or disease, type "**primary ciliary dyskinesia**" and press enter.

You'll see a list of clinical trials from around the world for people with PCD. Joining a trial is an important decision, and it's always a good idea to talk with your doctor to help decide if joining one is the right choice for you. Clinical trials involve trained doctors and researchers who guide participants and work hard to make sure they understand each element of a trial.

Be part of the journey. Be part of the solution.