CLINICAL TRIALS:
WHAT YOU NEED TO KNOW

National
Kidney
Foundation®

www.kidney.org

National Kidney Foundation's Kidney Disease Outcomes Quality Initiative

Did you know that the National Kidney Foundation's Kidney Disease Outcomes Quality Initiative (KDOQI®) offers guidelines and commentaries that help your doctor and healthcare team make important decisions about your medical treatment? The information in this booklet is based on those recommended guidelines.

Stages of Kidney Disease

There are 5 stages of kidney disease. They are shown in the table below. Your doctor determines your stage of kidney disease, based on the presence of kidney damage and your glomerular filtration rate (GFR), which is a measure of your level of kidney function. Your treatment is based on your stage of kidney disease. Speak to your healthcare professional if you have any questions about your stage of kidney disease or your treatment.

STAGES OF KIDNEY DISEASE

Stage	Description	Glomerular Filtration Rate (GFR)*
1	Kidney damage (e.g., protein in the urine) with normal GFR	90 or above
2	Kidney damage with mild decrease in GFR	60 to 89
3a	Moderate decrease in GFR	45 to 59
3b	Moderate decrease in GFR	30 to 44
4	Severe reduction in GFR	15 to 29
5	Kidney failure	Less than 15

*Your GFR number tells your healthcare professional how much kidney function you have. As chronic kidney disease progresses, your GFR number de-

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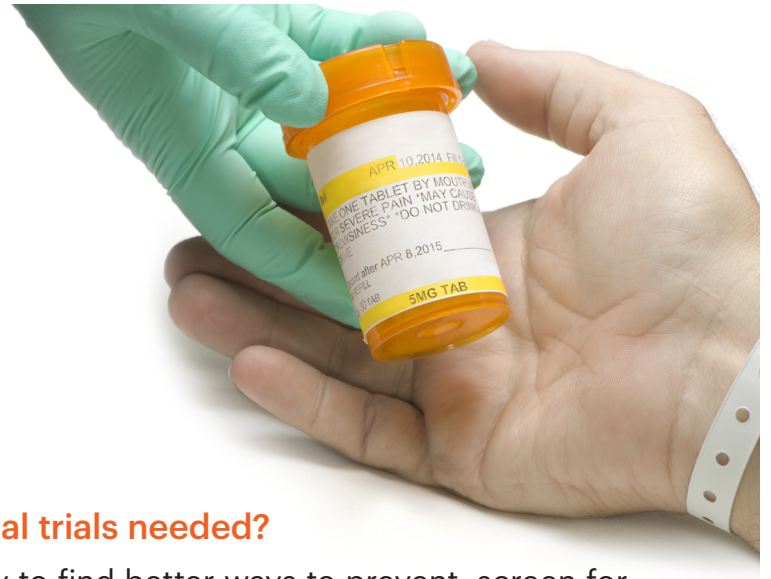
What is a clinical trial?

A clinical trial is a research study that looks at how well a new treatment works in people. This is called "efficacy." The treatment may be a drug, medical procedure, medical device, or even a lifestyle change. A clinical trial also makes sure the new treatment is safe, well tolerated, and does not cause harm or serious side effects in most people.

For example, a clinical trial may look at whether a new exercise routine helps people lose weight. Or it may look at whether a new drug helps lower blood pressure in people with high blood pressure.

In short, a clinical trial helps to answer two important questions:

- **Does the new treatment work in people?** If so, how well does it work? Is it better or the same as treatments that are currently available? If it is the same, does it have fewer side effects than other treatments? Does it help people who have not responded to other treatments?
- **Is the new treatment safe?** No treatment is completely safe for everyone; however, a clinical trial helps make sure the benefits outweigh the possible risks for most people.



Why are clinical trials needed?

Clinical trials try to find better ways to prevent, screen for, diagnose, or treat diseases and other health problems. Without them, we would not have new treatments or other advances in health and medicine.

> *"Hereditary polycystic kidney disease (PKD) runs in my family. This kidney disease has hit our family hard and I was happy to do anything I could to try to help researchers understand this disease better and try to find ways to delay kidney failure."*
>
> —A Kidney Patient

Who can participate in a clinical trial?

People who participate in clinical trials do so freely and of their own will. They are volunteers. In many studies, the volunteers will have a common health condition, such as kidney disease, high blood pressure, or diabetes. Each study has its own rules about who can — or cannot — participate in the study. This is called "eligibility." Your eligibility may be based on your age, gender, overall health, type and stage

of a disease, treatment history, and other conditions. Not everyone is chosen to participate. You may be turned down simply because you do not have certain characteristics, such your gender or age. Things that allow you to participate are called "inclusion criteria," and things that disqualify you are called "exclusion criteria."

Before you can participate in a trial, the researchers must make sure you understand all the possible risks, benefits, and alternatives to the study. As part of this process, you may be given verbal instructions, printed materials to read, questionnaires, and other forms of information. If you have any unanswered questions about the trial, be sure to ask.

At the end of this process—and if you are still willing to participate—you will be asked sign a document stating that you understand the risks, benefits, and alternatives. This is called "informed consent." Remember, the "informed consent" document is not a contract. It is meant to protect you, not bind you. You can withdraw from the study at any time, for any reason, even if the study is not over.

"I truly believe that participating in the research study helped me to gain a better perspective on my illness."

—A Kidney Patient

Who conducts clinical trials?

Each clinical trial is led by a principal investigator, who is usually a medical doctor. In addition, there is often a team of doctors, nurses, social workers, and other health professionals who will work directly with you and provide your medical care. To cover the costs involved, a study must also have financial support. This funding may come from a drug company, university medical center, government agency, or other organization. The group that provides funding is usually referred to as the "sponsor."

Are clinical trials safe?

In the United States, clinical studies are regulated by a government agency called the "Food and Drug Administration (FDA)." Each study is reviewed, approved, and watched over by an independent panel of qualified doctors, researchers, and members of the community called an "Institutional Review Board (IRB)." This review board makes

sure your rights, safety, and welfare is protected, and that the study itself is being done for honest and ethical reasons. It also makes sure that the health risks involved are reasonable compared to the possible benefits. Remember, no treatment is completely safe for everyone; however, a clinical trial helps make sure the benefits outweigh the possible risks for most people.

Before a new treatment can be tested in humans, it goes through years of research in laboratories, followed by testing in animals such as mice or rats. After each phase of research, the clinical trial team submits an "Investigation New Drug" (IND) application to the FDA. Along with an independent ethics committee, the FDA reviews the results and decides whether the study can continue to the next phase. Once approved for human testing, the new treatment is studied in a very small group of people (usually 20–100). These studies are called "Phase I" studies. Gradually, the number of people involved will be increased until the testing reaches Phase III studies. After completing Phase III, the trial team submits a "New Drug Application" (NDA) to the FDA for approval. If the new treatment receives FDA approval, it can be put on the market for consumer (public) use. In some cases, Phase IV trials might be conducted as well.

Why are clinical studies divided into phases?

Each phase has a different goal or certain questions it is trying to answer. Knowing which phase a trial is in can be helpful, especially if you are trying to decide whether to participate. In general, phase I studies have the most potential risk, but they can also be very helpful, especially if you need access to a new treatment that is not yet available for public use.

- **Phase I studies** are meant to find the best dosage that can be given without serious side effects. The first few people in the study will often get a low dose of the treatment and are watched very closely. If there are only minor side effects, the next few participants will get a higher dose. This continues until researchers find the dose that works best and with the fewest side effects.
- **Phase II studies** are generally intended to study the new treatment in a much larger number of people (usually 100–300). The main objective is to see if it works and is well-tolerated in many people.

- **Phase III studies** compare the new treatment to what is currently available. Does the treatment work better—or as well—as treatments that are currently on the market? If it does not work as well, does it have fewer side effects? Do some people improve with this new treatment who did not improve with treatments that are currently on the market? Phase III studies involve large numbers of patients (300–3,000 or more) and are aimed at being a more definitive test of how well the new treatment works compared to the known best treatment, or standard treatment.
- **Phase IV studies** take place after the FDA has approved a treatment for use by the public. Phase IV studies might look at the safety of a treatment in special populations, or over a longer period of time, or to address certain aspects that weren't included in the original studies. For example, a phase IV study might want to find out if an FDA-approved blood pressure drug can also slow the loss of kidney function in people with kidney disease.

What is a study protocol?

Each clinical trial approved by the FDA must have an action plan (called a "protocol"). The protocol describes what will be done in the study, how it will be conducted, and why each part of the study is needed. This helps make sure the study will answer certain questions about the treatment while trying to make sure the people taking part are kept as safe as possible. Among other things, a protocol describes:

- The reason for conducting the study
- Who may participate ("eligibility")
- How many participants are needed
- What tests and procedures will be done during the study and how often
- Information about the drugs that will be used, including the name and dosage
- The phase of the study (phase I – phase IV)
- How long the study will last

Actual study protocols can be very long (over 100 pages) and very technical. They are not written with patients in mind, so they can be hard for most patients to understand. Clinical trials must also have guidelines about who can be in them, and this information can be helpful if you are trying to decide whether you want to participate.

Should I participate in a clinical trial?

You are the best judge and the only person who can answer this question. Participating is not without risks, but it may give you an opportunity to improve your own health.
For example, you may have access to a treatment that's not otherwise available. Or the new treatment might be safer or work better than current treatment options. Some people who have participated in clinical trials believe they receive more attention from the healthcare staff, and more careful monitoring of their condition and possible side effects of treatment. You may even have all or part of your medical

care and other expenses paid for by the study sponsors. Also, you may be helping future generations that experience kidney problems.

But there are also some possible downsides to consider. The new treatment may have unknown side effects or risks, or it may not work for you even if it helps others. You may find some of the requirements inconvenient, such as traveling a long distance for appointments, frequent office visits and tests, or long time commitments.

One thing to ask yourself is whether the potential health benefits will outweigh the possible risks. You should learn as much as you can about the study and feel comfortable asking the research team questions. Be sure to ask who covers the costs and other expenses, such as travel. Tell your own healthcare professional that you are considering a trial, and discuss the pros and cons together.

How can I find a clinical trial?

There are many clinical trial opportunities and new ones are developed every day. Talk to your healthcare professional and let them know you would be willing to help in a research study. Your healthcare professional may know of opportunities in your area. You can also try the following:

- **The National Kidney Foundation**. Find information and links to other resources at kidney.org/patients/resources_ClinicalTrials
- **National Institutes of Health (NIH)**. Learn how you can participate in a clinical trial and find other important information at "NIH Clinical Trials and You." This website also includes a searchable registry to help you find a clinical trial. (Also available in Spanish) nih.gov/health-information/nih-clinical-research-trials-you

- **ResearchMatch.org**. Create a volunteer profile for yourself or for someone else as their parent or guardian. This NIH-funded resource is a free, secure registry that will keep you informed of clinical research studies that need volunteers matching your profile. researchmatch.org
- **Center Watch**. Use this listing service to find an industry-sponsored clinical trial that is currently seeking volunteers. centerwatch.com/ctrc/nkf/
- **ClinicalTrials.gov**. Search this database of publicly and privately supported clinical studies around the world. clinicaltrials.gov
- **Medline Plus**. Find basic information, newsletters, FAQs, glossaries, personal stories, a searchable database, and much more on this website from the U.S Library of Medicine. medlineplus.gov/clinicaltrials.html
- **Children and Clinical Studies**. Learn about children in clinical studies at childrenandclinicalstudies.org/

Learn more

The National Kidney Foundation (NKF) offers a toll-free patient help line for people affected by kidney disease, organ donation, or kidney transplant. Patients, families, and caregivers can speak with a trained professional who will help answer questions and concerns. Call 855.NKF.CARES (855.653.2273) or email nkfcares@kidney.org

The **National Kidney Foundation** (NKF) is the largest, most comprehensive and longstanding, patient centric organization dedicated to the awareness, prevention and treatment of kidney disease in the US.

Help us fight kidney disease. Learn more at kidney.org

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