

Learn the Basics to
Help You Decide
if a Clinical
Trial Is
the Right
Choice
For
You

Clinical Trials Are Definitely Worth a Second Look for Any Patient

Clinical Trials: What Patient Advocates Want You to Know

Patient advocates help patients avoid and fix roadblocks and work to ensure timely and affordable access to prescribed treatment and medication as part of their healthcare.

When working with patients, no matter what the problem is, experienced advocates know the option of clinical trials may come up in the conversation as a possible solution. But what is surprising to so many of us who work alongside patients everyday is the lack of accurate information surrounding this source of advanced treatment. Not only are patients too often unaware of the benefits of clinical trials, but many are also quick to dismiss the suggestion as a feasible avenue, relying solely on inaccurate or limited information.

In this guide, advocates from Patient Advocate Foundation bring forward some of the lesser known and

frequently misunderstood benefits of clinical trials and address the most frequently asked questions from patients.

We encourage all patients, and especially those at high risk of developing disease, to learn more about how they can benefit from new research, and maybe add it to the list of care options to consider in their future.

We know it is just too important of an option for advanced care that many patients are bypassing without realizing it.

MOST PEOPLE PREFERRED TO LEARN ABOUT CLINICAL TRIALS DURING DISCUSSIONS ABOUT TREATMENT OPTIONS WITH THEIR TREATING PHYSICIAN, BASED ON A STUDY PERFORMED BY THE CENTER FOR INFORMATION AND STUDY ON CLINICAL RESEARCH PARTICIPATION.

Center for Information & Study on Clinical Research Participation

Trials Are Not Just For Treatment

Some people only think of clinical trials as last resort treatment options, when other treatments have failed. Not only are many treatment trials geared towards those who have just been diagnosed, there are also numerous trials that are improving other aspects of our healthcare. There are six categories of clinical trials each designed with specific goals in mind:

- *Prevention trials* discover ways to keep from getting a specific disease, and look to identify unique genetic or inherited risk factors.
- *Screening trials* explore new ways to detect diseases or conditions.
- *Diagnostic trials* explore new testing procedures for an illness or condition.
- *Treatment trials* study new drugs, techniques, surgeries or combinations of treatments for those in any program of disease.
- *Quality-of-Life trials* evaluate ways to improve patient comfort, including addressing ways to reduce or eliminate side effects of treatment.
- *Observational studies* determine long term health outcomes by monitoring data over time.

Funding for this publication provided in part by
Amgen's Breakaway From Cancer and
Breakaway From Heart Disease initiatives

PAF Patient Advocate
Foundation

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Questions to Ask Your Doctor

- What are my options for taking part in a clinical trial?
- What are the eligibility requirements?
- When does the trial start? How long will the trial last?
- What is the purpose of the trial and how does this relate to me?
- Who will be in charge of my care? Will I be able to see my own doctor or a doctor in my network?
- How often will I need to visit a physician's office?
- What tests and treatments can I expect throughout the trial?
- What are the likely side effects from the treatment? How will these possible side effects impact my daily life?
- Are there treatments to manage any side effects I may experience?
- Will I need to be at a specific facility to receive the care? If so, how often and for how long?
- Which costs will my insurance cover? What costs are paid by the study? Can I get an estimate for my portion of the cost?
- Are there reimbursement options for the non-medical aspects of the trial, including transportation, parking, tolls, childcare, food, or other supplies?
- What will the information collected during the trial be used for? What are the research results so far for patients like me?
- Can I participate alone, or is it required that I bring a family member? What is required of my caregiver or family member?
- What support will be available for me and my caregivers during the trial? Can I talk to other people participating in the trial?
- What happens to my care after the trial is complete?
- Who can answer additional questions I might think of later?

You're Not a Guinea Pig

By the time a study begins recruiting volunteers, there has been significant research, frequently years of it, that indicated the new option being studied may provide beneficial outcomes. Researchers have rigorously tested, observed, and published medical aspects in many pre-clinical settings before bringing it to patients.

Always check with your health insurance company regarding your benefits PRIOR to joining a clinical trial.

Most Patients (And Doctors) Forget That It's Not Just for Treatment

There are lots of different types of studies that are trying to improve your overall healthcare experience, all included under the "clinical trial" name. Trials are very diverse in what they are studying.

For example, researchers may be looking to identify actions that help us prevent disease, develop easier tests that help detect and diagnose disease faster, investigate whether there are additional things that when added alongside modern medicine may improve our quality of life, or reduce side effects of today's treatment along with many other advances.

Sometimes studies are simply observational in nature and are recording trends or looking at genetic or behavioral connections between patients. Some might be adding non-medical components to medical care to improve the effectiveness of medications, like stress reduction, better sleep, emotional health, or complimentary techniques. Other studies also look at various educational tools or system processes to improve health outcomes and reduce patient frustration.

Some trials include people that are not sick or have not been diagnosed but have family history that makes screening techniques important.

Even treatment trials may simply be looking at whether an additional round or dose of the current medicine would lower your chances of returning, or if a drug approved for another diagnosis would work in your care.



On average, developing a new medication takes at least 10 years and costs 2.6 billion dollars.

What Is the Purpose of a Research Study?

Clinical trials are carefully designed science-driven studies that test the benefits and risks of specific medical treatments or interventions to prevent, treat, or monitor disease, including items such as a new drug or behavior change (e.g. diet).

Clinical trials are at the forefront of medical advancements against all types of disease. Every standard treatment and medicine available today was proven to be effective as a result of going through the clinical trial process. The ultimate goal of clinical trials is to determine if the new treatment, drug, or process being studied is safe and as effective as the current treatment being offered today.



Is it Right For You? What Does Participation Mean Exactly?

When exploring the possibility of joining a clinical trial, these are some factors to take into account as it relates to your particular situation:

- **You get it first** - Trial participants are given the opportunity to be among the first to benefit from a new treatment being studied that is otherwise not available outside of the trial.
- **You will be more involved** - Trial participants tend to be more informed every step of the way, meaning they play an active role and may gain a greater understanding of their disease or condition.
- **More signatures** - You are likely to have more paperwork to review for a trial and more to read. Plus, more medical details (and disclaimers) are shared with you during the process than you might have with the standard treatment.
- **Everyone benefits** - Participants volunteer and ultimately help advance medical research for other patients, even if they don't directly benefit from the results of the specific clinical trial.
- **Possibility of better results** - You could have better results, less side effects, or both compared to the usual care.
- **Experts for your diagnosis** - Trials are done by cutting edge research teams of doctors and specialists who understand your disease and work with patients just like you everyday.
- **Close monitoring** - You may require additional tests, monitoring, or doctor visits than if you were not in a trial. Some patients find this reassuring while others find it a burden.
- **Shared costs** - The costs for any trial-specific items are paid by the trial sponsor and your insurer. Non-medical costs may be eligible for reimbursement as part of the trial.
- **Locations vary** - Some trial locations are online, nearby, or even conducted by your current doctor. But sometimes the trial site may not be a location that is convenient to you, meaning you have more travel needs or have to change doctors.
- **Not always better** - Sometimes the new treatment or new process being investigated will not have any better results than the current standard treatment.
- **A lot is still unknown** - The way you will respond is not as predictable as standard treatments. This means that you may have fewer side effects or you may have more unexpected side effects. Early phase trials will have less information for the doctor to tell you what to expect than later trials. This is also why there is likely to be a requirement to bring someone with you for every treatment.
- **Costs can fall through the cracks** - Even when you ask upfront, there may turn out to be expenses that your insurance and the trial sponsor may not pay and you become responsible for those costs. Laws do require your insurance to cover services associated with routine care including what you would normally have covered outside of a trial for your diagnosis.
- **Random means random** - Sometimes trials may include the use of placebo, (and you must be notified if your study involves one), however in randomized trials you will not get to choose whether you have the standard treatment or the standard plus the trial-specific care. Cancer trials rarely involve a placebo.



According to CISCRP research, 94% of volunteers who participated in a clinical trial study would do so again.

Center for Information & Study on Clinical Research Participation

One Size Doesn't Always Fit All

We have known for a long time that this phrase isn't always true when it comes to our clothing sizes, but now medical researchers are seeing that your treatment path is not necessarily the same as other patients with the same disease name, but instead perhaps determined by how your individual body works. Today's research has shown that it is how your cancer, tumor or disease is behaving that can make a difference in whether your body gets better using a specific treatment.

Two new approaches to recruiting patients for trials have emerged as a result of this more personal approach. Known as **basket trials** and **umbrella trials**, researchers are designing trials that test the effectiveness of new therapies or targeted care when everyone with the same diagnosis is not grouped together and assumed to have the same biology. Instead these trials suspect that specific techniques and new treatment options will improve overall success rates when targeted to patient groups that are based on common cellular behavior or particular genetic or molecular trait instead.

Moving into this targeted and individualized style of treatment has already shown successful outcomes with fewer side effects because treatment is catered to a patient's specific biology, or the genetic or genomic profiles.

Similarly, one of the fastest growing areas of successful medicine is called **immunotherapy**. This is where your body's own natural defense mechanism is strengthened and then used to fight the diseased cells, instead of bringing in items that do not occur naturally in the body.

Understanding the Differences in Study Phases

Clinical trials are structured into three phases that must take place before a medical care option can be made available to all patients. Each phase is designed to fine-tune the answers to separate research questions. The knowledge gained by each stage together helps ensure that the product or procedure will benefit large populations after approval.

Phase 1 involves a small group of people (20-80) and are the first studies done to test if a new treatment is safe, identify possible side effects, and to look for the best way to give the treatment. Researchers also look for signs that diseased cells respond to the new treatment.

Phase 2 enrolls a larger group of people (100-300) to see if the diseased cells respond significantly to the tested drug or treatment

therapy. The second phase can last from several months to two years, and could involve up to several hundred patients.

Phase 3 randomly compares the new treatment or process against the current standard of care for a specific diagnosis. It includes a larger number of participants (100s - 1000s) spanning a variety of ages, ethnicities, and genders. This ensures the final results will apply to larger populations.

After a Phase 3 trial, the researcher can apply for approval from the Food and Drug Administration to market and provide the product to other patients. Long term trials and population monitoring, part of **Phase 4** studies, continue on for years after approval to gather information on larger and more diverse populations and demographics.

Learning To Be Your Own Best Advocate

Every clinical trial must meet a minimum set of standards and patient protection rules. In addition, they must be transparent and not hide anything from you as the patient. To ensure you are fully informed beforehand, trial sponsors must lay out ALL the details. This includes specific information on research goals, therapies that will be used, testing you will undergo, known risks, possible benefits, potential side effects, time line, and length of the study along with contact information for your medical team. This document is known as "informed consent" and your copy will serve as a good reference during the trial.

The informed consent document will also list elements that you are responsible for and can plan for, including arranging for caregivers, transportation, or overnight visits. In trials, you are given more information upfront about the care path ahead of you, giving you the power to be your own advocate. Use this information to ask questions and stay engaged with your care decisions.

Finding a Trial Doesn't Have to Be an Individual Task

Doing any kind of medical research can be overwhelming for many patients and family members. Finding trial options and figuring out if you match the eligibility requirements for participation can take time and usually includes unfamiliar technical medical terminology. But you are not alone. Members of the healthcare community can help do some of the legwork for you, including nurses, navigators at the doctor's office, patient advocates, healthcare social workers, and disease-specific organizations. With many tools online, you or your family members can narrow the list from home, and discuss possible options with the trial contact, your doctor, and family members.

In order to find a clinical trial for treatment you should be ready with some medical information, including your exact diagnosis, stage (if any), a list of previous treatments if any, radiology or lab results, molecular biomarkers or genetic characteristics, and other medical conditions you also have. For non-treatment trials, you may need information from your family history or other risk factors.

Your doctor can also help identify any local or regional options. Clinical trials are conducted in a variety of settings including cancer clinics, doctor offices, and larger medical centers. Look to see what's close to you, but also consider and inquire about transportation support before ruling out distant options.

If you do participate in a trial, you may be among the first to benefit from the new treatment being studied, but, regardless of your own outcome, your participation helps make a difference for future patients.

It May Be Good for Your Wallet

Compared to the standard treatment you would otherwise get from your doctor, there may be additional costs associated with clinical trials. The good news is that you may not be the one paying for all of them.

As with any medical care you receive, you are responsible for payment of doctors, treatment, and services received that are routine for your diagnosis. If you are insured, your health plan would help pay these charges according to your benefits, even if you were not participating in a clinical trial. This includes the standard medications, any side effects, doctor visits to network providers, lab tests, and imaging studies that are normal for treatment, prevention, or management of your diagnosis. The Affordable Care Act (ACA) requires most insurance companies to cover costs associated with receiving standard care in the plan's network, even for those participating in a research study, and not exclude you from receiving care you would receive if not participating in a trial.

When participating in a trial, there may be other study specific items you receive, including the medications, interventions, or

The Decisions is Yours to Make

The reasons why patients ultimately decide to participate in a clinical trial are varied and personal like all medical decisions we make.

Participating in a clinical trial involves a formal commitment to move forward with care that patients in standard paths may not have. Since your experience is being monitored and documented by researchers, they must ensure that you are comfortable with your decision.

When making your decision, it is okay to take time to talk to others and choose based on what is right for you. Discuss options with your specialist and the doctors you trust. It might also be a good time to get a second opinion from a new doctor that can give you a consultation with fresh eyes and share a recommendation on if you should proceed with a clinical trial based on your diagnosis and treatment history.

Following medical opinions you will want to consider your own views and talk to your family members about the possible medical and lifestyle impact of all treatment options. Share information you've learned about your options and encourage your family to ask questions as well. Consider any cost, time, and logistics issues for you and your caregivers and make a plan to address so your care is uninterrupted.

Remember that even if you decide to start a trial, if you feel the need to withdraw at any point for any reason, you do have the right to change your mind. You can stop participating in a trial at any time.

procedures that are being tested. The study sponsor pays for these and will share the list of specific care they pay for with you in the beginning during the informed consent process. The study may also provide stipends or reimbursement if there is travel, parking or tolls required, and to help with other costs like childcare, food, lodging, or caregiver support.

For those without insurance or with limited benefits, having the study pay for trial items reduces your own cost and provides a method of care that may have been too expensive otherwise.

However, be aware that there might be elements that neither the study nor your insurance will pay for. For example, look closely at the trial paperwork and informed consent documents to make sure you are not expected to see doctors outside of your network, or that there are not additional lab or imaging scans that are required along the way. You should also call your insurance company to double check their coverage of items you know you'll need during the trial to prevent any surprises down the road.

The Practical Side of Trial Participation

In addition to making the decision to participate in a trial based on the medical reasons of your care, patients must also determine if the requirements of participating in the trial fit into their lifestyle. While every treatment and medical care option presents its own practical, financial, and emotional challenges, it is important to learn as much as you can beforehand so you have time to decide your path without unexpected roadblocks.

Before you are able to finalize your enrollment into a study, the organizers will provide you with a list of the responsibilities you are expected to fulfill to ensure the trial results are captured the same for everyone. Be sure to read this closely and ask questions at the time.

Some of the biggest challenges patients report when it comes to participating in treatment trials relate to the financial cost. Expenses may come from the insurance plan's out-of-pocket costs for covered care, costs of any trial-related care that is not covered by insurance, additional financial strain that results from out-of-the-area travel, or reducing income if spending longer time away from home and/or work.

Additionally, trials can require more from your family and the caregivers around you, including larger time commitments when taking you to treatment or doctor visits. Asking questions will help you understand what to expect.

What's Next? How Do I Find a Trial?

You have decided to add the possibility of a trial to your list of treatment or care options and want to look further into the benefits as part of a research study. The first step is to identify if there are any current trials that are recruiting new patients that match your medical situation.

Although there is no single source, there are web matching tools as well as organizations that can help you sort through the treatment and

non-treatment trial options. When you have identified those that you meet the basic eligibility for, your doctor or the contact person for the trial can help you understand the medical terms.

While you don't need your doctor's permission to consider or engage in a trial, you will want to keep them advised of your ultimate decision so that your medical records will reflect all of the care or treatment you receive.

Clinical Trials Resources

National Institutes of Health
www.clinicaltrials.gov

Center Watch
www.centerwatch.com

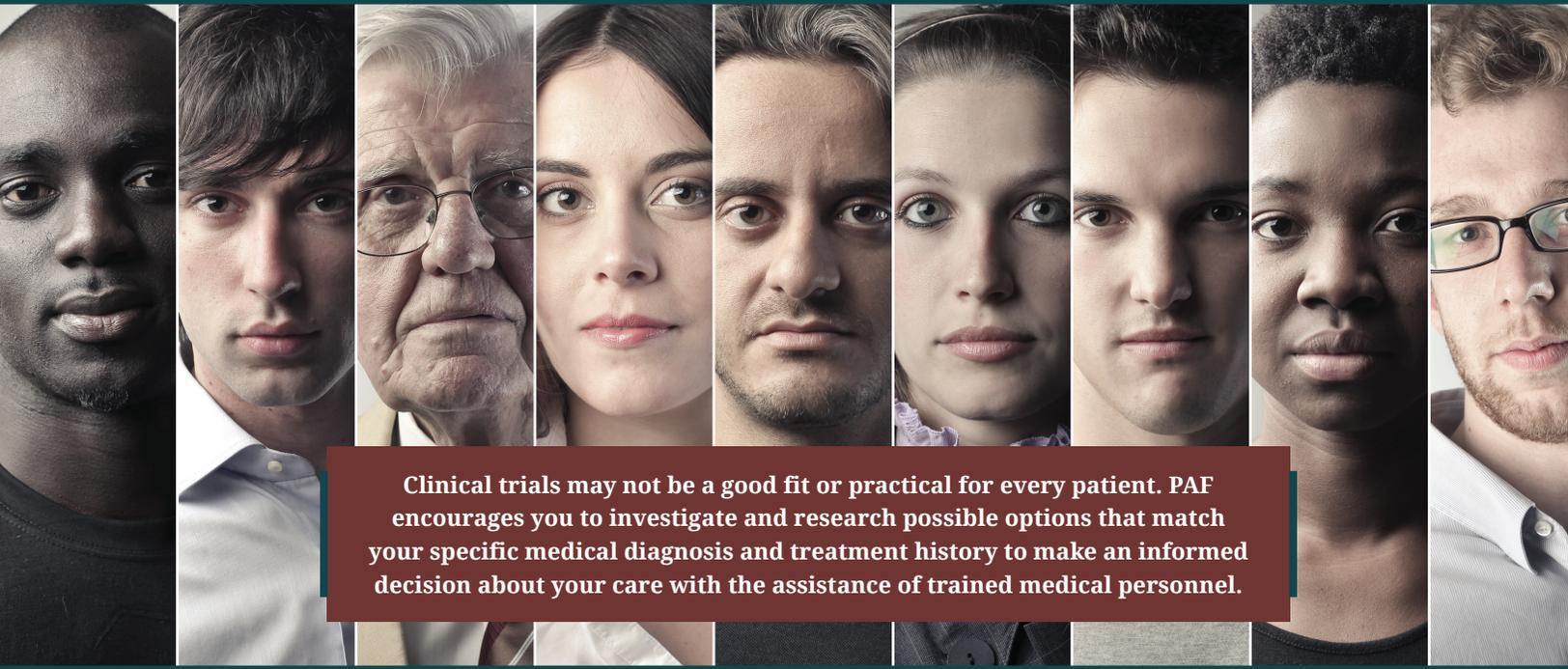
Center for Information and Study on Clinical Research Participation
www.ciscrp.org/services/search-clinical-trials

TrialCheck
www.eviticlinicaltrials.com/Services

EmergingMed Clinical Trial Navigation Service
app.emergingmed.com

National Cancer Institute
www.cancer.gov/about-cancer/treatment/clinical-trials

The Family and Medical Leave Act may protect your job related to medical illness or caregiving for others, allowing extended time away from work up to 12 weeks.



Clinical trials may not be a good fit or practical for every patient. PAF encourages you to investigate and research possible options that match your specific medical diagnosis and treatment history to make an informed decision about your care with the assistance of trained medical personnel.