Advocates Share the Basics to Help You Decide if a Clinical Trial Is Right For You

Clinical Trials:

What Patient Advocates Want You to Know

Clinical Trials Are Definitely Worth a Second Look for Any Patient

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 that its not surprising that the option of clinical trials frequently comes 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 less known and frequently misunderstood benefits of clinical trials and address the most frequently asked questions from patients.

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

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

100% of current FDA approved treatment went through a clinical trial, giving those first patients exclusive access to the benefits when others couldn't get it.

Trials Are Not Just For Treatment

Some people only think of clinical trials for 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:

Treatment trials **study new drugs, techniques, surgeries or combinations of treatments for those in any stage of disease.**

Prevention trials **discover ways to keep from getting a specific disease, and look to identify specific genetic or inherited risk factors.**

Screening trials **explore new ways to detect diseases or conditions.**

Diagnostic trials **explore better testing procedures for an illness or condition.**

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.**

PAF Patient Advocate Foundation

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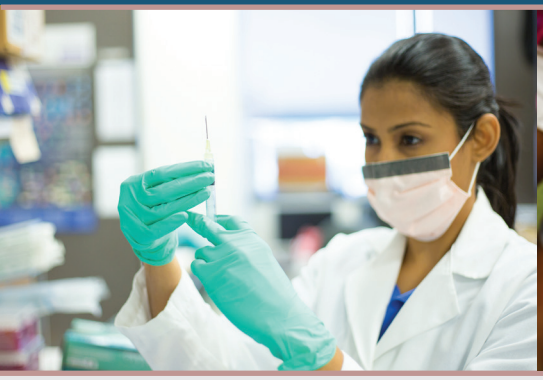
What Is the Purpose of a Clinical Trial?

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, including breast and metastatic breast cancer. 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 to replace or add to the current treatment being offered today.



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

You may be curious about learning more about clinical trials to explore whether volunteering to participate makes sense for you. Each trial is designed and monitored to ensure patient safety and is ultimately a step in the path to treat or cure disease, however every aspect of medical care also includes risks and may be different for everyone. While some people may consider a specific aspect a benefit, others consider the same element a concern, so it is important to weigh each as it relates to your 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 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 some additional 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 location may not be available at 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, or enough better results to be worth expanding to future patients.
- 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 unexpected or more 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 must pay. 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 - Cancer trials rarely involve a 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.

The total number of registered studies has grown 4556% since the year 2000. With over 260,000 medical studies active in 2017, millions of patients are enrolled with access to cutting edge medicine.

Understanding the Differences in Study Phases

There are 3 ways that clinical trial research is structured known as phases, that must take place before a medical care option can be sold and marketed to 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 involve a small group of people and are the first studies done to test if a new option is safe, identify possible side effects and to look for the best way to give the treatment (by mouth, injected into a vein, or injected into the muscle). Researchers also look for signs that diseased cells respond to the new treatment.

Phase 2 enrolls a larger group of people to see if the diseased cells respond significantly to the new drug or treatment therapy. The second phase can last from several months to two years, and involves up to several hundred patients.

Phase 3 randomly compares the new treatment against the current standard of care for a specific diagnosis. Includes a larger number of participants spanning a variety of ages, ethnicities & 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 sell 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

Just like in your doctor's office, every clinical trial must meet a minimum set of standards and patient protection rules. In addition, they must be transparent and not hide anything to you as a patient. To ensure you are fully informed beforehand, trial sponsors must lay out ALL the details including 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 trial 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.

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 specific body works. Today's research has shown that its how your cancer, tumor or diseased cells are forming and 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, researches 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 in the tumor itself.

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.

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 participating 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 cancer type, stage, a list of previous treatments if any, the test results from your cancer diagnosis such as 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 as well as 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.

The Decisions is Yours to Make

Why patients ultimately decide to participate in a clinical trial are varied and personal like all medical decisions we make during our life.

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

When making your decision, its ok to take time to talk to others and choose based on what is right for you. Discuss options with your oncologist 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 opinion 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 point you choose.

It May Be Good for Your Wallet

Compared to the standard treatment you would otherwise get from your doctor, there are 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 normal 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 to treat cancer and its 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 what 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 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. 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.

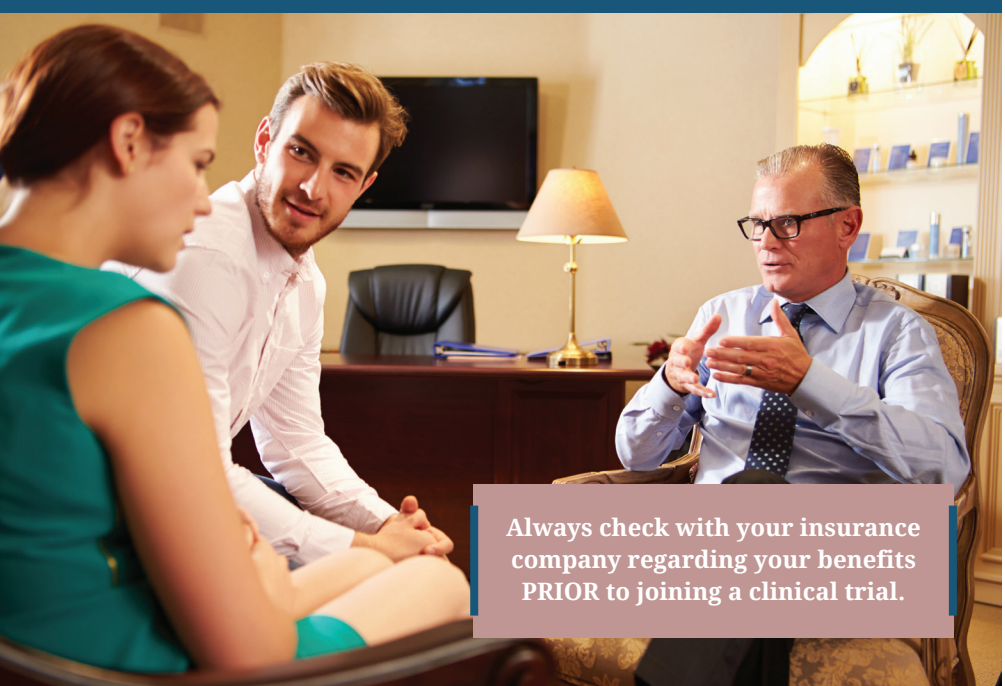
For those without insurance or with limited benefits, having the study pay for trial items reduces their own cost and provides a method of care that may have been too expensive otherwise.

However, be aware that there might be elements that neither the study nor your insurance will pay for that you should pay close attention to. For example, look closely at the list 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 Affordable Care Act requires non-grandfathered private group and individual insurers to cover the routine costs for all phases of clinical trials intended to prevent, detect, or treat cancers and other life threatening illnesses. In order to meet the requirements, the trial must be federally funded or being conducted by an organization that is federally funded (includes academic institutions, designated cancer centers and cooperative groups); and must also be conducted under an Investigational New Drug (IND) application. In addition to the ACA, there are 39 states that have added laws that addressed insurance coverage for participating in a clinical trial.

Questions to Ask

- 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 trial studying 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 affect my daily life?
- Are there treatments to manage any side effects?
- 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?
- Are there reimbursement options for the non-medical parts 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?



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

You're Not a Guinea Pig

By the time trials are approved by the official medical agencies to start recruiting patients, there has been significant research, *frequently years of research*, that indicates that this option could possibly improve your health. Researchers have rigorously tested, observed and published medical aspects in many pre-clinical settings before bringing it to patients.

Only 16% of cancer patients report having been aware that possible matching trial options exist. Even fewer were made aware by their doctor or specialist.

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 and care experience, all wrapped under the "clinical trial" name.

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

Sometimes studies are simply observational in nature and are recording trends or looking at DNA 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. Some studies also are looking 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 testing whether an additional round or dose of the current medicine would lower your chances of the cancer returning, or if a drug approved for a similar disease would work for yours. Trials are very diverse in what they are studying.

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.

Some of the biggest challenges patients report when it comes to participating in treatment trials relate to the financial cost. Expenses may come from affording the insurance plan's out-of-pocket costs, covering any trial-related care that is not covered by insurance, additional money strain that results from out of the area travel, or 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.

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.

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.

Some of the breast cancer specific clinical trial sites include:

Breast Cancer Trials
BreastCancerTrials.org

Metastatic Trial Search
www.breastcancer.org/treatment/clinical_trials

EmergingMed Clinical Trial Navigation www.emergingmed.com/

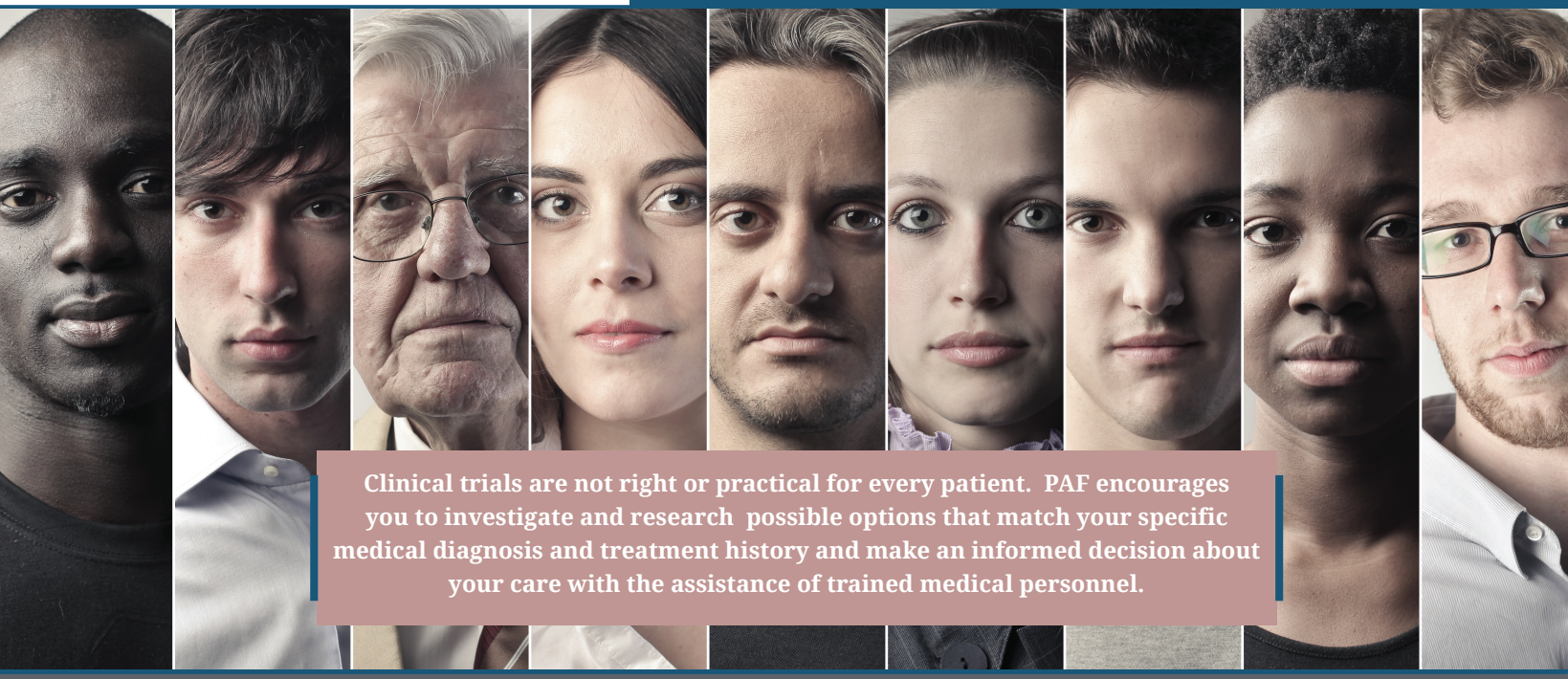
Metastatic Breast Cancer Network
www.mbcn.org/clinical-trial-information/

National Cancer Institute Clinical Trial Registry
www.cancer.gov/clinicaltrials

National Institute of Health
www.clinicaltrials.gov

CenterWatch
www.centerwatch.com/clinical-trials/

Triple Negative Breast Cancer Foundation
tnbcfoundation.org/clinicaltrials.htm



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