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Lighting The Way
A practical guide to Clinical Trials
Prepared by Patient Advocate Foundation
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Introduction

The future of medicine has never been so bright. Advances in treatments are being announced every day that can help eliminate or improve the life of patients suffering from chronic, life threatening and/or debilitating diseases. New drugs and treatments options are in development in medical facilities and laboratories across the country that have the potential to improve the health of individuals, communities and populations. These new treatments need to be studied to make sure they are safe and as effective, or more effective, than current standard treatment options.

When a person is diagnosed with a life threatening or chronic debilitating illness there are options to consider for treatment. Those options include standard of care treatment or clinical trials. NCI defines standard of care as: “In medicine, treatment that experts agree is appropriate, accepted, and widely used. Health care providers are obligated to provide patients with the standard of care. Also called best practice and standard therapy.”¹ In this booklet we will discuss what clinical trials are, how to screen for a clinical trial, how to talk to your physician and family about them, insurance issues related to reimbursement, obstacles, adult vs. pediatric participation, and underrepresented populations participation in clinical trials.

Trials are another resource for ensuring access to care and/or emerging medications. There are nine chapters in Lighting the Way. There are note pages as well as a personalized Q&A (Question and Answer) section in the back of the booklet for your use. We hope after reading this booklet you will gain a better understanding of Clinical Trials and feel comfortable with making choices concerning them.

Enjoy your reading!

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Chapter 1: Diagnosis Day

You or someone you love has been diagnosed with a serious medical condition. How is this possible? What exactly does “diagnosis” mean? A diagnosis is when a medical condition is identified after going through a process that looks at the presenting signs or symptoms along with looking at the results of tests or procedures that have been performed.

Typically, you see a doctor when there is something going on that is not normal for you, or you are having unusual symptoms. Your physician will take a complete medical history including when your symptoms started. He or she will do a complete physical examination checking for signs of disease. The physician may conduct further testing and or staging of the disease to confirm a diagnosis before recommending the most appropriate treatment.

An acute illness may be serious but can be diagnosed, treated, and you should recover fairly quickly. A chronic illness, even when treated appropriately, will continue long after the diagnosis is made. There are many different types of chronic illnesses, some are very serious and require frequent follow up, monitoring, and treatments. Most, however, require regular follow up and treatments, but allow you to lead a very normal and productive life.

Whether you are the patient or the caregiver of a patient newly diagnosed with a chronic illness you may feel overwhelmed with new responsibilities and may not know where to start. Do not be afraid to ask questions or get a second medical opinion, as you must be empowered to become the primary advocate for yourself or your loved one. Make sure you and your medical provider have discussed all of the available treatment options and be open to considering what you and your family determine may be the best treatment for you. The more you learn about the diagnosis and illness, the more in control you will feel. You will be asked to make decisions relating to treatment such as choosing your physician and where you will be treated. You will have many other important decisions to make such as financial issues, work and family support.

There are many resources available to you such as:

• Local or hospital libraries for books or lists of professional organizations
• Support groups
• Reliable internet websites
• Healthcare professionals
• Advocacy Organizations

As you begin to adjust to the day-to-day management of the illness, you will find that things you once thought were impossible have been accomplished. Over time you will learn to cope with the new challenges of managing your own or your family member’s illness. With proper, ongoing support, you will overcome the challenges forced upon you by the illness.
Chapter 2: Why Me?

You are never prepared to hear the words that you or a loved one has been diagnosed with a life threatening disease. You and your family may have many feelings after being given a diagnosis and you may not all be at the same place at the same time. Feelings of disbelief, sadness, loss of control, anger, worry or guilt are not unusual and you are encouraged to share your feelings with your family, friends, clergy or a support group. Over time, you will identify a new normal, and will be able to regain control of your life. Being a patient, or the caregiver of a sick patient, will take a large amount of time and energy and can prove to be both mentally and physically draining. You need to prepare yourself and your family so you are ready to face challenges or roadblocks that may lie ahead.

How to effectively maintain a positive attitude in difficult times

Staying upbeat when you, or someone who depends on you, are undergoing treatment can be an ongoing challenge. You may feel overwhelmed, but you need to remember you are not alone. People will assist you so give yourself permission to ask for help. Here are some suggestions that you may find useful.

• **Be prepared for the lows.** You may have days in which smiling will seem hard to do. Learning to accept a diagnosis often leads to anger, fear, and/or depression, which are natural feelings. Even though dealing with these emotions is not uncommon when dealing with an illness, you do not need to feel as if you have to suffer alone.

• **Seek counseling.** It is important that you are able to talk about your feelings. How you are mentally, can affect your body. You may be comfortable talking about how you feel, or you may find talking is too painful. Eventually, it is helpful to face your emotions head on. Talking about your feelings with a skilled and caring listener can assist you in building your own coping skills as well as provide you new ways to handle your emotions in a positive way.

• **Seek out support systems.** Do not be afraid to let others help you and do not be too proud to ask for help! The more people acting on your behalf, the less strain there will be on you. Develop relationships with members of the treatment team including social workers, nurses, physicians and church leaders. Talk with your family and friends about different ways they can help you with your daily activities. You may find it useful to seek out national and/or local non-profit organizations that offer support counseling. There are peer support groups available to assist you and your family members as you process your feelings about the illness. Support organizations are located in the Resource Appendix at the end of this guide.

• **Educate yourself about the illness.** Knowledge is power and being able to understand what you or someone you care about will be going through will help you prepare for the challenges to come. It is okay to ask questions when you do not understand something. You may have questions about the disease process, the proposed treatment plan, the time frame for having testing or treatment and
what if anything you are expected to be doing. Being an active participant during your treatment is important to having a successful outcome. Organizations such as Cancer Care, the American Cancer Society and the National Cancer Institute are great resources for information regarding your illness.

Challenge yourself to remain positive! Keep a sense of humor and focus on taking it one day at a time. Approach each challenge as if it is simply a stepping-stone to the next stage of your life. Your emotional state can serve as the determining factor in setting the tone during recovery.
Chapter 3: What Is A Clinical Trial?

A Clinical Trial is a study of new drugs, combinations of drugs (some already FDA approved for other purposes) and/or treatments to see how well they work – especially when compared with current standard of care treatment. Each study has rules about who can and cannot participate such as age, sex or stage of disease. “Clinical trials have protocols, or action plans, for conducting a trial. This helps the participant understand what will be done, how it will be done and why each part is needed. In the United States, an independent committee of physicians (Institutional Review Board), statisticians and members of the community must approve and monitor the protocol. They make sure that the risks are small and are worth the potential benefits.”¹

Each trial lists eligibility criteria for participation. For example, there are studies that need volunteers with a certain disease while others are looking for healthy people. Some trials want all female while others want all male participants. The sponsor of the study writes the protocol, which explains what the trial will do, how the trial will be conducted, location of the study, eligibility criteria, and how and when the participants will be evaluated. There are many sponsors of clinical trials. The National Cancer Institute has a webpage for locating clinical trials as well as a patient recruitment line to screen potential patients. Other sponsors include physicians, single institution, the Department of Defense, the Department of Veterans Affairs, and/or biopharmaceutical manufacturers.

Clinical trials are done to gather information for many purposes. The purpose of the trial defines how it will be conducted. The different types of trials include:

- Treatment trials test experimental treatments, new combinations of drugs, and new approaches to surgery or radiation therapy.
- Prevention trials look for better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. Approaches may include medicines, vaccines, vitamins, minerals, or lifestyle changes.
- Diagnostic trials are conducted to find better tests or procedures for diagnosing a particular disease or condition.
- Screening trials test the best way to detect certain diseases or health conditions.
- Quality of Life trials (or Supportive Care trials) explore ways to improve comfort and the quality of life for individuals with a chronic illness.¹

What are the phases of clinical trials?

Clinical trial phases are designed to test the new treatments being proposed. Phases of clinical trials go from phase 1 through phase 4. As results are obtained, the trial moves to the next phase. There are 4 phases.

Phase 1: When a drug or treatment is in a Phase 1 trial, the researchers are studying to determine how the drug or treatment will be administered (by mouth, through the vein (IV), etc). The researchers are looking for the proper dose and monitoring for side effects. The study focuses on a small group of participants.

Phase 2: When a drug or treatment advances to a Phase 2 trial, the researchers are studying the results to determine the effectiveness of the drug or treatment. Phase 2 trials collect information on the safety and benefit of the treatment. The study expands to 100 or more participants.

Phase 3: When a drug or treatment advances to a Phase 3 trial, the researchers are studying to determine if one treatment is better than another. The trial agent is studied in comparison to current standard of care treatment. Phase 3 trials expand in size to several hundred to thousands of participants.

Phase 4: When a drug or treatment advances to a Phase 4 trial, the researchers are monitoring long-term safety and effectiveness of the treatment. These are also known as Post Marketing Surveillance Studies.²

As a drug or treatment advances in each Phase of a clinical trial the number of institutions, or facilities, offering the trial increases. Major medical centers across the nation participate in Phase 2, 3 and 4 trials. In some situations, it may be possible for you to enroll in a clinical trial at a local facility.

In the United States, the FDA (Food and Drug Administration) requires all new drugs or treatments complete multiple years of testing before approving them for use as standard of care. All current drugs have been through Clinical Trial testing at some point to achieve the standard of care label from the FDA. Some consider clinical trials the most advanced cancer treatment therapy we have. Making the decision to enroll in a clinical trial is a personal one. Because of advances in medical science, clinical trials can offer you a chance to participate in cutting edge treatments, before they are available to the general public.

Chapter 4: Screening For Potential Participation

In Chapter 3, we discussed how Clinical Trials have protocols or study plans that describe what will be done in the study and how the study will be conducted. They are designed to safeguard the health of the participant as well as answer specific research questions.

To take part in a clinical trial there are specific criteria that must be met for participation. To ensure that all possible side effects as well as benefits of the drugs or treatments being proposed are thoroughly examined, study criteria must be adjusted.

Guidelines are developed by researchers to determine who can participate in a clinical trial. Some trials require participants to have specific medical conditions and some trials require healthy participants with no disease.

Guidelines that allow participation in a clinical trial are called “inclusion criteria” and those that disallow participation are called “exclusion criteria.” It is important to choose participants who meet the requirements for the trial to establish adequate and reliable results.

How will I know if I am eligible to participate in a clinical trial?

Each clinical trial has eligibility criteria, which are requirements that patients must meet before they can participate. Eligibility criteria might include information about:

- Age and gender
- Type of cancer
- Stage (extent) of the cancer
- Previous treatments that you must, or must not, have had
- Length of time since you last received treatment
- Results of certain laboratory tests
- Medicines that you are taking
- Other medical conditions
- Previous history of any other cancer
- Other conditions that are specific to each clinical trial

If you have found a clinical trial, you might qualify for, talk to your doctor, or contact the clinical trial’s principal investigator or research nurse. A member of the study team will ask you questions about your medical history to see if you meet the study’s eligibility criteria. These criteria are not used to reject potential participants, but rather to ensure safety. In addition, utilizing these criteria ensures the information obtained will answer the researcher’s questions.

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The Research team consisting of doctors and nurses will explain the trials’ purpose, procedures, risks and benefits. Informed consent must be obtained before you can participate in a clinical trial. This process allows potential participants to obtain all the facts on the study and make a well educated decision on whether to take part in the trial. The research team will provide you with an informed consent document that includes details of the study, purpose of the trial, duration or length of time the trial will run, and required testing such as lab work, scans and key contacts. Also included in the informed consent is an explanation of potential risks and benefits of the trial. Once informed, you may decide whether to accept or decline participation in the trial. Informed consent is not a contract. You have the right to leave the study at any time. By signing the consent you are not obligated to participate, you can always change your mind. If you choose to leave the study, your doctor will discuss other treatment options.

Understanding your rights through a clinical trial is important. You have the following rights:

• To understand all of your treatment options
• To know what is involved in the trial such as tests, risks and benefits
• To have the opportunity to discuss the trial with the investigator and research team
• To hear and read information in a language you can understand

There are benefits of participating in a clinical trial, including the following:

• Having access to promising new treatment approaches that are often not available outside a clinical trial setting.
• The approach being studied may be more effective than the standard approach.
• Receiving regular and careful medical attention from a research team that includes doctors and other health professionals.
• Being the first to benefit from the new method under study.
• Results from the study may help others in the future.

Along with benefits, there are risks associated with participating in a clinical trial including:

• New drugs or procedures under study are not always better than the standard care to which they are being compared.
• New treatments may have side effects or risks that doctors do not expect or that are worse than those resulting from standard care.
• Participants in randomized trials will not be able to choose the approach they receive.
• Health insurance and managed care providers may not cover all patient care costs in a study.
• Participants may be required to make more visits to the doctor than they would if they were not in the clinical trial.4

Clinical trials can be safe. Most clinical trials are federally regulated to protect the participants.

4Clinical Trial Benefits and Risks. Retrieved 3/30/2009
http://cancer.about.com/od/cancerclinicaltrials/a/trialriskbenefit.htm
Chapter 5: How To Talk To Your Doctor And Family About Clinical Trials

Once you have received a diagnosis you may wonder about potential clinical trial treatment. You and your physician can discuss current treatment choices and clinical trial options. When you are meeting with your physician you may benefit from bringing a family member or friend with you as you may be overwhelmed by the information you are provided. This can bring another perspective and that person may think of additional questions to ask during your visit.

Previously we discussed the screening process involved within a clinical trial. You may wish to contact the National Institutes of Health (NIH) to see if there are any current trials that you may potentially qualify for. During the call, you will be asked a series of questions and you will know if you meet the initial screening criteria. They may see one or two trials that may be potentials for you and provide you with what is known as the “Clinicaltrials.gov identifier” number to take to your physician. This will assist you in making the best decision for you.

Once you make the decision to participate in a clinical trial you will undoubtedly have many questions. You will need to speak with your treating physician if you have not already done so. Provide your treating physician with the information on the trial you would like to participate in and ask for their input. Prepare a notebook and keep a list of your questions. (Refer to the Q&A section at the back of this booklet). Take this to all physician appointments and ask those questions. Learn if your current physician can continue to treat you or whether you will need to change physicians for best results.

You will want to speak with your family and let them know why you made the decision you have made. Remember they are concerned and scared too. They will have many questions. Talk openly about your decision.

Some questions to keep in mind when considering clinical trials:

- How will I know if the treatment is working, and how soon?
- What are my options if the trial treatment does not work?
- How often will I have to travel long distances for treatment? (if applicable)
- How long does the trial last and what are my responsibilities for participating?
- What is my health insurance likely to cover and what is it likely to deny? What happens to those charges if insurance denies them – whose responsibility are they?
- Will there be out of pocket costs for me? If yes, how much? What if I cannot afford them?

As you go down the road of clinical trials, you will continue to have questions and seek answers. Be sure to keep a record of your questions so you can get the answers you need to make a well-informed decision. Some patients find that journaling or writing about their experience, is helpful.
Chapter 6: Insurance Issues Related To Clinical Trials

Many times participation in a clinical trial requires learning more about your insurance policy. Many policies do not provide coverage for clinical trials that are considered Experimental and/or Investigational. This means the insurance company does not recognize the proposed treatment as a standard of care and will not pay for it. Some policies may cover trials in certain stages – for example, coverage for Phase 3 or Phase 4 trials. This information will be explained in your insurance policy language. Health plans may specify specific criteria a trial must meet to be covered. The trial might have to be sponsored by a specified organization, be judged “medically necessary” by the health plan, not be significantly more expensive than treatments the health plan considers standard, or focus on types of cancer for which no standard treatments are available. In addition, the facility and medical staff might have to meet the plan’s qualifications for conducting certain procedures, such as bone marrow transplants.

How do I know if I have coverage?

Read your policy language to see what it says about Clinical Trials. You will want to review the Covered Benefits, Non-Covered Benefits and Exclusions of the plan language. In addition, you will want to read how the policy defines Experimental and/or Investigational in the definitions section of the policy. Your policy should provide coverage for current Standard of Care treatment.

Medicare reimburses patient care costs for its beneficiaries who participate in clinical trials designed to diagnose or treat cancer. Information about Medicare coverage of clinical trials is available at http://www.medicare.gov on the Internet, or by calling Medicare’s toll-free number for beneficiaries at 1–800–633–4227 (1–800–MEDICARE). The toll-free number for the hearing impaired is 1–877–486–2048. In addition, the National Cancer Institute (NCI) fact sheet More Choices in Cancer Care: Information for Beneficiaries on Medicare Coverage of Cancer Clinical Trials is available at:

http://www.cancer.gov/cancertopics/factsheet/support/medicare

After reviewing your plan language, you should have a better understanding of what is and is not covered. More information about insurance coverage can be found on the internet at NCI’s Clinical Trials and Insurance Coverage: A Resource Guide Web page at:

http://www.cancer.gov/clinicaltrials/learning/insurance-coverage

Many states have passed laws or developed policies requiring health plans to cover the costs of certain clinical trials. For more information, visit the NCI’s Web site at:


If your insurance company denies coverage of the trial, do not be discouraged. You need to submit an appeal to your insurance company, requesting that they reconsider the denial and provide coverage for the requested treatment. Your

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appeal should be based on the medical necessity of the clinical trial and why your physician feels that a clinical trial is the best treatment option for you. If there is enough information to show that the approach is safe and effective, your health plan may consider the approach “established” and cover some or all of the costs. If you find yourself in a situation where you need to appeal you may find “Your Guide to the Appeals Process” helpful. You can read or download the publication from our website, www.patientadvocate.org. You can contact Patient Advocate Foundation at 1-800-532-5274 and one of our Patient Service representatives is available to assist you. You can speak with the sponsor of the trial or one of the trial representatives. There may be funds available through the trial sponsor to assist you. In addition, there are fund raising organizations that may be of assistance.

What if your policy does not provide coverage?

The informed consent will outline costs that are covered under the trial and costs that are not. It will also outline what charges will be submitted to your insurance company for consideration. Speaking with your insurance company prior to your participation can help to prevent unforeseen financial costs to you. Again, be sure to speak with the Research Nurse or Principal Investigator to get an idea of the out of pocket costs to you, if any, for participation.

The biggest challenge in participating in a clinical trial if your insurance denies coverage, is obtaining coverage for those charges that would otherwise be covered if you were receiving standard treatment. Your insurance company may deny charges for blood work (complete blood count) or x-rays (e.g. CT scan) if the tests are ordered more often than they deem necessary. The insurance company will deny the tests as “Experimental/Investigational” feeling they are being done to monitor the trial. You will want to stay in close contact with your treating physicians’ office for assistance in appealing medical necessity claims of this nature. They will be able to help establish what is standard of care and what is related to the trial.

Financial assistance often is available via the hospital or treating facility. Remember, there are income guidelines that always must be considered.

Federal programs that help pay the costs of care in a clinical trial include those listed below:

- Beneficiaries of TRICARE, the Department of Defense’s health program, can be reimbursed for the medical costs of participation in NCI-sponsored Phase 2 and Phase 3 cancer prevention (including screening and early detection) and treatment trials. Additional information is available in the NCI fact sheet TRICARE Beneficiaries Can Enter Clinical Trials for Cancer Prevention and Treatment Through a Department of Defense and National Cancer Institute Agreement. This fact sheet can be found at: http://www.cancer.gov/cancertopics/factsheet/NCI/TRICARE

- The Department of Veterans Affairs (VA) allows eligible veterans to participate in NCI-sponsored prevention, diagnosis, and treatment studies nationwide. All Phases and types of NCI-sponsored trials are included. The NCI fact sheet The NCI/VA Agreement on Clinical Trials: Questions and Answers has more information. It is available at: http://www.cancer.gov/cancertopics/factsheet/NCI/VA-clinical-trials.6

Chapter 7: Obstacles To Clinical Trials

There are many reasons to participate in a clinical trial; sometimes there are obstacles. These obstacles can be overcome with education on your options and availability of resources. Lack of communication between your doctor and you could cause you to be excluded from participation in a clinical trial. The doctor may perceive a lack of interest or there is not an appropriate trial available. In addition insurance may not pay the cost of a trial.7

A lack of information regarding patient rights, accessibility, and choices to not participate can be an obstacle. A clinical trial may not be available locally requiring the patient to consider a trial some distance from their home. The need to travel a distance to obtain care creates an obstacle due to the inability to afford the cost of transportation to get to the trial. Transportation resources are located in the Resource Appendix at the end of this guide. Many hospitals or treatment facilities offer assistance to help with these costs. Some programs assist with lodging and meals free of charge or provide affordable rates. Many hotels offer a medical rate that is more affordable than the standard rate for lodging. Be sure to inquire at the treating facility for a list of local resources.

Another huge obstacle for potential participants is a loss of income, not only due to their illness, but sometimes their caregiver's loss of income. A spouse, child, or other significant other may become a caregiver and need to stay with the patient to provide support during the trial. This can be a challenge depending on the type of study and how often the patient needs to be seen. The patient or caregiver has options available with their employer. Discuss these options with your Human Resources Department regarding Short Term Disability (STD) or Long Term Disability (LTD), Family Medical Leave Act (FMLA), availability of sick pool donations, and/or altered work schedules. If you will not be able to work for at least one year due to treatment and recovery they should apply for Social Security Disability Income (SSDI) or Social Security Income (SSI).

The Social Security Administration (SSA) website, www.socialsecurity.gov can provide very useful information on this process. The SSA has developed a list of “compassionate allowances”, or diagnoses, that will receive approval for disability.

The compassionate allowances website at: http://www.ssa.gov/compassionateallowances/conditions.htm will provide you with a list of diagnoses.

Fear of the unknown can be an obstacle. A patient may feel that they are an experiment or a “guinea pig” or they will experience unpleasant side effects from the treatment. There can be unexpected side effects that were not anticipated and participants may need to make more trips for follow-up and testing that can add to expenses from increased lost time from their job and increased fuel costs.

7Questions to Ask about a Clinical Trial, University of Texas M.D. Anderson Cancer Center. Retrieved 2/28/2009 from http://www.mdanderson.org/patients_public/clinical_trials/display.cfm?id=91773241-08A5•4C4C-B484C69185DAFD25&method=displayFull
Child care costs also may be a factor. Hiring of private sitters or increased time in a day care add to these costs. Child care resources are located in the Resource Appendix at the end of this guide.

As discussed in Chapter 6, some health insurances and managed care providers do not cover all patient costs in a trial and this can cause unnecessary additional stress to your illness. You can contact your insurance company to find out what costs will be covered before the study begins.

Even though there are challenges when considering treatment in a clinical trial, there are numerous resources and options available to aide in the decision to participate. Do not let these challenges prevent you from considering all options. Clinical trials can offer life saving treatments for your disease and allow you to be a part of breakthrough technology, treatments and cures for your disease.
Chapter 8: Pediatric vs. Adult Participation

Why do so few adults participate in clinical trials?

There are many reasons why more children than adults participate in clinical trials, but one major point that is not in dispute, is advances in clinical trials have played a major role in the increase in cancer cure rates. Due to advances in availability of studies, improvement in outcomes along with the high quality standard of care being the rule not the exception, it is easy to understand why most parents choose to enroll their child in clinical trials.

Researchers are able to determine what can be done to save more lives or how to improve the quality of life and reduce long-term effects for patients undergoing treatment. It is widely recognized that clinical trials provide the best outcomes among childhood cancer patients. Because of this insurers typically pay for treatment in pediatric clinical trials. For adults, not all insurance companies cover the costs of clinical trials, such as costs associated with additional medical tests (See Chapter 6). What is covered will vary by insurance plan and the specific clinical trial. If you locate a trial you would like to participate in, ask for assistance in helping to determine what will be covered. Charges that are not covered by insurance may be paid by the study.

Since the 1950s, cooperative research has improved the survival rates for childhood cancer from less than 10% to over 77% overall. (Cure rates vary according to each specific type of childhood cancer.) Pediatric cancer research has also been beneficial by improving the basic understanding of cancer, treating adults with cancer and advances for treating other diseases of children and adults.8

Another reason for the high percentage of children being enrolled in clinical trials is that when a child is diagnosed with cancer they are usually referred to a major treatment facility, such as a Children’s Hospital, because childhood cancer is rare and is best treated by the specialists at these facilities. These pediatric oncology specialists are often members of Children’s Oncology Group, or COG. COG sponsors clinical trials for most childhood cancers.9

Unlike the increase seen in the past decade in pediatric clinical trial enrollment, approximately 20 percent of adult cancer patients would be eligible to participate in clinical trials however only 3 to 5 percent of this group choose to participate. Emerging adult cancer therapy is focused on drugs that kill only the cancer, while not harming the normal cells of the patient. This new therapy is referred to as “targeted therapeutics” which is a way of finding the weak spot of a tumor in a specific manner. Targeted therapeutics has shown great success in a number of

adult cancers. Unfortunately, pediatric cancers are so different from adult cancers that targeted therapeutics must be developed specifically for pediatric cancer.

Through the development of targeted drugs for children with cancer, children worldwide could benefit from cancer therapies that would require less ongoing care in contrast to today’s standard therapies. Children would benefit from additional drugs leading to a greater number of cures, and cures that would be associated with the life-long after effects currently caused by standard cancer treatments.\(^\text{10}\)

While there has been a lot of time devoted to recruiting more adults willing to participate in clinical trials, enrollment remains low. There are some valid reasons why patients are not able to participate in a trial. Some of these reasons may include:

- not meeting the eligibility criteria
- not being informed of the availability of a clinical trial
- not knowing how to locate a trial
- having practical or personal obstacles such as distance to the closest trial
- financial inability to participate
- suspicious of researchers
- not wanting to go against their doctor’s wishes.

As discussed in Chapter 7, Doctors themselves can be obstacles to patients’ considering clinical trials. Some of these reasons include:

- lack of knowledge about clinical trials
- unwilling to “lose control” of a person’s care
- belief that standard therapy is best
- concerned that clinical trials mean additional paperwork
- concerned about how the patient will react to the suggestion of a clinical trial.

If you are interested in a clinical trial, you should not hesitate to ask your doctor about whether there are trials that might be right for you. If your physician does not know how to find trials, clinical trial resources are located in the Resource Appendix at the end of this guide.\(^\text{11}\)


Chapter 9: Underrepresented Populations

Many aspects of clinical trials have been discussed in the previous chapters, and we would like to bring attention to the decreased enrollment of underrepresented populations. Underrepresented populations include adolescents, the elderly, individuals of lower socioeconomic status, people living in rural areas, and some ethnic groups. It is important to have a diverse group of participants in a clinical trial in order to best evaluate the effect on all groups including those considered in the underrepresented category. For example does the trial drug or treatment affect the elderly more than adolescents, and if so would dosage changes be required.

The problem of underrepresentation becomes even more striking in light of recent advances in the understanding of how your family history and your genetic makeup can influence your response to treatment. Researchers now recognize that people respond differently to medications based in part on their genetic makeup.  

Clinical trial roadblocks may include:

- Transportation issues to and from the treating trial center
- Additional costs associated with daily living expenses while undergoing treatment
- Lack of childcare while participating in the clinical trial
- Regional and/or family customs
- Language barriers are contributing factors to lower enrollment. Language lines are available for non-English speaking individuals should they wish to seek additional information on participation.
- Lengthy and complex consent forms
- Vague information about insurance coverage of clinical trials
- Doubts about safety of the trial also play a role in decreased enrollment
- Employment issues such as decreased pay or lack of paid time off

With increased awareness and education targeted at underrepresented populations the ultimate goal will be to ensure a more equal enrollment of that group in clinical trials.

Personalized Question And Answer Section

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10) Question

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Patient Resources

All website descriptions are copied from the respective websites themselves.

American Cancer Society (ACS), www.cancer.org, #800-ACS-2345, Road to Recovery Program. Provides transportation for cancer patients to and from treatment for people who have cancer who do not have a ride or are unable to drive themselves. Volunteer drivers donate their time and use of their cars so patients can receive life-saving treatments they need.

Cancer Care www.cancercare.org, #800-813-4673. Cancer Care provides limited grants for certain expenses related to treatment.

Candlelighters Childhood Cancer Foundation www.candlelighters.org, #800 366 2223. Provides advocacy, bereavement, counseling, financial assistance, insurance information, as well as information and referral.

Foundation for Children With Cancer www.childrenwithcancer.org. FCC Financial Assistance This program’s goal is to assist families with the financial burden that often accompanies an illness. FCC is unique in that it eliminates the “red tape” which often hinders families who need assistance. FCC networks with social workers, doctors, hospitals and clinics across the country to discover legitimate, unmet needs. In addition to dealing with the emotional trauma of having a child diagnosed with cancer, families of pediatric cancer patients are faced with high treatment bills, which can reach into the hundreds of thousands of dollars. FCC’s simple two-page application form, coupled with direct payment to vendors or mortgage institutions, eliminates additional stress for families, allowing them to focus on their child.

Joe’s House www.joeshouse.org provides a list of reduced or free lodging in the area of the treatment center.

Make a Wish Foundation www.wish.org, #800 722 9474. Grants wishes of children (up to 18) who have life-threatening illnesses.

National Brain Tumor Society (NBTS) www.braintumor.org/PatientServices Overview/#800-934-2873. Families are often faced with not only the overwhelming costs of treatment, but ongoing loss of income and increased expenses. The NBTS financial assistance program provides grants of up to $500 to assist with treatment related expenses and non-medical expenses including child care, home care, home adaptations, and transportation.

National Children’s Leukemia Foundation www.leukemiafoundation.org, #800 448 3467. A national non-profit organization whose sole purpose is to provide a wide variety of services, support and referrals to leukemia patients and their families. Primary goal is to ensure access to the state of the art treatment regardless of the ability to pay.

The Aubrey Rose Foundation www.aubreyrose.org. Provides financial assistance. Grants are awarded based on need. If a family has outstanding medical bills that insurance will not cover, this Foundation can possibly help out a family in need until annual funds have been exhausted.
The National Children's Cancer Society www.children-cancer.com, #1 800 5 FAMILY (#1 800 532 6459). Provides financial assistance to any child diagnosed with cancer on or before his/her 18th birthday. Assistance for ancillary costs may include transportation, childcare, household expenses, meals, lodging and insurance premiums.

The Tomorrow Fund www.tomorrowfund.org, #401-444-8811. In most cases when a child has cancer, one parent must quit their job in order to care for their ill child. Medical expenses not covered by insurance can be enormous, and ancillary costs, such as travel, child care for siblings, hospital meals and parking, and accommodations during out-of-state treatment are high. When coupled with reduced income, these expenses can be devastating. The Tomorrow Fund helps ease financial burdens through daily monetary stipends for meals, hospital parking passes, home expenses, medication and insurance co-pays.

Website Resources for Patients:
http://www.cancer.org/docroot/NWS/content/NWS_1_1x_Clinical_Trials_Optimal_Option_For_Childhood_Cancer.asp
http://www.cancer.org/docroot/ETO/content/ETO_1_4X_Monoclonal_Antibody_Therapy_Passive_Immunotherapy.asp?sitearea=ETO
http://www.cancer.gov/CLINICALTRIALS
http://www.lazarex.org/lookingforhelp.html
http://www.cancertrialshelp.org/trialcheck/default.aspx
Bibliography


PAF Works Cited

Too Young to Be Ill (PAF publication)
PAF Clinical Trials PowerPoint
Your Guide To The Appeals Process (PAF Publication)
Your Guide To The Disability Process (PAF Publication)