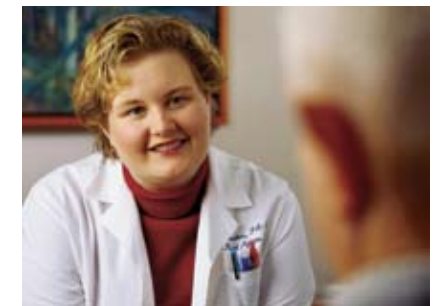
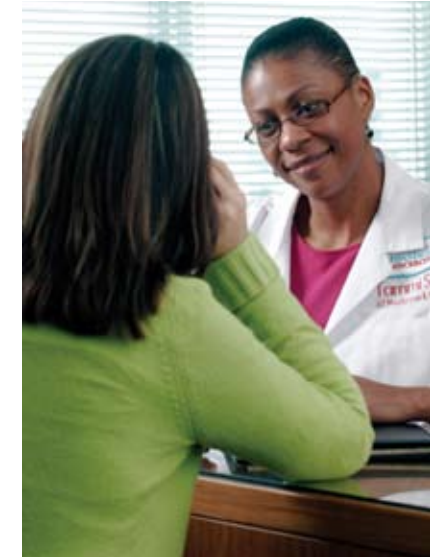


Clinical Trials at M. D. Anderson



THE UNIVERSITY OF TEXAS
MD ANDERSON
CANCER CENTER
Making Cancer History®

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Making Cancer History®

Mission

The mission of The University of Texas M. D. Anderson Cancer Center is to eliminate cancer in Texas, the nation and the world through outstanding programs that integrate patient care, research and prevention, and through education for undergraduate and graduate students, trainees, professionals, employees and the public.

Vision

We shall be the premier cancer center in the world, based on the excellence of our people, our research-driven patient care and our science. We are Making Cancer History®.

Core values

caring

By our words and actions, we create a caring environment for everyone.

integrity

We work together to merit the trust of our colleagues and those we serve.

discovery

We embrace creativity and seek new knowledge.



Clinical Trials at M. D. Anderson

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This booklet is about treatment (therapeutic) clinical trials for M. D. Anderson Cancer Center patients. A treatment trial studies how a specific anti-cancer treatment affects the patients who receive it. However, some of the information may be accurate for other types of clinical trials and trials performed at other hospitals.



What are clinical trials? ♦ Clinical trials are research studies that involve people. The main purpose of a clinical trial is to find a better way to prevent, diagnose or treat a disease. ♦ Clinical trials are part of a long, careful research process. Patients who participate in a clinical trial receive drugs or procedures that already have been researched in successful laboratory and/or animal studies. ♦ All patients who participate in clinical trials are **volunteers**. They can choose to stop their participation in a clinical trial at any time.



Most clinical trials study new drugs or procedures, but some clinical trials study drugs or procedures that have already received approval by the U.S. Food and Drug Administration.

For more information about other types of clinical trials, please ask your doctor. M. D. Anderson's Cancer Prevention Center conducts prevention and early detection trials. For more information, call 713-745-8040 or toll-free at 800-438-6434.

Why are clinical trials important? Clinical trials are important to develop new treatments for cancer. Many of today's standard cancer treatments – treatments that are accepted and widely used by medical experts – are based on the results of previous clinical trials.

Who are the members of the clinical trials team?

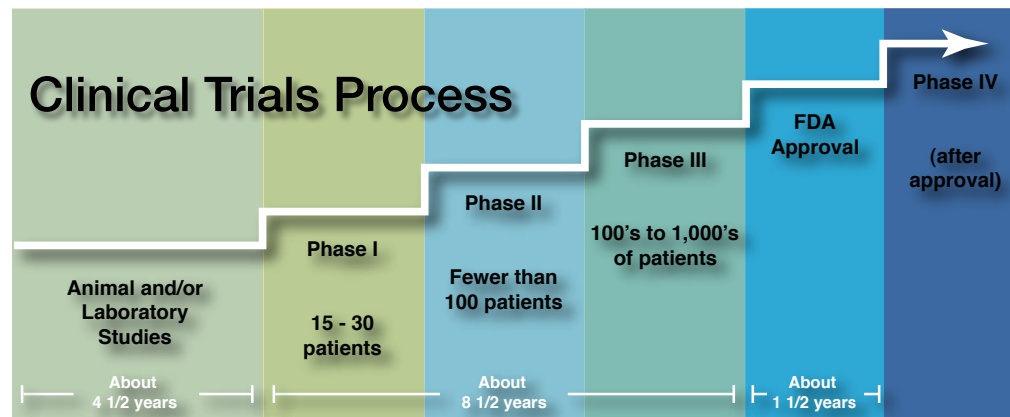
Principal Investigator: The principal investigator, usually a doctor, is responsible for the design, development and conduct of the clinical trial. He or she evaluates data and helps participating doctors manage the trial. (You will continue to see your primary doctor if he or she is not the principal investigator.)

Research Nurse: The research nurse directs and coordinates patient care during a clinical trial. He or she will teach you about participation in the clinical trial; make sure the clinical plan is followed; teach you about side effects; and help manage the clinical trial's data. The research nurse is a good contact for patients who have questions during a clinical trial.

Clinic Nurse: The clinic nurse coordinates general patient care regardless of whether you participate in a clinical trial. He or she will meet with you during your doctor visits. Because the clinic nurse is a part of your health care team, you also may ask him or her questions.

What are the types of clinical trials? As you research and learn about clinical trials, you may come across different types of trials:

- **Prevention trials** study how healthy people may prevent cancer. People who are at high risk of getting cancer may benefit from participation in a prevention trial.
- **Early-detection/screening trials** discover ways to find early-stage cancer.
- **Diagnostic trials** find new and better ways to determine if someone has cancer – and, if so, where the cancer is located in the body; how much cancer is there; and whether or not it has spread to other parts of the body.
- **Quality of life/supportive care trials** seek to improve the comfort and quality of life of patients and their families or caregivers.



The Drug and Approval Process in the 1990s as reported by the National Cancer Institute.

This guide focuses on M. D. Anderson's treatment trials. A treatment trial (also known as a therapeutic trial) studies how a specific anti-cancer treatment affects the patients who receive it.

What are the phases of clinical trials? After promising treatments are explored in animal and/or laboratory studies, researchers perform clinical trials. Once the drug, device or procedure enters the clinical trials process, it must go through several phases:

- **Phase I trials** determine the safety of a new treatment.
- **Phase II trials** determine whether a certain kind of cancer responds to a new treatment.
- **Phase III trials** study whether a new treatment is better than standard treatment.
- **Phase IV trials** find more information about a new treatment that has been already approved for use in patients.

Phase I Trials – Determine safety. The goal of a Phase I trial is to find the safest dose of a new drug that patients can receive without creating side effects that would be too harmful for the patients. During a Phase I trial, the researcher also examines the best way to give a new drug, such as by mouth or intravenously (through a vein).

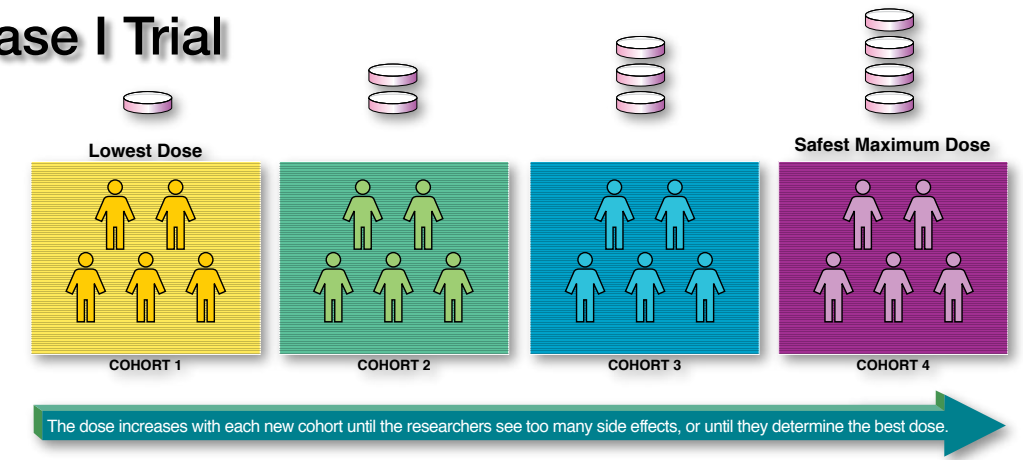
Throughout Phase I trials, researchers monitor whether the new drug shows any effect against cancer. However, because these trials often are testing drugs for the first time in people, the goal is to find out how to give the drug in a safe way, and not how well it fights cancer.

Phase I trials usually include 15 to 30 people. Usually patients who have already taken standard treatments without success and do not have other effective cancer treatment options are eligible to participate in a Phase I trial.

M. D. Anderson's Clinical Center for Targeted Therapies, which opened in late 2004, develops and runs Phase I clinical trials for patients with advanced cancer. If your care center does not have any clinical trials available for you, you may ask your doctor for a referral to the Clinical Center for Targeted Therapy to discuss further options.

In 2006, about 12,000 patients participated in treatment (therapeutic) clinical trials at M. D. Anderson.

Phase I Trial



Typically, patients in Phase I trials are divided into **cohorts** – small groups of patients – usually around three people. The first cohort receives a low dose of the new drug. Researchers may collect blood or urine samples to measure drug levels in the patients. If no severe side effects happen, then a new cohort receives a higher dose of the same drug. The dose increases with each new cohort until the researchers see too many side effects or until they determine the best dose.

If the treatment successfully passes through a Phase I trial, then it will move forward to be studied in a Phase II trial.

Phase II Trials – Determine whether the new treatment works.

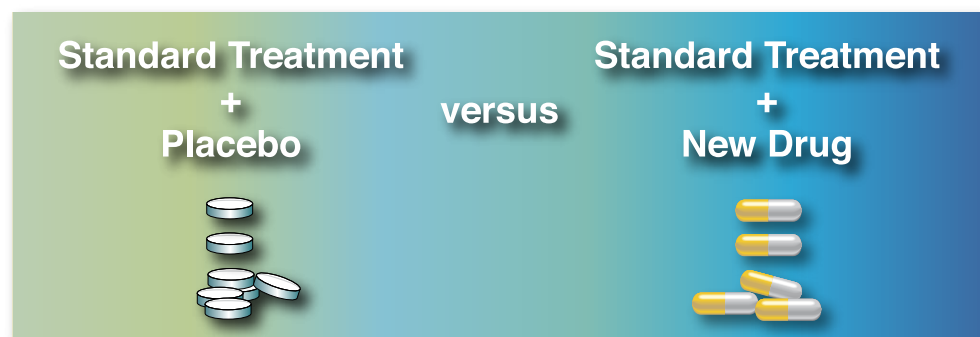
The main goal of a Phase II trial is to examine how well the new treatment works to fight a certain kind of cancer. Less than 100 patients usually participate in a Phase II trial. Patients who volunteer for a Phase II trial may have been treated with chemotherapy, biotherapy, surgery or radiation and still need further treatment.

In addition to evaluating how well the treatment works against the cancer, doctors continue to monitor patients' side effects. Since more patients participate in Phase II studies, some patients may have side effects that the patients in the Phase I clinical trial did not have.

If the new treatment seems to be effective against cancer in a certain percentage of patients, researchers may consider it successful enough to continue study in a Phase III clinical trial.

Phase III Trials – Study whether the new treatment is better than standard treatment.

The goal of a Phase III trial is to compare the new treatment with the standard treatment. Researchers track whether a new treatment is better than, the same as, or less effective than the standard treatment.



Cancer patients are given placebos in a randomized trial only under unusual circumstances.

*If you are offered a clinical trial as a treatment option, you will go through a process called **informed consent**. In this process you will learn about a specific clinical trial, including information about its design. See page 8 for more information.*

Phase III trials may include hundreds to thousands of patients around the country or world. In general, each patient enrolled in a Phase III clinical trial has an equal chance of participating in one of two or more **arms** (groups) of the study. In a clinical trial with two arms:

- One group gets the standard treatment; this is the **control group**.
- The other group gets the new treatment being tested; this is the **investigational or experimental group**.

Randomization – How are patients assigned to groups? The process of assigning participants to groups is called **randomization**. Randomization helps to avoid **bias** in the clinical trial. (Bias occurs when human choices or other factors not related to the treatment being tested change a study's results.)

Neither the patient nor the doctor can choose whether the patient is in the control group or the experimental group. Regardless of which group a patient is assigned to, either he or she will receive the best standard treatment available or the new treatment that researchers believe is as good as, or better than, the standard treatment.

Single Blind Versus Double Blind Trials. In **single blind** studies, patients do not know whether they are in the experimental or control group. In **double blind** studies, neither the patients nor the researchers know which patients are in each group (although this information is recorded and on file if needed). These approaches help researchers avoid bias.

Do cancer patients ever receive placebos (inactive medicines) in a clinical trial? Cancer patients in a clinical trial always receive the best standard treatment available or a new treatment that researchers believe is as good or better.

A **placebo** is a substance that looks like medicine, but is not. Cancer patients are given placebos in a randomized trial only under unusual circumstances. If a placebo is used, researchers may give patients in the control group a **placebo in**

If you have questions or concerns about safety while you are participating in a clinical trial, please contact a member of your clinical trial team. If you still have questions about the study or your rights as a study participant, you may contact the chairman of the Institutional Review Board (IRB) at 713-792-2933.



combination with standard treatment to compare standard treatment alone to standard treatment with a new drug.

FDA Approval The role of the U.S. Food and Drug Administration (FDA) is to make sure medical treatments are safe and effective for people to use. Researchers submit their clinical trial results to the FDA, and based on the information, the FDA may approve the drug or treatment. Then it becomes available to all patients and sometimes becomes the new standard treatment.

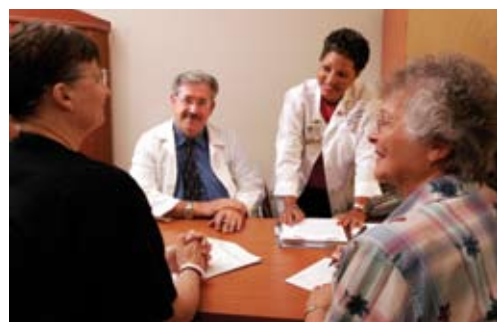
Phase IV Trials – Find more information about the new treatment. Phase IV clinical trials are not as common as Phase I, Phase II and Phase III trials. In Phase IV trials, researchers study drugs and/or treatments that have already received FDA approval. The goal of Phase IV trials is to study how safe and effective a drug or procedure is over time.

How does M. D. Anderson protect patients? When conducting clinical trials, M. D. Anderson's most important responsibility is to protect patients. M. D. Anderson protects patients through well-designed protocols, a dedicated Institutional Review Board (IRB) and a careful informed consent process.

Clinical Trial Protocol A **protocol** is a detailed plan that explains what will be done in a clinical trial and why. It outlines how many patients will take part in the clinical trial, what medical tests they will receive and how often, and the treatment and monitoring plan. Researchers must follow the protocol approved by M. D. Anderson's IRB.

The Institutional Review Board (IRB) M. D. Anderson has four Institutional Review Boards (IRBs). An IRB is a committee of people, such as doctors, nurses, scientists, dentists, chaplains, social workers, attorneys and patients, who are responsible for protecting clinical trial participants and making sure that the trials follow federal laws.

After a clinical trial begins, the IRB monitors the trial at least once a year and stops it if any safety concerns arise. For example, if a patient developed dangerous side effects, then the trial would be stopped.



Before a clinical trial is allowed to begin, the IRB reviews and approves the protocol to make sure that it is based on reliable scientific evidence. The IRB attempts to ensure that the protocol will not cause excessive harm to any patient.

After a clinical trial begins, the IRB monitors the trial at least once a year and stops it if any safety concerns arise. It also may stop a clinical trial early if it becomes clear that the new treatment is much more effective than standard treatment so that all the clinical trial participants may receive the better treatment.

The U.S. Food and Drug Administration regulates M. D. Anderson's IRBs, which report directly to the United States Office for Human Research Protections (OHRP). The OHRP, a part of the U.S. Department of Health and Human Services, is the central guardian of patient safety and welfare in federally funded clinical trials. The OHRP provides leadership to the IRBs and enforces important regulations for patient protection.

The FDA regulates M. D. Anderson's IRBs by auditing IRB minutes, staff and facilities every five years. FDA officials also can visit M. D. Anderson at any time and review anything they choose related to clinical trials.

The Informed Consent Process If your doctor offers you the option to participate in a clinical trial, you will first take part in a process called **informed consent**. In this process you will learn about a specific clinical trial so that you may decide whether you want to participate. The informed consent process is designed to make sure patients understand the clinical trial's plan before agreeing to participate.

The researcher or nurse from the clinical trial team will review the informed consent form in detail with you. This form explains a specific clinical trial's purpose, procedures, risks and benefits.

You will be encouraged to ask questions about terms or ideas that are confusing. A family member or friend may be helpful in the meeting by listening to the explanation, asking questions and recording answers. Some doctors also encourage patients to bring tape recorders so that they can review the information afterwards.

While you are participating in a clinical trial, you may receive updated information about the financial interests of any physician or M. D. Anderson personnel who have cared for you. Call the Conflict of Interest Coordinator at 713-792-3220.

Upon request, you will be given access to information disclosing whether The University of Texas System or M. D. Anderson has a conflict, and you will be given the identity of all physicians, administrators and/or M. D. Anderson personnel who have a financial interest in the clinical trial's sponsor.

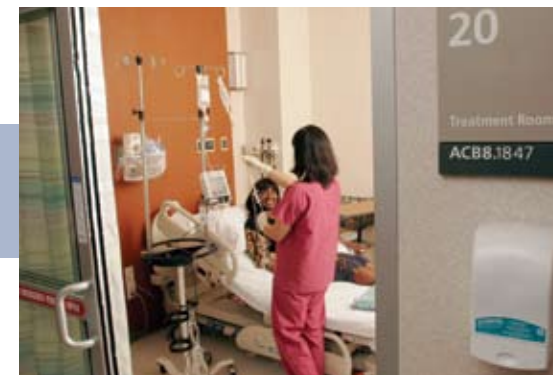
You can take the informed consent form with you to take time to think about whether you would like to participate in the clinical trial.

Information Found on the Informed Consent Form Informed consent forms are different, but they should include:

- The reason for the clinical trial (what the researchers hope to learn)
- Who is eligible to take part in the clinical trial
- What is known about the type of treatment being studied
- The possible risks and benefits (based on what is known so far)
- Other treatments that may be options
- The clinical trial's design (randomized, single blind, double blind, etc.)
- Types of tests, the number of tests and doctor's visits required
- Who is responsible for the costs of the clinical trial (tests, doctor's visits, etc.)
- Who is responsible for the costs if a patient needs additional care as a result of the clinical trial
- A statement about conflicts of interest
- A statement about protecting the patient's privacy
- A statement about the clinical trial being voluntary and the patient's rights to leave the clinical trial at any time
- Contact information for further questions

Signing the Form If you want to participate in a clinical trial after learning all that is involved and what you would be expected to do, then you, the trial's principal investigator (or nurse, if called for in the protocol) and a witness will sign and date the informed consent form. A copy of the form will be given to you.

The informed consent process does not end once you sign the informed consent form. If new benefits, risks or side effects are found during a trial, the doctor must inform all of the participants in the trial. You will be encouraged to keep asking questions throughout the trial.



M. D. Anderson will take appropriate steps to keep your personal information private. However, there is no guarantee of absolute privacy. The Food and Drug Administration (FDA), the IRB of M. D. Anderson and the clinical trial's sponsor might review your record to collect data or to see that the research is being done safely and correctly. Under certain circumstances, the FDA could be required to reveal the names of participants.

After the clinical trial ends, what happens to the information and results? Researchers give patients a number or code to protect their identities. When the clinical trial results are published, patients' names are not used. The research team may access a clinical trial's information to help the clinical trial sponsor submit data to the FDA to get the treatment approved.

If you want to read what has been published about a clinical trial that you participated in, contact your doctor. Ask for the name of the journal, date of publication, lead author and title of the study. The Learning Center staff can help you locate a copy of the article. (For more information about The Learning Center, please see page 15.)

Who sponsors M. D. Anderson's clinical trials? The U.S. National Cancer Institute (NCI) sponsors many of M. D. Anderson's clinical trials. Drug companies may also sponsor clinical trials. This is because these companies must show that their drugs and medical devices are safe and effective in order to receive FDA approval and become available to the public. M. D. Anderson and nonprofit organizations also sometimes sponsor clinical trials.

How do patients find a clinical trial at M. D. Anderson? There are several ways you can learn more about clinical trials conducted at M. D. Anderson:

- Ask your doctor.
- Call askMDAnderson at 877-MDA-6789.
- Visit The Learning Center at M. D. Anderson. (For more information about The Learning Center, please see page 15.)
- Go to <http://www.mdanderson.org>, and click on Clinical Trials.

For a complete list of resources about finding clinical trials, please see page 15.

M. D. Anderson is strongly committed to maintaining the privacy of your health information under the Health Insurance Portability and Accountability Act (HIPAA). For more information on HIPAA, please refer to M. D. Anderson's Notice of Privacy Practices or contact the Compliance Office at 713-745-6633. You may also find more information in the At Your Service guide, which is available at your center's Business Center or The Learning Center.

How will I know if I am eligible to participate in an M. D. Anderson clinical trial? Each clinical trial has eligibility criteria. **Eligibility criteria** are requirements that patients must meet before they can participate in a specific clinical trial. 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 think you might qualify for, talk to your doctor to see if you qualify, or contact the clinical trial's principal investigator or research nurse.

What should I consider before I decide to participate in a clinical trial?

Benefits and Risks Each clinical trial is unique and has its own benefits and risks. The following are ways you may benefit from participation in a clinical trial:

- You may have more treatment options.
- If the new drug or treatment works, you may be among the first to benefit.
- You may be able to help other cancer patients in the future.
- The trial sponsor may pay for some of your medical care or tests. (Ask your doctor or the research nurse about who is responsible for these costs before agreeing to participate.)



Some possible risks include:

- Side effects may be worse than those of standard treatment.
- Side effects may occur that the doctor does not expect.
- New treatments do not always turn out to be better than, or as good as, standard treatment.
- As with standard treatment, the new treatment may not work for you even if it works for other patients.

How do M. D. Anderson patients pay for clinical trials? The clinical trial sponsor (whether it is the government or a company) may pay for the experimental treatment, special testing, extra doctor visits, travel time and travel expenses.

Some health insurance companies will cover the costs of procedures that patients would have even if they were not in a clinical trial (routine costs). For Medicare patients, routine costs are covered in all Medicare-qualified clinical trials.

You will be responsible for any costs not covered by the clinical trial sponsor or your health insurance company. Therefore, be sure to ask the principal investigator or research nurse specifically about what costs will be your responsibility if you decide to participate in a clinical trial.

Questions to Ask About the Clinical Trial As the patient, it is your decision whether to participate in a clinical trial. Since it is your choice, ask as many questions as you need to ask, and continue to ask questions until the answers are clearly understandable to you. Some examples of questions you should ask about a clinical trial include:

Treatment-Specific Questions

- What is my prognosis? (Your **prognosis** is what the doctor thinks will happen with your cancer – your chance of recovery, the expected course of the cancer, or the length of time you are sick.)

You may feel anxious when considering a clinical trial because you may be used to your doctor making treatment decisions for you. At M. D. Anderson, we encourage a partnership between you and your health care team. We respect your right to make an individual decision that is based on your personal beliefs and values as well as on the available medical information. It is your right to decide whether you wish to be treated, and if so, by which method of treatment.

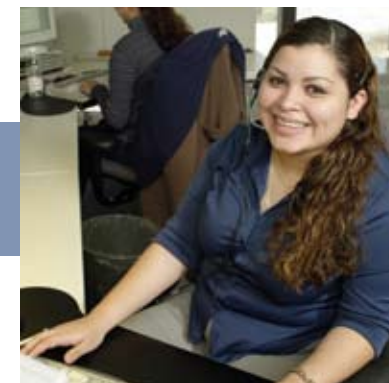
- What are my treatment options?
- How would the standard treatment affect my prognosis?
- How might the clinical trial affect my prognosis?
- What phase is the clinical trial?
- What is the purpose of the clinical trial?
- Why do researchers believe the new treatment being tested may be effective? Has it been tested before? Has anything been published about this treatment? If so, by whom?
- What are the possible short-term and long-term risks and/or side effects?
- How do the possible risks, side effects and benefits in the clinical trial compare with standard treatment?
- How often and for how long would I receive treatment?
- Who would be in charge of my care?
- Who would be my main contact if I have any problems, questions or an emergency?
- How long would I need to remain in the clinical trial?
- Would there be any follow-up after the clinical trial?

Logistical Questions

- Would I have to come to M. D. Anderson for all treatments, tests and procedures? How often would I have to come? How does this compare with standard treatment?
- Where would I receive treatment?
- How long would each treatment appointment last, including pretreatments?
- What would I need to do as a participant in the trial?
- Would I be responsible for additional costs of tests or travel?

Emotional and Relationship Needs Questions

- How would the clinical trial change or affect my daily activities?
- Can you put me in touch with other people who are in the clinical trial?
- Can you put me in touch with support services if I need them?



Whatever decision you make, you have the right to receive the best care available. Your relationship with your doctor will not be changed by your decision.

Decision-Making Suggestions After discussing questions with the doctor and/or nurse, take time to consider your options so that you feel comfortable with your decision. Some suggestions to consider when you are deciding about a clinical trial include:

- Learn as much as you can.
 - Talk to your doctor or nurse.
 - Go to The Learning Center at M. D. Anderson. The Learning Center is located in the Main Building and the Mays Clinic. (See more information on page 15.)
 - Search the resources listed on page 15 for more information.
- List the pros and cons of your options.
 - Determine benefits and risks that are most important to you. Is it likely that these benefits and risks will happen?
- Get your questions answered.
 - Keep a list of all of your questions and answers, and continue to add questions to your list.
 - Take your questions to your doctor. Make sure you get an answer for each question from your doctor or nurse.
- Define your priorities and your role in your family or community.
 - What or who is most important to you?
 - Consider making a decision that helps you continue doing what is most important to you (being a parent, working as an employee, being a friend, etc.).
- Talk to your family and friends.
 - Is it important for you to do what your family thinks is best?
 - Ask opinions of people you trust.
- Consider practical issues.
 - Determine travel time to meet your appointments (especially if you live outside of Houston).
 - If you would need to come to Houston often, would someone be able to come with you?
 - Would it be difficult for you to complete all that you are responsible for in the clinical trial?

- Would you dislike having to complete a patient diary if one is required? Consider the time commitment to participate in the trial.
- Think about how participating in a clinical trial would make you feel.
 - What decision would give you the least amount of stress?
 - Is it important to you to try anything that is new if you qualify?

Whatever decision you make, you have the right to receive the best care available. Your relationship with your doctor will not be changed by your decision.

Where can I find additional information?

Support by Phone

askMDAnderson

Call 877-MDA-6789.

Trained health information specialists provide information about M. D. Anderson's clinical trials.

National Cancer Institute's Cancer Information Service

Call 800-4-CANCER or 800-422-6237.

Trained information specialists can help callers determine whether they are eligible for various protocols.

The Learning Center

<http://www.mdanderson.org/departments/tlc/>

The Learning Center is a free consumer health library with the latest information on cancer care, support, prevention and general health and wellness issues. Locations provide a wide range of materials, and each site's resources are different. The Learning Center staff provides skilled, individualized service and will be happy to help you.

The Learning Center provides materials for information and convenience only. They are not to be substituted for medical advice. Medical information is often controversial and continually changes. Please consult your health care provider to discuss your specific concerns.



M. D. Anderson's Anderson Network, a program of the Department of Volunteer Services, is a unique cancer support group of current and former patients who can relate to what you're going through and offer patient-to-patient advice and encouragement. Services include Hospitality Centers in the Main Building and Mays Clinic, a patient and caregiver support line at 800-345-6324, weekly educational forums (PIKNIC) featuring M. D. Anderson and community experts, an online Ask the Expert message board, and more.
<http://www.mdanderson.org/departments/andersonnet/>

**Theodore N. Law Learning Center
Main Building**
Floor 4, near Elevator A
Room R4.1100
Monday - Friday, 9 a.m. - 4 p.m.
713-745-8063

**Levit Family Learning Center
Mays Clinic**
Floor 2, near The Tree Sculpture
Room ACB2.1120
Monday - Friday, 9 a.m. - 4 p.m.
713-563-8010

The following are resources recommended by The Learning Center:

Pamphlets

National Cancer Institute. *Taking Part in Clinical Trials: Cancer Prevention Studies, What Participants Need To Know*. NIH Publication No. 98-4250.
National Cancer Institute. *Taking Part in Clinical Trials: What Cancer Patients Need To Know*. NIH Publication No. 98-4250.

Videos

Clinical Trials at M. D. Anderson Cancer Center: What You Need to Know.

Internet Resources

The University of Texas M. D. Anderson Cancer Center Clinical Trials

http://www.mdanderson.org/patients_public/clinical_trials/
Find open M. D. Anderson trials listed by type of cancer, treatment, study number and physician name.

NCI Clinical Trials Database

<http://www.cancer.gov/clinicaltrials>
Click *Finding Clinical Trials*. Under *Use Other Web-Based Resources*, click *basic* or *advanced* to use the search form. You may also call 800-4-CANCER for access to these materials.