Clinical Trials at MD Anderson

THE UNIVERSITY OF TEXAS
MD Anderson Cancer Center
Making Cancer History®

“I’m also pretty excited about my involvement in this clinical trial. Because I was able to have this amazing treatment option first. And now, because of me and other clinical trial participants, more cancer patients may be able to have it someday, too.”

– Mallory Parish, hodgkin lymphoma patient

Table of Contents


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Is a clinical trial right for you?

In clinical trials, doctors test how new medicines and treatments work in people. This is how we find better ways to prevent, diagnose and treat cancer. Clinical trials may provide you with access to a new treatment for cancer. By joining a trial, you may also help future patients.

All patients who join clinical trials are volunteers. This means you do not have to take part if you do not want to, and you can stop taking part in a trial at any time.

The best way to know if you should consider a clinical trial is to talk with your care team. This booklet was created to help you and your loved ones learn more about how treatment clinical trials work. Throughout the booklet, you will find questions that other patients have asked when considering a trial. You may want to use the questions as a guide for talking with your doctor.

Ways to find a clinical trial at MD Anderson:

- Ask your doctor
- Call askMDAnderson at 877-632-6789
- Visit The Learning Center patient library
- Go to www.ClinicalTrials.org

"So, whenever anyone asks me what I think about clinical trials, I say that if you qualify to participate and they seem right for you, they are definitely worth it."

– Stephanie Watson, melanoma patient

Treatment Options

Standard treatment is the treatment most often used to treat a specific type of cancer.

- Standard treatment may also be called standard of care.
- Factors such as your health, stage of cancer, tumor size and family history determine standard treatment for you.
- Standard treatments are based on the U.S. Food and Drug Administration (FDA) approval and other medical guidelines.
- For most cancers, guidelines on standard treatments are published for doctors and patients.

Clinical trials may offer a treatment that is different than the standard treatment.

- Clinical trials aim to find new medicines or treatments that are more effective, or have fewer side effects than the standard treatment.
- Many clinical trials are for new medicines or treatments that the FDA has not yet approved. Some trials study treatments that the FDA has already approved.
- Successful treatments in clinical trials may become the new standard treatment.
- All patients receive treatment in cancer clinical trials. Placebos are rarely used, and if used, will be given with standard treatment as well.

Clinical trials can be for all types of patients. There are trials that involve people with any type of cancer and people at different stages of cancer. Most cancer clinical trials are treatment trials. Some trials are about managing symptoms, supportive care and improving quality of life.

Treatment clinical trials may test:

- New treatments
- New combinations of treatments
- New approaches to surgery or therapy

Clinical trials are designed to test if new treatments or procedures may be as good as or better than existing treatments.

Both standard of care treatment and clinical trials may involve:

- Chemotherapy
- Surgery
- Radiation
- Targeted therapy
- Immunotherapy
- Combined treatments (more than one kind of treatment used together)
- No treatment or delayed treatment (in some cases, doctors may watch a cancer and wait to see if it grows)

People join clinical trials for different reasons. When thinking about your treatment options, gather as much information and ask as many questions as you need. Use the questions in this booklet as a guide for talking with your doctor.

? What is the type and stage of cancer that I have?

? What are my treatment options?

? Are there any clinical trials that apply to me?

? How might the cancer respond to standard treatment?

? How might the cancer respond to treatment in a clinical trial?

How You Are Protected in Clinical Trials

Finding new cancer treatments is a long, careful process. Before a treatment is given to people, doctors know a great deal about it from other testing. For example, researchers may study cancer cells in a lab and then study the treatment in animals. Only treatments that show promise during these first steps are studied in people through clinical trials.

All treatments and procedures have some risk of harm. At MD Anderson, your safety is our top priority. Precautions and careful procedures are in place to make sure your risk is as low as possible. You will be informed of all potential benefits and risks before you agree to be part of a clinical trial.

Study Protocols

MD Anderson protects patients in clinical trials by following well-planned protocols. A protocol is like a recipe for a research study. It lays out every step of the clinical trial process to make sure the research is carried out in a certain way. Trials must follow strict laws, regulations and safety procedures.

A protocol explains:
- The goal of the trial
- Who is eligible to take part in the trial
- Details about the tests, procedures and treatment plan
- How long the trial may last
- How many patients may take part in the trial
- How patients are protected against possible risks

A committee called the Institutional Review Board (IRB) must approve clinical trials. You may only join a trial if the IRB has reviewed and approved it.

Institutional Review Board (IRB)
An IRB is a group of people who are responsible for protecting the rights and well-being of all volunteers in clinical trials. IRBs includes doctors, nurses, chaplains, social workers, lawyers and patients.

To protect patients, the IRB at MD Anderson:
- Reviews a clinical trial's protocol and informed consent form
- Makes sure that the trial follows federal laws
- Makes sure that the risks in the trial, when compared to the possible benefits, are reasonable for patients
- Monitors the trial from start to end
- Provides contact information to patients. If you are in a clinical trial, you may contact the IRB with any questions or concerns.

The FDA reviews and audits the IRB's files. FDA officials may visit MD Anderson at any time and review anything related to clinical trials.

Informed Consent
You join a trial as a volunteer. MD Anderson protects patients through a careful informed consent process to help you learn all that is involved in a trial before you join it. The research team explains the details of the trial to you, and you will receive an informed consent form that explains the details in writing. You may ask questions at any point in the trial.

Eligibility Criteria
Clinical trials must follow guidelines for who can qualify for the trial. These guidelines are called eligibility criteria. They describe conditions that must be shared by all patients in a trial. The conditions are different for each trial.

Eligibility criteria often include age, gender, medical history and current health status. To join a trial, patients often need to have a certain type and stage of cancer.

Placebos

A placebo is something that looks like the treatment being studied, but has no physical effect. Some patients worry about receiving a placebo if they join a clinical trial. **In cancer clinical trials, a placebo is rarely given by itself.**

Your doctor will always tell you if a clinical trial uses a placebo. If a placebo is used, it is given together with the standard treatment. This means that you will still receive treatment for your cancer.

To test a new combination of treatments, a trial may give some patients standard treatment with a placebo and give other patients standard treatment with the new medicine. All patients receive at least the standard treatment for their cancer.

STANDARD TREATMENT + PLACEBO

versus

STANDARD TREATMENT + NEW TREATMENT

If there is no effective treatment for a cancer, standard treatment may mean that doctors normally care for the patient by treating symptoms and monitoring the cancer. To find a treatment that works for these cancers, a clinical trial may involve giving a placebo to some patients and a new treatment to others. This is not common in cancer trials.

? How might standard treatment affect my outcomes?

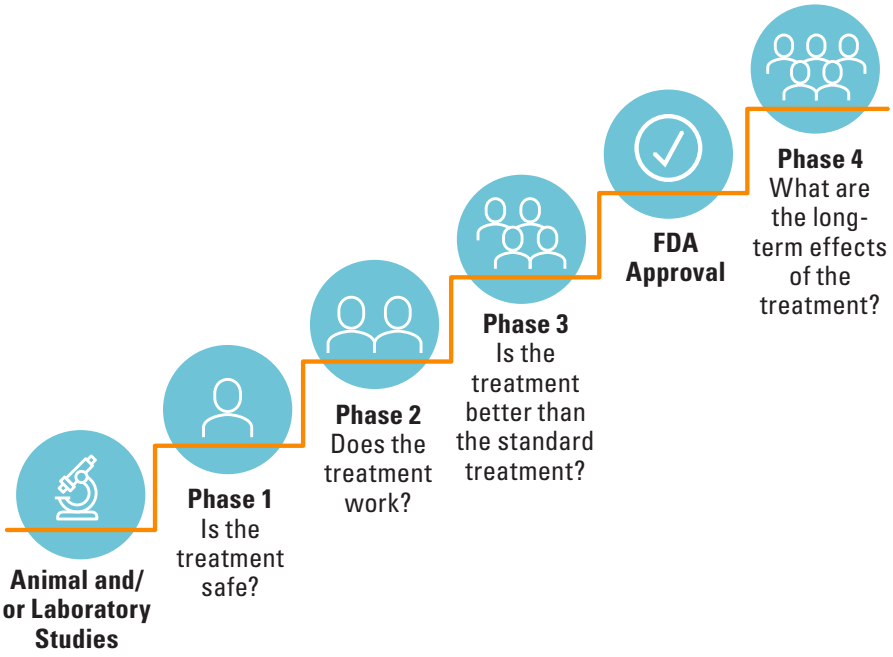
? How might the clinical trial affect my prognosis?

Phases of Clinical Trials

Testing a new treatment involves a series of clinical trials. Clinical trials are done in steps, called phases. Each clinical trial phase is designed to learn specific information about the new treatment. Knowing the phase or phases of a clinical trial may help you decide if the trial is right for you.

- The phases of clinical trials are done in order from 1 to 4.
- Successful treatments move through the phases.
- Doctors may stop a trial right away if they have concerns about the safety of the patients in the trial.

Each phase aims to find the answers to certain research questions and builds on knowledge gained in previous phases.



Many clinical trials only involve one phase of the research process. Some trials combine phases. For example, a trial may involve both phases 1 and 2.

? What phase is this clinical trial?

? What is the purpose of this clinical trial?

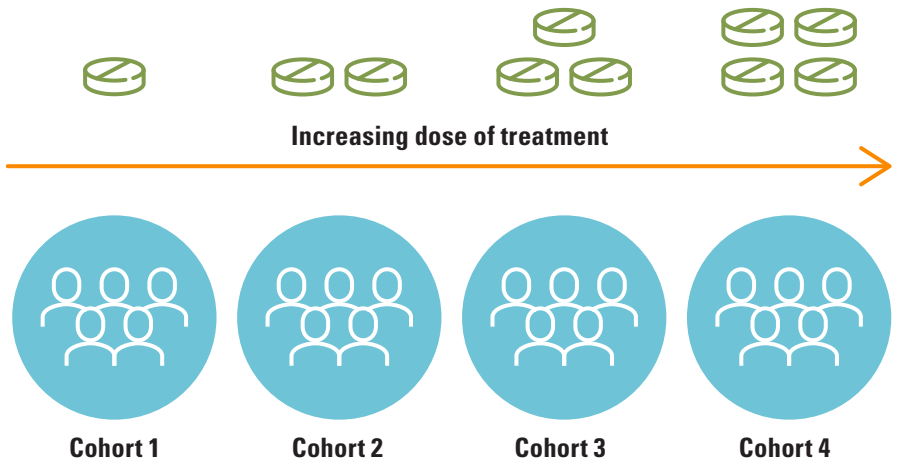
Phase 1 Trials

Phase 1 trials are the first step in testing a new treatment or combination of existing treatments in people. These trials often involve the greatest risk for patients. Most Phase 1 trials are small, and involve fewer than 30 patients.

In Phase 1 clinical trials, doctors:

- Test whether a new treatment is safe and look for the best dose of the treatment to use. (The dose is the amount of a medicine you receive)
- May look for the best way to give the treatment (such as through a pill or an injection)
- Watch for signs that the treatment is working

To test for the best dose of a treatment, patients are divided into small groups called cohorts. Patients in the first cohort get the first dose of the new medicine. If the first cohort does not have any severe side effects, then a new cohort gets a higher dose of the same medicine. The dose increases with each new cohort until doctors find the best dose. With each increasing dose, doctors also test to see if patients respond to the treatment.



If the doctors find that the treatment is safe, it moves to a Phase 2 trial for more testing.

Phase 2 Trials

The goal of a Phase 2 clinical trial is to see if the new treatment has an effect on a cancer. Doctors study the treatment in one type of cancer. Most Phase 2 trials involve fewer than 100 patients.

Even though the main goal is to see if the treatment works, doctors still closely watch for side effects. If the new treatment works, doctors may go on to study it in a Phase 3 trial.

Phase 3 Trials

Phase 3 trials may include hundreds to thousands of patients around the country or world. In this step of the research, doctors test if a new treatment is better than the standard treatment for a cancer. Safety is also closely monitored as the treatment is studied in more people.

In Phase 3 clinical trials, each patient in the trial will receive the standard treatment **or** the new treatment. Every patient in the trial has a chance to be put into one of 2 groups:

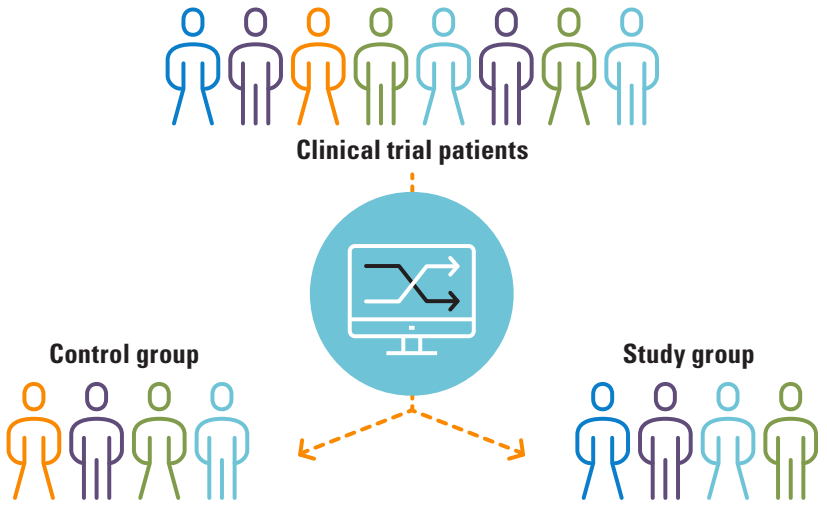
- The **control group** receives standard treatment.
- The **study group** receives the new treatment being tested.

At this step of the research, doctors do not know if the new treatment is better than the standard treatment. They believe the new treatment is as good as, or even better, than the standard treatment.

Doctors do not decide how patients are grouped. Instead, they use a computer to randomly assign the groups. This process is called **randomization**.

Randomization:

- Ensures all patients have equal chances of being in either group
- Allows doctors to compare outcomes between the study group and the control group
- Ensures if differences are found they are most likely due to the treatments and not other factors



After the computer assigns you to a group, you will **not** find out if you are in the study group or the control group. Your doctor may or may not know which group you are in. This depends on the design of the research study.

In single blind studies:
- The doctor knows the group the patient is in.
- The patients do not know which group they are in.

In double blind studies:
- The doctor does not know the group the patient is in.
- The patients do not know which group they are in.

In all studies, doctors can find this information in the case of an emergency.

A successful Phase 3 trial may lead to changes in the standard treatment for a condition. At this stage, the FDA reviews the study results to make sure the treatment is safe and effective. The FDA decides whether to approve the new treatment for patients who have that condition.

Phase 4 Trials

In Phase 4 trials, doctors study treatments that the FDA has already approved. The goal of these studies is to learn more about long-term side effects. Researchers may also look at how the treatment affects patients' quality of life and whether it is cost-effective. Phase 4 trials often involve thousands of people.

? Why do doctors believe this new treatment being tested might work for me?

? Has the new treatment been tested before?

? What has been written about this treatment (such as articles in medical journals that you may read)?

Benefits and Risks

Like all treatment options, clinical trials have possible benefits and risks. When thinking about joining a clinical trial, you may consider if the possible benefits are greater than the risks.

Possible benefits:

- The new treatment may work better than the standard treatment.
- The new treatment may only be available through the clinical trial.
- The results of the trial may advance research and help future patients.
- You receive close monitoring from an expert medical team.

Possible risks:

- The new treatment may not be better than standard treatment.
- The new treatment may not work for you, even if it works for other patients.
- There may be known and unknown side effects.

You can read about the possible benefits and risks of a specific clinical trial in the trial's informed consent form. The research team provides you with a copy of this form.

? What are the possible short-term and long-term benefits of this clinical trial?

? What are the possible short-term and long-term risks of this clinical trial?

? How do the possible benefits and risks of this clinical trial compare with the standard of care treatment for my cancer?

"Knowing I was a part of making that happen as a clinical trial participant feels really good. Because I believe that life is a process of overcoming challenges. Cancer was just the latest. And now, my experience may help someone else to overcome their own."

– Abdel Monem Said Aly, Ph.D., myelodysplastic syndrome patient

Joining a Clinical Trial

Not all clinical trials are right for all patients. Each clinical trial is designed with strict guidelines about who is able to join. These guidelines are called **eligibility criteria**. To join a trial, you must meet all the eligibility criteria.

Eligibility criteria may include information about:

- You and your overall health
  - Age
  - Gender
  - Results of medical tests
  - Medicines that you are taking or have taken in the past
  - Any other health conditions you have
- Your cancer
  - Cancer type
  - Stage
  - Other treatments you may have had
  - How long it has been since you were last treated

If you find a clinical trial you would like to join, contact your doctor. You and your doctor can review the eligibility criteria to see if you qualify for the trial.

Informed Consent Process

Before you join a trial, take time to learn what you may expect during the trial. The research team will help you do this during a process called informed consent.

During informed consent, you will meet with the research team to talk about all that is involved in the trial. You will also receive a form that provides the details in writing. The research team reviews this informed consent form with you and answers any questions you may have.

Review the Details
Look for the following information on the informed consent form.

Treatment
- The reason for the clinical trial (what the doctors hope to learn)
- Who is eligible to take part in the clinical trial
- What is known about the treatment being studied
- Possible benefits and risks
- Other treatment options

Tests
- What tests are involved
- How often the tests are done

Study Visits
- What other visits with the research team are involved
- How often you meet with the doctor
- How long you will be in the clinical trial

Costs
- Who pays for the clinical trial
- If the clinical trial causes you to need more medical care, who pays for those costs

Other
- Conflicts of interest (any direct financial benefit to MD Anderson Cancer Center or your doctor from the sponsor of the trial)
- How your privacy and information is protected
- What information about you is collected
- Who you may call if you have questions

The goal of informed consent is to make sure you know the details of a trial before you agree to join the trial. Take all the time you need to review the details and discuss them with your doctor and loved ones.
- Ask as many questions as you need.
- Bring a family member or friend to help you ask questions and write down answers.

- You may take the informed consent form home with you to review before signing it.

Signing the Informed Consent Form
If you choose to join the trial, you and the Principle Investigator (PI) will sign the informed consent form.

- You will receive a copy to keep for your records.
- Even after you sign the consent form, you can decide to stop and leave the clinical trial at any time. **You always have the right to leave a clinical trial.** Tell your team if you want to stop, and they will help you do so safely.

The informed consent process does not end once you sign the informed consent form. For example, your doctor must tell you if new risks or side effects of the treatment are found during the trial. You may ask questions at any time during the trial.

? To join this trial, will I need to gather any medical or personal records?

? Where will I receive treatment? How often will I have to come to MD Anderson? How does this compare to standard treatment?

? How often will I have visits for the clinical trial?

? How long will each visit last?

? What practical details do I need to consider (such as childcare, transportation, travel, time away from work)?

? How will this clinical trial affect my daily activities?

? Can I talk with other people who are in this clinical trial?

Your Care Team

If you join a clinical trial your primary care team will still care for you. You will also have a research care team.

The people on your research care team include:

- **Principal investigator (PI):** The PI is usually a doctor. The PI runs the clinical trial and makes sure the research plan is followed. You can find the PI's phone number in the informed consent form. Your doctor and your PI will communicate together about your care.
- **Research nurse:** A research nurse works closely with the PI to monitor your treatment and will help teach you about each step of the clinical trial. Your research nurse is a good person to contact if you have questions.
- **Study coordinator:** Study coordinators help arrange your care and guide you through the clinical trial process.

After a trial, you may continue to see your research team for treatment and follow-up care along with your primary care team.

Paying for Clinical Trials

The services you receive in a clinical trial are either routine costs or research costs.

- **You and your insurance provider are billed for routine costs.** You are responsible for out-of-pocket costs such as co-pays, deductibles and co-insurance.
- **You do not pay for research costs.**

Routine Costs

A clinical trial may include services or procedures that are standard care to manage your cancer. You would receive these routine services even if you were not in the trial.

Research Costs

Unlike routine costs, research costs are for services and procedures provided only for the purpose of the clinical trial.

Medicare

If you have traditional Medicare, your Medicare insurance covers routine costs for clinical trials. If you have a Medicare Advantage plan, traditional Medicare also covers your routine costs. You may be responsible for out-of-pocket costs such as co-pays, deductibles and co-insurance.

The routine and research costs of every trial are different. The MD Anderson Financial Clearance Center (FCC) can help answer your questions about insurance coverage and what you might have to pay. To talk with a financial counselor at the FCC, ask your research nurse or call 844-294-4322.

? Does my insurance company provide coverage to participate in a clinical trial?

? What costs am I responsible for?

Available Support

Clinical trials may involve more medical visits than standard treatment. These extra visits may mean you need to plan for more time, travel or other expenses such as childcare. Support may be available to help you with out-of-pocket costs related to clinical trial participation. Talk with the research team or ask your social work counselor about resources that may be available to you.

? Are resources available to help me with trial expenses?

Leaving a Trial

All patients in clinical trials are volunteers. You can choose to leave a clinical trial at any time, but talk with your doctor first. Your doctor can tell you how leaving the trial might affect your health and if there are other treatment options. Your doctor can also tell you the safest way to stop taking any clinical trial medicines and collect any remaining research study medicine or empty bottles that you may have. Your relationship with your care team is not changed by your decision.

? What happens if I decide to leave the trial?

Decision Guide

This decision guide is meant to help you think about your options, the people who support you and what matters most to you. Ask your care team any questions you need to help with your decision-making process.

What decision do I face?

When do I need to decide?

What is my progress on making a decision?

- ☐ I have not thought about my options.
- ☐ I am thinking about my options.
- ☐ I am close to making a decision.
- ☐ I have already made a decision.

How do you prefer to make this decision?

- ☐ I prefer to make the decision alone.
- ☐ I prefer to make the decision, after getting others' thoughts and opinions. Whose opinions are important to get?:

- ☐ I prefer to make the decision together with others, including:

- ☐ Other:

Support from Others

Your care team, family and friends may have a part in helping you decide. Fill out the chart below to help you think about the support you get from others.

? Do you have enough help from others to decide?

☐ Yes
☐ No

? Are you choosing without pressure from others?

☐ Yes
☐ No

Support Person Name:
Which option does this person prefer? Do you feel pressure from this person? How can this person support you?
Support Person Name:
Which option does this person prefer? Do you feel pressure from this person? How can this person support you?
Other Notes

If you feel pressure from others to make a certain choice:

- Focus on the opinions of those who matter most to you.
- Share this decision guide with others. You may also ask a family member or friend to complete this decision guide. Listen to each other's thoughts and opinions. Talk about what matters most to each of you.

Support is available:

- Talk about your options with a person you trust, such as doctor, nurse, chaplain, social worker, family member or friend.
- Find out what help is available to support your choice. For example, you may need help with things like financial resources, transportation, housing or childcare. Talk with the research team or ask your social worker about resources that may be available to you.

Know the Facts

It is important to know the facts before you decide. Resources are available to help you find information.

? Do you know all of your options?

☐ Yes
☐ No

Each option may have pros and cons. Pros are the reasons you may choose an option (benefits). Cons are the reasons you may avoid an option (drawbacks).

? Do you know the pros and cons of each option?

☐ Yes
☐ No

What Matters Most to You

Follow the steps below to help you think about what matters most to you.

To help you complete this exercise, it may help to:

- Talk with people who have experienced the pros and cons.
- Talk with others who have made a similar decision.
- Discuss what matters to you with others.

Steps

1. List your options. For each option, record the pros and cons you already know.

2. Rate how much each pro and con matters to you. Use a scale of 0 to 5 stars (★).
   - 5 stars (★★★★★) means it **matters a lot.**
   - No star means it does not matter at all.

Option 1:

The pros with the most stars are those that matter most to you.

Pros	How much it matters to you (0 to 5 stars)

The cons with the most stars are those that matter most to you. They may be the most important for you to avoid.

Cons	How much it matters to you (0 to 5 stars)

Option 2:

The pros with the most stars are those that matter most to you.

Pros	How much it matters to you (0 to 5 stars)

The cons with the most stars are those that matter most to you. They may be the most important for you to avoid.

Cons	How much it matters to you (0 to 5 stars)

Option 3:

The pros with the most stars are those that matter most to you.

Pros	How much it matters to you (0 to 5 stars)

The cons with the most stars are those that matter most to you. They may be the most important for you to avoid.

Cons	How much it matters to you (0 to 5 stars)

Next Steps

Based on what you recorded about your options, you may consider:

- How likely are the pros and cons to happen?
- Do you need more information about certain pros or cons?

List your questions and where you will look for the answers (such as your doctor, research nurse, social work counselor or The Learning Center patient library):

What other factors are making it hard to decide?

List anything else you might need to decide:

Notes

Notes

“My doctor told me ‘Your best hope is to participate in a clinical trial at MD Anderson. You’ll have access to potential treatments before they’re widely available.’

I said ‘Count me in. I’m 26 years old. I still have a lot of living to do.’”

– Connor Johnson, acute myeloid leukemia patient

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