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WHAT ARE THE GOALS OF MYELOMA CLINICAL TRIALS?

• Create truly curative therapies
• Develop therapies that can work when others have failed
• Decrease the side effects of treatment
• Improve the survival rate for multiple myeloma

TIPS FOR LEARNING ABOUT & FINDING CLINICAL TRIALS

• Know the stage of your myeloma and obtain a copy of your pathology report.
• Learn about eligibility criteria such as age and physical fitness.
• See if there are financial resources that can help assist with travel and other costs.
• Ask what the risks are to participating.
QUESTIONS TO ASK YOUR PROSPECTIVE CLINICAL TRIAL MEDICAL TEAM

- What kinds of tests, medicines, surgery, or devices are involved in the trial? Are any procedures painful?
- What are the possible risks or side effects of taking part in the study?
- What are the potential benefits of participating in this study? How would this be more beneficial than my current treatment option or the typical standard of care?
- How will this trial affect my daily life? Will I have to be in the hospital for a long period of time?
- Will I have to travel for this trial? Where and how often? Are telehealth visits an option?
- How long will the trial last?
- What will happen after the trial?

Cost-specific questions
- Who will pay for the tests and treatments I receive?
- Are there additional costs if I enroll in the trial?
- Does my insurance cover any of these trials?
- Will I be reimbursed by the trial sponsors for other expenses (for example, travel and childcare)?
WHAT TYPE OF TREATMENTS ARE USED IN A CLINICAL TRIAL?

Though research advances have improved myeloma survival rates and treatment response, several types of myeloma research and clinical trials are under way, especially to study treatment-resistant myeloma cells.

Specific drug therapies under study under research and in clinical trials for people with multiple myeloma include:

**Stem Cell Transplantation:**
Various approaches are under research, including the use of nonmyeloablative (reduced-intensity) allogeneic and autologous stem cell transplantation.

For more specific information about stem cell transplantation types, see Blood and Marrow Stem Cell Transplantation.
WHAT TYPE OF TREATMENTS ARE USED IN A CLINICAL TRIAL?

Proteasome Inhibitors (PIs): The mechanism of action for PI drugs block activity of proteasomes, which then leads to cancer cell death.

Many studies are researching the safety and efficacy of proteasome inhibitors, used in combination with other agents, for both the treatment of relapsed and refractory types of multiple myeloma and for newly diagnosed myeloma.

Immunotherapy:
Various forms of immunotherapy are being studied in clinical studies, including:

• **Chimeric Antigen Receptor (CAR) T-Cell Therapy:** CAR T-cell therapy is an immunotherapy that takes a patient’s own immune cells and then modifies them to be used to attack the cancer cells in their body. Chimeric antigen receptors (CARs) refer to the surface receptors that are generated from the genetic engineering of the patient’s T cells.
WHAT TYPE OF TREATMENTS ARE USED IN A CLINICAL TRIAL?

Immunotherapy (continued):

- Various ongoing research is looking at CAR T-cell therapies that target other cell antigens, such as CD38, CD19, and the signaling lymphocytic activation molecule F7 (SLAMF7), for myeloma treatment.

- The B-cell maturation antigen (BCMA) is being targeted by CAR T cells in clinical research for patients with relapsed or refractory multiple myeloma.

Dendritic Cell/Tumor Fusion Vaccines: Multiple myeloma cells have proteins on their surface that may be especially well-suited vaccine targets. Dendritic cells are generally Normally found in small amounts within the body, dendritic cells are responsible for immune responses against foreign invaders. For the creation of fusion vaccines, cells are taken from the patient’s tumor and fused (mixed) with dendritic cells removed from the blood, with the goal of stimulating a powerful antitumor response.
WHAT TYPE OF TREATMENTS ARE USED IN A CLINICAL TRIAL?

Immunotherapy (continued):

• **B-Cell Maturation Antigen (BCMA):** Also known as TNFRSF17 (part of the tumor necrosis factor protein superfamily), this antigen is a vital cell surface protein involved in supporting myeloma cell survival. It is expressed at drastically increased levels in all myeloma cells, but not on other normal tissues (except normal plasma cells). A drug under study is elranatamab (PF-06863135). Elranatamab is an investigational B-cell maturation antigen (BCMA) CD3-targeted bispecific antibody for patients with relapsed or refractory multiple myeloma.

• **Maintenance Therapy:** For newly diagnosed patients who are not candidates for stem cell transplant, recent research has shown that other drugs may be good options for maintenance therapy. Several ongoing maintenance therapy clinical trials are evaluating the efficacy of single and combination therapies.
OTHER CLINICAL TRIALS THAT MIGHT BE RIGHT FOR YOU

MyDRUG Trial:

The ongoing Myeloma Developing Regimens Using Genomics (MyDRUG) trial utilizes genomic profiling in identification of common genetic mutations and fluorescence in situ hybridization (FISH) to recognize common translocations.

Myeloma patients are then assigned to a specific arm of the study based on their results.

The study’s goal is to identify patients who have high-risk myeloma in order to administer treatment early in their disease course.
INSIGHT MM (Multiple Myeloma) Study:

This INSIGHT MM study aims to evaluate the efficacy of treatments for myeloma patients worldwide.

The study design involves the collection of data from over 4,000 patients with newly diagnosed or relapsed or refractory myeloma in 15 countries for a minimum of 5 years.

Patients submit the data directly and include issues such as the use of healthcare resources and quality of life. Though new patients are not being enrolled in the study, new data derived from the study are published regularly.

More information can be found at ClinicalTrials.gov (identification code NCT02761187).
OTHER CLINICAL TRIALS THAT MIGHT BE RIGHT FOR YOU

The PROMISE Study: Screening Individuals at High Risk of Myeloma

This PROMISE study identifies, screens, and tracks individuals who have a high risk of developing multiple myeloma.

The goal of the PROMISE study is to increase early detection of myeloma precursor conditions in order to develop new therapies to improve survival and to prevent disease progression. The study is seeking individuals from ages 45 to 75 who are African American, and/or individuals with a first-degree relative with a plasma cell disorder such as myeloma. All participation is either online or by mail.

To learn more, call, email, or visit the study’s website.
• Call: 617-582-7002
• Email: promisestudy@partners.org
• Website: promisestudy.org

(Source: https://www.lls.org/myeloma/treatment/clinical-trials)
Preparing for harvest - In Phase III trials, the study drug or treatment is given to large groups of people (1,000-3,000) to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, & collect information that will allow the drug or treatment to be used safely.

Planting the seed - In Phase I trials, researchers test a new drug or treatment in a small group of people (20-80) for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.

Laying down roots - In Phase II trials, the study drug or treatment is given to a larger group of people (100-300) to see if it is effective and to further evaluate its safety.

Preparing for harvest - In Phase III trials, the study drug or treatment is given to large groups of people (1,000-3,000) to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, & collect information that will allow the drug or treatment to be used safely.

Expanding the yield - In Phase IV trials, studies look at real-world experience over a long time and provide additional information on the drug’s risks, benefits, and optimal use.
At the age of 34, Thomas was diagnosed with multiple myeloma. After multiple treatments, including an autologous stem cell transplant, Thomas shares why it’s critical to increase participation in clinical trials so we can understand why multiple myeloma poses a greater risk to certain populations.

In Thomas' own words, “Participating in clinical trials is contributing to research for tomorrow's medicines, and an opportunity to make a difference for myself and for others facing multiple myeloma.”
My name is Thomas, and this is my multiple myeloma story.

Diagnosis
At age 34, I was healthy and enjoying life. I went to the gym daily, and when I wasn’t working out, I was shooting hoops with friends.

During a workout, I suddenly felt excruciating pain in my shoulder. My family physician declared I had bursitis, but I was so young and in such good physical shape, I knew by instinct that the diagnosis was wrong. I made an appointment with a sports medicine doctor, who ran CT and MRI scans of my upper body. The result wasn’t good. I was diagnosed with multiple myeloma, a cancerous tumor of plasma cells in my scapula.

Because the myeloma was localized to my shoulder, my oncologist recommended 6 weeks of radiation therapy. 10 months after the treatment, the agonizing pain returned, but this time in my lower back. The pain was so intense, I remember lying on the floor, unable to move. My myeloma has spread to my lower backbone and ribs. This time, I received an autologous stem cell transplant, but after 2 months, I relapsed again.

“I was diagnosed with multiple myeloma, a cancerous tumor of plasma cells in my scapula.”
Participating in Clinical Trials

Still determined, I agreed to participate in a clinical trial to receive an allogeneic stem cell transplant using human leukocyte antigen (HLA)-matched donor cells from my brother Earnest. This time, I lived cancer-free for two years before relapsing.

To keep the cancer from progressing, I joined another clinical trial to receive a second allogeneic transplant using my brother’s donor cells. This aggressive treatment also included an intensive conditioning regimen of high-dose chemotherapy plus total-body irradiation. Although the therapeutic effects were serious and kept me hospitalized for 127 days, the transplant was successful and pushed back my cancer for another two years.

The Road Ahead

Since then, my cancer has relapsed multiple times, but I refuse to accept defeat. Although my cancer is unlikely to be curable, my current treatment has been successful at keeping the myeloma from advancing. I’m hopeful that I can live a long life while treating it as a chronic disease.

It’s critical we increase the participation of Black people in clinical trials so we can understand why multiple myeloma poses a greater risk for us, and get closer to a cure.

Participating in clinical trials is contributing to research for tomorrow’s medicines, and an opportunity to make a difference for myself and for others facing multiple myeloma.

Know all of the facts about clinical trials before choosing whether or not to participate in one.

Ask your doctor about ongoing clinical trials and if you might be a suitable candidate.
Multiple myeloma patient Danielle was a very active person – and even went on vacation – right before receiving her diagnosis. Her myeloma journey unfolded with her myeloma symptoms, diagnosis, treatment, and participation in a life-altering clinical trial. “I decided to do the study trial because I also wanted to help individuals. If it wasn’t going to help me, then my data that they collect from the study trial will definitely help the scientists, researchers, the doctors. It would help them try to find a cure.”
Are multiple myeloma clinical trials becoming more critical for African, Hispanic, Asian, and other racial and ethnic groups as U.S. demographics change?

Watch as Dr. Sikander Ailawadhi from the Mayo Clinic explains the impact of low trial participation rates by African Americans and his experience with some of his myeloma patients.
At what point should a clinical trial be an option for myeloma treatment? Dr. Joshua Richter shares his perspective on the appropriate time to weigh clinical trial participation and the potential benefits.

Dr. Joshua Richter is director of Multiple Myeloma at the Blavatnik Family – Chelsea Medical Center at Mount Sinai. He also serves as Assistant Professor of Medicine in The Tisch Cancer Institute, Division of Hematology and Medical Oncology.
How has the COVID-19 pandemic changed multiple myeloma clinical trials, and how can telemedicine play a role in trials?

Dr. Sarah Holstein shares her perspective on how trials were altered and her suggestions for improvements in trials.
Myeloma patients who are African American and Hispanic typically get to the right treatment much later. In a lot of cases they may not get to the right treatment at all.

In this replay, a panel of distinguished myeloma experts and patient advocates tackle how we achieve health equity for multiple myeloma patients no matter geography.
Dr. Irene Ghobrial, a myeloma specialist and researcher, dispels common myths associated with clinical trials, including a review of each phase of the clinical trial process.

Dr. Ghobrial specializes in multiple myeloma (MM) and Waldenström macroglobulinemia (WM), focusing on the precursor conditions of monoclonal gammopathy of undetermined significance (MGUS) and smoldering myeloma.
Clinical Trial Resources

Clinicaltrials.gov
Provides information on publicly and privately supported clinical studies on a wide range of diseases and conditions. The website is maintained by the National Library of Medicine at the National Institutes of Health.

Each clinical trial record presents summary information about a study protocol and includes the following:

- Disease or condition
- Intervention (for example, the medical product, behavior, or procedure being studied)
- Title, description, and design of the study
- Requirements for participation (eligibility criteria)
- Locations where the study is being conducted
- Contact information for the study locations
Clinical Trial Finders
CenterWatch

Offers online tools to:
- Search clinical trials
- Receive email notifications about specific clinical trials
- Review results from completed clinical trials
- Search drug information
- Learn about the informed consent process
- Read an overview of the clinical trials process
- Find disease-specific health associations and other educational resources
- Review definitions of commonly-used clinical research terms
**NIH National Cancer Institute**

**Contact:** Call 1-800-422-6237, live chat through LiveHelp or email NCIinfo@nih.gov

Search portal to find National Cancer Institute (NCI)-supported clinical trials. Search by cancer type or keyword, your age (to determine which trials you could be eligible for) or U.S. ZIP code

**Contact:** 1-800-887-0639; email info@clinicalconnection.com

Founded by a team of medical researchers whose goal has been to efficiently connect patients with clinical trial opportunities that are relevant and timely. Options to create a free member account to be notified when clinical trials that match your health interests become available in your area.

Search for trials (both in U.S. and internally) by ZIP code, keyword, or distance (select distance ranges starting from within 5 miles up to over 250 miles)
CISCRP
(Center for Information and Study on Clinical Research Participation)
Contact: 877-MED-HERO (633-4376) or info@ciscrp.org.

Provides education and information about clinical trials. Search Clinical Trials is a free service designed to help people find clinical trials that are relevant to their needs.

CISCRP staff will work with you to understand your options and will help you find local clinical trials in your community, or as far as you would be comfortable traveling.

Antidote
1-888-509-1308 (US) or +44 808-196-0665 (UK)
Email: hello@antidote.me

Search for clinical trials by condition, city or ZIP code, age, and gender. Receive list of clinical trials that could be a match for you by answering series of questions. Watch educational webinars and patient stories.
Susan Lang Pay-It-Forward Patient Travel Assistance Program
Contact Us: (877) 557-2672 | FinancialAssistance@LLS.org

The fund provides financial assistance for care-related travel to patients diagnosed with a blood cancer, like multiple myeloma, who have significant financial needs.

For more information, visit: https://www.lls.org/support-resources/financial-support/susan-lang-pay-it-forward-patient-travel-assistance-program

Lazarex Cancer Foundation
Contact: 877-866-9523 or 925-820-4517
Other language(s): Spanish, Mandarin, Korean

Helps cancer patients navigate clinical trial options by offering financial assistance (such as lodging and transportation costs) for participation in FDA-approved clinical trials; call for eligibility details. Also provides community outreach and education.
Clinical Trial Finders

21st Century C.A.R.E.

Get immediate financial assistance for incidental expenses related to active cancer treatments. Must be referred by a physician to be considered for assistance.

Applications are processed without delay. Once the application is approved, then you are eligible for financial assistance for incidental expenses related to: transportation to and from treatments, follow-up visits related to cancer-care, childcare during treatment, temporary housing due to geographical distance from the treatment center, medical supplies, and much more.

Medicare and the National Cancer Institute provides information on Medicare coverage for clinical trials. Contact: 1–800–633–4227 or 1–877–486–2048 for hearing impaired

CenterWatch offers online tools to:

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- Receive email notifications about specific clinical trials
- Review results from completed clinical trials
- Search drug information
- Learn about the informed consent process
- Read an overview of the clinical trials process
- Find disease-specific health associations and other educational resources
When it comes to cancer treatment, you or a loved one may be considering participating in a clinical trial as a treatment option. Clinical trials are designed to evaluate the safety and effectiveness of a treatment. They may involve researchers administering drugs, taking blood or tissue samples, or checking the progress of patients as they take a treatment, according to a study’s protocol.

Learning about clinical trials can be a steep learning curve – not the least because the process comes with a lot of new terms, acronyms, and jargon. To help you, we’ve put together this list of the most common terms you will find when you are researching clinical trial information. This is not an exhaustive list, but it is a helpful starting point. At the end of this article, you will see links to find more information.
ADVERSE EFFECTS (AE)
Also called Adverse Events, or Adverse Drug Reaction, AEs are any harmful event experienced by a person while they are having a drug or any other treatment or intervention. In clinical trials, researchers must always report adverse events, regardless of whether or not the event is suspected to be related to or caused by the drug, treatment, or intervention.

ARM
Subsection of people within a study who have a particular intervention.
BIAS

Bias is an error that distorts the objectivity of a study. It can arise if a researcher doesn’t adhere to rigorous standards in designing the study, selecting the subjects, administering the treatments, analyzing the data, or reporting and interpreting the study results. It can also result from circumstances beyond a researcher’s control, as when there is an uneven distribution of some characteristic between groups as a result of randomization.

BLINDING

Blinding is a method of controlling for bias in a study by ensuring that those involved are unable to tell if they are in an intervention or control group, so they cannot influence the results. In a single-blind study, patients do not know whether they are receiving the active drug or a placebo. In a double-blind study, neither the patients nor the persons administering the treatments know which patients are receiving the active drug.
**COMPARATOR**
When a treatment for a specific medical condition already exists, it would be unethical to do a randomized controlled trial that would require some participants to be given an ineffective substitute. In this case, new treatments are tested against the best existing treatment, (i.e., a comparator). The comparator can also be no intervention (for example, best supportive care).

**COMPLETED**
A trial is considered completed when trial participants are no longer being examined or treated (i.e., no longer in follow-up); the database has been "locked" and records have been archived.

**CONTROL**
A group of people in a study who do not have the intervention or test being studied. Instead, they may have the standard intervention (sometimes called "usual care") or a dummy intervention (placebo). The results for the control group are compared with those for a group having the intervention being tested. The aim is to check for any differences. The people in the control group should be as similar as possible to those in the intervention group, to make it as easy as possible to detect any effects due to the intervention.
EFFICACY

How beneficial a treatment is under ideal conditions (for example, in a laboratory), compared with doing nothing or opting for another type of care.

A drug passes efficacy trials if it is effective at the dose tested and against the illness for which it is prescribed.

ELIGIBILITY CRITERIA/INCLUSION AND EXCLUSION CRITERIA

Eligibility criteria ensures patients enrolling in a clinical trial share similar characteristics (e.g., gender, age, medications, disease type, and status) so that the results of the study are more likely due to the treatment received rather than other factors.

FOLLOW-UP

Observation over a period of time of participants enrolled in a trial to observe changes in health status.
INFORMED CONSENT

A process (by means of a written informed consent form) by which a participant voluntarily agrees to take part in a trial, having been informed of the possible benefits, risks and side effects associated with participating in the study.

INTERVENTION

The treatment (e.g., a drug, surgical procedure, or diagnostic test) being researched. The intervention group consists of the study participants that have been randomly assigned to receive the treatment.

INVESTIGATOR

A person responsible for the conduct of the clinical trial at a trial site. If a trial is conducted by a team of individuals at a trial site, the investigator is the responsible leader of the team and may be called the principal investigator (PI).

MULTICENTER TRIAL

A clinical trial conducted according to a single protocol but at more than one site, and therefore, carried out by more than one investigator.
NUMBER NEEDED TO TREAT (NNT)
The average number of patients who need to receive the treatment or other intervention for one of them to get the positive outcome in the time specified.

OUTCOME MEASURES
The impact that a test, treatment, or other intervention has on a person, group, or population.

PLACEBO
A fake (or dummy) treatment given to patients in the control group of a clinical trial. Placebos are indistinguishable from the actual treatment and used so that the subjects in the control group are unable to tell who is receiving the active drug or treatment. Using placebos prevents bias in judging the effects of the medical intervention being tested.

POPULATION
A group of people with a common link, such as the same medical condition or living in the same area or sharing the same characteristics. The population for a clinical trial is all the people the test or treatment is designed to help.
PROTOCOL
A plan or set of steps that defines how something will be done. Before carrying out a research study, for example, the research protocol sets out what question is to be answered and how information will be collected and analyzed.

Randomized Controlled Trial (RCT)
A study in which a number of similar people are randomly assigned to two (or more) groups to test a specific drug, treatment, or other intervention. One group has the intervention being tested; the other (the comparison or control group) has an alternative intervention, a placebo, or no intervention at all.

Participants are assigned to different groups without taking any similarities or differences between them into account. For example, it could involve using a computer-generated random sequence. RCTs are considered the most unbiased way of assessing the outcome of an intervention because each individual has the same chance of having the intervention.
RELIABILITY
The ability to get the same or similar result each time a study is repeated with a different population or group.

SAMPLE
People in a study recruited from part of the study’s target population. If they are recruited in an unbiased way, the results from the sample can be generalized to the target population as a whole.

SUBJECTS
In clinical trials, the people selected to take part are called subjects. The term applies to both those participants receiving the treatment being investigated and to those receiving a placebo or alternate treatment.

TRIAL SITE
The location where trial-related activities are conducted.