

Aplastic Anemia and MDS International Foundation  
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*"Clinical Trials from A to Z: Understanding the Basics"*

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## What is a clinical trial?

- Clinical trials are research studies that involve people.
- Clinical trials versus observational studies
- Clinical trials are part of an extensive process that starts with research in a lab.
- Many of today's routine treatments are the results of past clinical trials.

<http://www.cancer.gov/about-cancer/treatment/clinical-trials/what-are-trials>

## What are Clinical Trials?

### What Are Clinical Trials

[www.Cancer.net](http://www.Cancer.net)

## Common Facts about Clinical Trials

- Taking part in any clinical trial is voluntary
- Not all clinical trials study treatments
- Not all clinical trials that do study treatments, study drugs
- When clinical trials do look at drugs, not all of them study new ones
- Very few cancer clinical trials use placebo

<http://www.cancer.org/treatment/treatmentsandsideeffects/clinicaltrials/whatyouneedtoknowaboutclinicaltrials/clinical-trials-what-you-need-to-know-facts-about-clin-trials>

## What are the types of clinical trials?

- Prevention trials
- Screening trials
- Diagnostic trials
- Treatment trials
- Quality of life trials
- Data Banks or Registries

## What are prevention trials?

- Prevention trials often look at ways to lower chances of getting cancer or help doctors learn more about preventing cancer.
- Large groups of people
- May be asked to do or take something
  - Exercise or diet
  - Medication or vitamin
- Comparison of groups

<http://www.cancer.gov/publications/patient-education/ctes-prevbrochenglishapp1.pdf>

## What are Screening Trials?

- Studies that look at whether certain screening tests are useful for early detection, diagnosis, and increased survival related to certain health conditions.
- Downsides to screening
  - Over-diagnosis
  - Overtreatment

<http://www.cancer.gov/research/areas/screening>

## What are Diagnostic Trials?

- Studies that look at certain tools that help doctors diagnose health conditions
  - Lab tests
  - Imaging studies (x-rays, CT scans, MRI)
  - Genomic analysis (looking at the DNA of cells)
- How can diagnostic trials help patients?
  - Risk of developing a health condition
  - Help determine the stage of disease
  - Classify groups of patients with similar genetic changes
  - Inform decisions about treatment
  - Assess response to treatment

<http://www.cancer.gov/research/areas/diagnosis>

## What are Treatment Trials?

- Treatment trials test new treatments or new ways of using existing treatments
  - Drugs, vaccines, approaches to surgery or radiation therapy, or combinations of treatments

<http://www.cancer.gov/about-cancer/treatment/clinical-trials/what-are-trials/types>

## What are Quality of Life Trials?

- These studies look at how your treatment or illness affects you.
- Many treatment studies include quality of life as part of the bigger trial.
- Quality of life studies typically use questionnaires and diaries to find out the answers to the research question.

<http://www.cancerresearchuk.org/about-cancer/finding-a-clinical-trial/what-you-should-be-told/about-a-clinical-trial/quality-of-life-studies>

## Questionnaires

- How are you managing to go to work?
- Do you feel more or less tired than usual?
- Do you feel more or less anxious or depressed?
- What types of side effects are you having?
- How do you feel in general?
- How are your personal relationships?

## What are the benefits of improving Quality of Life?

- Patients are more likely to continue with a treatment that has fewer side effects or if they have to go to the hospital/clinic less often.
- Inform the health care team of the impact of the health condition and its treatments.
- Can foster patient-provider communication, identify overlooked problems, and evaluate the impact of palliative care.

<http://www.cancernetwork.com/review-article/assessing-quality-life-research-and-clinical-practice>

## What is a bank or registry?

- A data bank or registry is an organized collection of information about individuals that is usually focused on a specific health condition or diagnosis.
- Can be composed of personal health information and/or tissue samples.
- Researchers can use this information to study trends in disease, risk of disease, gain insight to possible genetic and environmental risks, and increase general understanding of the disease or condition.

## Why participate in banks or registries?

- Likely to help researchers and other health care professionals increase their knowledge about a specific condition, help improve treatment, and help develop tests or new treatments.
- Is not intended to benefit the individual participant, but to help general knowledge.

## Types of Clinical Trials



[www.Cancer.net](http://www.Cancer.net)

## Clinical Research vs. Medical Treatment

- Clinical Research refers to studies in which people participate as patients or volunteers.
  - Clinical studies
  - Clinical trials
  - Studies
  - Research
  - Trials
  - Protocols

<http://www.fda.gov/ForPatients/ClinicalTrials/ClinicalvsMedical/default.htm>

## Clinical Research vs. Medical Treatment

- Clinical Research Goals:
  - Developing new treatments or medications
  - Identifying causes of illness
  - Studying trends
  - Evaluating ways in which genetics may be related to an illness or health condition

<http://www.fda.gov/ForPatients/ClinicalTrials/ClinicalvsMedical/default.htm>

## Clinical Research vs. Medical Treatment

- Clinical Research:
  - Strict rules by the National Institutes of Health (NIH) and FDA
  - May offer benefit to individual
  - May not offer benefit, but help scientist learn better ways to help people
  - Confidentiality is important
  - Results are not presented on an individual basis

## Clinical Research vs. Medical Treatment

	Clinical Research	Medical Treatment
Intent	Answers specific questions through research involving numerous research volunteers.	Addresses the needs of the individual patient.
Intended Benefit	Generally designed and intended to benefit future patients.	Intended to benefit the individual patient.
Funding	Paid by drug developers and Government agencies.	Funded by individual patients and their health plans.
Timeframe	Depends on protocols.	Requires real-time decisions

<http://www.fda.gov/ForPatients/ClinicalTrials/ClinicalvsMedical/default.htm>

## Clinical Research vs. Medical Treatment

	Clinical Research	Medical Treatment
Consent	Requires written informed consent.	May or may not require informed consent.
Assessment	Involves periodic and systematic assessment of patient data.	Based on as-needed patient assessment.
Protections	Protected by government agencies, institutional review boards, professional standards, informed consent, and legal standards.	Guided by state boards of medical practice, professional standards, peer review, informed consent, and legal standards.

<http://www.fda.gov/ForPatients/ClinicalTrials/ClinicalvsMedical/default.htm>

## Clinical Research vs. Medical Treatment

	Clinical Research	Medical Treatment
Certainty	Tests products and procedures of unproven benefit to the patient.	Uses products and procedures accepted by the medical community as safe and effective.
Access to Information	Considered confidential intellectual property.	Available to the general public through product labeling.
Release of Findings	Published in medical journals, after clinical research ends.	Individual medical records are not released to the general public.

<http://www.fda.gov/ForPatients/ClinicalTrials/ClinicalvsMedical/default.htm>

## What is informed consent?

- Federal law requires research institutions to get informed consent to participate from each person
- Consent is a process
- Not just a document
- Right of self-decision

## Who reviews and approves clinical trials?

- Institutional Review Board (IRB)
  - Independent Ethics Committee
  - Research Ethics Board
- Designated group that reviews and monitors biomedical research involving human subjects.
- Authority
  - Approve, require changes, or disapprove research
- Protection of the rights and welfare of human research subjects



### Phases of Clinical Trials

Preclinical  
Phase 1  
Phase 2  
Phase 3  
Phase 4

## Preclinical Research

- Research hypothesis
- Begins before clinical trials
- In Vitro research
- In Vivo research
- Detailed information

<http://www.cancer.net/navigating-cancer-care/how-cancer-treated/clinical-trials/phases-clinical-trials> and <http://www.fda.gov/ForPatients/Approvals/Drugs/ucm405658.htm>

## Phase 1 Clinical Trials

- First time the drug is used in people
- Small scale
- Dosing
- Safety
- Pharmacokinetics/Pharmacodynamics
- Who is offered participation in these studies?
- How many drugs move to the next phase?

<http://www.cancer.net/navigating-cancer-care/how-cancer-treated/clinical-trials/phases-clinical-trials> and <http://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm>

## Phase 2 Clinical Trials

- Medium scale
- Side effects
- Efficacy
- How many drugs move to the next phase?

<http://www.cancer.net/navigating-cancer-care/how-cancer-treated/clinical-trials/phases-clinical-trials> and <http://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm>

## Phase 3 Clinical Trials

- Large scale
- Compare to standard treatment
- Efficacy
- Side effects
- FDA approval
- How many drugs move to the next phase?

<http://www.cancer.net/navigating-cancer-care/how-cancer-treated/clinical-trials/phases-clinical-trials> and <http://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm>

## Phase 4 Clinical Trials

- Large scale
- Safety
- Efficacy

<http://www.cancer.net/research-and-advocacy/introduction-cancer-research/drug-discovery-and-development> and <http://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm>

## Who pays for clinical trials?

- Before patients agree to participate in a clinical trial, they should confirm whether they will be responsible for any study-related costs.
- Patient care costs (also referred to as routine care costs) are those costs related to treating your cancer, whether you are in a trial or receiving routine therapy. These costs are often covered by health insurance.
- Research costs are those related to taking part in the trial. Often these costs are not covered by health insurance, but they may be covered by the trial's sponsor.
- When patients takes part in a trial, they may have extra doctor visits that they may not have had with routine treatment

Study of Azacitidine in Combination with Drug X or Placebo in Subjects with Intermediate-1, Intermediate-2 or High-Risk MDS

#### Routine Care

- Azacitidine (and administration)
- Blood count checks
- Physical Exams
- Clinic Visits
- Administration of Drug X
- Bone Marrow tests
- Blood/Platelet transfusions

#### Research Procedures

- Drug X
- Special blood tests to see how Drug X works
- Extra visits to the clinic
- Extra bone marrow tests for research only

## Patient Protection and Affordable Care Act

- Effective: January 1, 2014
- What impact did it have on clinical trials?
  - Private insurers are required to cover the routine patient costs associated with clinical trials.

## What clinical trials are covered?

- Phase I, II, III, and IV trials meet the definition of an approved clinical trial if it is conducted for prevention, detection, or treatment of cancer or another disease or condition likely to lead to death unless the course of the disease is interrupted, AND
  - Federally funded OR
  - Conducted under an investigational new drug (IND) application or is exempt from the IND requirements

## Key Provisions

- “Routine patient care costs” means the costs of health care services, including drugs, items, devices, and services that would be covered under the patient’s health plan if they were provided outside of a cancer clinical trial.
- Not required to cover “research costs” such as the investigational drug or treatment, any procedure done only to collect data for the study, and anything that is no related to the routine treatment for the condition or disease being studied.

## Key Provisions

- **“Routine patient care costs” excludes the costs of:**
  - Drugs or devices not approved by the FDA for use associated with the cancer clinical trial;
  - Nonclinical expenses, such as travel, housing, and companion expenses;
  - Items or services provided solely for data collection and analysis purposes and not used in the patient’s clinical management;
  - Health care services that would otherwise be excluded from coverage under the patient’s health plan;
  - Health care services customarily provided free of charge to trial enrollees by the trial sponsors; and
  - The cost of an oncologic drug, if the trial’s purpose is to study the use of the drug in the particular cancer in question or to study the administration of the drug in a new manner.

## Benefits to Participating

- Access to a new treatment that is not available to people outside the trial.
- Close monitoring by the research team.
- If the treatment being studied is more effective than the standard treatment, the patient may be among the first to benefit.
- The trial may help scientists learn more about your health condition and help people in the future.

## Risks to Participating

- The new treatment may not be better than, or even as good as, the standard treatment.
- New treatments may have side effects that doctors do not expect or that are worse than those of the standard treatment.
- There may be more visits to the doctor than if the patient was receiving routine treatment. There may be additional expenses related to these extra visits, such as travel and childcare costs.
- There may be extra tests. Some of the tests could be uncomfortable or time consuming.
- Even if a new treatment has benefits in some patients, it may not work for everyone.
- Health insurance may not cover all patient care costs in a trial.

## How are trials are reviewed for safety?



[www.Cancer.net](http://www.Cancer.net)

## Who Can Join?

- **Common eligibility criteria include:**
  - Having a certain type or stage of cancer
  - Having received (or not having received) a certain kind of therapy in the past
  - Being in a certain age group
  - Medical history
  - Current health status

## Questions to Ask Your Doctor about Treatment Clinical Trials

- **About this trial**
  - What is the purpose of the trial?
  - Why do the researchers believe that the treatment being studied may be better than the one being used now? Why may it not be better?
  - How long will I be in the trial?
  - What kinds of tests and treatments are involved?
  - How will the doctor know if the treatment is working?
  - How will I be told about the trial's results?
  - How long do I have to make up my mind about joining this trial?
  - Who can I speak with about questions I have during and after the trial?
  - Who will be in charge of my care?
  - Is there someone I can talk to who has been in the trial?

## Questions to Ask Your Doctor about Treatment Clinical Trials

- **Risks and Benefits**
  - What are the possible side effects or risks of the new treatment?
  - What are the possible benefits?
  - How do the possible risks and benefits of this trial compare to those of the standard treatment?
- **Rights**
  - How will my health information be kept private?
  - What happens if I decide to leave the trial?
- **Costs**
  - Will I have to pay for any of the treatments or tests?
  - What costs will my health insurance cover?
  - Who pays if I'm injured in the trial?
  - Who can help answer any questions from my insurance company?

## Questions to Ask Your Doctor about Treatment Clinical Trials

- **Daily life**
  - How could the trial affect my daily life?
  - How often will I have to come to the hospital or clinic?
  - Will I have to stay in the hospital during the clinical trial? If so, how often and for how long?
  - Will I have to travel long distances?
  - Will I have check-ups after the trial?
- **Comparing choices**
  - What are my other treatment choices, including standard treatments?
  - How does the treatment I would receive in this trial compare with the other treatment choices?
  - What will happen to my cancer without treatment?

## Helpful Links

- [www.aamds.org](http://www.aamds.org)
- [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov)
- [www.CancerTrialsHelp.org](http://www.CancerTrialsHelp.org)
- [www.Cancer.gov](http://www.Cancer.gov)
- [www.asco.org](http://www.asco.org)
- [www.cancer.net](http://www.cancer.net)
- [www.cms.gov](http://www.cms.gov)

## Thank You!



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