Clinical Trials

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Where Anti-Cancer Drugs Come from

Sources of Anti-Cancer Drugs, 1940–2014

- Natural Product or Derived Directly From It (49%)
- Inspired by or Mimicking a Natural Product, Vaccines, Biologics (25%)
- Synthetic (26%)

Clinical Trials Are an Important Part of Drug Development for Any Disease including Cancer...

The Drug Pipeline

What Is a Clinical Trial?

• Study that aims to improve existing treatments or to replace them with new and better ones

• Before a new treatment is made widely available for patients, it must be tested in clinical trials for efficacy (how well it works) and safety
Why Do We Need Clinical Trials?

• Clinical trials are designed to help us learn more about the **benefits** and **side effects** of a new treatment.

• A treatment’s benefit and side effects can only be fully assessed after long-term use on patients in everyday clinical practice.
What Do the Different Phases Mean?

- Phase I: Safety, finding the safest dose
  - Small group

- Phase II: Safety, finding if it works (efficacy)
  - Medium group

- Phase III: Confirming efficacy, side effects
  - Large group
  - Compare to “gold standard”
  - Usually randomized, may be “Single-” or “Double-” blind
Why would **YOU** want to be part of a Clinical Trial?

Everybody’s reason may be different!

1. **Benefits?** Access to new approaches
2. Helping others
3. Moving science forward
4. **Risks??** New approach may not be of benefit to you:
   - Initial studies are small
   - Possible drug side effects
Patient Safety on a Clinical Trial

- Informed Consent (You/family)
- Physician oversight
- Institutional oversight (Institutional Review Board, “IRB”)
- National (FDA) oversight

**Multiple Safety Levels**

**Multiple Caring Levels**
Before You Enroll

You must **qualify**
- Inclusion criteria
- Exclusion criteria

You will go through **informed consent**
- Study purpose and details
- Risk/benefits

Know your **rights**
- You can stop participating **at any time for any reason**

IRB normally carefully reviews the consent form in detail to make sure it explains everything in an “easy-to-get” format . . . but ask questions!!
What Will Happen on a Trial?

- Protocol-driven treatment (aka “study plan”)
- Frequent monitoring
- Additional support and coordination
Facts about Clinical Trials

• What will happen to my data? The informed consent form will inform you how your data will be shared. There are laws that require your data to be kept securely.

• What are the costs to me of participating? Information about who is responsible for certain costs will be outlined in the informed consent

  − Patient care costs: Costs related to treating your cancer, whether you are in a trial or receiving standard treatment. The participant is usually expected to pay for these costs, which are often covered by health insurance.

  − Research costs: Costs associated with the trial itself, such as research medications or extra tests which are not part of your usual medical care. These costs are usually paid by the trial sponsor.
Who Are the Key People in a Trial?

- Your Doctor
- Principal Investigator
- Study Coordinator
- Your Family
What Happens after the Trial?

• Data is checked and audited to make sure it is correct
• Data is analyzed by a statistician
• Results are presented and/or published
• Where can I see the study results?
  − You may receive the study results from the study Principal Investigator (the study informed consent form will tell you if you will receive results)
  − Study results are required to be published on ClinicalTrials.gov
Good Questions to Ask

• Are clinical trials an option for me? If so, what types of clinical trials am I eligible for?

• What are my other options?

• What are my rights as a clinical trial participant?
Clinical Trial Resources

• University of Illinois Cancer Center: https://cancer.uillinois.edu/patients-survivors/#aboutclinicaltrials
• Food & Drug Administration (FDA): https://www.fda.gov/patients/clinical-trials-what-patients-need-know
• ClinicalTrials.gov
Questions?

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