

Understanding Cancer Research Studies



"A new study finds that eating 9 vegetables daily prevents colon cancer." "Results of a clinical trial show tamoxifen is effective in preventing breast cancer in high-risk women." Do you wonder what a clinical trial or an epidemiological study is after hearing these headlines?

With more and more of these studies appearing in the news, you have to understand some basic lingo to make heads from tails. Here is a primer on health research so you can follow along and better understand health headlines.

Making the connection

Americans are living longer thanks to medical advances. But living longer may mean developing chronic diseases, such as heart disease, cancer, and diabetes. Researchers are constantly searching for answers on how to prevent and treat disease, and improve the quality of life for those with disease.

Theories on the causes of disease may start in a laboratory. Scientists study cells in test tubes (also called *in vitro*) or animals to test their theories. They can easily control the environment of the cell or animal being studied. This means that the scientist decides exactly what the cell or animal is exposed to, such as temperature, diet, or chemicals. If that produces an interesting finding, scientists may propose a human study. This is because what happens in an animal may not happen the same way in people.

Researchers can also create theories about causes of disease by looking at large populations of people. For example, they may wonder why American women have higher rates of breast cancer than Japanese women. Researchers may compare the women's diets, amount of activity, or stress levels to find a reason.

Unfortunately, "people studies" are complicated. They are expensive, often take a long time, and present many factors not seen in a lab. There is the type of environment to consider (urban, rural), lifestyle factors (diet, amount of physical activity), psychological influences, and family genes. And the list goes on.

But research studies have helped to reduce many health risks. For instance, researchers have found that a diet lacking in certain vitamins and minerals causes specific diseases. They have discovered the link between smoking and lung cancer. Many infectious diseases, such as chickenpox and measles, are now preventable with vaccines.

Types of human studies

Epidemiological or population studies

Epidemiological or population studies look at a large group of people in a specific region. Researchers will observe a disease that is present and study why people have it. These are sometimes called observation studies. They find links between an exposure and a disease. Here are three common types of these studies:

Cross-sectional

Cross-sectional studies are useful in seeing how common a disease is in a certain region. Researchers may start with a cross-sectional study to decide if another type of population study is needed later on.

A group of people is gathered to observe certain information at one point in time. For example, researchers may observe 1,000 people in the state of California to see who has lung cancer on June 1, 2015. These studies are inexpensive and quick. But since they are like instant "snapshots" of people, they can't explain what led up to some people getting lung cancer.

Case-control

These studies are like looking at people's diaries. They gather two groups. In one group, the cases have the disease being studied. In the other group, or control group, they do not have the disease, but it is very similar to the cases in other ways. For example, participants may be the same age, live in the same area, or have other similar lifestyle habits.

Researchers then look back into the past, usually relying on the participants' memories, to see any differences between the cases and controls. The researchers look at a specific exposure or potential link to the disease. Did the two groups eat differently? Were they exposed to certain chemicals? If an exposure stands out in only the cases or only the controls, that exposure could be linked to the disease.

Cohort

This type of study looks ahead. Researchers choose an exposure and the disease (or condition) expected to happen because of the exposure.

A large group of people, called a cohort, is observed by researchers over a period of time to see who develops the disease and who does not. They could be followed for any length of time, such as 5 years or more than 20 years.

Clinical trials

Clinical trials are another type of human study. Clinical trials usually, but do not always, look at treatments.

Cancer clinical trials work with a group of people who have something in common (for example, a type of cancer or risk factor). Researchers decide on an agent or intervention for the group that may affect the disease or problem being studied. Examples of agents are a new medicine or high-dose vitamin.

You may hear the term placebo-controlled. This is when half the group is given an agent or intervention and the other half a placebo. A placebo is a fake or "dummy" intervention—something that looks like the agent but has no effect at all. A placebo is usually not used when there is already an effective treatment for the condition. With cancer, an agent or intervention is usually compared to a standard cancer treatment. Sometimes cancer prevention trials use placebos, such as looking at the effects of a high-dose nutrition supplement on the prevention of cancer. One group of people would get the supplement and another group the placebo.

If two groups are used (receiving either an agent or a placebo), it is important that the trial is fair and the groups are as equal as possible. So the scientists may "blind" the participants (single-blind) or even themselves (double-blind) so that no one knows who is getting the placebo or the agent. They may also randomize the participants in the beginning. This means they will use an unbiased system, often a computer, to randomly select who will get the agent being tested.

New cancer treatments develop in clinical trials through these phases:

- Phase I gives a small number of people a new treatment or medicine, observes side effects, and finds the best dose and way to administer the agent. People who are very sick and not responding to other treatments may enroll here.
- Phase II looks at how well the new treatment works on a specific condition or disease using a specific dose. It continues to observe side effects. People who choose to enroll here also may have used conventional treatments that no longer worked for them.
- Phase III uses a larger number of people. It compares the effectiveness of the new treatment or a combination of treatments to an existing or standard treatment to find out which one may be better.
- Phase IV trials evaluate medicines that have already been proven to be effective in Phase III trials. Phase IV trials focus on long-term safety and cost.

In addition to these four phases, the American Cancer Society identifies a Phase 0 for **drug trials**. Phase 0 clinical trials explore how a new medicine may work. Phase 0 information is included because more cancer volunteers may be asked to take part in these studies in the future. The goal of this phase is to speed up the approval of a new medicine.

- Humans are used in this trial, but Phase 0 is not like the other clinical trial phases.

- The biggest difference between Phase 0 and the other phases is that the patient has no chance of benefiting from this trial. People in the future are the ones who will benefit from Phase 0 research.
- Phase 0 trials are exploratory, and the medicine is given in very small doses and for a short period of time.
- Researchers look for medicine absorption, whether the medicine reaches the tumor, how the cancer cells respond, and if there are any adverse medicine actions within the body.

Phase 0 trials are very small (often with fewer than 15 people) and are not widely used. Phase 0 research is not a requirement for testing a new medicine.

Clinical trials for new cancer treatments are always emerging. Talk with your healthcare provider about whether you may benefit from a clinical trial.

Visit the [National Cancer Institute's Cancer Trials website](#) to find specific clinical trials. Or call the National Cancer Institute's Cancer Information Service at 800-422-6237 for more general information about clinical trials. You can also learn more about trials on the [www.clinicaltrials.gov website](http://www.clinicaltrials.gov), a service of the National Institutes of Health.

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