Understanding Cancer Research Study Design and How to Evaluate Results [1]

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Key Messages:

- Researchers design medical studies in different ways depending on the question they want to answer and the best way to answer it.
- The two main types of cancer research studies are experimental studies and observational studies. Researchers may also summarize and evaluate the available published research on a particular topic to draw conclusions.
- Experimental studies are generally considered more reliable than observational studies, although there are other factors to consider when evaluating research study results.
- Always talk with your doctor or another member of your health care team about the information you find in an abstract or study.

Doctors and scientists conduct research studies to discover more about the biology of cancer, investigate new treatments and diagnostic tests, and learn how to prevent the disease. Depending on the questions they want to answer, researchers can design these studies in a number of ways. No study design is perfect; each has strengths and drawbacks. Therefore, it is important to understand a study's design so you can evaluate the results and know if they apply to your situation.

In cancer research, there are two main types of research studies:

Experimental studies. As part of an experimental study, researchers provide an intervention (such as a treatment) to a group of individuals and compare their results to those of another group that does not receive the intervention (known as the control group). The researchers decide who receives the intervention and who does not either randomly (for reasons explained below) or through intentional selection. Experimental studies can investigate treatments, correlative science (testing whether specific genes or proteins affect the development or spread of cancer), new imaging techniques, and quality of life issues.

Observational studies. During an observational study, the researchers observe groups in which the intervention that each person receives is not controlled by the researchers. Observational

studies tend to be epidemiologic (relating to how various risk factors cause or affect the development of a disease in a population).

Types of experimental studies

Experimental studies are generally considered more reliable than observational studies because, in most cases, the volunteers who take part in an experimental study are randomly assigned to the intervention or control group, reducing the chance of having the researchers? or the subjects? biases (assumptions or preferences) affect the study results. In addition, with an experimental study design, the researchers can better identify and control unrelated factors, such as age, sex, and weight, that could influence or affect the results of the study. Researchers may also consider other factors, such as cancer type, stage of the disease, and status of the disease (whether the person is newly diagnosed or the cancer has started to grow and/or spread), when deciding who can participate in an experimental study.

One of the most common types of experimental studies is the clinical trial [3]. A clinical trial is a medical research study performed with people that tests the safety or effectiveness of a new drug, combination of existing treatments, approach to radiation therapy or surgery, or method of cancer prevention.

Doctors and researchers conduct clinical research in segments called phases. Each phase of a clinical trial is designed to provide different information about the new treatment, such as the dose, safety, and efficacy (how well it works). The phases are described as I, II, and III. Learn more about the phases of clinical trials [4].

Depending on experimental factors, researchers can keep clinical trial study participants and/or themselves from knowing whether specific study participants belong to the intervention or control group, eliminating subjective bias. This is a process known as ?blinding.?

Types of experimental studies include:

Double-blind randomized trial. This type of clinical trial is considered by most scientists to produce the best evidence because neither the study participants nor the researchers know who belongs to the intervention or control group until the study ends.

Single-blind randomized trial. In this type of trial, the participants do not know whether they belong to an intervention or control group, but the researchers do.

Open/unblinded trial. In this type of trial, both the participants and the researchers know who belongs to each test group within the study. This usually occurs when it isn't possible to use blinding; for example, if the study was comparing a surgical treatment to a medication.

Types of observational studies

In observational studies, researchers have less control over the characteristics of the study participants, which means certain factors could unintentionally affect the results. These studies, however, are useful in providing preliminary evidence that can help guide future experiments.

Types of observational studies include:

Case-control studies. These observational studies compare two groups of people, such as those who have cancer (the case) and those who do not (the control). For example, researchers may look for lifestyle or genetic differences between the two groups that may explain why one group developed cancer and the other did not. These studies are done retrospectively, meaning that the circumstances they are evaluating have already happened.

Cohort studies. These studies are prospective, which means that the event is studied as it occurs. The researchers monitor a group of people for a long time and track, for example, any new cases of cancer. This approach is often used to study whether certain nutrients or behaviors can prevent cancer. In addition, this approach can be used to identify cancer risk factors, such as the link between the use of postmenopausal hormone replacement therapy and an increased risk of breast cancer.

Case reports and case series. These studies are compilations of detailed descriptions of a patient's diagnosis and treatment history. These individual patient descriptions are called case reports. If a number of patients are given a similar treatment, the case reports may be combined into a case series. The results and conclusions of case series studies are descriptions of patients' experiences within a specific population and should not be used to determine treatment options.

Cross-sectional studies. These studies examine the relationship between diseases and other factors (such as exposures or behaviors) within a specific population at one point in time. However, because these studies only measure circumstances at one point in time, they cannot absolutely prove that something causes cancer.

Types of review articles

With the huge number of cancer research studies being published every year, it is challenging, if not impossible, for doctors and researchers, as well as interested patients and caregivers, to keep up with the latest advances. Also, since the research studies published in journals are constantly shaping and reshaping the scientific understanding of that subject, no single study is ever considered the final word on a particular topic, cancer type, or treatment. As a result, review articles, which evaluate and summarize the findings of all the available published research on a particular topic, can be extremely helpful.

Types of review articles include:

Systematic reviews. These articles summarize the best available research on a specific topic. The researchers use an organized method to locate, gather, and evaluate a number of research studies on a particular topic. By combining the findings of a number of studies, the researchers are able to draw more reliable conclusions.

Meta-analyses. These studies combine the statistical data collected in several research studies on the same topic. By combining these data, a meta-analysis has the ability to find trends that may not be seen in smaller studies. However, if the individual studies were poorly done, the results of a meta-analysis may not be useful.

Evaluating research studies

In addition to considering the study design, here are some tips for evaluating the information you find in a research study:

Find out if the journal uses a peer-review process. Results from a study are more reliable if they are peer-reviewed, meaning that other researchers not affiliated with the study have looked over and approved the design and methods.

Look at the length of the study and the number of people involved. A study is more useful and believable if the same results occur in many people across a long time. An exception to this rule can be made for studies of rare cancer types or cancers with a poor prognosis (chance of recovery) because there may only be a small number of patients to study. Also, when considering the length of the study, it may be appropriate for some clinical trials to be shorter. For instance, cancer prevention trials are often much longer than treatment clinical trials.

Consider the phase of the study, especially for new treatments. Earlier phase studies provide early information about the safety of a new treatment (phase I) and an initial evaluation of how well it works (phase II). These studies tend to have a smaller number of participants compared to phase III trials, which compare the effectiveness of a new treatment to the current (standard) treatment.

Determine if the study supports or contradicts information that is already available. New results are exciting, but other researchers must validate the results before the medical community accepts them as fact. Review articles like systematic reviews may be of particular interest because they provide an analysis and draw conclusions across all of the published research on a specific topic.

Watch out for conclusions that overstate the results. Each study is a small piece of the research puzzle, and medical practice rarely changes because of the results of one study.

Talking with your doctor

Always talk with a member of your health care team about the information you find in an abstract or study. Even if you have read a reputable study that suggests a different approach to cancer treatment, do not stop or change your treatment before discussing your treatment plan with your doctor.

To start a conversation with your doctor you may want to ask:

- I recently heard about a study that used a new treatment. Is this treatment applicable to my type and stage of cancer?
- What type of journals should I read to learn more about my type of cancer?

- Should I consider being a part of a clinical trial?
- What clinical trials are open to me?
- Where can I learn more about clinical trials?

More Information

Understanding the Publication and Format of Cancer Research Studies [5]

Journal Links [6]

patientACCESS [7]

Research Summaries for Patients [8]

Medical News: How to Know If It?s Accurate [9]

Links:
[1] http://www.cancer.net/research-and-advocacy/introduction-cancer-research/understanding-cancer-research-study-design-and-how-evaluate-results
[2] http://www.cancer.net/about-us
[3] http://www.cancer.net/node/24863
[4] http://www.cancer.net/node/24880
[5] http://www.cancer.net/node/24718
[6] http://www.cancer.net/node/25373
[7] http://www.cancer.net/node/29231
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