



UNDERSTANDING SCIENTIFIC STUDIES & REPORTS: A GUIDE TO DETERMINING WHAT'S GOOD & WHAT'S NOT

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Many things are changing in medicine, including the increasing desire for patients and family members to learn as much about a disease as possible. This often results in reading on the Internet and listening to news reports. Too often, however, the news media almost exclusively focuses on the study results, paying little attention to the study design or quality of the study. For those reading the scientific study itself, the average person will find the information from the medical journals difficult to understand or critique. This article will focus on some general information about scientific reports and studies that should help individuals without medical training determine if the medical information is good or bad.

Prospective, Randomized, Controlled Trial: The best of the best – this study design has been called the ‘Gold Standard’ for research study designs. So, what do all those words mean? ‘*Prospective*’ means that the information is collected going forward once the study begins; ‘*Randomized*’ means that neither the patient nor the physician can determine which treatment will be given; usually a computer randomly selects the treatment and makes that decision and ‘*Controlled*’ means that the study is restricted to patients with similar specific characteristics meaning that not everyone can join. Why is this design the best? Because using it to compare two different treatments is the only way to truly prove that one treatment is better than another. If two groups of patients are essentially identical in every way possible except that one group received a different treatment and had a better outcome, then it is reasonable to conclude that the better result occurred *because* of the treatment given. For example, suppose a new drug has been developed for men with advanced prostate cancer. If it is tested in a prospective randomized, controlled trial and is compared to a placebo or inactive drug and the average survival is longer, then one may conclude that the drug improves survival. Based on that result it can be recommended for patients with the same characteristics as those tested in the study. **This is the only study design that permits such a recommendation.**

In an ideal world, all research trials would use this design. Unfortunately, that does not occur. You are probably wondering why not. The reasons are cost and time and patient acceptance. These studies usually cost several hundred million dollars to perform and take a number of years to complete. They are just too costly and time-consuming to be done. Drug companies do them because that is the only way a drug can be approved. But in the case of other therapies like surgery or radiation, they are much less likely to be done. The other reason is that in the case of surgery or radiation, too few patients would be willing to let a computer choose the therapy. They want to help make that decision which is not permitted in this study design.

Retrospective Study: Not good enough! In a retrospective study, someone decides to look back at patients treated at one or more institutions over many years. Specific characteristics are tabulated including the treatment they received. The results are then reported. As an example, in a retrospective study of men treated for localized prostate cancer at Hospital X, 100 men received surgery and 80 received radiation. Alternatively, all the men at Hospital X received radiation and the results were compared to another report published last year from Hospital Y in which the patients were treated by surgery. The 5-year survival for the radiation patients was 85% but only 80% for the men treated by surgery. So, does that prove that radiation is a better treatment? Absolutely not! You are probably wondering why not. There are many reasons why a retrospective study is not valid. First, the patients may be different due to general health issues their Gleason score or PSA results at diagnosis are different. There may be other differences between the groups that are not known. Second, there is the bias of how patients chose their treatment.

Perhaps healthier patients chose the radiation or their disease was less aggressive. Also, the doctor could have recommended radiation because they felt there was not much cancer present. Another shortcoming is there is no way to validate the accuracy of the information gathered about each patient. There are many examples showing that when information is gathered from medical records and rechecked, there are often errors. For example, the Gleason scores on the biopsy may be inconsistent if different pathologists interpret them. In a prospective study, however, a single pathologist usually will review the biopsies leading to better consistency. Another example is the cause of death, which is sometimes misinterpreted when not done by someone involved in the study.

In summary, in a retrospective study there are too many possible factors that could have affected their survival than the treatment they received. Without randomly assigning patients to surgery or radiation, a valid conclusion about which is better is just not possible. In some ways it is like comparing apples and oranges!

Because of the problems of a retrospective study design, the FDA does not approve treatments based on this approach. Unfortunately, the news media rarely reports which study design was used or the study's weaknesses. The public is led to believe the study provides important information that should influence their treatment decision. However, if you find out the study was done retrospectively, you can now appreciate that the conclusions and any recommendations made may not be valid. Only a prospective study can provide valid results.

Case-controlled Study: Also not good enough. Another study design with similarities to the retrospective study is the case-controlled study. A case-controlled study can be done either retrospectively or prospectively. With this design, data about patients from an institution who will be or were treated in a similar fashion are collected and then compared to another group with the same disease who were treated by a different method at a different institution. The weaknesses of a case-controlled study are the same as the retrospective study; the potential bias in how the treatment was selected, recognized or unrecognized differences in the patient groups and potential inaccuracies in the data collected. Even if done prospectively, these problems still exist. The bottom-line is no conclusion is justified and a treatment recommendation cannot be made.

Epidemiological Study: Garbage in, garbage out. Have you ever heard a news report presenting the results of an epidemiological study saying food X or vitamin Y is good for you and then you hear about another report saying food X or vitamin Y is bad for you? Why such confusion and how should you interpret these reports?

The best advice is to ignore them. Why? The reason is that epidemiological studies prove nothing. They may be correct or they may be incorrect. There is absolutely no way to know the truth. They are conducted by collecting lots of information from a group of individuals such as what they ate, how often they ate it, what vitamins and supplements they ingested, how much they exercised, etc or what treatments they received. The patients are then divided into groups, based in part on how long they lived, or what diseases they developed. The researchers then try to determine whether any particular characteristic or characteristics was (were) associated with living longer, or getting a disease, or avoiding one. They may find that food X was associated with a particular disease; those eating a lot of it were more likely to develop cancer than those eating little or none of it. The greater the amount people ate, the greater the likelihood of getting the disease.

There are many problems with trying to make conclusions and recommendations based on epidemiological studies. Some of it is the accuracy of the data gathered. There may be other unknown factors that influenced someone getting or avoiding a disease. The most significant criticism of an epidemiological study is that it does not prove cause and effect. There is no way to conclude that the reason a result occurred is because of any particular intervention. Epidemiological studies are useful, however, for designing prospective studies. They generate an idea for a prospective study to prove or disprove the theory.

The next time you hear the result of an epidemiological study presented, say thank you very much and be hopeful that a proper prospective study will eventually be done to determine if the result was correct. Is there any harm in incorporating the findings from an epidemiological study about a food or supplement into your own life? No, of course not, as long as you realize that it may good for you or it may be bad for you and there is no way to know if there are any harmful effects. But if you do hear about the results of an epidemiological study, realize that nothing has been proven.

Commentary: There is much more to learn about research study designs but this information should begin to make it possible for individuals who are not doctors to be a little more critical about what you hear on the news or read in the papers or on the internet. If at all possible, try to determine how the study was performed. If the report is discussed in a news article, send an e-mail to the author or try to search for that article on the Internet. That is quite easy to do these days and then look specifically at the study design so you can decide if the results are valid and useful. While all studies do provide some useful information, they do not all provide patients with guidance on the best form of treatment. Only a prospective randomized controlled trial can do that.

For those with prostate cancer, the challenge is even greater because treatments can and do become available even without a randomized controlled study. Such is the case for radical prostatectomy, radiation therapy, brachytherapy, cryosurgery, hormone therapy and HIFU. Not one of these treatments was ever compared to a placebo or control before it was used for patients nor have any of them been compared to each other. The FDA did not have to approve these therapies. As discussed earlier, however, for new medications, drug companies must perform

randomized studies because the FDA regulates how drugs can be marketed. In order to claim a drug is effective for the treatment of X disease, they must conduct a prospective randomized trial to prove it. The FDA does have to certify that a new device is safe for patients, but it is not necessary to show how a treatment compares to other treatments in terms of efficacy for treating a disease. That is why there is so much disagreement between doctors as to which option is best for a given patient with localized prostate cancer. **WE HAVE NO PROSPECTIVE RANDOMIZED STUDIES.** This is important for you to recognize when being counseled about your therapy. If a doctor recommends any of these treatments, it is based on his/her opinion and **NOT** on the kind of study (prospective randomized study) that proves it is really better than any of the other options available. There is physician bias. Unfortunately, this is not likely to change because there is no incentive for those studies to be done.

In summary, medical science is making important progress all the time. More good studies are being conducted that truly help guide treatment for patients. Yet, there are still too many studies that are not well designed but we hear about them anyway and they get publicity. It is the nature of news reporting. Patients need to be aware that just because something is in the news or on the Internet does not make it correct or useful. And since no one restricts the kind of studies that can be published or what is written on the Internet, we are far more likely to hear about retrospective, case controlled or uncontrolled studies because they are so common.

In the absence of good studies, what should a patient do who is trying to decide about treatment? Become informed about all the options, learn what are good and bad about each and then share in the choice, recognizing that no one can prove at this time that one is better than the other.

About Us TOO International

Us TOO International is a grassroots, 501-c-3 non-profit prostate cancer education and support network of 325 chapter support groups worldwide, providing men and their families with FREE information, materials and peer-to-peer support so they can make better decisions on detection, treatment options and coping with ongoing survivorship.

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