Assessing the value of clinical trial results

Introduction

Not all clinical trials are equal. In deciding how much weight to give to clinical trial results, it is worth asking a few key questions:

How well was the trial designed?

There is no one correct design for a clinical trial – it is more a question of whether the design used was appropriate to the circumstances. While large trials are general more reliable than small ones, this must be interpreted with common sense. For example, a trial of a rare inherited enzyme deficiency will never be able to include 5,000 patients, as frequently seen in trials of heart-attack medicines. Similarly, a follow-up period of a few weeks is perfectly adequate for a pneumonia trial but would be inappropriate for a contraceptive pill. Placebo control groups, while very helpful in the interpretation of results, are clearly unethical in some situations (e.g. life-threatening illnesses for which effective treatments exist). While comparative trials are the best way of assessing efficacy, larger and longer open-label trials may offer more insight into real-life medicine safety.

Each trial design must be approached through the question: ‘Was this the best way to do things under the circumstances?’

Does the patient population studied

correspond to the one I am interested in?

Information from a trial conducted in adults aged 18 to 65 may be of limited relevance to very elderly patients, and will almost certainly be inadequate for guiding the treatment of small babies. Similarly, people with severe or very advanced disease may respond quite differently to those with milder or earlier illness.

How relevant are the endpoints?

Some diseases and symptoms lend themselves more readily than others to study in a clinical trial. If a new cancer medicine is increasing median survival by a year, there can be little doubt that this is a relevant measurement. A new painkiller used to treat the same patients will be much more difficult to assess because there are no clear ‘standard units of pain’. Again, all that can be done is to question whether the approach taken is appropriate to the circumstances.

Were the effects of the medicine clinically valuable?

Generally, the bigger the effect of the medicine, the better. All medicines come with costs in terms of both money and side effects. The aim is to find the greatest possible benefit in return for those costs. It is worth remembering, however, that a result that is modest overall may be made up of a dramatic improvement in some patients and no change in others. If further research can help identify the sub-group likely to do especially well, then the new medicine may have much to offer this target population.

How do the clinical trial results fit into the pattern of previous knowledge?

It is very unusual for a clinical trial to stand alone as the only information available in a particular area of medicine. When this does happen, it usually represents the first use of a radically new approach to treatment and all one can do is to note the results with interest and wait to see if subsequent trials support them. Much more commonly, there will have been previous trials conducted with the same medicine or of the same class in the same illness or in related diseases. The new clinical trial results can then be viewed in light of the previous body of knowledge. Findings that mesh well with what is already known are generally easier to accept than those that directly contradict earlier results. However, it is important to keep an open mind.

Attachments

- Assessing the Value of Clinical Trial Results

  Size: 367,550 bytes, Format: .pptx

  A presentation describing how to assess the value of clinical trial results, which can be adapted for own use.

A2-4.34.3-v1.2