Good sources

As of 2023, and probably for a long time to come, the most robust evidence of a treatment’s efficacy comes from comparative trials, conducted in accordance with a rigorous methodology to ensure that the comparison was not biased. In other words, when seeking to determine whether a treatment is more effective than another, or more effective than no treatment, nothing is as robust and reliable as double-blind randomised comparative trials that use meaningful clinical endpoints (see “Proving a treatment’s efficacy” p. 162 of this issue). It may not be popular among those who consider that this step delays patients’ access to “innovations”, and propose saving time by using the results of “real-world” observations together with new methods for their statistical analysis, or by using artificial intelligence to create “virtual control groups”, but it is nevertheless true.

As of 2023, and probably for a long time to come, well-designed and well-conducted comparative clinical trials, carried out in flesh-and-blood patients, are necessary for making informed choices between different treatments, in the interests of all patients.

However, not all double-blind randomised comparative trials provide the same level of evidence: some are subject to bias that skews their results. And sometimes, trials providing similar levels of evidence produce conflicting results. All these factors complicate their analysis. Yet few health professionals have both the time and experience required to analyse trials themselves and put their results into perspective. Because it takes time to learn how to identify a trial’s methodological flaws, to search for and compare all the data, and to make sure that the results of some trials have not been buried through publication bias.

It is therefore quite legitimate to rely on someone else in order to save time. But who? Opinion leaders, whose potential selectivity and bias when weighing the evaluation data is difficult to gauge? Or teams working independently of the pharmaceutical industry and lobby groups, who seek to produce systematic analyses of all the available data?

If they apply a critical attitude in their choice of information sources and learn the basics of analysing clinical trials, healthcare professionals can distinguish with confidence between purported innovations and true therapeutic advances, with benefits that matter to patients and that justify the risk of experiencing the harms they can cause.

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