Ineffective yet Helpful: Thoughts about the Placebo Effect

Whenever a complementary therapy has failed to pass the test of scientific scrutiny, one often hears from its proponents that it may be ineffective but it still helps a lot of people. This apparent paradox is in many ways fascinating and is sometimes used by those who want to show us that clinical trials are not a useful means of assessing CAM – but what does it mean?

At first glance, one might detect several meanings. Most obviously it could imply that a certain therapy failed to reach a positive result for the primary outcome measure of that particular trial, say survival of cancer patients, but showed a positive effect in a more subtle way, for instance, quality of life. Thus one could say it was ineffective for what it was tested, while it could still be helpful for many patients.

This interpretation of the ‘ineffective yet helpful’ principle must, however, be rejected – not because the scenario outlined above does not exist but because it does not describe an ineffective therapy. The scenario would merely refer to a trial with the wrong primary outcome measure. If we re-designed that study using quality of life as a primary endpoint, we would doubtlessly demonstrate effectiveness. ‘Ineffective yet helpful’ would thus not apply.

Another meaning of the ‘ineffective yet helpful’ principle could refer to the inadequacy of the randomised clinical trial (RCT) for proving effectiveness of complementary therapies. We all know that the RCT is not perfect. It certainly can, under certain circumstances, produce false negative results. When this is the case, the ‘ineffective (as suggested by the results of a false negative RCT) yet helpful (as demonstrated by patients’ experience)’ notion could rightly apply. In such cases the solution would lie in defining what element of the trial design was inappropriate and to plan a study, randomised or not, that overcomes the problem(s).

The most frequent use of the ‘ineffective yet helpful’ principle is to suggest that a therapy has no specific effect but a substantial non-specific (placebo) response. Thus a scientist testing it against placebo is likely to call it ‘ineffective’. Nevertheless patients experience benefit from it. There could be several reasons for such an experience. The most obvious is that patients benefit from a placebo effect. In everyday practice, neither the patient nor the therapist normally have reliable ways

of differentiating the precise cause for clinical improvement. It could be a placebo effect, the natural history of the disease, concomitant therapies, a false impression altogether (e.g. the patient only claiming improvement to please the doctor) and, of course, a specific effect of the therapy applied. Generally speaking, doctors tend to claim that it was the therapy that helped, while neglecting the other possibilities.

It would be foolish to deny the power of the placebo or to think of it as an inferior or not really useful effect. But it is probably best to be clear about what we are dealing with and differentiate between specific and non-specific therapeutic effects as clearly as we can. A treatment without specific effects (i.e. not superior to placebo) can be called ineffective by investigators, while patients and clinicians find it helpful. This may be just a question of terminology and for the patient this may look like splitting hair – all the patient wants is to get better. However, for medical research, and I would say for progress, it matters greatly.

Why? Depending on what type of therapy was used as a ‘vehicle’ to ‘transport’ the placebo effect, different reasons come into mind. If a therapy carries significant risks, we have to ask ourselves whether the placebo response might not be ‘transported’ via a less risky treatment. If it is expensive, we might look for a cheaper and equally effective placebo. If it means that patients are led to believe in the paranormal or absurd, we probably could find a placebo where this is less likely.

The more important reasons, however, are scientific honesty and progress. Honesty is a value in itself that, in my view, cannot be rated high enough. Progress is probably equally important. Significant therapeutic advances could be made if we started seriously wondering what exactly the placebo response is. If we simply are content with the view ‘as long as it helps patients, all is fine’ and if we don’t try to define the exact causes of clinical improvement, we are unlikely to progress. As the placebo effect is potentially powerful we should try to unpack the contents of this ‘black box’. Once we understand it better, we probably can use it better – and this would really be progress.

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