

Evidence in Medicine

Preliminary Remarks

- Last week: Is there a Philosophy of Medicine?
- The prospect of scientific revolutions in medicine raises questions about evidence-theory relations.
- This week: Evidence and Medicine

Evidence and Medicine

Some Central Questions:

- What counts as evidence in Medicine?
- How weighty different kinds of evidence ought to be?
- Can the philosophy of science give useful advice in this context?
- What is the role and evidential power of clinical experience, judgment and expertise?
- What is the role and evidential power of control trials and randomisation?

Evidence-Based Medicine

John Worrall:

- Interested in the question ‘What is the relationship between evidence and theory?’
- In particular, interested in the question ‘What is the role and evidential power of randomisation?’

Controlled Clinical Trial:

Typically involves two groups one of which is given a test treatment and the other a control ‘treatment’ (often a placebo).

Randomised Controlled Trial (RCT):

Same as above but with a random process assigning patients to the different treatments.

Randomised Controlled Trials

- Dominant view: RCT represents the ‘gold standard’. Other evidence is available and should be used but it is less desirable.
- Why is RCT considered the ‘gold standard’ if
 - non-RCT evidence can be equally effective and compelling?
 - it is not required in diagnosis and prognosis?
 - even for therapy it is not always required?
- In general, what justifies a hierarchy of evidence where RCT is at the top?

Reasons to Randomise (1)

Worrall: The epistemic power of RCTs is overstated.

- Why randomise? Four answers:

(1) Fisherian Argument: Classical frequentist statisticians (following Fisher) argue that the statistical significance test requires randomisation.

A *significance test* determines the probability of a given result arising by chance. If the chance is low, the significance is high.

Objection: Many deny that classical significance-testing has epistemic validity, e.g. Henkel and Morrison (1970) .

Reasons to Randomise (2)

(2) Randomisation controls for ALL variables.

Basic logic of controlling trials:

- Avoid the *post hoc ergo propter hoc* fallacy.

Example: Suppose people recover from a cold after being given vitamin C. That by itself does not constitute evidence for the efficacy of vit. C against a cold unless there is a control group.

- Not just any control group will do. Confounding factors must be eliminated.

Example: If the health of the members of the experimental group was better than those of the control group the same pattern would emerge.

Reasons to Randomise (3)

Initial claim: *All* possible confounding factors (known and unknown) are controlled. Fisher, Giere, etc.

Objection: This claim is patently false because randomisation might still be allowing for confounding factors.

Revised claim: Given randomisation, it is *improbable* that an unknown factor's distribution is highly distorted in the sample population (as opposed to the general population). The greater the size of the trial the more improbable it becomes.

Objection: RCTs usually performed only once. Moreover, there are indefinitely many possible confounding factors. The probability that there is some confounding factor in a given study might be high.

Reasons to Randomise (4)

(3) Selection Bias:

In cases where clinicians decide how to divide patients up into the control and experimental groups, they can adversely affect the outcome of the trial (deliberately or inadvertently).

Indeed, eligibility criteria to participate in a trial are in part always subjective.

Randomisation helps eliminate selection bias:

If a random process divides the patients into the two groups, then such selection bias can be eliminated.

NB: The important thing here is to blind the clinician. Randomisation is just one of the ways to achieve this.

Reasons to Randomise (5)

(4) Non-RCTs exaggerate treatment effects.

Studies in 70's and 80's compared non-RCTs with RCTs. It 'emerged' that non-RCTs routinely exaggerate the real effect.

Objections:

- a) Hidden premise that RCTs measure the real effect.
- b) The non-RCT trials might not have been done properly.
- c) More recent studies (Kunz and Oxman (1998) and Benson and Hartz (2000)) show that *some* non-RCTs are as reliable as RCTs; indeed repeated application of certain non-RCTs reveals more consistent results than RCTs.

Upshot

Worrall's conclusions:

- Clinical practice should be based on best evidence.
- Best evidence must control against plausible alternatives.
- There are indefinitely many such alternatives.
- Background knowledge indicates plausible alternatives.
- Randomisation cannot control all possible confounding factors.
- Randomisation at its best in guarding against selection bias.
- Selection bias, however, can be controlled in other ways.
- We justifiably have a more positive attitude towards non-RCTs.
- We are justifiably moving towards a more unified account of clinical evidence.

Food for Thought

- Can the philosophy of science teach us anything about how to evaluate non-scientific theories, hypotheses, conjectures?

Reading

- Worrall, J. (2002) ‘What Evidence is Evidence-Based Medicine?’, *Philosophy of Science*, vol. 69(3): S316-S330.