

## May2002: Distinguish Between Observational and Experimental Studies (Rule 1.1)

Rules of the month are numbered in accordance with the numbering in the book. Thus, Rule 1.1 refers to the first rule in Chapter 1. And so on. These comments do not repeat the material in the book but highlights and amplifies it.

### 1 Remarks

As indicated in the discussion for this rule, the key distinction is whether or not randomization has taken place in a study. Randomization is crucial to obtaining a representative sample. An observational study, by definition, is a take-it-as-it-comes activity.

The best statistical discussion of the challenge to the inferential process in non-random (i.e. observational) samples can be found in Copas (1997). He states that “observational studies are often analyzed as if they had resulted from a controlled study, and yet the tacit assumption of randomness can be crucial for the validity of inference.” Precisely.

Recent papers by Concato et al. (2000) and Benson et al. (2000) arguing for the equality of observational studies with randomized, controlled trials (RCTs)—based on meta-analyses—raised valuable points but have not convinced me of their equality although there is gradation. The argument that a well-designed study with a cohort or case-control design is comparable to an RCT ignores this gradation. A flurry of letters criticized the conclusions. Concato et al. (2000) make a remarkable statement about the criticism of selection bias in their review of some 99 articles that these articles, “may not be exhaustive but should be representative of the available literature in those journals.” This is an example of the argument in Rule 1.1 that lack of randomness leads to arm waving.

### 2 References

- Benson, K. and Hartz, A.J. (2000). A comparison of observational studies and randomized, controlled trials. *New England Journal of Medicine*, **342**: 1876–1886. Discussion in (2000) **342**: 1907–1909 and **343**: 1194–1197.
- Concato, J., Shah, N. and Horwitz, R.I. (2000) Randomized, controlled trials, observational studies, and the hierarchy of research design. *New England Journal of Medicine*, **342**: 1887–1892. Discussion in (2000) **342**: 1907–1909 and **343**: 1194–1
- Copas, J.B. and Li, H.G. (1997). Inference for non-random samples (with discussion). *Journal of the Royal Statistical Society*, **59**: 55–95.
- Copas, J.B. and Shi, J.Q. (2000). Reanalysis of epidemiological evidence on lung cancer and passive smoking. *British Medical Journal*, **320**:417–418. Responses

in *British Medical Journal*, **321**:1221–1222.

- Hayes, B. (2001). Randomness as a resource. *American Scientist*, **89**: 300–304.
- Kruskal, W.H. and Mosteller, F. (1979a). Representative sampling. I. Scientific literature. *International Statistical Review*, **47**: 13–24.
- Kruskal, W.H. and Mosteller, F. (1979b). Representative sampling. II. Scientific literature, excluding statistics. *International Statistical Review*, **47**: 111–128.
- Kruskal, W.H. and Mosteller, F. (1979c). Representative sampling. III. Scientific literature, current statistical literature. *International Statistical Review*, **47**: 245–265.
- Kruskal, W.H. and Mosteller, F. (1980). Representative sampling. IV. The history of the concept in statistics, 1895–1939. *International Statistical Review*, **48**: 169–195.

### 3 Responses

This section is intended to contain reader comments and perhaps responses from me. It provides a forum for discussion and further reflection.