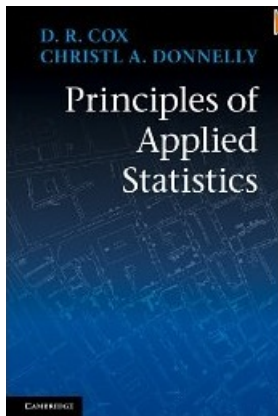


# General concepts



Chapters 1 and 2 uploaded to blackboard.  
All other material on [my page](#).

# Preliminaries

- ▶ we need statistics when we have “unexplained and haphazard variation”
- ▶ distinguish between natural variability and measurement error
- ▶ one is of interest, the other needs to be accommodated
- ▶ example: blood pressure varies over time scales of minutes, hours, days, even in healthy individuals. *Measurements* of blood pressure are also imprecise, but this variability is not of especial interest, although we need to be aware of it

# Components of investigation

- ▶ “the ideal sequence”
  - ▶ formulation of research questions
  - ▶ search for relevant data
  - ▶ design and implementation of investigations to obtain data
  - ▶ analysis of data
  - ▶ interpretation of the results
- ▶ “The essence of our discussion will be on the achievement of individually secure investigations. These are studies which lead to unambiguous conclusions ... Yet virtually all subject-matter issues are tackled sequentially ... typically the important and challenging issue of synthesizing information of very different kinds, so crucial for understanding, has to be carried out informally”
- ▶ Examples: Northern Hemisphere temperature time series; investigations of bovine tuberculosis; evidence for HIV as the cause of AIDS

## ... components of investigation

- ▶ very focused research question – ideal
- ▶ research questions emerge as the study develops – “consequent reformulation of the detailed statistical model used for analysis... usually causes no conceptual problem ... Major changes of focus... ideally need confirmation in supplementary investigations”
- ▶ “An extreme case of departure from the ideal sequence ... a large body of administrative data become available, and there is a perception that it must contain interesting information about something... the term ‘data mining’ is often used in such context... how much effort should be spend on such issues beyond the simple tabulation of frequencies and pairwise dependencies must depend in part on the quality of the data ... any conclusions are in most cases likely to be tentative and in need of independent confirmation”
- ▶ “ A large amount of data is in no way synonymous with a large amount of information”
- ▶ Grove *Science* Sept 23 editorial – replace Phase 2 and 3 clinical trials with data mining

## Design and Analysis

- ▶ choice of material/individuals to study – “units of analysis”
- ▶ “For studies of a new phenomenon it will usually be best to examine situations in which the phenomenon is likely to appear in the most striking form, even if this is in some sense artificial”
- ▶ statistical analysis needs to take account of the design (even if statistician enters the project at the analysis stage)
- ▶ need to be clear at the design stage about broad features of the statistical analysis – more publicly convincing **and** “reduces the possibility that the data cannot be satisfactorily analysed”
- ▶ example: Female faculty salary survey
- ▶ “it is unrealistic and indeed potentially dangerous to follow an initial plan unswervingly ... it may be a crucial part of the analysis to clarify the research objectives”

## Experimental and observational studies

- ▶ experiment is a study in which all key elements are under the control of the investigator
- ▶ in an observational study key elements cannot be manipulated by the investigator.
- ▶ “It often, however, aids the interpretation of an observation study to consider the question: what would have been done in a comparable experiment?”
- ▶ Example: hormone replacement therapy and heart disease
- ▶ observational study – strong and statistically significant reduction in heart disease among women taking hormone replacement therapy
- ▶ women’s health study (JAMA, 2002, p.321) – statistically significant **increase** in risk among women randomized to hormone replacement therapy

# Principles of measurement

- ▶ “construct validity – measurements do actually record the features of concern”
- ▶ “record a number of different features sufficient to capture concisely the important aspects”
- ▶ reliable – i.e. reasonably reproducible
- ▶ “cost of the measurements in commensurate with their importance”
- ▶ “measurement process does not appreciably distort the system under study”

## Types and phases of analysis

- ▶ “A general principle, sounding superficial but difficult to implement, is that analyses should be as simple as possible, but no simpler.”
- ▶ the method of analysis should be transparent
- ▶ Reference: *Science* Dec.2, 2011; special issue on Data Replication and Reproducibility
- ▶ main phases of analysis: data auditing and screening; preliminary analysis; formal analysis; presentation of conclusions



## “what are the principles of applied statistics?”

- ▶ “formulation and clarification of focused research questions of subject-matter importance
- ▶ design of individual investigations and sequences of investigations that produce secure answers and open up new possibilities
- ▶ production of effective and reliable measurement procedures
- ▶ development of simple, and where appropriate, not-so-simple methods of analysis, with suitable software, that address the primary research questions, often through a skilful choice of statistical model, and give some assessment of uncertainty
- ▶ effective presentation of conclusions
- ▶ structuring of analyses to facilitate their interpretation in subject matter terms and their relationship to the knowledge base of the field.”
- ▶ “somewhat in contrast, the role of work in the theory of statistics is to develop concepts and methods that will help