Beyond classic epidemiological designs (full day)

Abstract

Recent years have seen an increase in population-based health registers, and clinical trial registers, as well as in biobanks, motivated by the increasing interest in investigating genomic or molecular biomarkers. While this is undoubtedly an area of great interest and expansion in medical research, the statistical analysis for exploring and developing new biomarkers often faces limited availability of biological samples. In this context, it is crucial to develop novel study designs, and associated statistical analysis methods, for a parsimonious use of available resources.
Epidemiologists and biostatisticians are familiar with matched and unmatched cohort and case-control designs, but there are numerous other subsampling designs that are useful, and perhaps more efficient, when a sample is selected from a well-defined cohort or population, such as an electronic register. Moreover, despite the availability of methods and software for implementing many of these designs, they are rarely used in practice.

The course begins with extensions to the classic designs for binary outcome from both a sampling and analysis perspective: intentional and unintentional two-stage designs, balanced and optimal sampling at the second stage. The cohort, nested case-control and case-cohort designs for time-to-event outcomes will be reviewed, their interrelationship explored through examples, and extensions to more efficient and optimal designs presented. The practical sessions will include computational exercises using statistical software (STATA and R). The course is based on the forthcoming book "Controlled Epidemiological Studies".

Course outline

1. review of classic designs (cross sectional, cohort, case-control) matching, risk measures
2. two-stage design (recognition, examples and analysis)
3. balanced and optimal sampling of two-stage data in different settings
4. review of classic designs for time-to-event outcome and their interrelationship
5. novel applications of case-cohort and nested case-control designs
6. extended and optimal sampling for studies with time-to-event outcome

After this course, you should be able to:
- select & justify a suitable design for a specific research question concerning a binary and time-to-event outcome
- compare and interpret risk estimates from different sampling strategies
- recognise, analyse and design a two-stage study of a binary outcome
- implement and analyse a nested case-control or case-cohort study in various settings
- design and analyse a two-stage study of a time-to-event outcome

Target audience

Applied biostatisticians / epidemiologists or graduate students familiar with logistic and Cox model.

Presenters:

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