INSTITUTE OF ENVIRONMENTAL MEDICINE

C6F6031, Transporting Treatment Effects from Randomised Trials to Target Populations: Causal Inference for External Validity, 1.5 credits (hec)

Transportera behandlingseffekter från randomiserade prövningar till målpopulationer:kausal inferens för extern validitet, 1,5 högskolepoäng

Third-cycle level / Forskarnivå

Approval

This syllabus was approved by The Committee for Doctoral Education on 2024-09-13, and was last revised on 2025-04-25. The revised course syllabus is valid from spring semester 2025.

Responsible department

Institute of Environmental Medicine, Faculty of Medicine

Prerequisite courses, or equivalent

Knowledge equivalent to "Epidemiology I: Introduction to epidemiology", "Biostatistics I: Introduction for epidemiologists", "Epidemiology II: Design of epidemiological studies", and "Biostatistics II: Logistic regression for epidemiologists" or corresponding courses.

Purpose & Intended learning outcomes

Purpose of the course

To understand how to assess and quantitatively address external validity problems in randomized trials

Intended learning outcomes

After successfully completing this course, the student is expected to be able to:

- Explain external validity and discuss the scenarios in which trials have external validity issues
- Identify suitable target populations using real world observational data

- Interpret and communicate results that have been transported from a randomized trial to a target population
- Motivate how these methods fit into the drug regulatory and health technology assessment landscap

Course content

There is a lot of interest in understanding how externally valid or "generalizable" randomized trial results are to populations that are underrepresented or not recruited into the trial itself. There is a group of novel causal inference methods, namely transportability methods, that allow us to take the results from a randomized trial and assess whether the results would be the same in other clearly defined target populations.

In this course we will discuss the theory underlying external validity, present several methods used for transporting treatment effects from randomized trials to defined target populations (weighting and outcome modelling), and consider where and how these methods fit into the drug regulatory and health technology assessment landscape. Participants will be provided with analytic R code to undertake transportability analyses that use simulated data from a randomized trial and various target populations. We will also discuss how to interpret results.

Forms of teaching and learning

Lectures, computer labs, and individual and group work involving analysis of simulated real-life research problems related to the external validity of treatment effects estimated in randomized trials.

Language of instruction

The course is given in English

Grading scale

Pass (G) /Fail (U)

Compulsory components & forms of assessment

Compulsory elements
Individual written examination (summative assessment)

Forms of Assessment
To pass the course, the student must show that the intended learning outcomes have been achieved. The assessment methods used in this course are individual and group assignments (formative assessment) and an individual take-home examination (summative assessment). The focus will be to understand transportability methods to address specific research questions and interpret analytic results, rather than a detailed focus on mathematical principles. The examination is viewed as contributing to the development of knowledge, rather than a test of that knowledge. Students who do not obtain a passing grade in the first examination will be

offered a second examination within two months of the final day of the course.

Course literature

Recommended literature:

Cole, S.R., Stuart, E.A., 2010. Generalizing Evidence From Randomized Clinical Trials to Target Populations: The ACTG 320 Trial. Am. J. Epidemiol. 172, 107–115. https://doi.org/10.1093/aje/kwq084

Dahabreh, I.J., Robertson, S.E., Steingrimsson, J.A., Stuart, E.A., Hernán, M.A., 2020. Extending inferences from a randomized trial to a new target population. Stat. Med. 39, 1999–2014. https://doi.org/10.1002/sim.8426

Dahabreh, I.J., Robertson, S.E., Tchetgen, E.J., Stuart, E.A., Hernán, M.A., 2019. Generalizing causal inferences from individuals in randomized trials to all trial-eligible individuals. Biometrics 75, 685–694. https://doi.org/10.1111/biom.13009

Westreich, D., Edwards, J.K., Lesko, C.R., Stuart, E., Cole, S.R., 2017. Transportability of Trial Results Using Inverse Odds of Sampling Weights. Am. J. Epidemiol. 186, 1010–1014. https://doi.org/10.1093/aje/kwx164