WORKSHOP

Transporting treatment effects from randomized trials to real-world target populations

Date: Tuesday 16th November 2021, 13.30-17.00
Place: Zoom (details will be provided nearer the time)
Programme: Arranged within the doctoral programme in Epidemiology

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Overview: Randomized clinical trials (RCTs) are considered the gold standard for assessing efficacy of new therapies and are required for regulatory approval. However, patients enrolled on trials are often not representative of the patients in whom treatment will ultimately be delivered in clinical practice settings. When response to therapy varies across subgroups, differences between trial and clinical population composition contribute to the "efficacy-effectiveness gap" – where a treatment's efficacy in a trial differs from its effectiveness in clinical practice. Methods for generalizability and transportability can help bridge this gap. These methods combine RCT and clinical patient data to generate evidence that directly addresses therapy effectiveness in target populations. Such approaches leverage the enhanced internal validity of RCTs with the external validity of clinical practice data to better inform real-world decision-making.

Content: Methods used for generalizing and transporting treatment effects from RCTs to defined target populations will be presented, and we will discuss the theory underlying external validity, and where and how these methods fit into the Swedish health research landscape. Participants will apply SAS and/or R code to combine data from a publicly available phase III RCT and various simulated target populations to estimate transported treatment effects. Participants will use data visualization to guide decisions about transport model specification and performance. Analytic code will be provided directly to participants.

Target group: This workshop is of relevance to doctoral students and postdoctoral students, as well as Master students and junior and senior researchers, interested in expanding their epidemiological study design and analysis toolkit. The workshop may be of particular interest to individuals seeking additional training in the application of causal inference methods, pharmacoepidemiology, and comparative effectiveness research. It is recommended to have at least intermediate level knowledge in epidemiology and biostatistics, and familiarity with statistical programming in R or SAS.

Instructors
- Anthony Matthews, PhD, Postdoctoral Researcher, Institute of Environmental Medicine, Karolinska Institutet (anthony.matthews@ki.se)
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Please register here.