8 SESSION MODULE

Observational research: Introduction to epidemiology, real-world data, prognostic models, machine learning, and health economics

One of the key principles of evidence-based medicine is that there exists a hierarchy of evidence, often represented by a pyramid with study types ordered according to their comparative level of validity. Systematic reviews and meta-analyses sit at the top, followed by randomised controlled trials (RCTs) and then various observational study designs. The high costs and limitations of RCTs, together with the ever-growing availability of observational or routinely collected data and data science tools make routinely collected data increasingly relevant in evidence-based medicine and healthcare decision-making. The Centre for Statistics in Medicine at NDORMS is home to interdisciplinary teams conducting observational research, producing studies that advance healthcare practice and policy focusing on improving safety, vigilance, equity, effectiveness and cost-effectiveness. Topics of interest include prognosis, clinical predictions, analysis of routinely-collected health data, and health economics and outcomes research. This 8-session module will provide DPhil students with an introduction to the methods and practice of observational research which can help support their research and will enhance their skills in their future research careers.

Overview

This module will provide students with the opportunity to learn about the foundations of a series of key related topics in observational research over eight consecutive session. The course is spread over approximately 4 weeks between 20 April and 23 May 2022, with occasional work to be done between sessions to help prepare and enrich the level of discussion in subsequent sessions. The module will cover basic and real-world epidemiology, sources of real-world data in the UK, development and validation of prognostic models, machine learning, foundations of health economics, and economic analyses of MSK diseases and interventions. No prior knowledge is required.

Learning outcomes

With the series of eight sessions, this module is aimed to help students become familiar with some of the key methods, data sources, strengths, limitations and applications of observational research, with particular attention to their use in musculoskeletal diseases and interventions.

By the end of this 8-session module, students will:

- be familiar with the principles and scope of epidemiology, the benefits and limitations of various epidemiological studies, and understand how causal associations can be examined by real world epidemiology;
- understand the role, strengths and weaknesses of real-world big clinical sources, with special attention to the UK setting, as well as recognise challenges and identify solutions to give observational studies robust foundations;
- understand what prediction models are as well as the processes required to develop and validate them, and be able to critically appraise the methodological quality of studies reporting on those;
- be familiar with machine learning methods in healthcare and their application using real-world data;
- understand the basic rationale of health economic analyses as well as how and why economic evaluations are used to inform decision-making in healthcare, especially for the assessment of MSK interventions using routinely-collected data.

Sessions

Session 1 – Introduction to Epidemiology

Speakers: Trishna Rathod, Annika Jodicke, Cheryl Tan, and Maria Sanchez-Santos
When: 20 April 2022 10.00 – 13.00

Students will learn the principles and scope of epidemiology and examine the benefits and limitations of epidemiological studies. Concepts such as 'PICO', 'confounding' and 'bias' will be introduced and the differences between various study designs such as cohort and case-control examined. Leading to the following session, students will be introduced to real-world epidemiology.

Session 2 – Real-world Epidemiology

Speakers: Trishna Rathod, Annika Jodicke, Cheryl Tan, and Maria Sanchez-Santos
When: 28 April 2022 10.00 – 13.00

Students will learn about real-world data and how the causal association can be examined by real world epidemiology. The definitions of exposures, outcomes and confounders will be examined as well as specific bias inherent in these type of data. We will provide a high level introduction of approaches used to deal with confounders in real-world epidemiologic studies. The advantages and disadvantages of cohort and case-control study designs in real world epidemiology will be discussed through a group debate, closing with a discussion on the challenges and pitfalls of real-world epidemiology.

Session 3 – Real-world data sources in the UK: CPRD (GOLD AND AURUM), ONS and HES

Speaker: Antonella Delmestri
When: 10 May 2022 14.00 – 16.30

Students will learn about the most influential data sources available in the UK: why they are collected, how they are structured and linked, and how to gain permission to use them, including ISAC applications. The challenges of real-world big clinical data will be made apparent together with solutions through the usage of DataBase Management Systems (DBMS, e.g. MySQL) and high-level programming languages (e.g. Python) to implement advanced data curation and variable extraction.

Session 4 – Development of prognostic models

Speakers: Michael Schlussel, Paula Dhiman

When: 11 May 2022 13.00 – 16.00

Prediction models are widely used to inform clinical practice and to help guide physicians' treatment decisions. To be effective in improving patient care, prediction models need be robustly developed to provide accurate predicted probabilities for the health outcome of interest. In this two-session talk on prediction modelling, we will first cover the study design, analysis and reporting considerations when developing a prediction model. This will include how to handle predictors, modelling techniques, and options for simplifying a prediction model so it is presented in an easy-to-use format.

Session 5 – Validation of prognostic models

Speakers: Michael Schlussel, Paula Dhiman

When: 12 May 2022 13.00 – 16.00

In this second session, we will build on previously presented concepts of developing a prediction model, and introduce the study design, analysis, and reporting considerations when validating a prediction model. This will include how to predict from the model, how to estimate a model predictive performance using the same or different individuals from those used to develop the model (internal or external validation, respectively), and how to interpret the predictive accuracy of a model.

Session 6 – Introduction to machine learning

Speaker: Sara Khalid

When: 16 May 2022 14.00 – 16.30

This session will offer an overview of machine learning methods for healthcare applications including supervised and unsupervised learning approaches, followed by real-world examples of data analysis using routinely-collected data.

Session 7 – Health economics: the basics

Speaker: Rafael Pinedo-Villanueva

When: 18 May 2022 10.00 – 12.30

Treatment effects are key outcomes of clinical research, but when it comes to healthcare systems making decisions about their implementation then treatment costs, and relevant outcomes, must be assessed together to help inform the decision-making process. We will explore why and how this is done, and introduce the foundations of economic evaluations in healthcare, including the concept of quality-adjusted life years.

Session 8 – Economic analyses of MSK diseases and interventions

Speakers: Rafael Pinedo-Villanueva

When: 23 May 2022 10.00 – 12.30

Many MSK diseases are chronic and this requires assessments that consider long periods of time. It is also often the case that evaluations must be conducted using evidence from various studies. We will introduce the basics of decision-analytic modelling in health economics, which offer a framework to conduct simulations over the lifetime of patients and using different sources of data. We will discuss examples of economic analyses of MSK diseases and interventions using descriptive analysis and modelling methods.

Registration

For registration, please contact graduate.studies@ndorms.ox.ac.uk.