PROGRAMME Population Health Management Advanced Risk Stratification 14 – 18 May 2018

Short description content

Diagnostic and prognostic models are increasingly published in the medical literature each year. But are the results relevant for decision making in practice? How can models be used for risk stratification in populations? What are the critical elements of a well-developed diagnostic or prognostic model? How can we assume that the model makes accurate predictions for our population, and not only for the sample that was used to develop the model (generalizability, or external validity)? Are big data and advanced statistical techniques the solution for the problem of poor generalizability?

In the course we will address these and other questions from an epidemiological, statistical and decisionmaking perspective, using examples from the clinical literature. The participants will be encouraged to participate in interactive discussions and in practical computer exercises, starting with basic approaches and extending to advanced modelling.

Overall aim of the course (in terms of knowledge, application and attitude of the students):

After the course students:

- Understand the roles that diagnostic and prognostic models may play in risk stratification, and ultimately medical decision-making.
- Know the critical factors that determine the validity of predictions from diagnostic and prognostic models.
- Have insight in the pitfalls of model development with standard statistical techniques.
- Have both theoretical and practical knowledge on advanced methods in model development and validation, specifically on regression modelling.
- Understand the possibilities of using Electronic Health Records data for risk stratification.

Embedding in total PHM curriculum

This course is the 3rd course in data-analysis track. It is recommended that students have followed the courses "Fundamentals of Population Health Management: Moving from Volume to Value", "Study Design and Risk Parameters", and "Responsible Data Analysis for Population Health Management".

Faculty:

- David van Klaveren PhD (Biomedical data sciences, LUMC)
- Marta Fiocco PhD (Biomedical data sciences, LUMC)
- Prof Ewout Steyerberg PhD (Biomedical data sciences, LUMC)
- Ross Williams MSc (Bio Informatics, Erasmus MC)
- Henrik John MSc (Bio Informatics, Erasmus MC)
- Alexandros Rekkas MSc (Biomedical data sciences, LUMC)

Coordinators:

- Prof Ewout Steyerberg PhD (Medical Statistics & Medical Decision Making, LUMC)
- Prof Marc Bruijnzeels PhD (Public Health & Primary Care, LUMC)

Monday 14 May 2018 Room 219

Topic: Screening and diagnosis Faculty: David van Klaveren

Short description

Choosing the best treatment. Interpreting diagnostic test results. Deciding when to test. Multiple test results

Learning objectives:

After this day, students:

- Understand trade-offs of harms vs benefits
- Are able to calculate: no treat treat threshold
- Understand Bayesian thinking and perform probability revision
- Understand the trade-offs of harms vs benefits in deciding when to test
- Be able to calculate the no treat test threshold and the test treat threshold
- Know how to use diagnostic prediction rules

Programme

Lecture 1: Choosing the best treatment

The aim of this lecture is to make the participants acquainted with the basic concept of medical decision making: choosing the best treatment and using the information of (perfect) diagnostic tests.

Lecture 2: The value of diagnostic information

The aim of this lecture is to make the participants acquainted with the use of imperfect diagnostic tests. We will use Bayesian revision to update the prior probability of having a disease with diagnostic test results. We will analyse the question when to apply, or not to apply, an imperfect diagnostic test.

Literature

Decision Making in Health and Medicine, Hunink & Weinstein, Cambridge University Press 2014

Group assignment

Practical with Excel.

Reflection

Discuss practical. Paper discussion.

Capita Selecta

Prof Ewout Steyerberg. Biomedical data sciences, LUMC

Tuesday 15 May 2018 Room 219

Topic: Prognosis and prediction **Faculty:** David van Klaveren

Short description

Learning objectives:

• Have both theoretical and practical knowledge on methods in model development and validation, specifically on regression modelling.

Programme

Lecture 3: Introduction to prognostic research

The aim of this lecture is to make the participants acquainted with prognostic research. The concept of optimism and overfitting will be explained. Numerous examples of prognostic research will be presented.

Lecture 4: Introduction to prognostic modelling

A 7-step recipe for prediction model development is introduced. The first steps of developing a prediction model are discussed.

Literature: Clinical Prediction Models, Steyerberg, Springer 2011

Group assignment

Practical with SPSS

Reflection

Discuss practical. Paper discussion.

Capita Selecta

Prof Ewout Steyerberg. Biomedical data sciences, LUMC

Wednesday 16 May 2018 Room 364

Topic: Survival Analysis Faculty: M. Fiocco

Short description

In these lectures the focus is on the predictive value of a Cox model. In the first part we shall discuss about the discriminative ability of the Cox model; measure the prediction error; how to deal with overfittig; internal calibration. In the second part the focus will be on dynamic prediction models by landmarking.

Learning objectives:

- Understand how to assess the performance of a survival model
- Learn how to visualize the relation between predictor and survival
- Measure the discriminative ability of the model
- Measure the prediction error
- To assess the validity of the model by calibration, internal and external calibration
- Understand prediction in a dynamic setting by using landmarking

Programme

Lecture 5: Cox survival models

In this lecture we will first discuss prognostic models for survival data using information available at baseline, based on the Cox model. Then we will discuss about how to assess the performance of a pre-specified model. Here the visualization of the model, its discriminative ability and the prediction error of the model will be illustrated. The concept of model calibration (internal and external) will be presented

Lecture 6: Dynamic prediction

In this lecture dynamic prediction by landmarking will be discussed. The concept of landmarking will be first introduced. Reasoning about the construction of a dynamic prediction model will be provided. Then a dynamic prediction model for survival in a specific context will be presented Group assignment

practical with SPSS

Group assignment

Practical with SPSS

Reflection

Good statistical practice. Summary and take-home messages

Capita Selecta

Dr. Marta Fiocco, department of Medical Statistic, LUMC

Thursday 17 May 2018 Room 219

Topic: Advanced prognosis & prediction **Faculty:** David van Klaveren

Short description

On Tuesday the first steps of developing a prognostic model were introduced. Today we complete the 7-step recipe for prediction model development.

Heterogeneity across patients is generally considered to generate differences in treatment effects across individuals (heterogeneity of treatment effect). We will explain why prediction models may play a very important role in the quantification of treatment effect heterogeneity.

Learning objectives:

After this day, students:

- Deeper understanding of opportunities and problems with prediction models
- Conceptual insight in advanced statistical issues
- Increase experience with advanced modeling
- Understand the concept of heterogeneity of treatment effect
- Understand the limitations of conventional subgroup analyses
- Understand the rationale of a risk-based subgroup analysis

Programme

Lecture 7: Prognostic modelling

In this lecture you will extend your knowledge of prognostic modelling with advanced topics: Estimation of regression coefficients; evaluation of model performance; validation and presentation of a prediction model.

Lecture 8: Heterogeneity of treatment effect

Heterogeneity of treatment effect is often analysed with subgroup analyses. You will learn the limitations of conventional subgroup analysis and you learn the advantages of a risk-based subgroup analysis.

Group assignment

Practical with SPSS

Reflection

Discuss practical. Paper discussion.

Capita Selecta

Mar Rodríguez-Girondo: "The role of omics in prediction research"

Friday 18 May 2018 Room 219

Topic: Predictive analytics & big data **Faculty:** Ross Williams/Hendrik John/Alexandros Rekkas

Short description

If we want to achieve quality, efficiency, and transparency of EHR research, we need standardized Electronic Health Records (HER) data. The OMOP common data model (CDM) is used as a platform. Today you will learn how data is extracted from an EHR database in OMOP Common Data Model format. You will learn how prediction models are developed, using a large set of covariates, including drugs, diagnoses, procedures, demographics and comorbidity indexes. Of course, performance evaluation of these prediction models is an essential aspect.

Learning objectives:

After this day, students:

- Understand the nature and structure of EHR data
- Understand possibilities of using Electronic Health Records data for risk stratification

Programme

Lecture 9: Prediction in EHR data

We will show how data is extracted from a database in OMOP Common Data Model format. Using a large set of covariates, including drugs, diagnoses, procedures, as well as age and comorbidity indexes, we will show how to fit prediction models with large scale regularized regression. We will also show how to evaluate the performance of these prediction models.

Lecture 10: Case studies of prediction in EHR data

A number of case studies of prediction models in EHR data will be presented. In one of these case studies will use a prediction model to analyse potential heterogeneity of treatment effect.

Literature

Group assignment

General assignment Advanced Risk Stratification. Are big data the solution?