

Observational Study in Multiple Sclerosis



Challenges in the DIFUTURE Use-Case

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DIFUTURE

Data Integration for Future Medicine

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DIFUTURE

Data Integration for Future Medicine

Clinic



Data Lake



Research



Notation and Definitions

MS Multiple sclerosis

cMRT Cerebral magnetic resonance tomography (images of the brain)

sMRT Spinal magnetic resonance tomography (images of the spine)

t_i Month i after first relevant cMRT (= cMRT before treatment start)

Develop a model which can be used for making individual treatment decisions in patients with MS

- **Treatment success if no new or enlarged lesions** visible in sMRT and cMRT between months 6 (t_6) and 24 (t_{24})
- **3 treatment options:**
 - ▶ No treatment
 - ▶ Basic treatment
 - ▶ Strong treatment
- A variety of patient characteristics can potentially influence treatment success

Data

Data collected as part of clinical routine at MRI (TU Munich).

Outcome

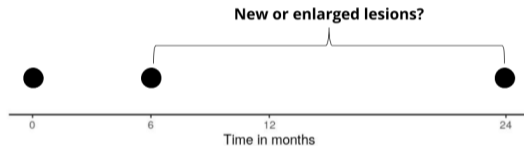
Treatment

Baseline patient characteristics

Data

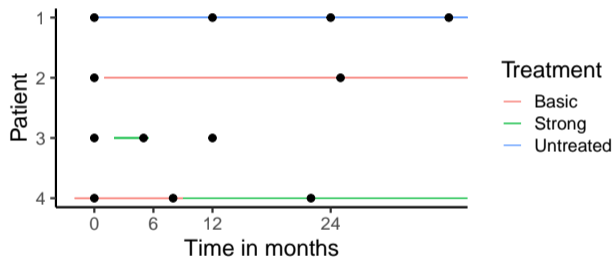
Outcome:

New or enlarged lesions visible in sMRT and cMRT between t_0 and t_{24} (yes/no)

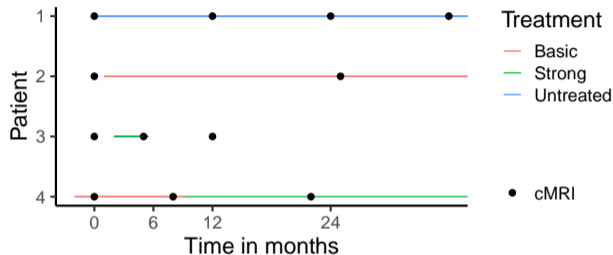


- No sMRT
- cMRT "usually" around t_0 , t_{12} and t_{24} (not as needed t_0 , t_6 and t_{24}).

Data



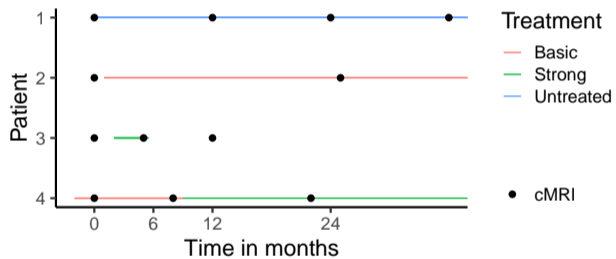
Data



Treatment:

- If no treatment within the first 2 years after diagnosis: treatment = no treatment
- Otherwise: treatment = first treatment given
- Treatment switches are possible

Data



Data

Baseline patient characteristics:

- Many possible characteristics available
- Many missing values (potentially not missing at random)
- Data quality potentially low

Challenges

How to deal with the issue of censoring in the outcome?

- We are interested in new or enlarged lesions between t_6 and t_{24}
- MRTs are mostly not done at these time points
- Can we use imputation methods?
- Can we use methods that can deal with censoring?

How to deal with the fact that for most patients we have only one sMRT?

- Clinician say that we can assume that there is no spinal progression if no sMRT was conducted.
- Is there a way to account for the insecurity?

How to obtain causal treatment effects?

- Data is observational
- We want to be able to say why a patient should receive which treatment

What to do in case of treatment switches?

- Patients sometimes switch from one treatment to another during the study period
- Is there a way to account for this?
- Would it be ok to ignore treatment switches? If so, why?

What to do with missing values in patient characteristics?

What to do with other data issues?

- Treatment starts before first cMRT
- What to do if data quality in certain patient characteristics is bad?
- ...