Medical Innovation Day

4-5 October 2018

Aarhus University

Background of the challenge:

- Read the article "Data skal løfte kvaliteten i den kliniske praksis", Perspektiv&Debat
- The current standard data set for approval of new medicines by authorities are data from Randomized Clinical Trials (RCTs)
- With rare biomarker-defined populations this is getting increasingly difficult as populations often are very small, prolonging the clinical development time considerable
- When running trials in for instance oncology the comparator arm would traditionally be an active control arm – but regulatory approval using single-arm Phase II studies are seen more and more often
- If using a comparative arm in the study, patients are often allowed to switch to active treatment (the investigational drug) if progressing during the trial, which makes interpretation of overall survival data difficult
- In Denmark we have more than 16 databases collecting oncology data of which a few are very comprehensive
- Region Midtjylland collects research data on all Non-Small Cell Lung Cancer (NSCLC) patients – The Database has a core of variables, with a potential for development and modulation when a center wants new variables
- Research Article: comparative effectiveness from a single-arm trial and RWD: https://www.futuremedicine.com/doi/pdf/10.2217/cer-2018-0032
- RWD explained. Real World Data (RWD) is all health data registered in clinical data-bases and electronic health records. More specifically, RWD is data on real patients in treatment in contrast to select patient populations included in clinical trials.

Details about the challenge

- Which challenges/barriers do you see when using RWD as comparator arm in clinical trials?
- Any suggestion on how to overcome the above?
- How do we ensure the quality of the RWD is acceptable for usage as comparator arm in trials?
- What would a trail design using RWD as comparator arm look like? Choose a specific disease.

What values and effect goals can be imagined by solving the challenge?

- Time to market of new innovative medicines would be shortened
- More patients could get exposed to new innovative treatments
- Good quality RWD would enable us to follow the treatment efficacy in broader populations than those included in RCTs after launch

Optional background

- Example of an attempt to realize the potential of RWD: https://flatiron.com/