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Session 2: Methodological issues

Challenges

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Utilization of observational data (RWD)

- Randomized controlled trials in oncology drug development
  - take a long time
  - conducted in a selected patient population
  - expensive
  - often too small to study predictive (bio)markers

  - drug efficacy unfortunately often marginal
  - MA often based on only one pivotal trial

- At the time of licensing knowledge on true benefit/risk balance often limited

- Additional information from observational studies valuable
  - **provided we can trust efficacy estimates**

RWD – some pros and cons

- Sources of data
  - data quality
- Can we get better estimates of efficacy and safety with data on use in the general patient population?
  - sample size
  - bias
- Potential to study predictive biomarkers
  - sample size
  - bias
- Handling of (unmeasured) confounding?
  - confounding by indication

Potential solutions – data collection/data analysis