Registry based studies

Overview of Registries

- Large, non-experimental cohort studies
- No randomization of treatment
- Minimal or no exclusion criteria in order to capture real-world patients and practices
- Safety endpoints and duration of follow up selected according to disease and product MOA
- Disease-based or product-based registries

Registry studies allow:

1. Assess natural history, including estimating the magnitude of a problem
   - Underlying incidence or prevalence of condition
2. Measuring or monitoring safety and harm associated with specific treatment/products
   - Safety
   - Effectiveness
   - Cost-effectiveness
   - Comparative effectiveness

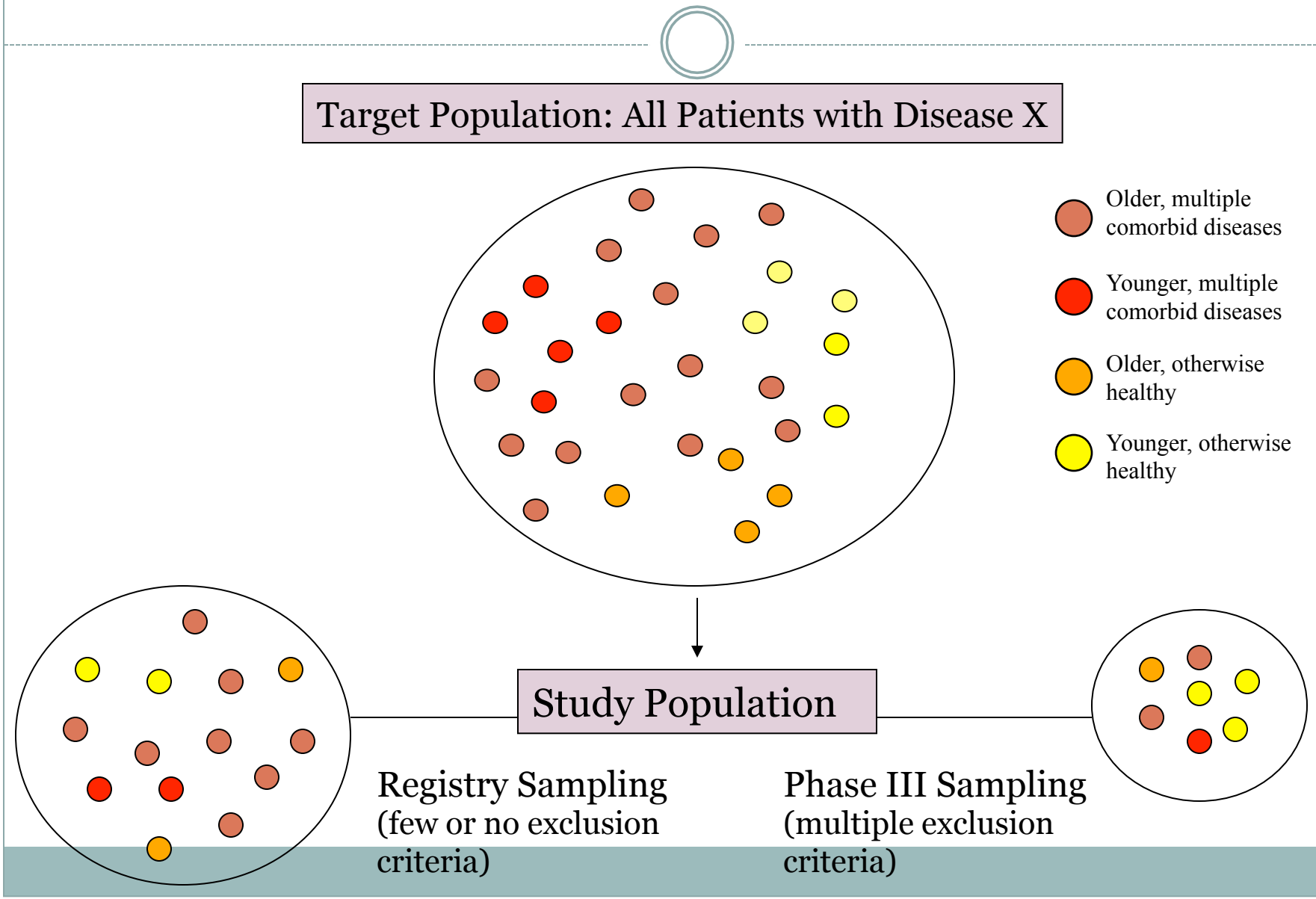
Study design for registries

- The framework for how the data will be analyzed drives the data collection and choices of patients for inclusion into the study.
- The study models of case series, cohort, case-control, and nested case control are commonly applied to registry data

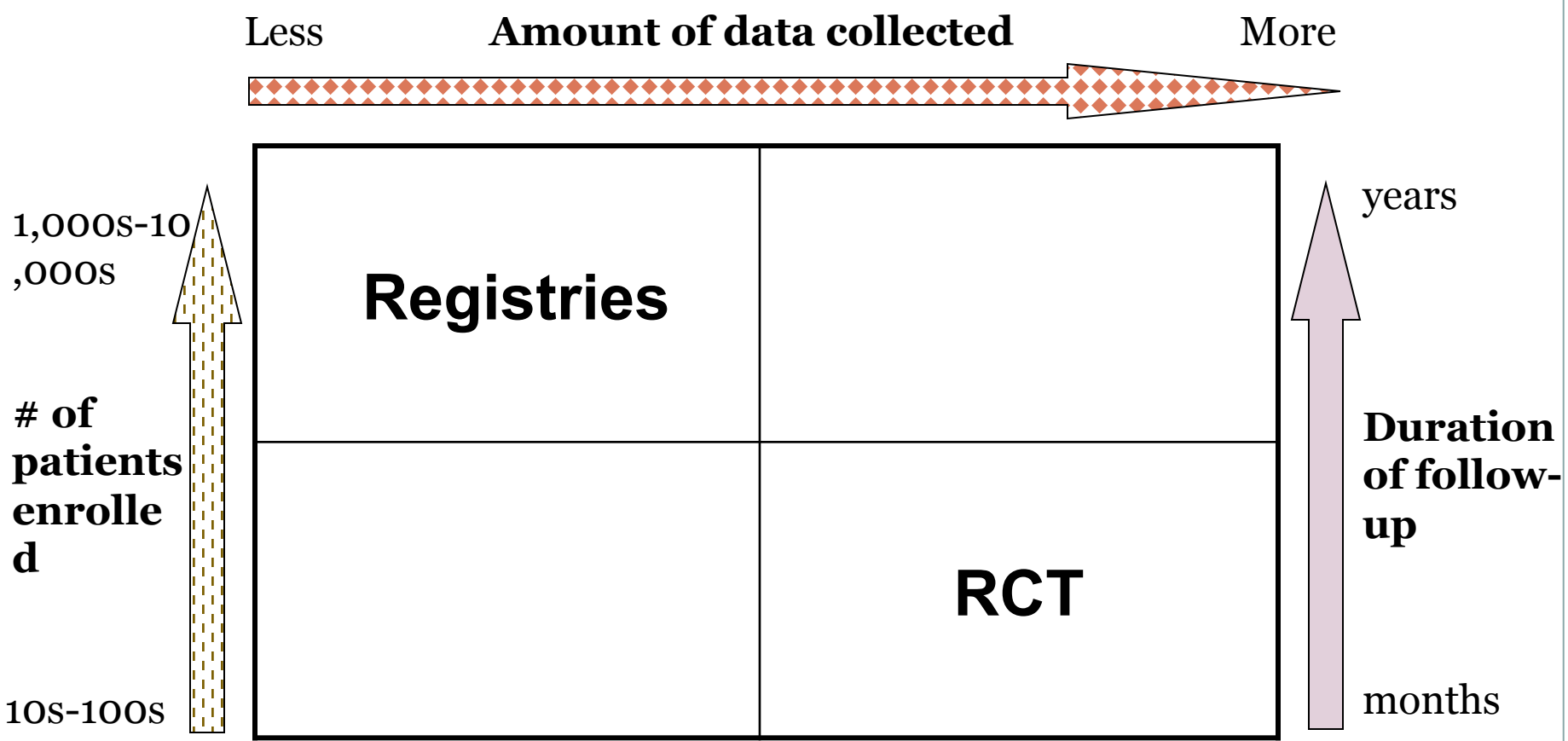
Unique benefits of registry studies

- Measure clinical effectiveness related to compliance.
- Subgroup differences in treatment effects due to population heterogeneity
- Surveillance of rare events or rare diseases
- Follow-up for delayed or long-term benefits/harm thanks to their longer observation periods
- Allow study of treatments where randomization is unethical
- Evaluate actual standard medical practice

Registries vs. RCT: Generalizability



Registries vs. RCT: Scope of Study



Potential bias in Registry Studies

- Channeling bias (Confounding by indication): form of selection bias.
  - Drugs with similar therapeutic indications are prescribed to groups of patients with prognostic differences.
    - Propensity score and blinded prospective review are used to adjust for confounding by indication
- Loss to followup
  - Differences in patients remained vs. loss is series when nonrandom