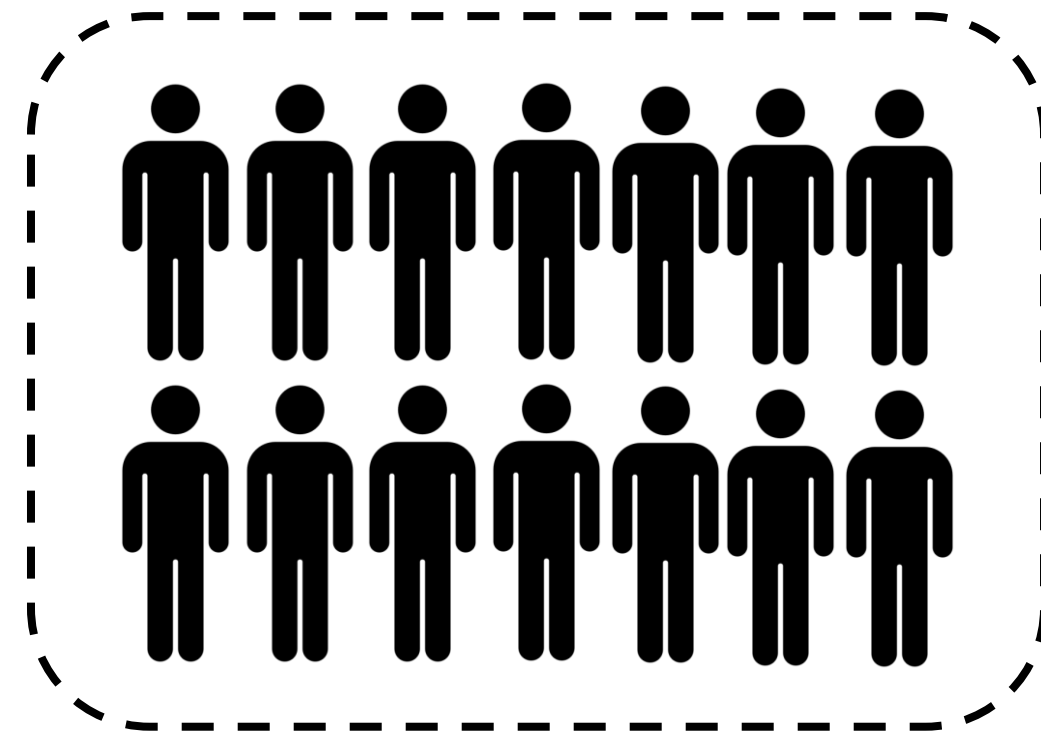


Dynamic Subgroup Identification in Covariate-adjusted Response-adaptive Randomization Experiments

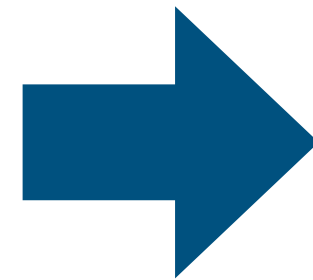
Yanping Li, Nankai University, Statistics
Joint work with Jingshen Wang and Waverly Wei

@ NeurIPS 2024

Motivation



Uniform treatment
for all subjects

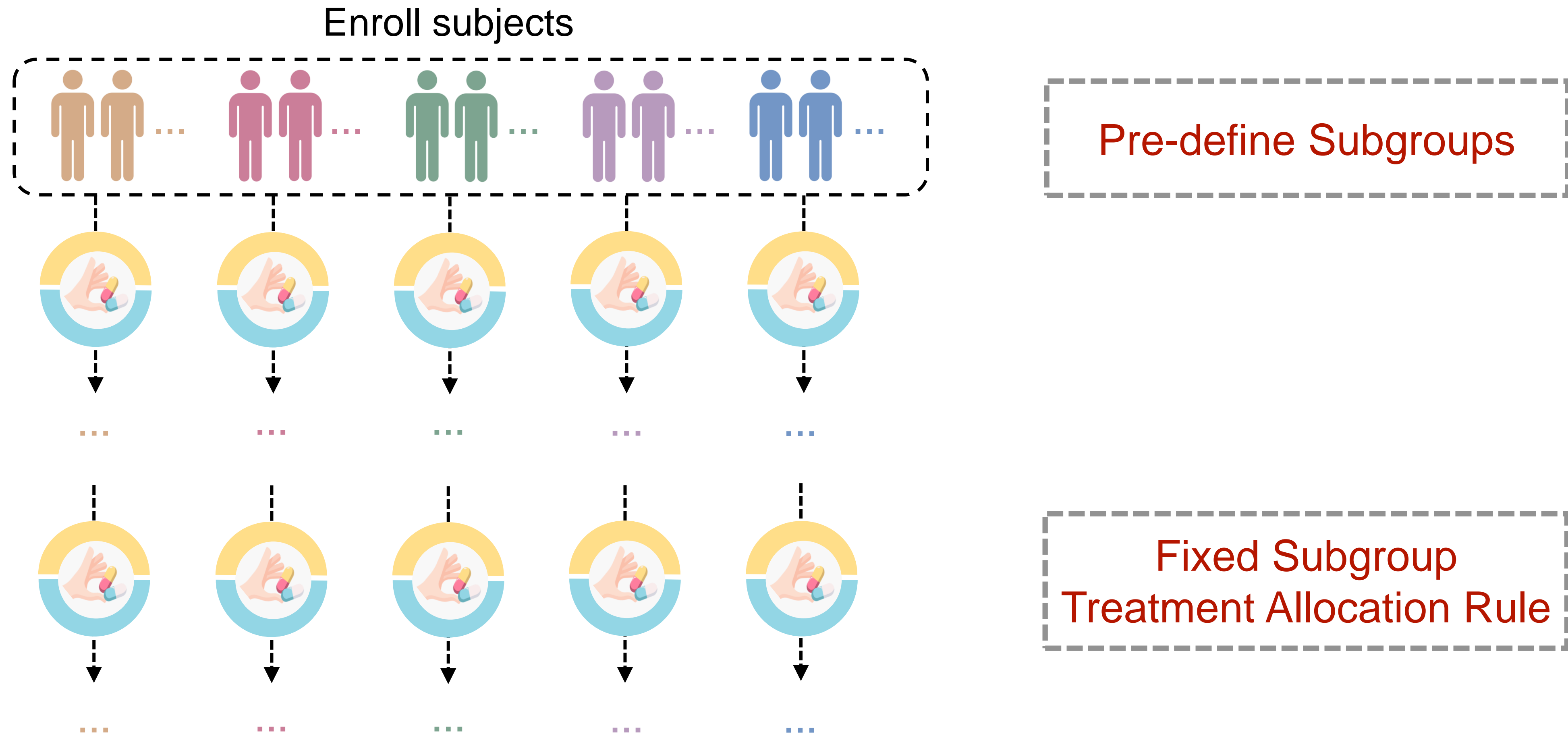


Tailored treatments
by subgroups

Advanced personalized
medicine

Motivation

- ▶ Traditional clinical trial designs

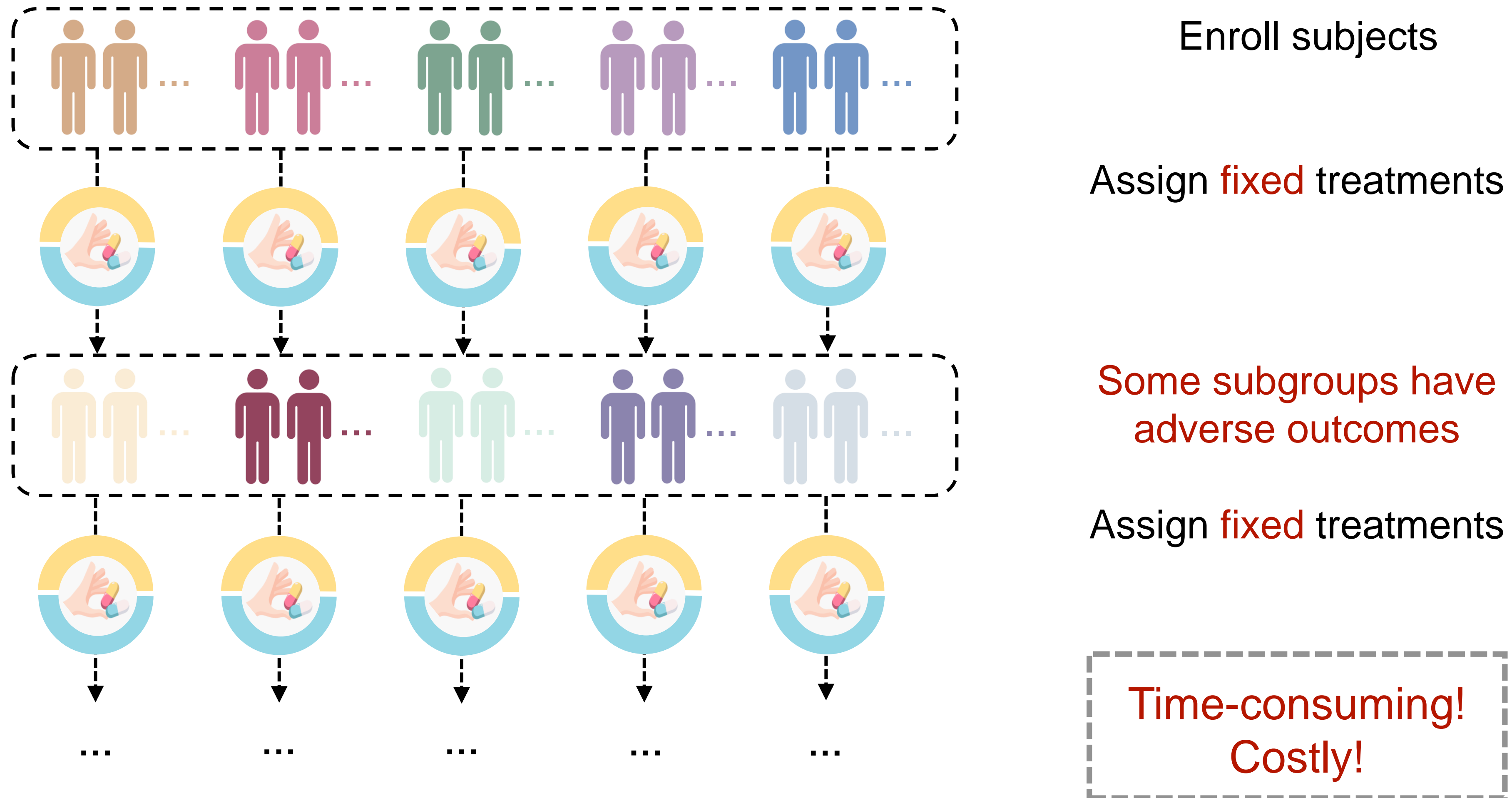


Limitation of Traditional Clinical Trials

- ▶ Traditional clinical trial designs
- ▶ At the beginning of the trial, subgroups are **pre-defined**;
- ▶ The **subgroup treatment allocation rule** is fixed;
- ▶ **Issue**: Traditional clinical trial designs make **inefficient** use of experimental data;

Limitation of Traditional Clinical Trials

Allocate experimental resources within a constrained budget



Limitation of Traditional Clinical Trials

- ▶ Traditional clinical trial designs
- ▶ At the beginning of the trial, subgroups are **pre-defined**;
- ▶ The **subgroup treatment allocation rule** is fixed;
- ▶ **Issue**: Traditional clinical trial designs make **inefficient** use of experimental data;
- ▶ How to **efficiently** allocate experimental resources within a **constrained** budget?
- ▶ **Solution**: Dynamic subgroup identification CARA design.

Assumptions:

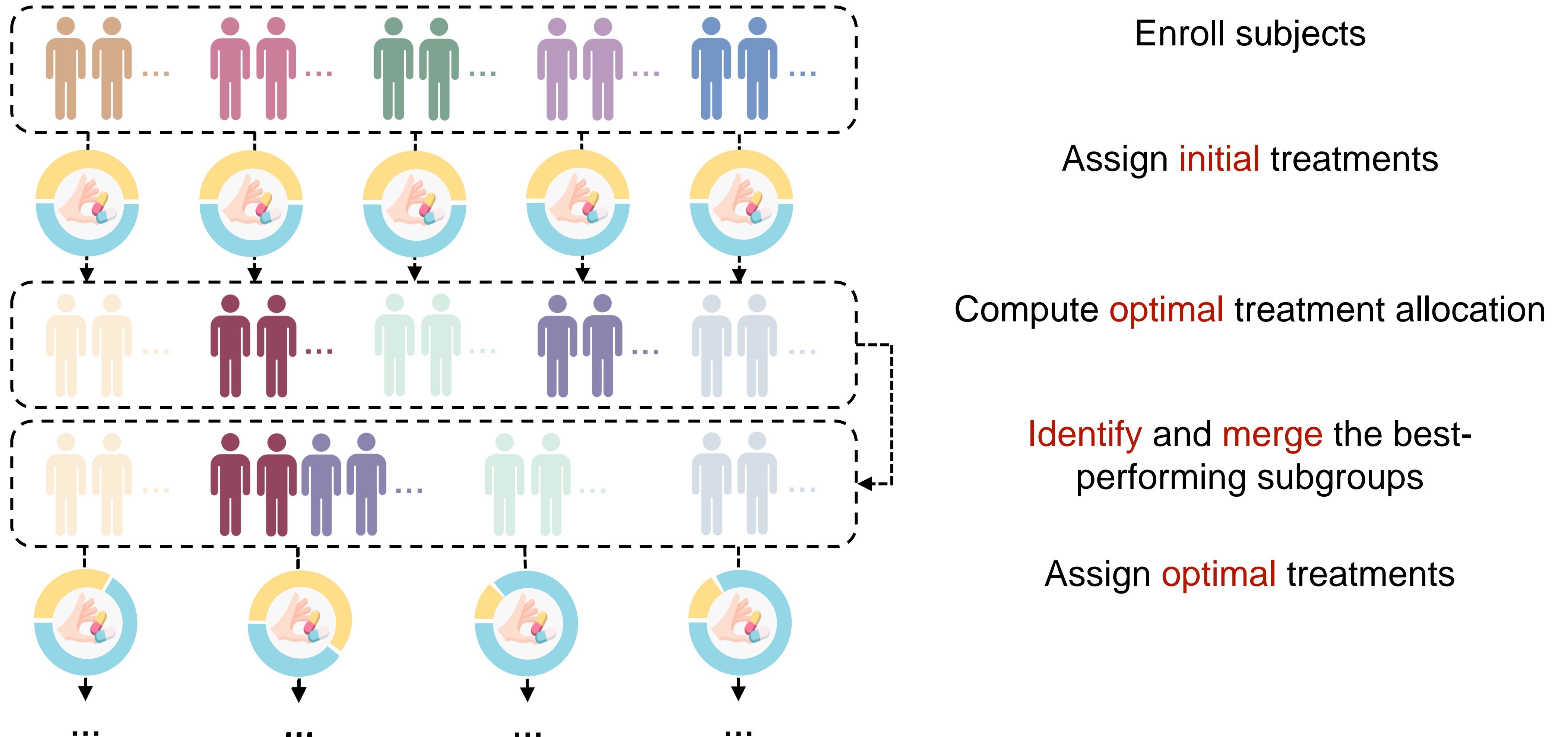
- ▶ Outcomes are observed **immediately** at the end of each stage **without delay**;
- ▶ The underlying distributions of outcomes do not shift over time.

Tasks:

- ▶ Adaptively **update treatment allocation** and treatment effect estimates;
- ▶ Dynamically **identify the best-performing subgroups**;
- ▶ **Select hyperparameters** that help with dynamic identification.

Dynamic Subgroup Identification Strategy

Experimental Goal: Maximize correct selection probability



Best-performing Subgroups Identification

- ▶ Generate **bootstrap samples** from the accrued data;
- ▶ Identify best subgroups within each bootstrap sample;
- ▶ Choose final subgroups with the **highest occurrence frequency**.
- ▶ **Hyperparameter Selection:** data-adaptively

Stage	Method	
Stage 1	Single Bootstrap	Double Bootstrap
Stage t ($t > 1$)	Naive Bootstrap	Separate Bootstrap

Summary

We propose a **dynamic subgroup identification CARA design** to optimize treatment assignments under resource constraints.