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JOHNS HOPKINS  
BLOOMBERG  
SCHOOL *of* PUBLIC HEALTH

## Study Populations

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# Identifying the Study Population

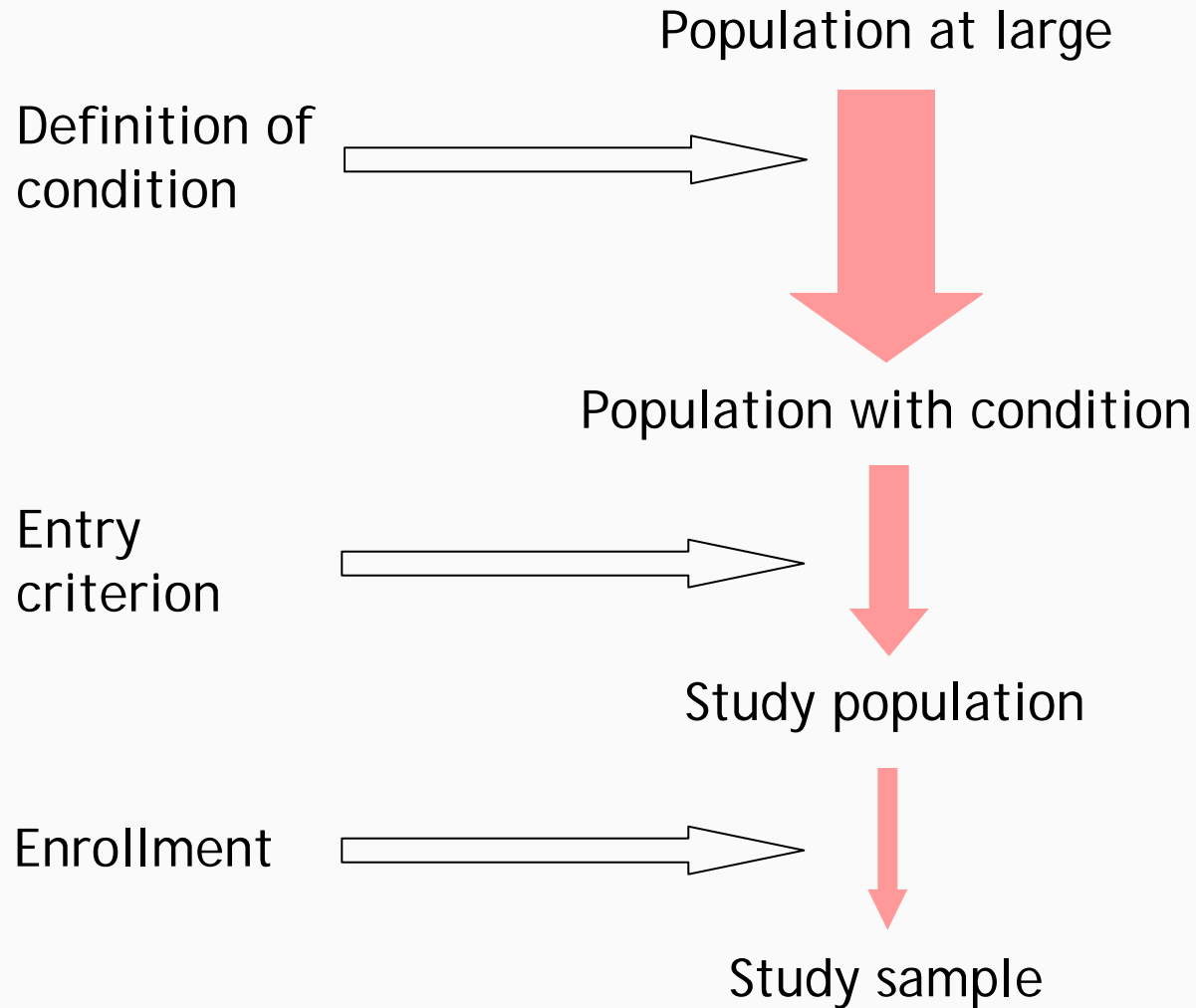
“The study population should be defined in advance, stating unambiguous inclusion (eligibility) criteria. The impact that these criteria will have on study design, ability to generalize, and participant recruitment must be considered.”

—*Friedman, et al., pg. 30*

# Study Population

- Eligibility = inclusion + exclusion criteria
- Consider . . .
  - Feasibility
  - Generalizability
  - Risk/benefit
  - Subject characteristics
  - Disease characteristics
  - Enrichment

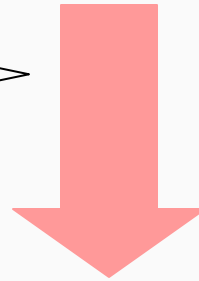
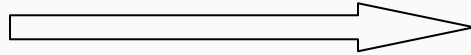
# Overall Picture



# Example: Women with Breast Cancer

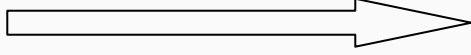
Population at large

Definition of condition



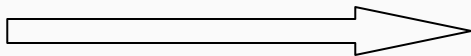
Women with breast cancer

Entry criterion



HER2+, distant metastases, no prior chemotherapy, no serious intercurrent illness

Enrollment

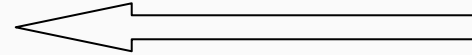
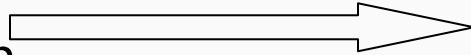


Study sample

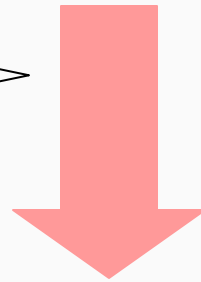
# How It Happens in Practice

Population at large

Population  
w/o condition

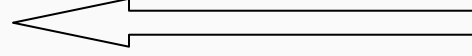
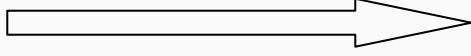


Definition of  
condition



Population with condition

With  
condition  
but ineligible

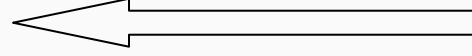
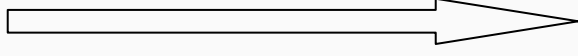


Entry  
criteria



Study population

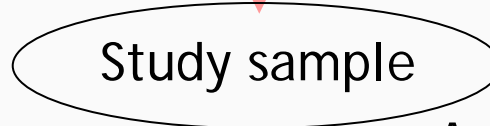
Eligible  
but not  
enrolled



Enrollment



Study sample



And these we randomize

# Eligibility Criteria

- Identify subjects:
  - Likely to benefit
  - In whom benefit can be discerned
  - In whom risks are acceptable
- Function of trial phase and objectives
- Basis for generalizability of results
- Improve precision of effect
- Quantitative or otherwise clearly interpretable criteria should be used



# Eligibility Criteria

## ■ Inclusion

- Hospitalized patients 18 years-of-age or older
- Oral temperature  $>38.5^{\circ}$  C

## ■ Exclusion

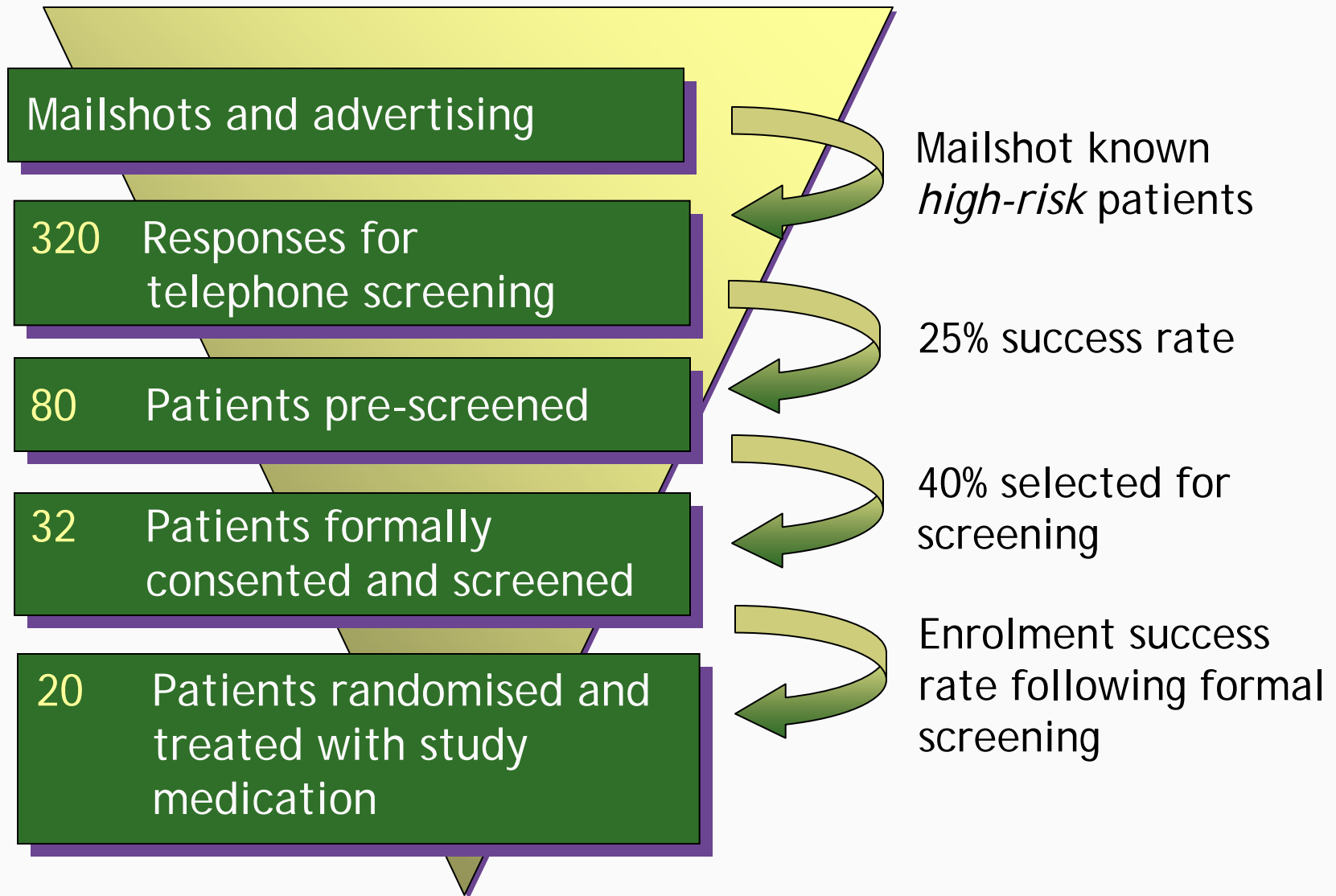
- History of hypersensitivity to new treatment (and control)
- Pregnant or breastfeeding
- Hospitalized for CHD or for treatment of endocarditis

# An Example in Obesity

- The epidemiology
  - 20% adults BMI > 30—increases with age
  - At least 20% of all patients would fall into a high-risk category
  - 50% of these high-risk patients will have a BMI > 27

- Taking any 100 adults on GP list:
  - 20 patients will have a BMI > 30
  - 10 patients will have at least one risk factor and BMI > 27
- Allowing for overlap and ineligible, it was estimated that 20 adults out of every 100 will qualify

# Anticipated Recruitment Funnel



# A Critical Question . . .

- To whom do the results apply?
  - All patients?
  - All patients who meet the disease criteria?
  - All patients who meet the disease criteria but are not subject to any of the exclusion criteria?
  - All patients who . . . [as above] . . . and who are willing to take part in a trial?
  - Your next patient?

# In the Next Lecture We'll Look at . . .

- Study designs issues
  - Randomization
  - Blinding (masking)
  - Stratification, blocking