

# UNDERSTANDING THE EVIDENCE FOR GOOD TREATMENTS

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#PatientNetwork

# Outline

- Discuss concepts important to understanding the evidence from clinical trials
- Small group activity, with case studies to illustrate this information

# CLINICAL TRIALS

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# Many different types of clinical trials

## Randomized Double Blind Clinical Trial

- **Gold Standard**
- **Patients randomly assigned to get drug 1 or drug 2 (or placebo)**
- **Patient doesn't know which drug**
- **Doctor/researcher doesn't know which**



# Many different types of clinical trials

## Randomized Single Blind Clinical Trial

- **Gold Standard**
- Patients randomly assigned to get drug 1 or drug 2 (or placebo)
- Patient doesn't know which drug
- ~~Doctor/researcher~~ doesn't know which



# Many different types of clinical trials

## Randomized Controlled Clinical Trial

- **Gold Standard**
- **Patients randomly assigned to get drug 1 or drug 2 (or placebo)**
- **Patient doesn't know which drug**
- **Doctor/researcher doesn't know which**



# Many different types of clinical trials

## Controlled Clinical Trial

- **Gold Standard**
- Patients **randomly** assigned to get drug 1 or drug 2 (or placebo)
- Patient ~~doesn't know which drug~~
- Doctor/researcher ~~doesn't know which~~
- 2 patient groups are similar or matched on age, sex, diagnosis.



# Many different types of clinical trials

## Uncontrolled Clinical Trial

- **Gold Standard**
- **Patients randomly assigned to get drug 1 or drug 2 (or placebo)**
- **Patient doesn't know which drug**
- **Doctor/researcher doesn't know which**
- **2 patient groups are similar or matched on age, sex, diagnosis.**





# Types of Controls

- New drug compared to PLACEBO (or device compared to a sham)
- New drug compared to an old drug
- New drug compared to historical controls

SIGNIFICANCE DOES NOT  
EQUAL EFFECTIVENESS

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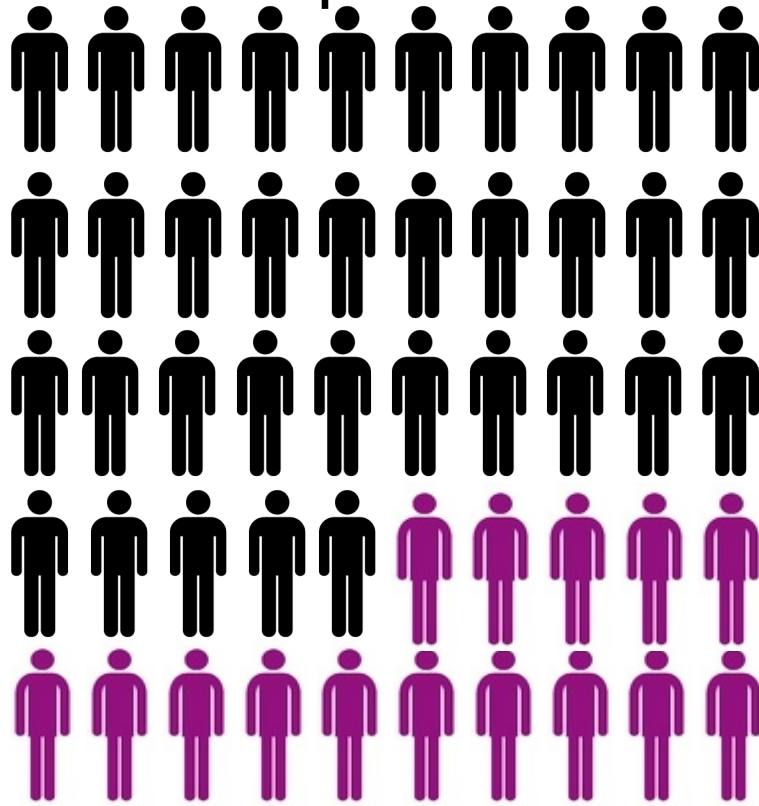
# Statistically significant $\neq$ important

- Statistically significant does NOT necessarily mean that the difference is large or important. It means it probably didn't happen by chance.

# Results in a small sample could be due to chance

## CONTROL

30% effective out of 50 patients



## DRUG

40% effective out of 50 patients

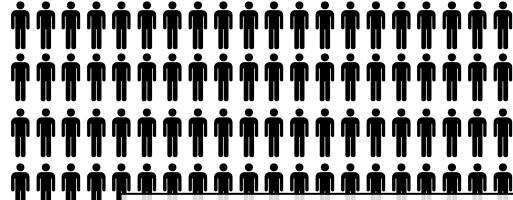


10% difference between groups

$P=0.18$

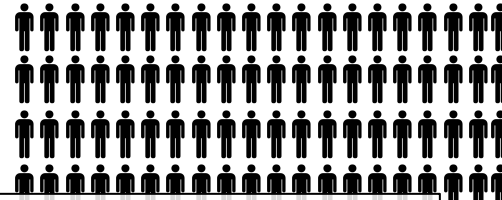
Not Significant

# Large sample with the same percent difference is significant



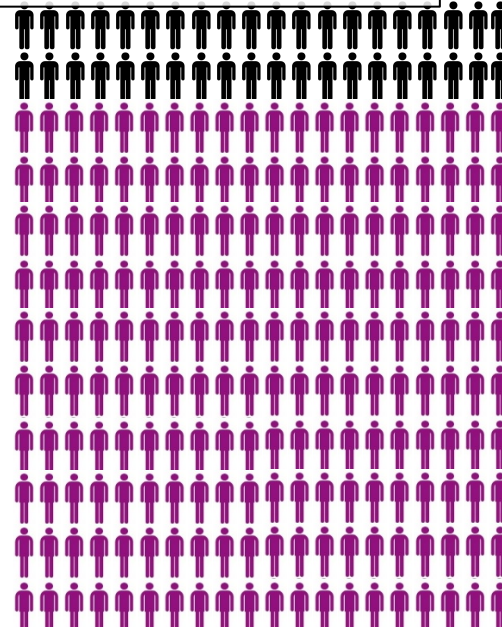
**CONTROL**

**30% effective out of 500 patients**



**DRUG**

**40% effective out of 500 patients**



10% difference between groups

$P=0.001$

Statistically significant

# Important questions to ask about trial design and results:

- Will we be able to tell if there is a difference between the treatment group and the control?
- Is the study well controlled to reduce bias, such as differences between treatment and control groups?
- Is the statistically significant result meaningful to patients?

# Group activity #1: Identify the Clinical Trial

- **Options:**

- Randomized Double Blind Clinical Trial
- Randomized Single Blind Clinical Trial
- Randomized Controlled Clinical Trial
- Controlled Clinical Trial
- Uncontrolled Clinical Trial

# Group activity #2: Importance of Sample Size

- **Example based on the real study of Vytorin, a cholesterol lowering medication**