

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

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**

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

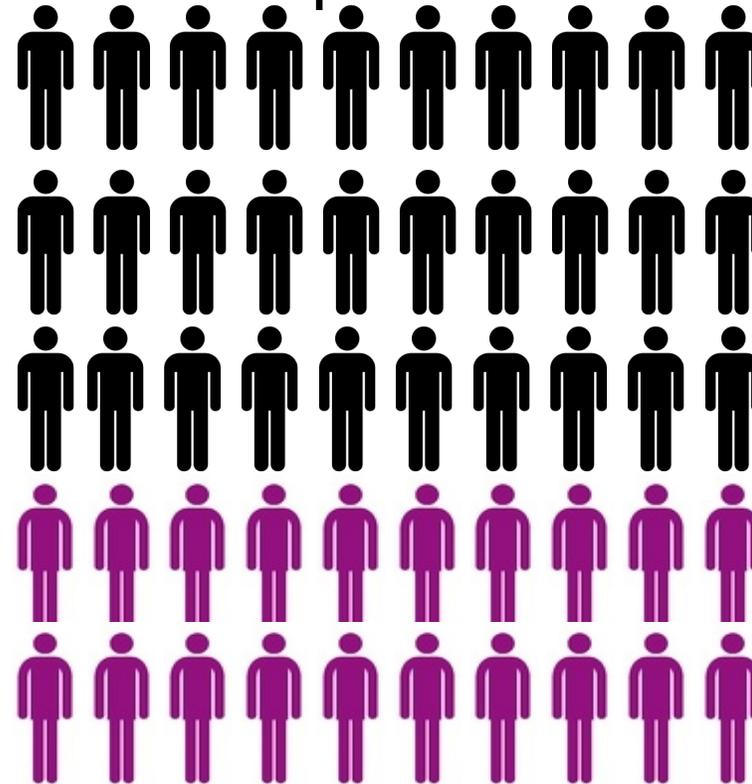
CONTROL

30% effective out of 50 patients



DRUG

40% effective out of 50 patients

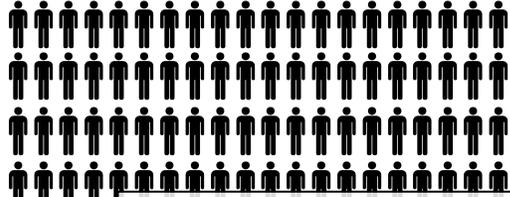


10% difference between groups

$P=0.18$

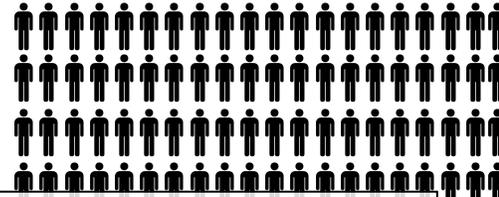
Not Significant

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**