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
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 ≠ 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