Experiments, Good and Bad
Chapters 5 & 6 (till page 108)

Jan 25, 2013

- Childhood Polio: Background
- The Vital Statistics Approach
- The Method of Comparison
- Two Experiments
- Statistical Significance
1.0 Childhood Polio: Background

- Polio is an epidemic disease: spread by contact.

- It is a disease of hygiene. Children from better economical backgrounds are more susceptible.

- Timeline:
  - 1916: First polio epidemic in the U.S.
  - 1952: 60,000 cases.
  - 1953: 30,000 cases.
  - 1954: Jonas Salk’s vaccine is ready for field testing by the National Foundation for Infantile Paralysis (N.F.I.P.).

- Subjects: children in grades 1, 2 and 3.
2.0 The Vital Statistics Approach

- Distribute the vaccine as widely as possible.
- Monitor the rate of reported polio in subsequent seasons.
- In the case of polio, this approach would fail to give clear-cut evidence. Why?

Figure 1  Poliomyelitis in the U.S., 1930–56. Source: Rutstein (1957)
3.0 The Method of Comparison

- The only way to “show” the vaccine is effective is by comparing it to something else.

<table>
<thead>
<tr>
<th>TREATMENT</th>
<th>CONTROL</th>
</tr>
</thead>
<tbody>
<tr>
<td>vaccinated group</td>
<td>unvaccinated group</td>
</tr>
</tbody>
</table>

- In some cases, if there is a STANDARD OF CARE, then the new drug must be compared with it.

- Ethical considerations can also play a role in the construction of comparison groups.
4.0 Some Vocabulary

- Subjects: individuals in a study.
- Response Variable: measures **outcome** of interest.
- Explanatory Variables: thought to **explain** or **cause** changes to the response variable.
- Treatment Variable: Explanatory variable of experimental interest. It is usually qualitative.
4.1 Example

- **Background:** Oregon has an experimental boot camp program to rehabilitate prisoners before their release. The goal is to reduce the “recidivism” rate – the percentage who will be back in prison within three years. Prisoners volunteer for the program, which lasts several months.

- **Study:** To evaluate the program, researchers compared the recidivism rate for the volunteers who completed boot camp with those who dropped out.

- **Subjects? Response Variable? Explanatory Variable(s)? Treatment Variable? Study Design?**
Does swearing increase pain tolerance? Researchers tested 64 students’ tolerance to pain by asking them to submerge their hand in ice water for as long as they could while repeating a series of swear words of their choice. They were then asked to carry out the task again while repeating non-offensive words. They found that volunteers who swore were able to keep their hand submerged in the water for an average of 40 seconds longer. When questioned about their perceived pain, they also rated it as being lower. The researchers also measured the volunteers’ heart rate and found that it increased while swearing.

Subjects? Response Variable? Explanatory variable(s)? Treatment Variable? Study Design?
5.0 Two Designed Experiments for the Vaccine Study

N.F.I.P. Study
950,000 kids from grades 1, 2, 3.

Group 1
(225,000 children from grade 2 whose parents consented to vaccine)

Group 2
(725,000 children from grades 1 & 3)

Treatment 1 (vaccine)

Compare polio rates

Field Trial:
400,000 kids from grade 2 only. All parents must consent to kid receiving some type of injection.

Group 1
(200,000 children)

Random assignment (1:1)

Group 2
(200,000 children)

Treatment 1 (vaccine)

Compare polio rates

Treatment 2 (saline injection)

Group 1
(200,000 children)

Treatment 1 (vaccine)

Compare polio rates

Treatment 2 (no vaccine)
5.1 Random Assignment

- In the field trial, the subjects are randomly assigned to one of the two comparison groups. That is, a chance procedure is used for impartial assignment.

- What else does random assignment accomplish? It ensures that the two groups are similar in all respects, at least if the sample sizes are large. Why is this important?

- To carry out random assignment label all eligible participants with a unique number. Select a S.R.S. of appropriate size to receive treatment 1, and so on.
An experiment was carried out to determine the effect of providing free milk to school children in a certain district (Lanarkshire, Scotland). Some children in each school were chosen for the treatment group and got free milk. Assignment to treatment or control was done at random to make the groups comparable in terms of family background and health.

After randomization, teachers felt there were too many small kids in the control group and they were allowed to use their judgment in switching children between treatment and control, to equalize the two groups. Was it wise to let the teachers use their judgment in this way. Answer yes or no, and explain briefly.
5.3 Example

Random assignment is used in designed experiments (when possible) because it:

1. reduces bias
2. creates groups of equal sizes
3. mitigates the effect of “lurking” variables
4. enables cause-effect conclusions to be drawn

Choose all that apply.
In the field trial, the control group was given a saline solution or a placebo – a dummy drug that looks like the real thing. What does this accomplish?

A survey of physicians found that some doctors can give a placebo to a patient who complains of pain for which the physician can find no cause. If the patient’s pain improves, these doctors conclude that it had no physical basis. The investigators conducting the survey claimed that these doctors do not understand the placebo effect. Why?
5.5 Example

A placebo is often included for comparison purposes because it:

1. reduces bias
2. “blinds” the subject to their group assignment.
3. enables cause-effect conclusions to be drawn

Choose all that apply.
5.6 Example

A researcher claims that the chemical capsaicin, the ingredient that makes chilli peppers hot, helps relieve pain. He randomly assigns subjects with chronic back pain to two groups. In one group he injects capsaicin near the sciatic nerve. In the other, he injects a placebo. The subjects did not know what treatment they received, but the researcher did. After an hour, he interviewed the patients and rated the change in their pain based on their comments. Critics said the results were suspect. What could one criticism be?
6.0 The Salk Field Trial

The Salk field trial was a randomized, placebo controlled, double blind study.

<table>
<thead>
<tr>
<th></th>
<th>The Salk Field Trial</th>
<th>The N.F.I.P. study</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Size in 1,000</td>
<td>Rate per 100,000</td>
</tr>
<tr>
<td>Grade 2 (vaccine)</td>
<td>200</td>
<td>28</td>
</tr>
<tr>
<td>Grade 2 (saline)</td>
<td>200</td>
<td>71</td>
</tr>
<tr>
<td>Grade 2 (vaccine)</td>
<td>225</td>
<td>25</td>
</tr>
<tr>
<td>Grades 1 &amp; 3 (nothing)</td>
<td>725</td>
<td>54</td>
</tr>
</tbody>
</table>

- The Salk Field Trial provides **stronger** proof of the effectiveness of the vaccine.
- It also enables us to isolate the vaccine as the cause of the reduction in polio cases.
6.1 Statistical Significance

Definition
An observed effect of a size that would rarely occur by chance is called “statistically significant”.

- The effect is the result we are comparing between the groups.
  - e.g., the difference in polio rates between the vaccinated and placebo groups. (0.043%)

- How large must an effect be in order to be statistically significant?
  - depends on sample size

- However, an effect is only meaningful if it is practically significant.
6.2 Example

It has long been thought that eating a healthier diet reduces the risk of bowel cancer. A large study case doubt on this advice. The subjects were 2,079 people who had polyps removed from their bowels in the past six months. The subjects were randomly assigned to a low-fat, high-fiber diet or to a control group where the subjects ate their usual diets. Did polyps re-occur during the next four years?

Surprisingly, the occurrence of new polyps “did not differ significantly” between the two groups. Explain clearly what this means.
6.3 Example

A study compares two groups of mothers with young children who were on welfare two years ago. One group attended a voluntary training program that was offered free of charge at a local vocational school and was advertised in the local news media. The other group did not choose to attend the training program. The study finds a statistically significant difference between the proportions of the mothers in the two groups who are still on welfare: 21%.

- Explain in simple language what a statistically significant difference means.

- Does this study provide good evidence that requiring job training of all welfare mothers would greatly reduce the percentage who remain on welfare?