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# Statistics

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# Statistics

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# Preface

*What song the Syrens sang, or what name Achilles assumed when he hid among women, though puzzling questions, are not beyond all conjecture.*

— SIR THOMAS BROWNE (ENGLAND, 1605–1682)

## TO THE READER

We are going to tell you about some interesting problems which have been studied with the help of statistical methods, and show you how to use some of these methods yourself, explaining why the methods work and what to watch out for when other people use them. Mathematical notation only seems to confuse things for most people, so we are going to do it with words, charts, and tables—and hardly any  $x$ s or  $y$ s. Even when professional mathematicians read technical books, their eyes often skip over the equations despite their best efforts. What they really need is a sympathetic friend who will explain the ideas and draw the pictures behind the equations. We are trying to be that friend for those who read our book.

## WHAT IS STATISTICS?

Statistics is the art of making numerical conjectures about puzzling questions.

- How should experiments be designed to measure the effects of new medical treatments?

- What causes the resemblance between parents and children, and how strong is that force?
- How is the rate of inflation measured? The rate of unemployment? How are they related?
- Why does the casino make a profit at roulette?
- How can the Gallup poll predict election results using a sample of only a few thousand people?

These are difficult issues, and statistical methods help quite a lot in analyzing them. These methods were developed over several hundred years by people who were looking for the answers to hard questions. Some of these people will be introduced later on in the book.

#### AN OUTLINE

Part I is about designing experiments properly, so that meaningful conclusions can be drawn from the results. It draws examples from medicine, education, and urban policy, and analyzes some studies which are badly designed and misleading. And it explains some of the questions to ask when judging the quality of a design. Most studies produce so many numbers that it is impossible to absorb them all: summaries are needed. Descriptive statistics is the art of describing and summarizing data. This branch of the subject is introduced in Part II. The discussion is continued in Part III, where the focus is on analyzing relationships—for instance, how income depends on education.

Much statistical reasoning depends on the theory of probability, discussed in Part IV. The connection is through chance models, which are developed in Part V and used in Part VII to solve some problems about measurement error and genetics. Parts VI and VIII, statistical inference, explain how to make valid generalizations from samples. Part VI deals with estimation and answers the questions: How does the Gallup poll take a sample to estimate the percentage of Democrats in the population? How far off is the estimate likely to be? Using tests of significance, Part VIII explains how to judge whether a sample confirms or denies a hypothesis about the population from which it is drawn.

Nowadays, inference is the branch of statistics most interesting to professionals in the field. However, nonstatisticians usually find descriptive statistics the more useful branch—and the one which is easier to understand.

The bare bones of the subject are presented in Chapters 1 to 6, 16 to 21, 23 and 26. After digesting these, the reader can browse anywhere; the next chapters to read might be 8, 10, and 13.

#### EXERCISES

The numbered sections in each chapter usually end with a set of exercises, the answers being at the end of the book. If you work these exercises as they come along and check the answers against the back of the book, you will get



practice in your new skills—and find out the extent to which you have mastered them.

Every chapter except 1 and 7 ends with a set of review exercises; the book does not give answers for these exercises. When working them, you may be tempted to flip backward through the pages until you find the relevant formula. However, reading this book backward will prove very frustrating. The review exercises demand much more than formulas. They call for rough guesses and qualitative judgments, and require a good intuitive understanding of what is going on. The way to develop that understanding is to read the book forward.

Why does this book include so many exercises that cannot be solved by plugging into a formula? The reason is that few real-life statistical problems can be solved that way. Blindly plugging into statistical formulas has caused a lot of confusion. So this book teaches a different approach: thinking.

#### ACKNOWLEDGMENTS

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PART I

# Design of Experiments



# 1

## Controlled Experiments

*Always do right. This will gratify some people, and astonish the rest.*

—MARK TWAIN (UNITED STATES, 1835–1910)

### 1. THE SALK VACCINE FIELD TRIAL

A new drug is introduced. How should an experiment be designed to test its effectiveness? The basic method is *comparison*. The drug is given to subjects in a *treatment group*, but other subjects are used as *controls*—they aren't treated. Then the responses of the two groups are compared. Experience shows that subjects should be assigned to treatment or control *at random*, and the experiment should be run *double-blind*: neither the subjects nor the doctors who measure the responses should know who was in the treatment group and who was in the control group.<sup>1</sup> These ideas will be developed in the context of an actual field trial.<sup>2</sup>

The first polio epidemic hit the United States in 1916, and during the next forty years polio claimed many hundreds of thousands of victims, especially children. By the 1950s, several vaccines against this disease had been discovered. The one developed by Jonas Salk seemed the most promising. In laboratory trials, it had proved safe, and had caused the production of antibodies against polio. A large-scale field trial was needed to see whether the vaccine would protect children against polio outside the laboratory. In 1954, the Public Health Service decided to organize this kind of experiment. Nearly two million children were involved, and about



half a million were vaccinated. About a million were deliberately left unvaccinated. And another half a million refused vaccination. The field trial was conducted on children in the most vulnerable age groups—grades 1, 2, and 3. It was carried out in selected school districts throughout the country, those where the risk of polio was believed to be the worst.

This is an example of the method of comparison. The treatment (vaccination) is given only to some of the subjects, who form the treatment group: the others do not get the treatment and are used as controls. The responses of the two groups can then be compared to see if the treatment makes any difference. Here, the treatment group and control group were of different sizes, but that did not matter. The investigators were going to compare the rates at which children got polio in the two groups—cases per hundred thousand. Looking at rates instead of absolute numbers adjusts for the differences in the size of the two groups.

There is a troublesome question of medical ethics here. Shouldn't all the children have been given the protection of the vaccine? One answer is that with new drugs, even after extensive laboratory testing, it is often unclear whether the benefits outweigh the risks. A field trial is needed to find out what the treatment does when it is used in the real world. Now giving the vaccine to a large number of children might seem to provide decisive evidence, even without controls. For instance, if the incidence of polio in 1954 had dropped sharply from 1953, that would seem to be proof of the effectiveness of the Salk vaccine. But it really wouldn't be. Polio is an epidemic disease, whose incidence varies a lot from year to year. In 1952, there were about 60,000 cases; in 1953, there were only half as many. Without controls, low incidence in 1954 could have meant one of two things: either the vaccine worked, or there was no epidemic that year.

The only way to find out whether the vaccine worked was to leave some children unvaccinated. Of course, children could be vaccinated only with their parents' permission. So one possible design was this: the children whose parents consented would form the treatment group and get the vaccine. The other children would form the control group. But it was known that higher-income parents would consent to treatment much more readily than lower-income parents. And this would have created a bias against the vaccine—because children of higher-income parents are more vulnerable to polio than children of lower-income parents. This seems paradoxical at first, but polio is a disease of hygiene. Children who live in less hygienic surroundings tend to contract very mild cases of polio early in childhood, while still protected by antibodies from their mother. After being infected, they generate their own antibodies, which protect them against more severe infection later. Children who live in more hygienic surroundings are less likely to contract these early mild infections, do not develop antibodies, and are less likely to be protected against severe infection later.

The statistical lesson is that to avoid bias, the treatment group and control groups should be as similar as possible—except for the treatment. That makes it possible to conclude that any difference in response between the two groups is due to the treatment, rather than some other factor. If the

two groups differ with respect to some factor other than the treatment, the effects of this other factor might be *confounded* (mixed up) with the effects of the treatment. Separating these effects is often difficult or even impossible. Confounded effects are a major source of bias.



Coming back to the Salk vaccine field trial, several designs were proposed. The National Foundation for Infantile Paralysis (NFIP) wanted to vaccinate all grade 2 children whose parents would consent, leaving the children in grades 1 and 3 as controls. And this NFIP design was accepted by many school districts. However, it had two serious flaws. First, polio is a contagious disease, spreading through contact. So the incidence could be much higher in grade 2 than in grades 1 or 3. This would have biased the study against the vaccine. Or the incidence could have been much lower in grade 2, biasing the study in favor of the vaccine. Second, children in the treatment group (where parental consent was needed) were bound to have different family backgrounds from those in the control group (where parental consent was not required).

With the NFIP design, the treatment group would include too many children from the higher-income families, making this group more vulnerable to polio than the control group. Here was a definite bias—against the vaccine.

Many school districts saw these flaws in the NFIP design, and as a result adopted a different design. To make a fair comparison, the control group had to be chosen from the same population as the treatment group: children whose parents consented to vaccination. Otherwise, the effect of family background would have been confounded with the effect of the vaccine. The next question was how to assign the children to treatment and control. It seems that delicate human judgment was called for to make the treatment group and