

## PHASES OF STATISTICAL ANALYSIS

### 1. Initial Data Manipulation

Assembling data

Checks of data quality - graphical and numeric

### 2. Preliminary Analysis: Clarify Directions for Analysis

Identifying Data Structure:

What is to be regarded as an “individual”?

Are individuals grouped or associated?

What types of variables are measured on each individual?

Response versus Explanatory Variables

Are any observations missing?

### 3. Definite Analysis – Formulation of Model, Fitting, ...

### 4. Presentation of Conclusions

## STYLES OF ANALYSIS

- Descriptive Methods

  - Graphical

  - Numerical summaries

- Probabilistic Methods

  - probability model for data

  - probability model for any uncertain quantities*

  - probabilistic properties of estimates and uncertainties

    - graphics help convey results

    - numerical statements for results/conclusions

## TYPES OF STUDIES

- Experiments:
  - RCT: Randomize Treatment A and B; outcome survival
  - Difference in responses due to “treatment”
- Observational Studies (no control by investigator)
  - Hospital Records of Treatments and Survival
  - Other causes?
  - Caution with Conclusions!
- Prospective Studies (Individuals selected by investigator)
  - random sample of smokers/non-smokers;
  - followup response cancer/non-cancer
- Retrospective Studies (Individuals selected by investigator)
  - sample of cancer/non-cancer;
  - identify differences in explanatory variables

## ANALYSIS GOALS

- Estimation of uncertain quantities
- Prediction of future events
- Tests of hypotheses

Theoretical framework based on probability models

**Probability:** Measurement of *uncertainty*

Interpretations?

- **Counting:** equally likely outcomes (card and dice games, sampling)
- **Frequency:** Hurricane in September? Relative frequency from “similar” years
- **Subjective:** measure of belief, judgment
  - Can include counting, frequency cases
  - also applies to unique events – the Duke/E.NC game, probability of terrorist attacks
  - expert opinion
  - computer simulations

## Probability Models

At least Two Physical Meanings for Probability Models

- Well defined Population and individuals (the sample) are drawn from the population in a “random” way
  - could conceivably measure the entire population
  - probability model specifies properties of the full population
- Observations are made on a system subject to random fluctuations
  - probability model specifies what would happen if hypothetically observations were repeated again and again under the same conditions.

Population is hypothetical

Models almost always involve unknown parameters!

Models are almost always an approximation!

“Simplest” statistical inference context: DIAGNOSIS example

- $x = 0, 1$ 
  - Data (to be observed) — results of experiment/observation
  - (Binary) Sample Space  $x \in \{0, 1\}$  — discrete, simple
  - test outcome
- $\theta = 0, 1$ 
  - Parameter, “Truth,” state of nature, uncertain/unknown
  - Primary objective: Inference on  $\theta$
  - (Binary) Parameter Space  $\theta \in \{0, 1\}$  – discrete, simple
  - Disease state
- Uncertainty about outcomes described by **probabilities**  $p(x|\theta)$  (conditional on  $\theta$ )

NOTATION:  $p(x|\theta)$ ,  $p(x = 1|\theta = 1)$  ,  $Pr(x = 1|\theta = 1)$

$p(x|\theta)$  defines the **Sampling model**

Where do these probabilities come from?

- Measures of **likelihood** of observed data given states of nature
  - $x = 1$  is 9.9 times as likely when  $\theta = 1$  than when  $\theta = 0$
  - Likelihood ratios  $r(x) = p(x|\theta = 1)/p(x|\theta = 0)$
  - $r(1) = 9.9$ ,  $r(0) = 0.0111\dots$
- Evidence, Information to compare  $\theta = 1 : 0$  given data  $x$

- Odds of disease (initial or prior odds),  $\text{Odds} = \text{Prob}/(1-\text{Prob})$

or

$$o(\theta = 1) = \frac{p(\theta = 1)}{1 - p(\theta = 1)} = \frac{p(\theta = 1)}{p(\theta = 0)}$$

- $\text{Prob} = \text{Odds}/(1+\text{Odds})$  or  $p(\theta = 1) = o(\theta = 1)/(1 + o(\theta = 1))$

$$p(\theta = 1) = .5 \text{ and } o(\theta = 1) = 1$$

$$p(\theta = 1) = 0.01 \text{ and } o(\theta = 1) = 0.0101\dots$$

$$p(\theta = 1) = 0.99 \text{ and } o(\theta = 1) = 99.$$

JOINT PROBABILITY DISTRIBUTION:  $p(x, \theta) = p(x|\theta)p(\theta)$

- Marginal distribution for  $x$  via *Law of Total Probability*:

$$p(x) = p(x|\theta = 0)p(\theta = 0) + p(x|\theta = 1)p(\theta = 1)$$

- Bayes' theorem:

$$p(\theta|x) = \frac{p(\theta)p(x|\theta)}{p(x)}$$

- Bayes' theorem reverses conditioning
- Now  $x$  is fixed, conditioned upon
- Initial/prior probability distribution for  $\theta$  is revised/updated
- Final probability = Posterior probability  $p(\theta|x)$

In odds form,

$$o(\theta = 1|x) = o(\theta = 1)r(x)$$

based on observed likelihood ratio  $r(x) = p(x|\theta = 1)/p(x|\theta = 0)$

Posterior odds = Prior odds  $\times$  Likelihood ratio

- Scientific learning/Statistical Inference:  $p(\theta) \rightarrow p(\theta|x)$
- Mapping via **likelihood function**  $p(x|\theta)$  from sampling model
- Formally,  $p(\theta|H) \rightarrow p(\theta|H, x)$  where  $H$  = prior information
- *Technically*: Simply apply Bayes' theorem

“Final” inferences depend on both data  $x$  and prior information  $H$

**Sensitivity to prior:**

- Any prior  $a = p(\theta = 1)$  implies posterior  $p(\theta|x)$
- Variation with respect to  $a$  for given data, hence fixed  $r(x)$  ... ?
- graph  $p(\theta = 1|x)$  versus  $a$

## Statistics By Example

Your mischievous brother owns a coin which you know to be loaded so that it comes up heads 70% of the time. He comes to you with a coin and wants to make a bet with you. You don't know if this is the loaded coin or a fair one. He lets you flip it 5 times to check it out, and you get 2 heads and 3 tails. Which coin do you think it is?

1. What is our **unknown parameter of interest**?
2. What are our **data**?
3. What is our **probability model** for the data?
4. How do we **estimate** our unknown parameter?

## The Unknown Parameter of Interest

We want to know **which coin it is**. We could denote the coin type by  $\theta$ , where  $\theta \in \{\text{fair}, \text{loaded}\}$ .

## The Data

Our **data** are the **5 flips** of the coin. The **observed values** are **2 heads and 3 tails**. We could write  $X$  for the number of heads observed in 5 flips.

## The Probability Model for the Data

We use a **binomial model** for the number of heads, where the probability of a single flip being heads equals  $\theta$  and flips are independent of outcomes of other flips:

$$P(X = x | \theta = \text{fair}) = \binom{5}{x} \left(\frac{1}{2}\right)^5$$

$$P(X = x | \theta = \text{loaded}) = \binom{5}{x} (.7)^x (.3)^{5-x}$$

For a particular value of  $\theta$ , the model gives us a probability model for  $X$  (the probability of observing different values of  $x$ ). Once we have observed  $X$  (here  $X = 2$ ), we can think of the probability model as a function of  $\theta$  alone. This gives the probability of the particular observed data as a function of  $\theta$ . This function is called the **likelihood** and is denoted  $L(\theta)$ .

## Estimating the Unknown Parameter - I

One way to make statements about the value of  $\theta$  is **Maximum Likelihood**. The idea is that our best guess of the true value of  $\theta$  is the value that maximizes the likelihood.

$$L(\theta|X = 2) = \begin{cases} 0.3125 & \text{if } \theta = \text{fair} \\ 0.1323 & \text{if } \theta = \text{loaded} \end{cases}$$

So in this example, we would guess that the coin is fair.

Some problems with this approach:

- It can be hard to make useful statements of our confidence/estimation error. (In particular, we can't give a probability of being right.)
- It can be hard to incorporate other information.

## Estimating the Unknown Parameter - II

A different (and our preferred) approach to making statements about the value of  $\theta$  is the **Bayesian** approach. In this case we express prior uncertainty about  $\theta$  using a probability distribution. We start with a **prior** distribution for  $\theta$ ,  $P(\theta)$ , (representing our beliefs before seeing the data). We then observe the data and compute a **posterior** distribution,  $P(\theta|X)$ , by using **Bayes' Theorem**.

$$\begin{aligned} P(\theta|X) &= \frac{P(\theta, X)}{P(X)} \\ &= \frac{P(X|\theta)P(\theta)}{\int P(X|\theta)P(\theta)d\theta} \\ &\propto L(\theta)P(\theta) \end{aligned}$$

## Example

Since you know your brother reasonably well, you might think that there is a 60% chance that this is the loaded coin, before you get to flip it 5 times. You then get 2 heads and 3 tails. What is your posterior probability that the coin is loaded?

$$\begin{aligned} Pr(\text{loaded}|X) &= \\ &= \frac{Pr(X|\text{loaded})Pr(\text{loaded})}{Pr(X|\text{loaded})Pr(\text{loaded}) + Pr(X|\text{fair})Pr(\text{fair})} \\ &= \frac{\binom{5}{2}(.7)^2(.3)^3(.6)}{\binom{5}{2}(.7)^2(.3)^3(.6) + \binom{5}{2}(.5)^2(.5)^3(.4)} \\ &= \frac{0.07938}{0.07938 + 0.125} = 0.388 \end{aligned}$$

## Prior Sensitivity

We could have made a different choice for a prior:

- $Pr(\text{loaded}) = .9 \Rightarrow Pr(\text{loaded}|X) = 0.792$
- $Pr(\text{loaded}) = .5 \Rightarrow Pr(\text{loaded}|X) = 0.297$

Note that the “non-informative” prior gives the “same” answer as the MLE. Also note that since the sample size is small, the result is highly sensitive to the choice of prior. As the sample size increases, the prior matters less.

Advantages of this approach:

- Our answer is a probability distribution for  $\theta$ , so we can make probability statements and error estimates.
- We can incorporate other information into the prior.

