Contents

Introduction to Bayesian Modeling

2.1 Probability as quantification of uncertainty about observables. Binary outcomes

Case Study: *Hospital-specific prediction of mortality rates*. Let's say you're interested in measuring the **quality of care** (e.g., Kahn et al., 1990) offered by one particular hospital.

I am thinking of the **Dominican Hospital** (DH) in Santa Cruz, CA; you may have a different hospital in mind.

As part of this you decide to examine the medical records of all patients treated at the DH in one particular time window, say **January 2002–December 2005**, for one particular medical condition for which there is a strong *process-outcome link*, say **acute myocardial infarction (AMI; heart attack)**.

(**Process** is what health care providers do on behalf of patients; **outcomes** are what happens as a result of that care.)

In the time window you're interested in there will be about $n = 400$ **AMI patients** at the DH.

The Meaning of Probability

To keep things simple let's ignore process for the moment and focus here on one particular outcome: **death status (mortality)** as of 30 days from hospital admission, coded 1 for dead and 0 for alive.

(In addition to process this will also depend on the **sickness at admission** of the AMI patients, but for simplicity let's ignore that initially too.)

From the vantage point of December 2001, say, **what may be said** about the roughly 400 1s and 0s you will observe in 2002–05?

The meaning of probability. You are definitely **uncertain** about the 0–1 death outcomes $Y_1, \ldots, Y_n$ before you observe any of them.

Probability is supposed to be the part of mathematics concerned with quantifying uncertainty; can probability be used here?

In part 1 I argued that the answer was **yes**, and that three types of probability—**classical**, **frequentist**, and **Bayesian**—are available (in principle) to quantify uncertainty like that encountered here.

2.2 Review of Frequentist Modeling

I'll focus on the approaches with the most widespread usage—**frequentist** and **Bayesian**—in what follows.

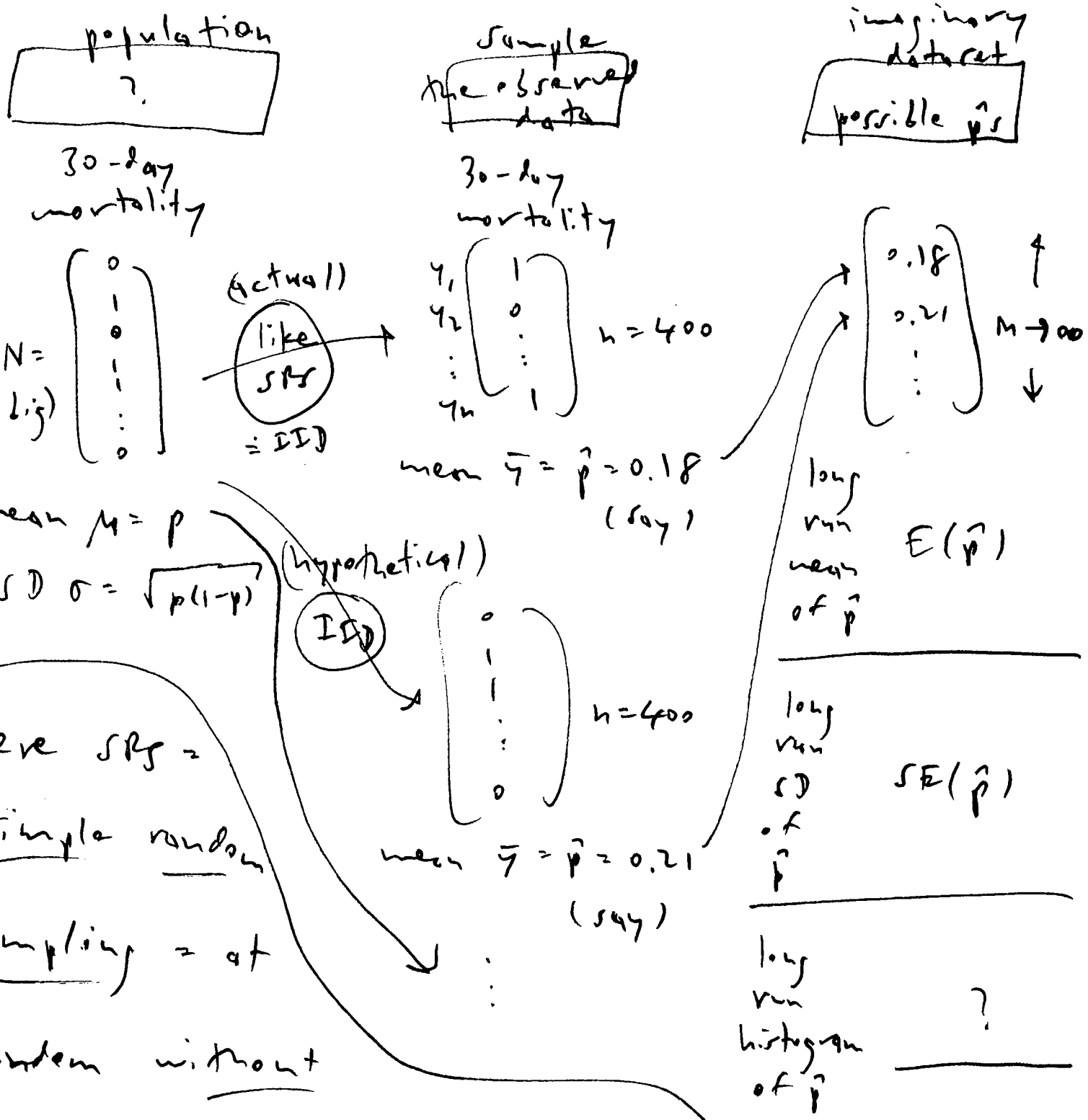
How can the **frequentist** definition of probability be applied to the hospital mortality problem?

By definition the frequentist approach is based on the idea of **hypothetical or actual repetitions** of the process being studied, under conditions that are as close to **independent identically distributed (IID)** sampling as possible.

When faced with a data set like the 400 1s and 0s $(Y_1, \ldots, Y_n)$ here, the usual way to do this is to think of it **as a random sample**, or **like** **a random sample**, from some **population** that is of direct interest to you.

Then the **randomness** in your probability statements refers to the **process** of what you might get if you were to repeat the sampling over and over—the Y_i become **random variables** whose probability distribution is determined by this hypothetical repeated sampling.

Frequentist Modeling (continued)



Here SRS = simple random sampling = at random without replacement; when $n << N$ (n is a lot smaller than N), SRS ≈ IID = at random with replacement.

Frequentist Modeling (continued)

On the previous page **SD** stands for **standard deviation**, the most common measure of the extent to which the observations y_i in a data set **vary**, or are spread out, around the center of the data.

The **center** is often measured by the mean $\bar{y} = \frac{1}{n}\sum_{i=1}^{n} y_i$, and the SD of a sample of size n is then given by

$$SD = \sqrt{\frac{1}{n-1}\sum_{i=1}^{n}(y_i - \bar{y})^2}. \quad (1)$$

The **population size** is denoted by N; this is often much larger than the **sample size** n.

With 0/1 (**dichotomous**) data, like the mortality outcomes in this case study, the population mean μ simply records the **proportion** p of 1s in the population (check this), and similarly the sample mean $\bar{y}$ keeps track automatically of the **observed death rate** $\hat{p}$ in the sample.

As $N \to \infty$ the **population SD** σ with 0/1 data takes on a **simple form** (check this):

$$\sigma = \sqrt{p(1-p)}. \quad (2)$$

It's common in frequentist modeling to make a notational distinction between the **random variables** Y_i (the placeholders for the process of making IID draws from the population over and over) and the **values** y_i that the Y_i might take on (although I'll abuse this notation with $\hat{p}$ below).

Frequentist Modeling (continued)

In the diagram on page 6 the **relationship between the population and the sample** data sets can be usefully considered in each of two directions:

- If the population is known you can think about how the sample is likely to come out under IID sampling—this is a **probability** question.

Here in this case p would be known and you're trying to figure out the **random behavior** of the sample mean $\bar{Y} = \hat{p}$.

- If instead only the sample is known your job is to infer the likely composition of the population that could have led to this IID sample—this is a question of **statistical inference**.

In this problem the sample mean $\bar{y} = \hat{p}$ would be known and your job would be to **estimate** the population mean p.

Suppose that $N \gg n$, i.e., that even if SRS was used you are effectively dealing with IID sampling.

Intuitively both SRS and IID should be "good"—**representative**—sampling methods, so that $\hat{p}$ should be a "good" estimate of p, but what exactly does the word "**good**" mean in this sentence?

Evidently a good estimator $\hat{p}$ would be **likely to be close to the truth** p, especially with a lot of data (i.e., if n is large).

In the frequentist approach to inference quantifying this idea involves imagining how $\hat{p}$ would have come out if the **process** by which the observed $\hat{p} = 0.18$ came to you were **repeated** under IID conditions.

This gives rise to the **imaginary data set**, the third part of the diagram on page 6: we consider **all possible** $\hat{p}$ values based on an IID sample of size n from a population with $100p\%$ 1s and $100(1-p)\%$ 0s.

Frequentist Modeling (continued)

Let M be the **number of hypothetical repetitions** in the imaginary data set.

The long-run mean (as $M \to \infty$) of these imaginary $\hat{p}$ values is called the **expected value** of the random variable $\hat{p}$, written $E(\hat{p})$ or $E_{\text{IID}}(\hat{p})$ to emphasize the mechanism of drawing the sample from the population.

The long-run SD of these imaginary $\hat{p}$ values is called the **standard error** of the random variable $\hat{p}$, written $SE(\hat{p})$ or $SE_{\text{IID}}(\hat{p})$.

It's natural in studying how the hypothetical $\hat{p}$ values vary around the center of the imaginary data set to make a **histogram** of these values: this is a plot with the possible values of $\hat{p}$ along the horizontal scale and the frequency with which $\hat{p}$ takes on those values on the vertical scale.

It's helpful to draw this plot on the **density scale**, which just means that the vertical scale is chosen so that the total area under the histogram is 1.

The long-run histogram of the imaginary $\hat{p}$ values on the density scale is called the **(probability) density** of the random variable $\hat{p}$.

The values of $E(\hat{p})$ and $SE(\hat{p})$, and the basic shape of the density of $\hat{p}$, can be determined **mathematically** (under IID sampling) and verified by **simulation**.

It turns out that

$$E_{\text{IID}}(\hat{p}) = p \quad \text{and} \quad SE_{\text{IID}}(\hat{p}) = \frac{\sigma}{\sqrt{n}} = \sqrt{\frac{p(1-p)}{n}}, \qquad (3)$$

and the density of $\hat{p}$ for large n is well approximated by the **normal curve** or **Gaussian distribution** (this result is the famous **Central Limit Theorem (CLT)**).

Frequentist Modeling (continued)

Suppose the sample of size $n = 400$ had **72 1s** and 328 0s, so that $\hat{p} = \frac{72}{400} = \mathbf{0.18}$.

Thus you would estimate that the population mortality rate p is **around 18%**, but how much uncertainty should be attached to this estimate?

The above standard error formula is not directly usable because it involves the unknown p, but we can **estimate** the standard error by plugging in $\hat{p}$:

$$\widehat{SE}(\hat{p}) = \sqrt{\frac{\hat{p}(1-\hat{p})}{n}} = \sqrt{\frac{(0.18)(0.82)}{400}} \doteq 0.019. \qquad (4)$$

In other words, I think p is around **18%**, give or take about **1.9%**.

A **probabilistic uncertainty band** can be obtained with the frequentist approach by appeal to the CLT, which says that (for large n) in repeated sampling $\hat{p}$ would fluctuate around p like draws from a normal curve with mean p and SD (SE) 0.019, i.e.,

$$\begin{aligned} 0.95 &\doteq P_F\left[p - 1.96\,\widehat{SE}(\hat{p}) \le \hat{p} \le p + 1.96\,\widehat{SE}(\hat{p})\right] \\ &= P_F\left[\hat{p} - 1.96\,\widehat{SE}(\hat{p}) \le p \le \hat{p} + 1.96\,\widehat{SE}(\hat{p})\right]. \end{aligned} \qquad (5)$$

Thus (Neyman 1923) a 95% (frequentist) **confidence interval** for p runs from $\hat{p} - 1.96\,\widehat{SE}(\hat{p})$ to $\hat{p} + 1.96\,\widehat{SE}(\hat{p})$, which in this case is from $0.180 - (1.96)(0.019) = 0.142$ to $0.180 + (1.96)(0.019) = 0.218$, i.e., I am **"95% confident that p is between about 14% and 22%"**.

But **what does this mean**?