

# 1 (Binomial) Model for (Sampling) Variability of Proportion/Count in a Sample

## The Binomial Distribution: what it is

- The  $n + 1$  probabilities  $p_0, p_1, \dots, p_y, \dots, p_n$  of observing  $0, 1, 2, \dots, n$  “positives” in  $n$  independent binary trials (*such as in s.r.s of  $n$  individuals*)
- Each of the  $n$  observed elements is binary (0 or 1)
- There are  $2^n$  possible *sequences* ... but only  $n + 1$  possible *values*, i.e.  $0/n, 1/n, \dots, n/n$  (*can think of  $y$  as sum of  $n$  Bernoulli r. v.’s*)
- Apart from sample size ( $n$ ), the probabilities  $p_0$  to  $p_n$  depend on only 1 parameter:
  - the probability that a selected individual will be ‘+ve’ i.e.,
  - the proportion of “+ve” individuals in sampled population
- Usually denote this (un-knowable) proportion by  $\pi$  (sometimes  $\theta$ )<sup>1</sup>

Author	Parameter	Statistic
C & H	$\pi$	$p = D/N$
Hanley et al.	$\pi$	$p = y/n$
M & M	$p$	$\hat{p} = y/n$
Miettinen	$P$	$p = y/n$

- Shorthand:  $y \sim \text{Binomial}(n, \pi)$ .

## How it arises

- Sample Surveys
- Clinical Trials
- Pilot studies
- Genetics
- Epidemiology ...

<sup>1</sup>M & M use  $p$  for *population* proportion and  $\hat{p}$  or “ $p$ -hat” for observed proportion in a *sample*. Others use Greek letter  $\pi$  for population value (parameter) and  $p$  for the sample proportion. Greek letters make the distinction clearer, some textbooks do not use them consistently: e.g., for the population proportion and population mean respectively, M & M use the Arabic letter  $p$  and the Greek letter  $\mu$  (mu)! Some authors (e.g., Miettinen) use UPPER-CASE letters, [e.g.  $P$ ,  $OR$ ] for PARAMETERS and lower-case letters [e.g.,  $p$ ,  $or$ ] for statistics (*estimates* of parameters).

## Use

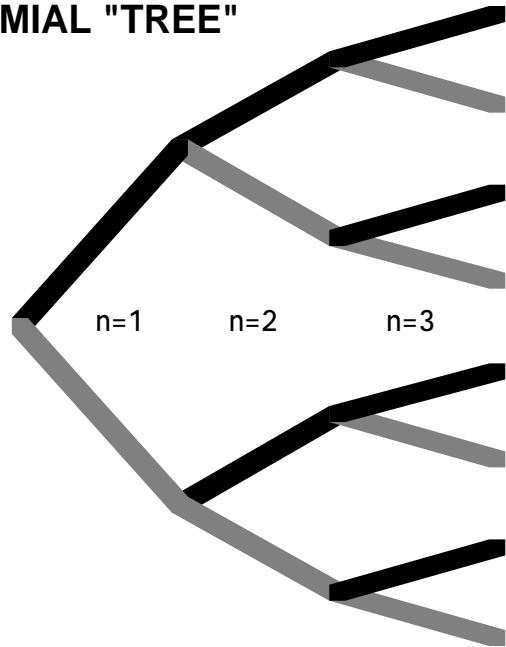
- to make inferences about  $\pi$  from observed proportion  $p = y/n$ .
- to make inferences in more complex situations, e.g. ...
  - Prevalence Difference:  $\pi_1 - \pi_0$
  - Risk Difference (RD):  $\pi_1 - \pi_0$
  - Risk Ratio, or its synonym Relative Risk (RR):  $\pi_1 / \pi_0$
  - Odds Ratio (OR):  $[\pi_1 / (1 - \pi_1)] / [\pi_0 / (1 - \pi_0)]$
  - Trend in several  $\pi$ ’s

## Requirements for $y$ to have a Binomial ( $n, \pi$ ) distribution

- Each element in “population” is 0 or 1, but we are only interested in estimating proportion ( $\pi$ ) of 1’s; we are not interested in individuals.
- Fixed sample size  $n$ .
- Elements selected at random and independent of each other; each element in population has same probability of being sampled.
- Denote by  $y_i$  the value of the  $i$ -th sampled element.  $\text{Prob}[y_i = 1]$  is constant (it is  $\pi$ ) across  $i$ . It helps to distinguish the  $N$  *population* values  $Y_1$  to  $Y_N$  from the  $n$  *sampled* values  $y_1$  to  $y_n$ . In the ‘What proportion of our time do we spend indoors?’ example (in Resources), it is the *random* sampling of the temporal and spatial patterns of 0s and 1s that makes  $y_1$  to  $y_n$  independent of each other. The  $Y$ s, the elements in the population can be related to each other [e.g. spatial distribution of persons] but if elements are chosen at random, the chance that the value of the  $i$ -th element chosen is a 1 cannot depend on the value of  $y_{i-1}$  or any other  $y$ : the sampling is ‘blind’ to the spatial location of the 1’s and 0s.

**Binomial Tree (overleaf):** Even though there are  $2^n$  possible sequences of 0s and 1s, each with its probability, the calculation of the probability that the sequence in the selected sample contains  $y$  1’s and  $(n - y)$  0’s is greatly simplified by the fact that  $\text{Prob}[y_1 = 1] = \text{Prob}[y_2 = 1] = \dots = \text{Prob}[y_n = 1] = \pi$ . Thus we can calculate prob. of any one sequence that contains  $y$  1’s and  $(n - y)$  0’s. Since all the sequences have same probability, namely  $\pi^y(1 - \pi)^{n-y}$ , we can, in lieu of adding all such probabilities, simply multiply this one probability by the number,  ${}^nC_y$ , of unique paths to terminal node.

### BINOMIAL "TREE"



### EXAMPLE WITH $\pi = 0.5$

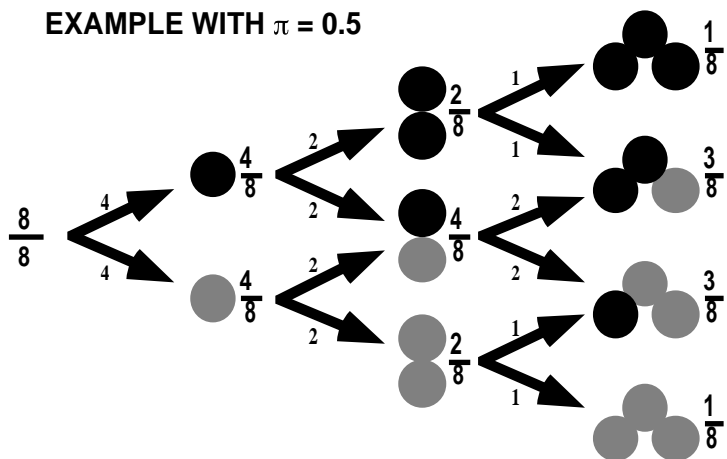


Figure 1: Binomial( $n = 3, \pi = 0.5$ )

## 1.1 Does the Binomial Distribution Apply if... ?

Interested in	$\pi$	the proportion of 16 year old girls in Québec protected against rubella
Choose	$n = 100$	girls: 20 at random from each of 5 randomly selected schools ['cluster' sample]
Count	$y$	how many of the $n = 100$ are protected
• Is $y \sim \text{Binomial}(n = 100, \pi)$ ?		
"SMAC"	$\pi$	Prob['abnormal'   Healthy] = 0.03 for each chemistry in Auto-analyzer with $n = 18$ channels
Count	$y$	How many of $n = 18$ give abnormal result.
• Is $y \sim \text{Binomial}(n = 18, \pi = 0.03)$ ? (cf. Ingelfinger: Clin. Biostatistics)		
Interested in	$\pi_u$ $\pi_e$	proportion in 'usual' exercise classes and in expt'l. exercise classes who 'stay the course'
Randomly Allocate	4 <u>25</u> $n_u = 100$ 4 <u>25</u> $n_e = 100$	classes of students each to usual course classes of students each to experimental course
Count	$y_u$ $y_e$	how many of the $n_u = 100$ complete course how many of the $n_e = 100$ complete course
• Is $y_u \sim \text{Binomial}(n_u = 100, \pi_u)$ ? Is $y_e \sim \text{Binomial}(n_e = 100, \pi_e)$ ?		
Sex Ratio	$n = 4$ $y$	children in each family number of girls in family
• Is variation of $y$ across families Binomial ( $n = 4, \pi = 0.49$ )?		
Pilot Study		To estimate proportion $\pi$ of population that is eligible & willing to participate in long-term research study, keep recruiting until obtain who are. Have to approach $n$ to get $y$ .
• Can we treat $y \sim \text{Binomial}(n, \pi)$ ?		

## 1.2 Calculating Binomial probabilities:

### Exactly

- pdf: formula  $\text{Prob}[y] = {}^n C_y \pi^y (1 - \pi)^{n-y}$ .
- cdf:
  - direct summation of terms in pdf
  - Using link between this sum and the cdf of the  $F$  distribution<sup>2</sup>.
- Tables: CRC; Fisher and Yates; Biometrika Tables; Documenta Geigy
- Spreadsheet — Excel function `BINOMDIST(y, n, π, cumulative)`
- Statistical Packages:
  - SAS `PROBBNML(p, n, y)` function
  - Stata function `Binomial(n,k,p)`
  - R functions `dbinom()`, `pbinom()`, `qbinom()`: probability, distribution, and quantile functions.

### Using an approximation

- Poisson Distribution ( $n$  large; small  $\pi$ )
- Normal (Gaussian) Distribution ( $n$  large or midrange  $\pi$ )
  - Have to specify *scale* i.e., if say  $n = 10$ , whether summary is a

	r.v.	e.g.	E	SD
count:	$y$	2	$n \times \pi$	$\{n \times \pi \times (1 - \pi)\}^{1/2}$ $n^{1/2} \times \sigma_{Bernoulli}$
proportion:	$p = y/n$	0.2	$\pi$	$\{\pi \times (1 - \pi)/n\}^{1/2}$ $\sigma_{Bernoulli}/n^{1/2}$
percentage:	$100p\%$	20%	$100 \times \pi$	$100 \times SD[p]$

– same core calculation for all 3 [only the *scale* changes]

<sup>2</sup>Fisher 1935: see Resources

## 2 Inference concerning a proportion $\pi$ , based on s.r.s. of size $n$

The **Parameter**  $\pi$  of interest: the proportion, e.g., ...

- with undiagnosed hypertension / seeing MD during a 1-year span
- who would respond to a specific therapy
- still breast-feeding at 6 months
- of pairs where response on treatment > response on placebo
- of US presidential elections where taller candidate expected to win
- of twin pairs where left-handed twin dies first
- able to tell imported from domestic beer in a “triangle taste test”
- who get a headache after drinking red wine
- of all in an RCT who would become HPV-infected, what proportion of them had been vaccinated:

e.g. in RCT of HPV16 Vaccine, NEJM Nov 21, 2002: 0 seroconversions in 11084.0 W-Y in vaccinated group vs. 41 in 11076.9 W-Y in placebo group.

[this proportion is a function of the parameter of interest, the efficacy of the vaccination]

Inference via **Statistic**: the number ( $y$ ) or proportion  $p = y/n$  ‘positive’ in an s.r.s. of size  $n$ .

### Frequentist

- based on  $\text{prob}[\text{data} | \theta]$ , i.e.
- probability statements about data

Evidence (P-value) against  $H_0: \pi = \pi_0$   
 Test of  $H_0$ : Is P-value < (preset)  $\alpha$ ?  
 CI: interval estimate

### Bayesian

- based on  $\text{prob}[\theta | \text{data}]$ , i.e.,
- probability statements about  $\pi$

- point estimate: (mean/median/mode) of posterior distribution of  $\pi$   
 - (credible) interval

See “Bayesian Inference for a Proportion (Excel)” under Resources Ch 8 cf also A&B §4.7; Colton §4.

## 2.1 (Frequentist) Confidence Interval for $\pi$ , based on an observed proportion $p = y/n$

### 2.1.1 “Exact”, first-principles, Confidence Interval

This is not as “awkward to work with” as M & M p. 586 imply.

*Example 1*

Q: What fraction  $\pi$  of a population would return a 4-page questionnaire?

Data: In a pilot test, ( $y =$ ) 11 of ( $n =$ ) 20 return it, i.e.  $p = \hat{\pi} = 11/20 = 0.55$

**Logic behind the exact Clopper-Pearson  $100(1 - \alpha)\%$  CI:**

Limits are calculated so that Binomial Prob [ $\geq y \mid \pi_{lower}$ ] = Prob [ $\leq y \mid \pi_{upper}$ ] =  $\alpha/2$ , exactly. [See Biometrika Tables for Statisticians]

*How one can obtain exact CI:*

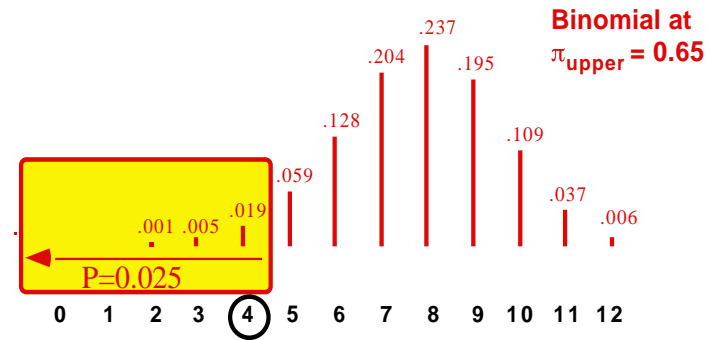
- Tables [Homegrown table<sup>3</sup>, Documenta Geigy, Biometrika Tables]
- Nomograms<sup>4</sup> (see example on next page).  
 Read **horizontally**, nomogram shows the variability of proportions from s.r.s samples of size  $n$ . Read **vertically**, it shows: (i) CI  $\rightarrow$  symmetry as  $p \rightarrow 0.5$  or  $n \rightarrow \infty$  [in fact, as  $n \times p$  &  $n(1 - p) \rightarrow \infty$ ] (ii) widest uncertainty at  $\pi = 0.5 \Rightarrow$  can use as a ‘worst case scenario’
- software
  - by trial and error, via the Binomial cdf in Excel, R, SAS

<sup>3</sup>To save space, table gives CI’s only for  $p \leq 0.5$ , so get CI for  $\pi$  of non-returns: point estimate is 9/20 or 45%, CI is 23% to 68% (1st row, middle column of the  $X = 9$  block. Turn this back to  $100 - 68 = 32\%$  to  $100 - 23 = 77\%$  returns]

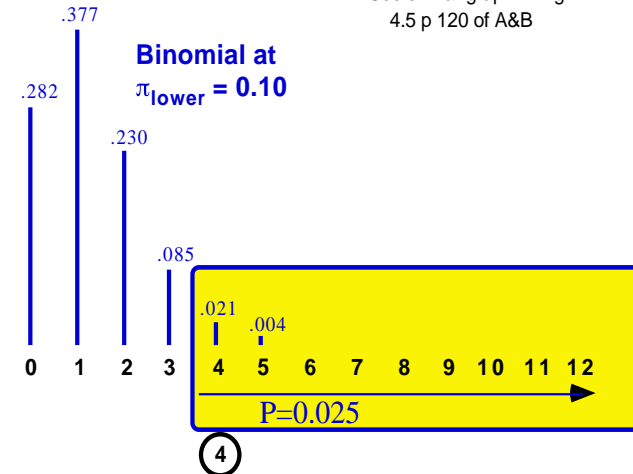
<sup>4</sup>95% CI (Biometrika nomogram) 32% to 77%. Nomogram uses  $c$  for numerator; enter through lower  $x$ -axis if  $p \leq 0.5$ ; in our case  $p = 0.55$  so enter nomogram from the top at  $\frac{c}{n} = 0.55$  near upper right corner; travel downwards until you hit bowed line marked 20 (the 5th line from the top) and exit towards the rightmost border at  $\pi_{lower} \approx 0.32$ ; go back and travel downward until hit the companion bowed line marked 20 (the 5th line from bottom) and exit towards the rightmost border at  $\pi_{upper} \approx 0.77$ .

Notice link between  $100(1 - \alpha)\%$  CI and two-sided test of significance with a preset  $\alpha$ . If true  $\pi$  were  $< \pi_{lower}$ , there would only be less than a 2.5% probability of obtaining, in a sample of 20, this many (11) or more respondents; likewise, if true  $\pi$  were  $> \pi_{lower}$ , there would be less than a 2.5% probability of obtaining, in a sample of 20, this many (11) or fewer respondents. The  $100(1 - \alpha)\%$  CI for  $\pi$  includes all those parameter values such that if the observed data were tested against them, the p-value (2-sided) would not be  $< \alpha$ .

**Clopper-Pearson 95% CI for  $\pi$  based on observed proportion 4/12**



See similar graph in Fig 4.5 p 120 of A&B



Notice that Prob[4] is counted twice, once in each tail.

The use of CI’s based on Mid-P values, where Prob[4] is counted only once, is discussed in Miettinen’s Theoretical Epidemiology and in §4.7 of Armitage and Berry’s text.

Figure 2: Logic behind Clopper-Pearson CI for proportion  $\pi$

- using function that gives the inverse of the cdf of the F distribution.<sup>5</sup>
- directly in R, Stata etc

*Example 2*

Experimental drug gives  $y = 0$  successes in  $n = 14$  patients<sup>6</sup>;  $\Rightarrow \pi = ??$

**2.1.2 “Approximate”, first-principles, Confidence Interval**

$$\pi = \frac{1 - \frac{n}{n+z^2} + \frac{2np}{n+z^2} \pm \frac{z\sqrt{4np-4np^2+z^2}}{n+z^2}}{2}$$

This asymmetric CI (see later) was used to create CI’s in nomogram below.

**2.1.3 CI based on Gaussian approximation to sampling distribution of  $p$ , or function of  $p$**

- **CI:**  $p \pm z \times SE[p] = p \pm z \times \{p(1-p)\}^{1/2}$
- e.g.  $p = y/n = 300/1000 = 0.3$
- 95% CI:  $0.30 \pm 1.96 \times \{0.3 \times 0.7/1000\}^{1/2} = 0.30 \pm 0.03 = 30\% \pm 3.0\%$
- **NB:** The  $\pm 3.0\%$  is pronounced and written as “ $\pm 3$  percentage points” to avoid giving the impression that it is 3% of 30%.
- **“Large- $n$ ”: How Large is large?**
  - A rule of thumb: when the expected no. of positives,  $n \times \pi$ , and the expected no. of negatives,  $n \times (1 - \pi)$ , are both bigger than 5 (or 10 if you read M & M).
  - JH’s rule: when you can’t find the CI tabulated anywhere!
  - if the distribution is not ‘crowded’ into one corner (cf. the shapes of binomial distributions in the Binomial spreadsheet – in Resources), i.e., if, with the symmetric Gaussian approximation, neither of the tails of the distribution spills over a boundary (0 or 1 if proportions,

<sup>5</sup>See spreadsheet “CI for a Proportion (Excel spreadsheet, based on exact Binomial model)” under Resources. In this sheet one can obtain the direct solution, or get there by trial and error. Inputs in bold may be changed. See article by Fisher

<sup>6</sup>95% CI for  $\pi$  (from table) 0% to 23%. CI “rules out” (with 95% confidence) possibility that  $\pi > 23\%$ . [might use a 1-sided CI if one is interested in putting just an upper bound on risk: e.g. what is upper bound on  $\pi =$  probability of getting HIV from HIV-infected dentist? see JAMA article on “zero numerators” by Hanley and Lippman-Hand (in Resources).

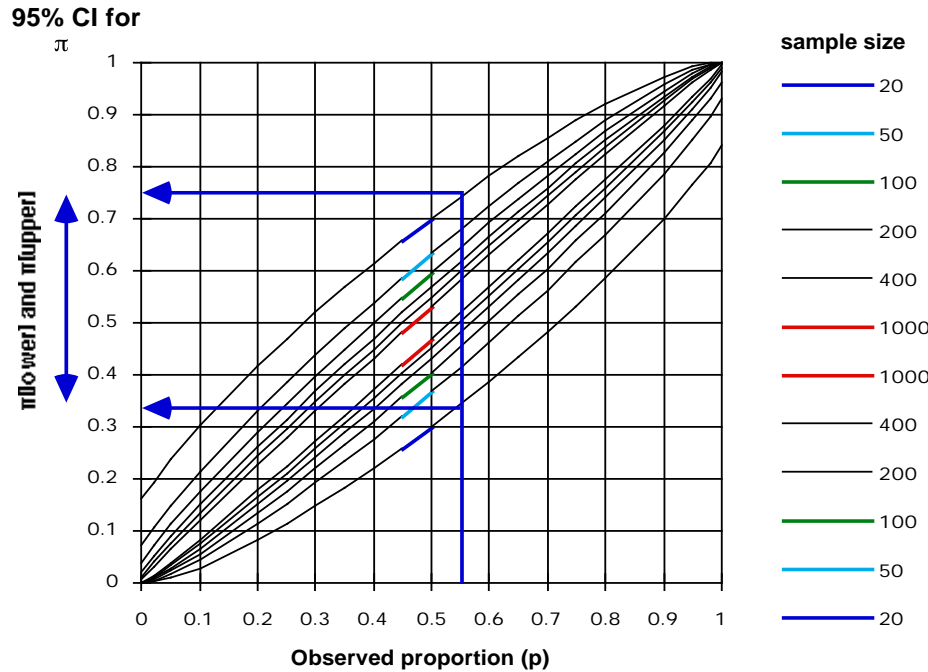


Figure 3: Small and large-sample Clopper-Pearson CIs for proportion  $\pi$

or beyond 0 or  $n$  if on the count scale), See M & M p383 and A&B §2.7 on Gaussian approximation to Binomial.

- SE-based (sometimes referred to in texts and software output as “Wald”) CI’s use the *same* SE for the *upper* and *lower* limits – they calculate one SE at the point estimate, rather than two separate SE’s, calculated at each of the two limits.

• In SAS

```
DATA CI_propn;
INPUT n_pos n;
LINES;
300 1000;
PROC genmod data = CI_propn; model n_pos/n = /
dist = binomial link = identity waldci; RUN;
```

• In Stata

```
immediate command: cii 1000 300
clear *Using datafile
input n_pos n
140 500 * glm doesn't like file with 1 'observation'
160 500 *so ..... split across 2 'observations'
end
glm n_pos , family (binomial n) link (identity)
```

2.1.4 Other, more accurate and more theoretically correct, large-sample (Gaussian-based constructions)

The “usual” approach is to form a symmetric CI as

$$\text{point estimate} \pm \text{a multiple of SE}$$

This is technically incorrect in the case of a distribution, such as the binomial, with a variance that changes with the parameter being measured. In construction of CI’s [see diagram on page 1 of material on Ch 6.1] there are *two* distributions involved: the binomial at  $\pi_{upper}$  and the binomial at  $\pi_{lower}$ . They have different shapes and different SD’s in general. Approaches A and B (below) take this into account.

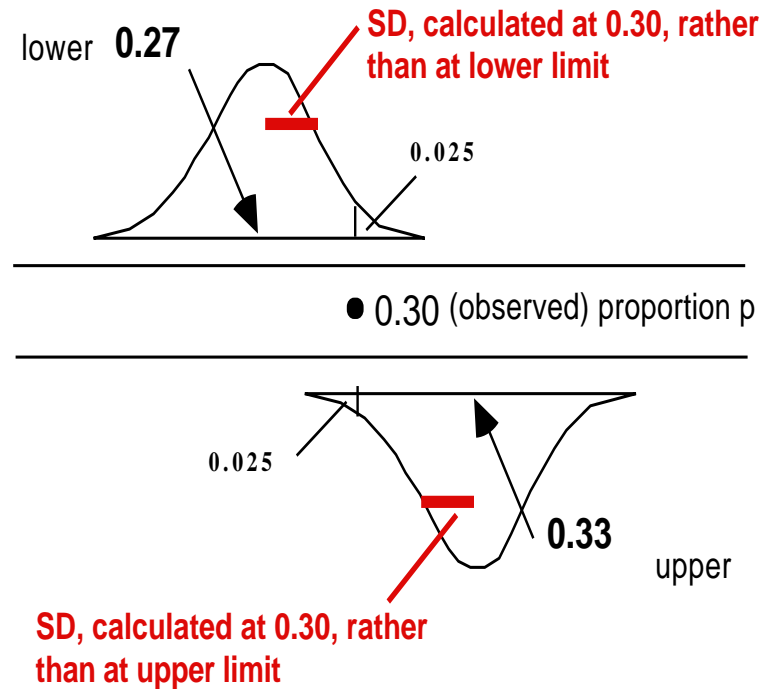


Figure 4: Closer to a ‘first-principles’ CI

**Method A.** Based on Gaussian approximation to binomial distribution, but with SD’s calculated separately at the lower limit  $\pi_{LOWER}$  and upper limit  $\pi_{UPPER}$  of  $\pi$ .

The “usual” (not-a-first-principles) CI uses a *common*  $SD = \{p[1 - p]/n\}^{1/2}$ , i.e. the one calculated at the point estimate, i.e., at  $\pi = p$ .

Define CI for  $\pi$  as the pair of  $\pi$  values  $\{\pi_{LOWER}, \pi_{UPPER}\}$  such that,

$$\text{Prob}[p \geq p_{obs} | \pi_{LOWER}] = \alpha/2 \quad \& \quad \text{Prob}[p \leq p - obs | \pi_{UPPER}] = \alpha/2.$$

Use Gaussian approximations to Binomial( $n, \pi_{LOWER}$ ) and

Binomial( $n, \pi_{UPPER}$ ), and solve

$$p = \pi_{LOWER} + z_{\alpha/2} \{ \pi_{LOWER} \times [1 - \pi_{LOWER}] / n \}^{1/2}$$

for  $\pi_{LOWER}$  and

$$p = \pi_{UPPER} + z_{\alpha/2} \{ \pi_{UPPER} \times [1 - \pi_{UPPER}] / n \}^{1/2}$$

for  $\pi_{UPPER}$ .

This leads to **asymmetric**  $100(1 - \alpha)\%$  limits of the form<sup>7</sup>

$$\{ \pi_{LOWER}, \pi_{UPPER} \} = \frac{1 - \frac{n}{n+z^2} + \frac{2np}{n+z^2} \pm \frac{z\sqrt{4np-4np^2+z^2}}{n+z^2}}{2}$$

Rothman (2002 - p.132) attributes this method ?? to Wilson 1907

**Method B:** Based on Gaussian distribution of a **variance-stabilizing transformation** of the binomial, again with SD’s calculated at the limits rather than at the point estimate itself.

$$\sin[\sin^{-1}[\sqrt{p}] - \frac{z}{2\sqrt{n}}]^2,$$

$$\sin[\sin^{-1}[\sqrt{p}] + \frac{z}{2\sqrt{n}}]^2$$

as in most calculators,  $\sin^{-1}$  & the \* in  $\sin[*]$  measured in radians.

**E.g. with  $\alpha = 0.05$ , so that  $z = 1.96$ , we get:**

Method	n = 10 p = 0.0	n = 10 p = 0.3	n = 20 p = 0.15	n = 40 p = 0.075
1	[0.00, 0.28]	[0.11, 0.60]	[0.05, 0.36]	[0.03, 0.20]
2	[0.09, 0.09]	[0.07, 0.60]	[0.03, 0.33]	[0.01, 0.18]
“usual”	[0.00, 0.00]	[0.02, 0.58]	[-0.01, 0.31]	[-0.01, 0.16]
Binomial*	[0.00, 0.31]	[0.07, 0.65]	[0.03, 0.38]	[0.02, 0.20]

\* from Mainland

<sup>7</sup>References: Fleiss, Statistical Methods for Rates & Proportions; Miettinen, Theoretical Epidemiology, Ch 10. See also `binconf` function in (downloadable) `Hmisc` library in R.

**Method C:** Based on **Gaussian distribution of the logit transformation** of the estimate ( $p$ , the observed proportion) and of the parameter  $\pi$ .

**Parameter:** <sup>8</sup>

$$\text{logit}\{\pi\} = \log\{ODDS\}^9 = \log\left\{\frac{\pi}{(1-\pi)}\right\} = \log\left\{\frac{\text{PROPORTION “Positive”}}{\text{PROPORTION “Negative”}}\right\}$$

$$\textbf{Statistic: } \text{logit}\{p\} = \log\{odds\} = \log\left\{\frac{\text{proportion “Positive”}}{\text{proportion “Negative”}}\right\}.$$

**Reverse transformation** (to get back from LOGIT to  $\pi$ ) ...

$$\pi = \frac{ODDS}{1 + ODDS} = \frac{\exp[LOGIT]}{1 + \exp[LOGIT]}$$

likewise...

$$p = \frac{odds}{1 + odds} = \frac{\exp[logit]}{1 + \exp[logit]}$$

$$\pi_{LOWER} = \frac{\exp\{\text{LOWER limit of LOGIT}\}}{1 + \exp\{\text{LOWER limit of LOGIT}\}} = \frac{\exp\{\text{logit} - z_{\alpha/2} SE[\text{logit}]\}}{1 + \exp\{\text{logit} - z_{\alpha/2} SE[\text{logit}]\}}$$

$\pi_{UPPER}$  likewise.

$$SE[\text{logit}] = \left\{ \frac{1}{\# \text{ positive}} + \frac{1}{\# \text{ negative}} \right\}^{1/2}$$

**e.g.**  $p = 3/10 \Rightarrow odds = 3/7 \Rightarrow logit = \log[3/7] = -0.85$ .

$$SE[\text{logit}] = \{1/3 + 1/7\}^{1/2} = 0.69$$

$$\Rightarrow 95\% \text{ CI in LOGIT}[\pi] \text{ scale: } -0.85 \pm 1.96 \times 0.69 = \{-2.2, 0.5\}$$

$$\Rightarrow \text{CI in } \pi \text{ scale: } \{\exp(-2.2)/(1 + \exp(-2.2)), \exp(0.5)/(1 + \exp(0.5))\}$$

SAS	Strata
DATA CI_propn;	clear
INPUT n_pos n;	input n_pos n
LINES;	1 5
3 10	2 5
;	end
PROC genmod;	glm n_pos,
model n_pos/n = /	family (binomial n) link (logit)
dist = binomial	
link = logit waldci;	
<hr/>	
Anti-logit[logit] = $\frac{\exp[\text{logit}]}{1 + \exp[\text{logit}]}$	Greenland calls it the “expit function”.

**Method D:** Based on Gaussian distribution of estimate of **log** [ $\pi$ ]

<sup>8</sup>UPPER CASE / Greek = parameter; lower case / Roman = statistic.

<sup>9</sup>Here,  $\log = \text{‘natural’ log}$ , i.e. to base e, which some write as  $\ln$ .

**Parameter:**  $\log[\pi]$

**Statistic:**  $\log[p]$

**Reverse transformation**  $\pi = \text{antilog}[\log[\pi]] = \exp[\log[\pi]]$

Likewise  $p \leftarrow \log[p]$

$\pi_{\text{LOWER/UPPER}} = \exp\{\text{LOWER/UPPER limit of } \log[\pi]\}$ .

$$SE[\text{logit}] = \left\{ \frac{1}{\# \text{ positive}} - \frac{1}{n} \right\}^{1/2}$$

Limits for  $\pi$  from  $p = 3/10$  :  $\exp[\log[3/10] \pm z \times \{1/3 - 1/10\}^{1/2}]$

Exercises:

1. Verify that you get same answer by calculator and by software
2. even with these logit and log transformations, the Gaussian distribution is not accurate at such small sample sizes as  $\frac{3}{10}$ . Compare their performance (against the exact methods) for various sample sizes and numbers positive.

SAS	Stata
DATA CI_propn;	input n_pos n
INPUT n_pos n;	1 5
LINES;	2 5
3 10;	end
;	
PROC genmod;	glm n_pos,
model n_pos/n = /	family (binomial n) link (logit)
dist = binomial	
link = log waldci;	

### 3 Applications, and notes

#### 3.1 95% CI? IC? ... Comment dit on... ?

[La Presse, Montréal, 1993] L’Institut Gallup a demandé récemment à un échantillon représentatif de la population canadienne d’évaluer la manière dont le gouvernement fédéral faisait face à divers problèmes économiques et général. Pour 59 pour cent des répondants, les libéraux n’accomplissent pas un travail efficace dans ce domaine, tandis que 30 pour cent se déclarent de l’avis contraire et que onze pour cent ne formulent aucune opinion.

La même question a été posée par Gallup à 16 reprises entre 1973 et 1990, et ne n’est qu’une seule fois, en 1973, que la proportion des Canadiens qui se disaient insatisfaits de la façon dont le gouvernement gérait l’économie a été inférieure à 50 pour cent.

Les conclusions du sondage se fondent sur 1009 interviews effectuées entre le 2 et le 9 mai 1994 auprès de Canadiens âgés de 18 ans et plus. Un échantillon de cette ampleur donne des résultats exacts à 3,1 p.c., près dans 19 cas sur 20. La marge d’erreur est plus forte pour les régions, par suite de l’importance moindre de l’échantillonnage; par exemple, les 272 interviews effectuées au Québec ont engendré une marge d’erreur de 6 p.c. dans 19 cas sur 20. Notice the emphasis on ‘a sample of this size’ and the procedure.

#### 3.2 1200 are hardly representative of 80 million homes / 220 million people!

**The Nielsen system for TV ratings in U.S.A.**

(Excerpt from article on “Pollsters” from an airline magazine)

“...Nielsen uses a device that, at one minute intervals, checks to see if the TV set is on or off and to which channel it is tuned. That information is periodically retrieved via a special telephone line and fed into the Nielsen computer center in Dunedin, Florida. With these two samplings, Nielsen can provide a statistical estimate of the number of homes tuned in to a given program. A rating of 20, for instance, means that 20 percent, or 16 million of the 80 million households, were tuned in. To answer the criticism that 1,200 or 1,500 are hardly representative of 80 million homes or 220 million people, Nielsen offers this analogy:

Mix together 70,000 white beans and 30,000 red beans and then scoop out a sample of 1000. the mathematical odds are that the number of red beans will be between 270 and 330 or 27 to 33 percent of the sample, which translates to a “rating” of 30, plus or minus three, with a 20-to-1 assurance of statistical reliability. The basic statistical law wouldn’t change even if the sampling came from 80 million beans rather than just 100,000.” ...

**Why, if the U.S. has a 10 times bigger population than Canada, do pollsters use the same size samples of approximately 1, 000 in both countries?**

**Answer:** it depends on WHAT IS IT THAT IS BEING ESTIMATED. With



$n = 1,000$ , the SE or uncertainty of an estimated PROPORTION 0.30 is indeed 0.03 or 3 percentage points. However, if interested in the NUMBER of households tuned in to a given program, the best estimate is  $0.3N$ , where  $N$  is the number of units in the population ( $N=80$  million in the U.S. or  $N=8$  million in Canada). The uncertainty in the ‘blown up’ estimate of the TOTAL NUMBER tuned in is blown up accordingly, so that e.g. the estimated NUMBER of households is

U.S.A.	80, 000, 000	$[0.3 \pm 0.03]$	=	24, 000, 000	$\pm$	2, 400, 000
Canada	8, 000, 000	$[0.3 \pm 0.03]$	=	2, 400, 000	$\pm$	240, 000

2.4 million is a 10 times bigger absolute uncertainty than 240,000. Our intuition about needing a bigger sample for a bigger universe probably stems from absolute errors rather than relative ones (which in our case remain at 0.03 in 0.3 or 240,000 in 2.4 million or 2.4 million in 24 million i.e. at 10% irrespective of the size of the universe). It may help to think of why we do not take bigger blood samples from bigger persons: the reason is that we are usually interested in *concentrations* rather than in absolute amounts and that concentrations are like proportions.

### 3.3 The “Margin of Error blurb” introduced (legislated) in the mid 1980’s

#### 3.3.1 Number of Smokers rises by Four Points: Gallup Poll

The Gazette, Montreal, August 8, 1981

Compared with a year ago, there appears to be an increase in the number of Canadians who smoked cigarettes in the past week - up from 41% in 1980 to 45% today. The question asked over the past few years was: **“Have you yourself smoked any cigarettes in the past week”** Here is the national trend:

*Smoked cigarettes in the past week*

Today	.....	45%
1980	.....	41%
1979	.....	44%
1978	.....	47%
1977	.....	45%
1976	.....	Not asked
1975	.....	47%
1974	.....	52%

Men (50% vs. 40% for women), young people (54% vs. 37% for those > 50) and Canadians of French origin (57% vs. 42% for English) are the most likely smokers. **Today’s results are based on 1,054 personal in-home interviews with adults, 18 years and over, conducted in June.**

**Had the percentage in the population really risen?** Without a SE (or margin of Error, ME) for each percentage, we are unable to judge whether the ‘jump’ from 41% to 45% is real or maybe just sampling variation. By 1985, margins of error in the reporting of polls had become mandatory...

#### 3.3.2 39% of Canadians Smoked in Past Week: Gallup Poll

The Gazette, Montreal, Thursday, June 27, 1985

Almost two in every five Canadian adults (39 per cent) smoked at least one cigarette in the past week - down significantly from the 47 percent who reported this 10 years ago, but at the same level found a year ago. Here is the question asked fairly regularly over the past decade: **“Have you yourself smoked any cigarettes in the past week?”** The national trend shows:

*Smoked cigarettes in the past week*

1985	.....	39%
1984	.....	39%
1983	.....	41%
1982*	.....	42%
1981	.....	45%
1980	.....	41
1979	.....	44%
1978	.....	47%
1977	.....	45%
1975	.....	47%

(\* Smoked regularly or occasionally)

Those < 50 are more likely to smoke cigarettes (43%) than are those 50 years or over (33%). Men (43%) are more likely to be smokers than women (36%). Results are based on 1,047 personal, in-home interviews with adults, 18 years and over, conducted between May 9 and 11. **A sample of this size is accurate within a 4-percentage-point margin, 19 in 20 times.**

Again, notice the emphasis on ‘*a* sample of this size’ and the *procedure*.

## 4 Test of $H_0 : \pi = \pi_{NULL}$

### 4.1 $n$ small enough $\rightarrow$ Use Exact Binomial probabilities

- Testing  $H_0: \pi = \pi_0$  vs  $H_a: \pi \neq \pi_0$  [or  $H_a: \pi > \pi_0$  ]
- Observe  $p = y/n$ .
- Calculate Prob[observed  $y$ , or a  $y$  that is more extreme |  $\pi_0$ ] using  $H_{alt}$  to specify which  $y$ 's are more extreme i.e. provide even more evidence for  $H_a$  and against  $H_0$ .  
or...  
use correspondence between a  $100(1 - \alpha)\%$  CI and a test which uses a level of  $\alpha$  i.e. check if CI obtained from CI table or nomogram includes  $\pi$  value being tested  
  
[there may be slight discrepancies between test and CI: the methods used to construct CI's don't always correspond exactly to those used for tests]

#### Examples

1. A common question is whether there is evidence against the proposition that a proportion  $\pi = 1/2$  [Testing preferences and discrimination in psychophysical matters e.g., therapeutic touch, McNemar's test for discordant pairs when comparing proportions in a paired-matched study, the (non-parametric) Sign Test for assessing intra-pair differences in measured quantities, ...]. Because of the special place of the Binomial at  $\pi = 1/2$ , the tail probabilities have been calculated and tabulated. See the table entitled “Sign Test” in the chapter on Distribution-Free Methods.  
  
M&M (2nd paragraph p 592) say that “we do not often use significance tests for a single proportion, because it is uncommon to have a situation where there is a precise proportion that we want to test”. But they forget paired studies, and even the sign test for matched pairs, which they themselves cover in section 7.1, page 521. They give just 1 exercise (8.18) where they ask you to test  $\pi = 0.5$  vs  $\pi > 0.5$ .
2. Another example, dealing with responses in a setup where the “null” is  $\pi_0 = 1/3$ , example 4.3 is described below.
3. The First Recorded P-Value??? (by a physician no less!) <sup>10</sup>

“AN ARGUMENT FOR DIVINE PROVIDENCE, TAKEN FROM THE CONSTANT REGULARITY OBSERVED IN THE BIRTHS OF BOTH SEXES.”

*John Arbuthnot, 1667-1735 physician to Queen Anne*

Arbuthnot claimed to demonstrate that divine providence, not chance, governed the sex ratio at birth.

To prove this point he represented a birth governed by chance as being like the throw of a two-sided die, and he presented data on the christenings in London for the 82-year period 1629-1710.

Under Arbuthnot's hypothesis of chance, for any one year male births will exceed female births with a probability slightly less than one-half. (It would be less than one-half by just half the very small probability that the two numbers are exactly equal.)

But even when taking it as one-half Arbuthnot found that a unit bet that male births would exceed female births for eighty-two years running to be worth only  $(1/2)^{82}$  units in expectation, or

$$\frac{1}{4\ 8360\ 0000\ 0000\ 0000\ 0000}$$

a **vanishingly small number**.

”From whence it follows, that it is Art, not Chance, that governs.”

### 4.2 Large $n$ : Gaussian Approximation

**Test:**  $\pi = \pi_0$

**Test Statistic:**  $(p - \pi_0)/SE[p] = (p - \pi_0)/\{\pi_0 \times (1 - \pi_0)/n\}^{1/2}$

**Note:**

- The *test* uses the *NULL* SE, based on the (specified)  $\pi_0$ .
- The “usual” *CI* uses an SE based on the *observed*  $p$ .

#### 4.2.1 (Dis)Continuity Correction

Because we approximate a discrete distribution [where  $p$  takes on the values  $\frac{0}{n}, \frac{1}{n}, \frac{2}{n}, \dots, \frac{n}{n}$  corresponding to the integer values  $(0, 1, 2, \dots, n)$  in the numerator of  $p$ ] by a continuous Gaussian distribution, authors have suggested a ‘continuity correction’ (or if you are more precise in your language, a ‘discontinuity’ correction). This is the same concept as we saw back in §5.1, where we said that a binomial count of 8 became the interval  $(7.5, 8.5)$  in the interval scale.

<sup>10</sup>related by Stigler in his History of Statistics

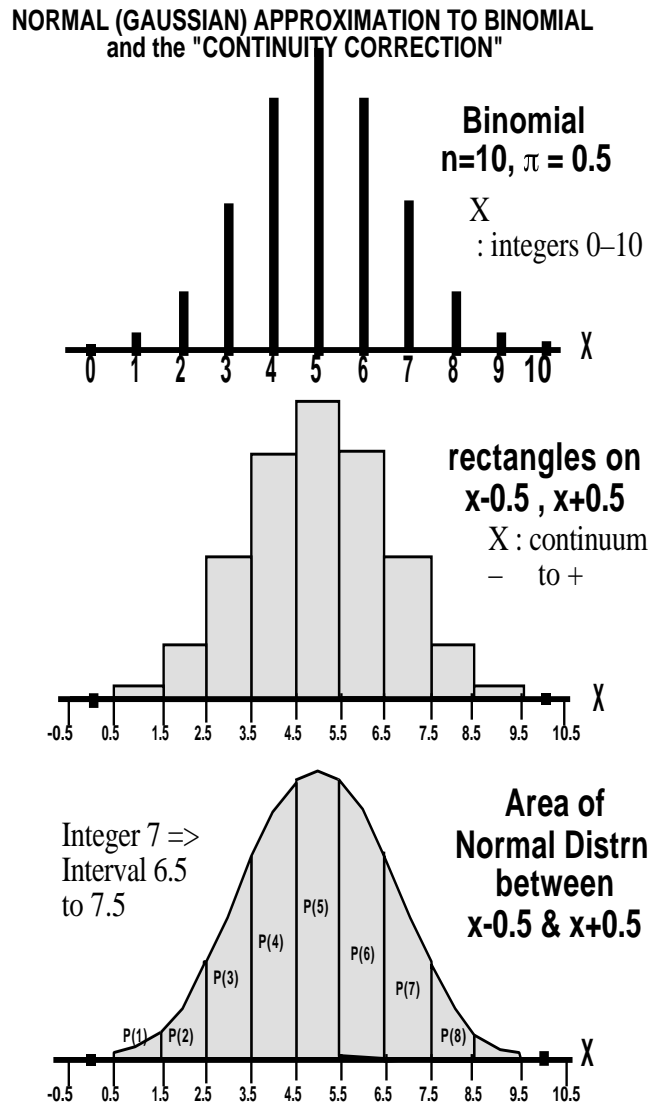


Figure 5: From discrete to continuous

Thus, e.g., if we want to calculate the probability that proportion out of 10 is  $\geq 8$ , we need probability of  $\geq 7.5$  on the continuous scale.

If we work with the *count* itself in the numerator, this amounts to reducing the absolute deviation  $y - n \times \pi_0$  by 0.5 . If we work in the *proportion scale*, the absolute deviation is reduced by  $\frac{0.5}{n}$  viz.

$$z_c = \frac{|y - n\pi_0| - 0.5}{SE[y]} = \frac{|y - n\pi_0| - 0.5}{\sqrt{n\pi_0[1 - \pi_0]}}$$

or

$$z_c = \frac{|p - n\pi_0| - \frac{0.5}{n}}{SE[p]} = \frac{|p - n\pi_0| - \frac{0.5}{n}}{\pi_0[1 - \pi_0]/n}^{1/2}$$

†Colton [who has a typo in the formula on p ...] and A&B deal with this; M&M do not, except to say on p386-7 “because most statistical purposes do not require extremely accurate probability calculations, we do not emphasize use of the continuity correction”. There are some ‘fundamental’ problems here that statisticians disagree on. The “Mid-P” material (below) gives some of the flavour of the debate.

### 4.3 Example of Testing $\pi$ : The Triangle Taste Test

As part of preparation for a double blind RCT of lactase-reduced infant formula on infant crying behaviour, the experimental formulation was tested for its similarity in taste to the regular infant formula.  $n$  mothers in the waiting room at MCH were given the TRIANGLE TASTE TEST i.e. they were each given 3 coded formula samples – 2 containing the regular formula and 1 the experimental one. Told that “2 of these samples are the same and one sample is different”,  $p = y/n$  correctly identify the odd sample. Should the researcher be worried that the experimental formula does not taste the same? (assume infants are no more or less taste-discriminating than their mothers) [study by Ron Barr, Montreal Children’s Hospital]

The null hypothesis being tested is

$$H_0: \pi(\text{correctly identified samples}) = 0.33 \text{ against } H_a: \pi() > 0.33$$

[here, for once, it is difficult to imagine a 2-sided alternative – unless mothers were very taste-discriminating but wished to confuse the investigator]

We consider two situations (the real study with  $n=12$ , and a hypothetical larger sample of  $n=120$  for illustration)

**Data:**  $y = 5$  of  $n = 12$  mothers correctly identified the odd sample., i.e.  $p = 0.42$ .

Degree of evidence against  $H_0$  :

$$\begin{aligned}
 &= \text{Prob}(5 \text{ or more correct} | \pi = 0.33) \dots \text{(a } \sum \text{ of 8 probabilities)} \\
 &= 1 - \text{Prob}(4 \text{ or fewer correct} | \pi = 0.33) \dots \text{(a shorter } \sum \text{ of only 5)} \\
 &= 1 - [P(0) + P(1) + P(2) + P(3) + P(4)] \\
 &= 0.37*
 \end{aligned}$$

Using  $n = 12$ , and  $p = 0.30$  in Table C gives 0.28; using  $p = 0.35$  gives 0.42. Interpolation gives 0.37 approx. \*

Can also obtain the exact probability (0.03762) directly via Excel, using the function BINOMDIST(4, 12, 0.333, TRUE), or using 1-sum(dbinom(1:4,12,1/3)) in R.

So, by conventional criteria (Prob < 0.05 is considered a cutoff for evidence against  $H_0$ ) there is not a lot of evidence to contradict the  $H_0$  of taste similarity of the regular and experimental formulae.

With a sample size of only  $n = 12$ , we cannot rule out the possibility that a sizable fraction of mothers could truly distinguish the two.

Our observed proportion of 5/12 projects to a one-sided 95% CI of “as many as 65% in the population get it right”. In this worst-case scenario, assuming that the percentage of right answers in the population is a mix of a proportion  $\pi_{can}$  who can really tell and one third of the remaining  $(1 - \pi_{can})$  who get it right by guessing, we equate  $0.65 = \pi_{can} + (1 - \pi_{can})/3$ , giving us an upper bound  $\pi_{can} = (0.65 - 0.33)/(2/3) = 0.48$ , or 48%.

\*These calculations can be done easily on a calculator or spreadsheet without any combinatorials:

$P(0) =$	$0.67^{12}$	$= 0.008$
$P(1) =$	$\frac{12 \times 0.33 \times P(0)}{[1 \times 0.67]}$	$= 0.048$
$P(2) =$	$\frac{11 \times 0.33 \times P(1)}{[2 \times 0.67]}$	$= 0.131$
$P(3) =$	$\frac{10 \times 0.33 \times P(2)}{[3 \times 0.67]}$	$= 0.215$
$P(4) =$	$\frac{9 \times 0.33 \times P(3)}{[4 \times 0.67]}$	$= 0.238$
$\Sigma$		$= 0.640$

so Prob[5 or more correct |  $\pi = 0.33$ ] =  $1 - 0.64 = 0.36$ .

%said that = 0.32 which is mathematically incorrect

**What if 50 of 120** mothers identified odd sample?

Test  $\pi = 0.33$  :  $z = (0.42* - 0.33) / \{0.33 \times (1 - 0.33) / 20\}^{1/2} = 2.1$ .

So  $P = \text{Prob}[\geq 50 | \pi = 0.33] = \text{Prob}[Z \geq 2.1] = 0.018$

\* We treat the proportion 50/120 as a continuous measurement; in fact it is based on an integer numerator 50; we should treat 50 as 49.5 to 50.5 so  $\geq 50$  is really  $> 49.5$ , and we are dealing with the probability. of obtaining 49.5/120 or more. With  $n = 120$ , the continuity correction does not make a large difference; however, with smaller  $n$ , and its coarser grain, the continuity correction [which makes differences smaller] is more substantial.

## 5 Planning: Sample Size for CI’s and Tests

### 5.1 $n$ to yield (2-sided) CI with margin of error ME at confidence level $1 - \alpha$

(see M&M p. 593, Colton p. 161)



- see CI’s as function of  $n$  in tables and nomograms
- (or) large-sample CI:  $p \pm Z_{\alpha/2}SE(p) = p \pm ME$

$$SE(p) = \{p[1 - p]/n\}^{1/2},$$

so ...

$$n = \frac{p[1 - p] \times Z_{\alpha/2}^2}{ME^2}$$

If unsure, use largest SE i.e. when  $p = 0.5$  i.e.

$$n = \frac{0.25 \times Z_{\alpha/2}^2}{ME^2} \tag{1}$$

### 5.2 $n$ for power $1 - \beta$ to “detect” a population proportion $\pi$ that is $\Delta$ units from $\pi_0$ ; type I error = $\alpha$ .

(Colton, p. 161)

$$\begin{aligned} n &= \frac{\left\{ Z_{\alpha/2} \sqrt{\pi_0 [1 - \pi_0]} - Z_{\beta} \sqrt{\pi_1 [1 - \pi_1]} \right\}^2}{\Delta^2} \\ &\approx \left\{ Z_{\alpha/2} \right\}^2 \left\{ \frac{\sqrt{\pi [1 - \pi]}}{\Delta} \right\}^2 [*] \\ &= \left\{ Z_{\alpha/2} - Z_{\beta} \right\}^2 \left\{ \frac{\sigma_{0,1}}{\Delta} \right\}^2 \end{aligned} \tag{2}$$

\* where  $\pi$  is average of  $\pi_0$  and  $\pi_1$ .

Notes:  $Z_{\beta}$  will be negative; formula is same as for testing  $\mu$

#### 5.2.1 Worked Example 1: sample size to test for preferences $\pi = 0.5$ vs. $\pi \neq 0.5$ or Sign Test that median difference = 0

**Test:**

$H_0$ : Median<sub>D</sub> = 0 vs  $H_{alt}$ : Median<sub>D</sub>  $\neq$  0;  $\alpha = 0.05$  (2-sided);

or

$H_0$ :  $\pi(+)$  = 0.5 vs  $H_{alt}$ :  $\pi(+)$  > 0.5

For Power  $1 - \beta$  against:  $H_{alt}$ :  $\pi(+)$  = 0.65 say.

At  $\pi = ave$  of 0.5 & 0.65,  $\sqrt{\pi[1 - \pi]} = 0.494$ .

$$n \approx \left\{ Z_{\frac{\alpha}{2}} - Z_{\beta} \right\}^2 \left\{ \frac{0.494}{0.15} \right\}^2$$

$\alpha = 0.05$  (2-sided) &  $\beta = 0.2 \Rightarrow Z_{\alpha} = 1.96$ ;  $Z_{\beta} = -0.84$

$(Z_{\frac{\alpha}{2}} - Z_{\beta})^2 = \{1.96 - (-0.84)\}^2 \approx 8$ , i.e.

$$n \approx 8 \left\{ \frac{0.494}{0.15} \right\}^2 = 87$$

#### 5.2.2 Worked Example 2: sample size for $\Delta$ Taste Test:

$\pi_{correct} = 1/3$  vs.  $\pi > 1/3$

If set  $\alpha = 0.05$  (hardliners might allow 1-sided test here), then  $Z_{\alpha} = 1.645$ ; If want 90% power, then  $Z_{\beta} = -1.28$ ; Then using equation 2 above...

$\pi_{correct}$ :	0.4	0.5	0.6	0.7	0.8
$n$ for 90 Power against this $\pi$	400	69	27	14	8

## 0 Exercises

### 0.1 (m-s) Working with logits and logs of proportions

In order to have a sampling distribution that is closer to Gaussian (sample proportions, odds, and ratios of them tend to have nasty sampling distributions), we often transform from the (0,1)  $\pi$  i.e., proportion, scale to the  $(-\infty, 0)$   $\log[\pi]$  scale, or the  $(-\infty, -\infty)$   $\log[\pi/(1 - \pi)]$  scale. The latter transformation is called the *logit* transform.

Thus, we do all our inference (SE calculations, CI’s, tests) on the log or logit scale, then transform back to the proportion or odds or ratio scale.

1. Suppose  $y \sim \text{Binomial}(n, \pi)$  and that  $p = y/n$ . Derive the (approx.) variance for the random variables  $\log[p]$  and  $\text{logit}[p] = \log[p/(1 - p)]$ . Assume  $n$  and  $\pi$  are such that we can ignore the possibility of obtaining  $y = 0/n$  or  $y = n/n$ : people often add 0.5 to  $y$  and 1 to  $n$  to avoid such complications.
2. The variance of  $p$  is largest when  $\pi = 0.5$  and smallest when  $\pi = 0.0$ . At what value of  $\pi$  is the variance of  $\text{logit}[p]$  largest? smallest?
3. How large would the ‘amplitude’<sup>11</sup> be in a series of yearly proportions of male births in a country or province with about (i) 1 million (ii) 10,000 (iii) 100 births per year? What, if instead of a proportion, the series plotted the sex ratio (males:females, typically 1.04:1, or 104:100)? the log of this ratio? Do the different amplitudes on different scales fit with the wider and narrower ranges of the different scales?

If interested to see annual fluctuations, the Canadian data from 1931-1990 are available in the Resources web page under ‘**Data / Miscellaneous**’.

4. Clayton and Hills, Chapter 9.2-9.4, page 80-85, describe “Approximate likelihoods for transformed parameters”, and illustrate them using the log likelihood for the rate parameter  $\lambda$ . They then give, without proof, the results of the calculation of the curvature for the Bernoulli log-likelihood, when plotted against the log of the odds. *Exercise:* Repeat the ‘longer’ and ‘shorter’ calculations for what they call the ‘risk’ parameter.

### 0.2 (m-s) Greenwood’s formula for the SE of an estimated Survival Probability

In survival analysis, we often estimate the surviving proportion  $S$  after a fixed number  $k$  of time intervals as a product of (estimated) conditional probabilities, ie  $\hat{S} = \prod_1^k \hat{S}_i$ . The  $i$ -th element is the conditional probability of surviving the  $i$ -th interval, given that one survived the previous intervals.

For inference regarding  $S$ , we need  $\text{SE}[\hat{S}]$ . To derive this, it is easier to work in the  $\log[S]$  scale, so that  $\log \hat{S} = \sum_1^k \log[\hat{S}_i]$ , to calculate the SE and CI in this scale, and then transform back to the (0,1)  $S$  scale.

*Exercise:* Treat  $\hat{S}_i \sim (1/n_i) \times \text{Binomial}(n_i, S_i)$ , with  $n_i$  fixed (in practice, the  $n_i$ ’s are random, but there are good reasons to treat them as fixed for the variance calculation). Derive the variance for  $\log[\hat{S}]$ , and from this (via the same math applied to the reverse, i.e., antilog, transform) the variance for  $\hat{S}$ .

### 0.3 (m-s) Link between exact tail areas of Binomial and F distributions

Re-write the proof<sup>12</sup> in your own notation (see Fisher 1935, under Resources).

### 0.4 (m-s) Link between exact tail areas of Poisson and Chi-Square distributions

Re-write the proof in your own notation (see Fisher 1935, under Resources). See also, under the REPRINTS web page, the article and the diagram in Accromath.

*To more easily follow Fisher’s sometimes cryptic results, it can help to examine the P-value calculations in Pearson’s 1900 paper on the Chi-Square goodness of fit statistic (available under Resources for proportions). His paper contains several worked illustrations; in some of them he shows the formula he used to calculate the (upper) tail of the Chi-Square square distribution. The formula clearly shows the link with the Poisson tail area. If we did not have ready access to the tail areas of Poisson distributions, we could arrive at them if we had access to tables of the tail areas of the Chi-Square square distribu-*

<sup>12</sup>This one is more complicated than the one for the link between the Poisson and Chi-Square distributions, since there is one extra ‘parameter’ or ‘modulator’. As the denominator df. goes to infinity, the F distribution approaches the Chi-Square distribution: with near infinite df., we can estimate  $\sigma^2$  almost perfectly.

<sup>11</sup>If you wish, use the SD or IQR rather than range.

tions. Indeed, before the widespread availability (e.g., in Excel, R, Stata and SAS) of ways to compute these Poisson tail areas, this link was widely used in epidemiology.

Or use the insights gained in the exercise that links the **number** of tire-ruptures in a trip of a **given distance** and the **distance** travelled before a **given number** of tire-ruptures occur.

## 0.5 Clusters of Miscarriages [based on article by L Abenheim]

Assume that:

- 15% of all pregnancies end in a recognized spontaneous abortion (miscarriage) – this is probably a conservative estimate.
- Across North America, there are 1,000 large companies. In each of them, 10 females who work all day with computer terminals become pregnant within the course of a year [the number who get pregnant would vary, but assume for the sake of this exercise that it is exactly 10 in each company].
- There is no relationship between working with computers and the risk of miscarriage.
- a “cluster” of miscarriages is defined as “at least 5 of 10 females in the same company suffering a miscarriage within a year”

**Exercise:** Calculate the number of “clusters” of miscarriages one would expect in the 1,000 companies. Hint: begin with the probability of a cluster.

## 0.6 “Prone-ness” to Miscarriages ?

Some studies suggest that the chance of a pregnancy ending in a spontaneous abortion is approximately 30%.

1. On this basis, if a woman becomes pregnant 4 times, what does the binomial distribution give as her chance of having 0,1,2,3 or 4 spontaneous abortions?
2. On this basis, if 70 women each become pregnant 4 times, what number of them would you expect to suffer 0,1,2,3 or 4 spontaneous abortions? (Think of the answers in (i) as proportions of women).

3. Compare these theoretically expected numbers out of 70 with the following observed data on 70 women, each of whom had 4 pregnancies:

No. of spontaneous abortions:	0	1	2	3	4
No. of women with this many abortions:	23	28	7	6	6

4. Why don’t the expected numbers agree very well with the observed numbers? i.e. which assumption(s) of the Binomial Distribution are possibly being violated? (Note that the overall rate of spontaneous abortions in the observed data is in fact 84 out of 280 pregnancies or 30%).

## 0.7 Automated Chemistries (from Ingelfinger et al)

At the Beth Israel Hospital in Boston, an automated clinical chemistry analyzer is used to give 18 routinely ordered chemical determinations on one order (glucose, BUN, creatinine, ..., iron). The “normal” values for these 18 tests were established by the concentrations of these chemicals in the sera of a large sample of healthy volunteers. The normal range was defined so that an average of 3% of the values found in these healthy subjects fell outside.

1. Using the binomial formula [even if it is naïve to do so here], compute the probability that a healthy subject will have normal values on all 18 tests. Also calculate the probability of 2 or more abnormal values.
2. Which of the requirements for the binomial distribution are definitely satisfied, and which ones may not be?
3. Among 82 normal employees at the hospital, 52/82 (64%) had all normal tests, 19/82 (23%) had 1 abnormal test and 11/82 (13%) had 2 or more abnormal tests. Compare these observed percentages with the theoretical distribution obtained from calculations using the binomial distribution. Comment on the closeness of the fit.

## 0.8 Binomial or Opportunistic? Capitalization on chance... multiple looks at data (Question from Ingelfinger et al.)

Mrs A has mild diabetes controlled by diet. Her morning urine sugar test is negative 80% of the time and positive (+) 20% of the time [It is never graded higher than +].

1. At her regular visit she tells her physician that the test has been + on each of the last 5 days. What is the probability that this would occur

if her condition has remained unchanged? Does this observation give reason to think that her condition has changed?

2. Is the situation different if she observes, between visits, that the test is positive on 5 successive days and phones to express her concern?

### 0.9 Can one influence the sex of a baby?

These data are taken from an article in the NEJM 300:1445-1448, 1979.

1. Consider a binomial variable with  $n = 145$  and  $\pi = 0.528$ . Calculate the SD of, and therefore a measure of the variation in, the proportions that one would observe in different samples of 145 if  $\pi = 0.528$ .
2. Then consider the following, abstracted from the NEJM article: and answer the question that follows the excerpt.

The baby’s sex was studied in births to Jewish women who observed the orthodox ritual of sexual separation each month and who resumed intercourse within two days of ovulation. The proportion of male babies was 95/145 or 65.5% (!) in the offspring of those women who resumed intercourse two days after ovulation (the overall percentage of male babies born to the 3658 women who had resumed intercourse within two days of ovulation [i.e. days -2, -1, 0, 1 and 2] was 52.8%).”

3. How does the SD you calculated above help you judge the findings?

### 0.10 It’s the 3rd week of the course: it must be Binomial

In which of the following would Y not have a Binomial distribution? Why?

1. The pool of potential jurors for a murder case contains 100 persons chosen at random from the adult residents of a large city. Each person in the pool is asked whether he or she opposes the death penalty; Y is the number who say “Yes.”
2.  $Y =$  number of women listed in different random samples of size 20 from the 1990 directory of statisticians.
3.  $Y =$  number of occasions, out of a randomly selected sample of 100 occasions during the year, in which you were indoors. (One might use this design to estimate what proportion of time you spend indoors)

4.  $Y =$  number of months of the year in which it snows in Montral.
5.  $Y =$  Number, out of 60 occupants of 30 randomly chosen cars, wearing seatbelts.
6.  $Y =$  Number, out of 60 occupants of 60 randomly chosen cars, wearing seatbelts.
7.  $Y =$  Number, out of a department’s 10 microcomputers and 4 printers, that are going to fail in their first year.
8.  $Y =$  Number, out of simple random sample of 100 individuals, that are left-handed.
9.  $Y =$  Number, out of 5000 randomly selected from mothers giving birth each month in Quebec, who will test HIV positive.
10. You observe the sex of the next 50 children born at a local hospital; Y is the number of girls among them.
11. A couple decides to continue to have children until their first girl is born; Y is the total number of children the couple has.
12. You want to know what percent of married people believe that mothers of young children should not be employed outside the home. You plan to interview 50 people, and for the sake of convenience you decide to interview both the husband and the wife in 25 married couples. The random variable Y is the number among the 50 persons interviewed who think mothers should not be employed.

### 0.11 Tests of intuition

1. A coin will be tossed either 2 times or 20 times. You will win \$2.00 if the number of heads is equal to the number of tails, no more and no less. Which is correct? (i) 2 tosses is better. (ii) 100 tosses is better. (iii) Both offer the same chance of winning.
2. Hospital A has 100 births a year, hospital B has 2500. In which hospital is it more that at least 55% of births in one year will be boys.

### 0.12 Test of a proposed mosquito repellent

An entomologist carried out the following experiment as a test of a proposed mosquito repellent. Thirty-five volunteers had one forearm treated with a



small amount of repellent and the other with a control solution. The subjects did not know on which forearm the repellent had been used. At dusk the volunteers exposed themselves to mosquitoes and reported which forearm was bitten first. In 10/35, the arm with the repellent was bitten first.

1. Make a statistical report on the findings.
2. How would you analyze the results if: (a) some arms were not bitten at all? (b) some people were not bitten at all?

### 0.13 Triangle Taste test

In its 1974 manual “Laboratory Methods for Sensory Evaluation of Food”, Agriculture Canada described tests (the triangle test, the simple paired comparisons test,...) to determine a difference between samples

In the triangle test, the panelist receives 3 coded samples and is told that 2 of the samples are the same and 1 is different and is asked to identify the odd sample. This method is very useful in quality control work to ensure that samples from different production lots are the same. It is also used to determine if ingredient substitution or some other change in manufacturing results in a detectable difference in the product. The triangle test is often used for selecting panelists.

Analysis of the results of triangle tests is based on the probability that - IF THERE IS NO DETECTABLE DIFFERENCE - the odd sample will be selected by chance one-third of the time. Tables for rapid analysis of triangle test data are given below. As the number of judgements increases, the percentage of correct responses required for significance decreases. For this reason, when only a small number of panelists are available, they should perform the triangle test more than once in order to obtain more judgements.

The results of a test indicate whether or not there is a detectable difference between the samples. Higher levels of significance do not indicate that the difference is greater but that there is less probability of saying there is a difference when in fact there is none.

Chart: Triangle test difference analysis [Table starts at  $n = 7$  and ends at  $n = 2000$ ; selected entries shown here]

Number of correct answers necessary to establish...

No. Tasters	level of significance		
	5%	1%	0.1%
7	5	6	7
10	7	8	9
12	8	9	10
30	16	17	19
60	28	30	33
100	43	46	49
1000	363	372	383

1. Show how one arrives at the numbers 7, 8 and 9 of correct answers necessary to establish the stated levels of significance for the case of  $n=10$  tasters. Hint: you can work them out from the BINOMDIST function in Excel or [since we are only interested in the principles involved, and not in getting answers correct to several decimal places] you should be able to interpolate them from probability distributions tabulated in the text [the setup here is similar to the therapeutic touch study, but with  $\pi = 1/3$  rather than  $\pi = 1/2$ ].
2. Calculate the exact 90, 98 and 99.8 percent 2-sided CI's for the proportions 7/10, 8/10 and 9/10 respectively, and from these limits verify that indeed 7/10, 8/10 and 9/10 are significantly greater than 0.33, at the stated levels of significance. (I am presuming that their  $H_a$  is 1-sided, ie. 0.33 vs.  $> 0.33$   
You can obtain these CI's from the spreadsheet “CI for a proportion”, under Resources for Ch 8.
3. Show how one arrives at the numbers 43, 46 and 49 of correct answers necessary to establish the levels of significance for the case of 100 tasters. Hint: you should be able to use a large-sample approximation.
4. How well would this large-sample approximation method have done for the case of  $n = 10$ ?
5. If you set the  $\alpha$  at 0.05 (1-sided), what number of tasters is required to have 80 percent power to ‘detect’ a ‘shift’ from  $H_0 : \pi = 1/3$  to (i)  $H_a : \pi = 1/2$  (ii)  $H_a : \pi = 2/3$ ? Use the sample size formula in section 8.1 of the notes.

Notes: See worked example 2 in notes on Chapter 8.1. This is a good example where a one-sided alternative is more easily justified, so with  $\alpha = 0.05$  1-sided,  $Z_\alpha = 1.645$ . Note that power of 80 percent means that  $Z_\beta = -0.84$ . The  $Z_\beta$  is always one-sided, since one cannot be on both sides of  $H_0$  simultaneously!

## 0.14 Variability of, and trends in, proportions

The following data are the proportion of Canadian adults responding YES to the question “Have you yourself smoked any cigarettes in the past week?” in Gallup Polls for the years 1974 to 1985.

	1974	'75	'76	'77	'78	'79	'80	'81	'82	'83	'84	85
%	52	47	.	45	47	44	41	45	42*	41	39	39

. question not asked in 1976;

\* question worded “occasionally or regularly” in 1982.

Results are based on approximately 1050 personal in-home interviews each year with adults 18 years and over.

1. Plot these percentages along with their 95 confidence intervals.
2. Is there clear evidence that the trend is downward? To answer this, try to draw a straight line through all (or most of) the confidence intervals and ask can the straight line have a slope of zero i.e. be parallel to the horizontal axis. You might call this a “poor-person’s test of trend.”

For recent national and provincial figures, see

<http://www.statcan.gc.ca/tables-tableaux/sum-som/101/cst01/health74b-eng.htm>

## 0.15 A Close Look at Therapeutic Touch

[Rosa L et al., JAMA. 1998;279:1005-1010; for those interested, there is considerable follow-up correspondence]

See the full article under Resources..

In the last paragraph of Methods the authors state (italics by JH):

“The odds of getting 8 of 10 trials correct by chance alone is *45 of 1024* ( $P=.04$ ), a level considered significant in many clinical trials. We decided in advance that an individual would “pass” by making *8 or more correct selections* and that those passing the test would be retested, although the retest results would not be included in the group analysis.”

1. Use statistical software, or Table C of M & M3, or first principles, to verify that the probability of getting exactly 8 of 10 correct is indeed 45 of 1024.
2. In the next sentence the authors state that in fact they used “8 or more correct” as their criterion. Explain why this definition of “evidence for

the therapeutic touch” (or, if you prefer, “against the skeptic’s null hypothesis”) is more logical than the “exactly 8” for which they calculate the  $P=0.04$  [ Hint: See the second half of the first paragraph (about specific outcomes) under P-values in M & M page 457. In our context, imagine that there were 400 trials: then the probability of – by chance alone – getting exactly 320 is indeed, in Dr. Arbuthnot’s words, “vanishingly small.” but the probability of getting specifically 200 (a value that provides no evidence against  $H_0$ , is also small (0.04)]

3. Calculate – under the “null” hypothesis, the probability of “8 or more correct”. Is it indeed less than the arbitrary “level considered significant” of 0.05? If not, then what would the criterion need to be so that the probability – again calculated under “ $H_0$ ” – of reaching this criterion is  $< 0.05$ .
4. Figure 2 shows the scores of the 28 subjects. Multiply the set of Binomial probabilities with  $n=10$  and  $p = 0.5$  (i.e.,  $p[0/10 \text{ correct} - p = 0.5]$  to  $p[10/10 \text{ correct} - p = 0.5]$  by 28 to obtain theoretical frequencies. These are the numbers of subjects, out of 28, one would expect to get 0/10, 1/10, ... 10/10 trials correct if all they were doing in each trial was guessing. Compare the theoretical frequencies of subjects with the observed “No. of subjects” with each score. Comment. Ignore for the moment the fact that the 28 people tested were really only 21 distinct people – 14 tested once (10 trials each) and 7 tested twice (10 trials, twice)

## 0.16 Is this the correct way to calculate a CI for a proportion?

Using an observed  $\hat{\pi} = 2/1094$  ‘positivity rate’, a sociologist calculated the lower and upper 95% limits for the theoretical proportion positive ( $\pi$ ) using the following method:

$\{\pi_L, \pi_U\} = \text{qbinom}(c(0.025, 0.975), \text{size} = 1094, \text{prob} = 2/1094)/1094$

1. Calculate the limits using the ‘exact’ method described in section 2.1.1. [i.e., instead of obtaining the upper 95% CI using the point estimate, one should vary the upper limit until the probability of 2 or fewer is 0.025; and conversely for the lower limit.] Compare your answer with that of the sociologist, and comment.
2. What limits would the sociologist have obtained had the observed proportion been 0/1094?

3. What (posterior) limits would you obtain had the observed proportion been 0/1094?
4. What limits would Laplace, with his 'law of succession,' have obtained had the observed proportion been 0/1094?
5. You probably obtained the limits by trial and error, varying the limit until you obtained the appropriate tail areas. Can you see how to use Fisher's shortcut (which uses the equivalence between the Binomial tail area and the tail area of an F distribution with certain numerator and denominator degrees of freedom – see question 0.3) to avoid the trial and error? In other words, can you obtain the limits for  $\pi$  directly, using the  $qf(p, df_{num}, df_{denom})$  function?
6. How far off would you be if you treated the numerator (the 2) as the realization of a Poisson (rather than Binomial) random variable with mean (expectation)  $\mu$ , obtained the 95% CI for  $\mu$  [see next chapter], and then divided its limits by 1094 to get the 95% CI for  $\pi$ ?