Where do the (big) data come from?
2. Statistical Inference, and Probability sampling

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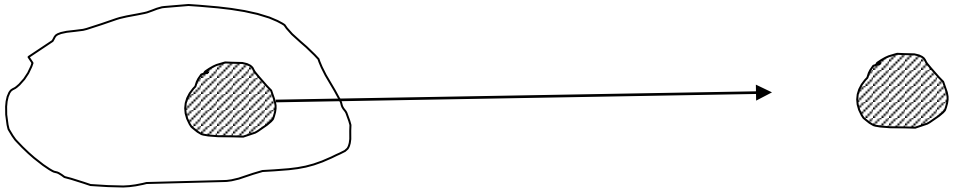
Inference for a population based on a sample

- Statistical inference: the process of making inferences about parameters of a population based on sample data.
- Usually use Roman symbols for the sample quantities, and Greek symbols for the population quantities



• Population		• Sample	
•Mean	μ	•Mean	$\bar{x}$
•SD	σ	•SD	s

- Inference crucially requires that sample is randomly selected from population (or an assumption that it is)

The two main tools of classical (frequentist) inference

- **Hypothesis testing:** key output is the P-value
- **Confidence interval:** a random interval that includes the true value of a parameter in a given proportion of repeated samples (e.g. 95%)
- Concepts are related: a 95% confidence interval includes the set of hypotheses that are “consistent with the data” – $P > 0.05$

Hypothesis testing

- A scientific hypothesis is converted into a null hypothesis H_0 about the value or values of one or more parameters.
- The key output of a hypothesis test is a P-value between 0 and 1 that measures whether the observed data are consistent with the null hypothesis.
  - Small P-Value (say less than 0.05) indicates evidence against the null: either the null hypothesis is false or an unlikely event has occurred. The null hypothesis is "rejected“
  - Large P-Value indicates lack of evidence against the null. The null hypothesis is "accepted", or more precisely, "not rejected".
- *Important:* “Accepting" the null hypothesis does *not* imply that the null hypothesis is true, only that data do not contradict it.

Elements of a hypothesis test

- A scientific hypothesis, e.g. “new treatment is better than old treatment”
- An associated null hypothesis H_0. The null hypothesis is often counter to the scientific hypothesis, e.g. “the average difference in outcomes between treatments is zero”.
- An alternative hypothesis H_a: legitimate values of the parameter if H_0 is not true.
- A test statistic T computed from the data, which (a) has a known distribution if the null hypothesis is true and (b) provides information about the truth of the null hypothesis.
- The P-Value for the test is:

$$P = \Pr(\text{test statistic the same or more extreme than } T \mid H_0)$$

- Small P-values are evidence against the null hypothesis

More on P-Value

P-Value = $\Pr(\text{"data"} \mid H_0)$

"data" = "values of T at least as extreme as that observed".

Measures consistency of data with H_0

P-Value is *not* $\Pr(H_0 \mid \text{data})$

That is, is not the probability that H_0 is true given the data

(Latter is computed in Bayesian hypothesis testing)

Strength of evidence against null

As measures of statistical evidence, we can informally divide P-Values into intervals, as follows:

- $P < 0.01$: strong evidence against null (but some argue for $P < 0.005$)
- $0.01 < P < 0.05$: weak evidence against null
- $0.05 < P < 0.1$: at best marginal evidence against null
- $P > 0.1$: data consistent with null, different values of P above 0.1 (e.g. 0.2, 0.7) have little impact on conclusions

Smaller deviations from the null can be detected with larger sample sizes, so the P-Value is strongly dependent on sample size – it is <u>not</u> a good measure of the size of the effect.

Problems with P-Values

- P-value is not the probability that the null hypothesis is true, $p(H_0|data)$; it measures consistency of the data with the null hypothesis, $p(data|H_0)$
- P-value is poor measure of the size of an effect –
  - mixes estimate of effect and its uncertainty
  - size of P-value has no clinical meaning
  - P value is strongly determined by sample size – since nothing is exactly zero, anything is significant with a large enough data … so P-Values have limited use for big data!
  - The more important question is the size of the effect, not whether it differs from zero

Problems with P-Values

- The conventional cut-off for statistical significance – $P < 0.05$ – is weak evidence – when translated to the Bayes factor for reasonable choices of alternative, it is too weak to establish effects.
- Some (e.g. Val Johnson) advocate the more stringent cut-off $P < 0.005$. Hence my limerick:

"In statistics, one thing do we cherish
P .05 we publish else perish
Val says, that's so out-of-date
Our studies don't replicate
P .005, then null is rubbish!"

Confidence intervals

- A confidence interval -- estimate with associated measure of uncertainty
- Confidence interval property – in hypothetical repeated samples, the 95% interval includes the true value of the parameter at least 95% of the time. Here 95% is the "nominal coverage" of the CI
  - Example: 95% CI for population mean in a normal sample of size n with mean $\bar{x}$, sd s is

$$\bar{x} \pm t_{.975} s / \sqrt{n}$$

where $t_{.975}$ is the 97.5th percentile of the t distribution with n – 1 degrees of freedom. In particular

$t_{.975} = 1.96$ if $n > 50$, $t_{.975} = 2.447$ if $n = 7$.

Roughly "estimate +/- two se's" for moderate size n

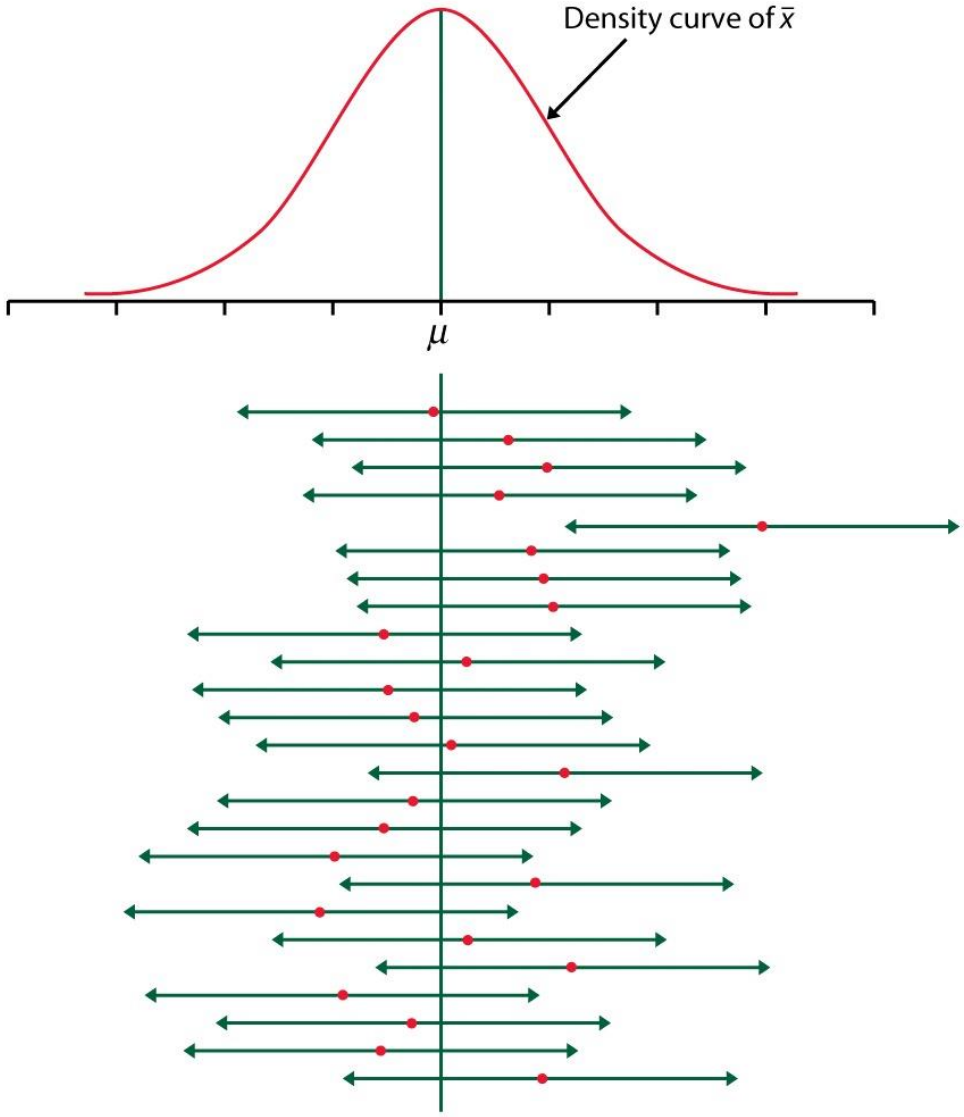
Ex: Confidence interval for population mean

- A confidence interval (CI) is a measure of uncertainty for a sample estimate (e.g. $\bar{x}$) of a population quantity (e.g. μ)
- range of values computed from the sample within which the population quantity is likely to lie
- A 95% confidence interval for a population mean μ (if the sample size *n* is large, say greater than 30) is

$$C_{.95}(\mu) = \bar{x} \pm 1.96\left(s / \sqrt{n}\right)$$

- Works because of the *Central Limit Theorem*, which shows that means have a normal distribution in repeated samples
- Random sample from population is a key assumption
- Confidence interval interpretation: in 95% of repeated samples, this random interval covers the true mean

CI interpretation: “in 95% of repeated samples, this random interval covers the true mean”



Confidence Intervals: better than P-values

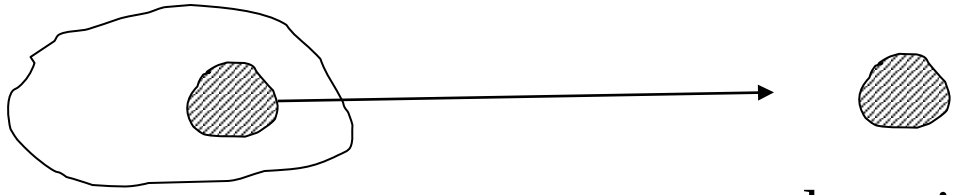
- Estimate has clinical meaning – closer to the science. Good measurement is the heart of statistics
- width of interval captures uncertainty in a natural way
- Confidence interval summarizes the evidence in a natural way
- But confidence intervals are peculiar objects: the interval is random, but the parameter is fixed
- *Bayesian statistics* provide credibility intervals where interval is fixed, parameter is random

Some quotes about data

- Data always have errors --
- “If a statistic is interesting, it’s probably wrong!”
  - Sir Claus Moser, UK Central Statistics Office
- For “found” big data, not collected with any particular objective or design, beware of the GIGO principle:
- “garbage in, garbage out”
- “It ain’t so much the things we know that get us into trouble. It’s the things we know that just ain’t so” -- Artemis Ward

Root mean squared error of an estimate

- Statistical inference: the process of making inferences about parameters of a population based on sample data



population quantity θ sample estimate $\hat{\theta}$

- Statistical measures of quality of the estimate include:

Bias: $B(\hat{\theta}) = E(\hat{\theta}) - \theta$

Variance: $Var(\hat{\theta})$, Standard Error: $SE(\hat{\theta}) = \sqrt{Var(\hat{\theta})}$

Mean Squared Error: $MSE(\hat{\theta}) = B^2(\hat{\theta}) + Var(\hat{\theta})$

Root Mean Squared Error $RMSE(\hat{\theta}) = \sqrt{B^2(\hat{\theta}) + Var(\hat{\theta})}$

Precision and accuracy

- An estimate is precise if it has low uncertainty -- small standard error, narrow confidence interval
- An estimate is accurate if it is precise and close to the true value – small bias and standard error, small RMSE, narrow confidence interval around true value
- E.g. suppose a true target proportion is **T=0.4**
- Estimate = 0.5, confidence interval = (0.1, 0.9) [-----T------]

  - low precision and accuracy, but no evidence of bias
- Estimate = 0.5, Confidence interval (0.47, 0.53) T [---]

  - high precision, low accuracy (biased)
- Estimate = 0.42, Confidence interval = (0.39, 0.45) [-T-]

  - high precision, high accuracy (no evidence of bias)

Big data: precise but potentially inaccurate?

- Generally speaking, as sample size *n* increases:
- Precision increases, but bias stays constant (or may even increase)
- With small sample sizes, maximizing precision is important
- With large sample sizes, minimizing bias is important

Big Data

- “Big Data” – large data sets, often not collected for a specific research objective with a statistical design – e.g. internet data, administrative data
- Large implies high statistical precision, but high potential for bias (that is, estimates may be inaccurate – and get the wrong answer)

Two roles of randomization to avoid bias

- Random selection of participants
  - Ensures unbiased selection
  - Enhances external validity – inference for population based on sample
- Random allocation of treatments/factors
  - Ensures unbiased assignment
  - Enhances internal validity – valid treatment effect for individuals in the sample
  - Absent in observational studies
- Ideally we would like both, but this is very rarely achieved

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Properties of a good sampling scheme

- "representative" of the population (... whatever that means)
- demonstrably free of selection bias
- repeatable (at least in theory)
- efficient: lowest cost for given level of precision
- measurable precision: e.g., can quantify how close the sample mean is to the population mean it is estimating.
- Only probability (or random) sampling designs have these properties. Probability samples are characterized by the following two properties:
  - every sample has a known (maybe zero) probability of selection
  - every element (individual) in the population has a (known) positive probability of selection.

Probability sampling defined

- Probability samples are characterized by the following two properties:
  - every sample has a known (maybe zero) probability of selection
  - every element (individual) in the population has a (known) positive probability of selection
  - Examples to follow
- "scientific" sampling – allows statements about uncertainty for estimates of population quantities
  - In practice, frame errors and nonresponse can reduce effectiveness

Non-random sampling methods

- Examples of non-random sampling methods are:
  - Convenience sampling: Sample readily accessible individuals
  - Purposive or judgmental sampling (???)
  - Self-selected samples – e.g. open-access internet surveys
  - Quota sampling
  - Opt-in sampling for internet surveys
  - Snowball sampling
- These methods are less scientific and less trustworthy than probability sampling, since they are subject to hidden biases.
- Note: “big data” are usually not based on random samples

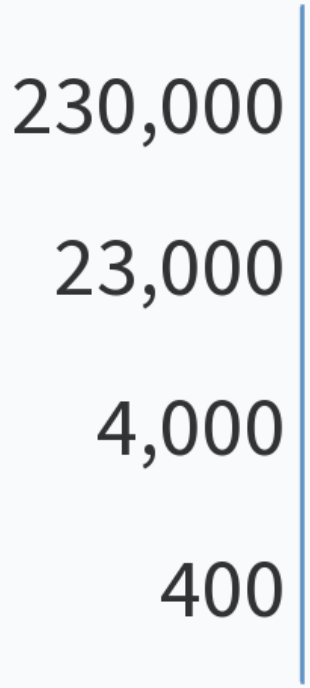
Probability sampling and "big data"

Xiao-Li Meng's (2018) "Law of Large Populations" (LLP) suggests that the value of probability sampling increases with the sample size, and is surprisingly high:

Meng's "data-defect correlation" $\rho_{R,X}$ is the correlation between inclusion in a sample (R) and a survey outcome (X). For random sampling $\rho_{R,X} = 0$, non-zero values indicate selection bias.

A sample of 2,300,000 *self-reporting* voters from the Cooperative Congressional Election Study (CCES) of the 2016 US presidential election had a miniscule $\rho_{R,X} \approx -0.005$ for voting for Donald Trump. This (seemingly minuscule) data-defect correlation implies that for proportion voting for Trump, this sample of **n ≈ 2,300,000**, has the **same mean squared error** as the sample proportion from a **genuine simple random sample of size n(srs) ≈ ???**

n =2.3 million from CCES has equivalent RMSE to a simple random sample size n(srs) of



Simple Random Sampling

- The most familiar form of probability sampling (but there are others)
- With and without replacement
- Simple random sampling without replacement corresponds to selecting *n* balls out of a well-mixed urn containing *N* balls (like some lotteries). For this method:
  - All possible samples of size *n* have an equal probability of being selected.
  - All samples of size not equal to *n* have zero probability of selection
  - every individual has probability *n*/*N* of selection

SRS example

- Example. Suppose the urn contains $N = 5$ balls, labeled {A B C D E}; this is our population. We select a simple random sample of $n = 2$ balls. There are 10 possible samples of size 2, namely:
- AB, AC, AD, AE, BC, BD, BE, CD, CE, DE
- Since all these samples have the same chance of being selected,
  - Pr(any size 2 sample selected) = 0.1
  - Pr(any other sample selected) = 0
  - Pr(any particular ball is included) = 0.4

Neyman's famous paper

ON THE TWO DIFFERENT ASPECTS OF THE REPRESENTATIVE METHOD: THE METHOD OF STRATIFIED SAMPLING AND THE METHOD OF PURPOSIVE SELECTION.

By JERZY NEYMAN

(Biometric Laboratory, Nencki Institute, Soc. Sci. Lit. Varsoviensis, Warsaw).

[Read before the Royal Statistical Society, June 19th, 1934, the PRESIDENT, the RT. Hon. LORD MESTON of Agra and Dunottar, K.C.S.I., LL.D., in the Chair.]

Probability Sampling versus "Purposive Sampling"

- Initially, probability sampling was equated with its basic form, simple random sampling (SRS)
  - Every sample of size *n* has *equal* chance of being selected, hence an equal probability of selection method (*epsem*)
  - Samples of size other than *n* have no chance of being selected
  - With and without replacement

“Purposive Sampling”

- “Non-probability sampling” – but hard to define a negative.
- Units are picked so that sample matches distribution of a characteristic known for the population.
- E.g. if we know distribution of age and gender in population, choose sample cases to match this distribution.
- A common form is *quota sampling*: interviewers are given a quota for each age group and gender and interview individuals until this quota is met

The Controversy

- Under simple random sampling, distribution of a known characteristic in the sample can deviate considerably from its (known) distribution in the population, purely by chance
- This “lack of representativeness” led some to prefer purposively picking the sample to match the population distribution

Neyman's "Resolution"

- Neyman (1934) showed that we can get the best of both worlds by stratified sampling:
  - Create strata by the classifying population according to the known characteristics
  - Select a simple random sample of known size n_j from population of size N_j in stratum j
- If $f_j = n_j/N_j$ = const., results in epsem sample, retains probabilistic selection, and sample matches distribution of strata in population
- Also one can vary f_j and weight sample cases by $1/f_j$: Neyman's optimal allocation

Footnote to Neyman's paper

- Neyman's paper is also famous for introducing (in English) the idea of confidence intervals– intervals with at least the nominal coverage in repeated samples.
- Ushered in the era of Neyman and Pearson significance testing
- Fisher notably fought with Neyman over this idea, calling it a "confidence trick"

More Complex Designs

- Neyman's paper helped to set the stage for extensions to cluster sampling, multistage sampling, greatly extending the practical feasibility and utility of probability sampling in practice
- E.g. simple random sampling of people in the US is not feasible – we do not have a complete list of everyone in the population from which to sample
- Work of Mahalanobis, Hansen, Cochran, Kish, ….

Checking "Representativeness"

- One way of assessing representativeness is to compare distributions of known variables for the sample and the population
  - e.g. target population = U.S. Civilians
  - compare sample distribution of age, race, and sex with the population distribution from the nearest census.
  - should be done if possible, but are of limited value: really need to compare variables closely associated with the variables of interest

Summary

- Random sampling: a scientific way of achieving representativeness (on average)
- Big data that are not a random sample of the population may yield biased answers – proceed with caution

Tomorrow's topic

- Random selection of participants
  - Ensures unbiased selection
  - Enhances external validity – inference for population based on sample
- Random allocation of treatments/factors
  - Ensures unbiased assignment
  - Enhances internal validity – valid treatment effect for individuals in the sample
  - Absent in observational studies
- Ideally we would like both, but this is very rarely achieved

For Wednesday

- Clinical trials for comparing treatments
- A little homework before tomorrow: read the (a) Cameron and Pauling and (b) Creagan et al. articles on Vitamin C as a treatment of advanced cancer, in the course site
- Why do they give some different conclusions? What are strengths and weaknesses