Sample Size, Power and Sampling Methods

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- Designed for physicians and health care professionals
- Intended to strengthen capacity and leadership in research related to respiratory conditions, critical care and sleep medicine in middle and low income countries

Testing a hypothesis

- We want to make inferences about a population from a sample.
  - i.e., we have a hypothesis we want to test

- We need to choose the number of observations to include in a study sample.
  - The larger the sample, the greater the power of the statistical test

=> Sample size determines statistical power

Testing a hypothesis

- What is (statistical) power?

$\Rightarrow$ It is the probability that we will observe an intervention effect (based on data from our sample) when an intervention effect actually exists.

Testing a hypothesis: Example

- Say we want to test a text messaging intervention to remind diabetes patients to take their meds on schedule, which should result in lower HbA1c levels.
  - We randomly assign patients to the **text message intervention** or to the "**usual care" control** groups.
  - Define the mean difference between HbA1c scores in the intervention and non intervention groups as our effect size:

$$\delta_{diff} = \delta_{tx} - \delta_{no\ tx}$$

δ_{diff} is the "effect size"

Testing a hypothesis: Example

- We specify mutually exclusive "null" and "alternative" hypotheses":
  - Null hypothesis: there is no difference in mean HbA1c between patients receiving the text messages and those not:

$$H_0: \delta_{diff} = 0$$

  - Alternative (2-sided) hypothesis : mean HbA1c differs between patients who do and do not get the text intervention:

$$H_A: \delta_{diff} \neq 0$$

The truth vs what we observe

- What is the truth?
  - Either the intervention is effective or it isn't (we don't know which)
- What do we observe (we only do the study in a sample of the intended population)
  - Either we will observe an effect on HbA1c level when we analyze the data from our sample or we won't
- We want to optimize our chances of:
  - Observing an effect in our data **if** the truth is that the intervention really works.
  - NOT observing an effect **if** the truth is that the intervention really doesn't work

Testing a Hypothesis

What we see (in our sample)	The "Truth" (unknown to us) Our text intervention is:	
	Effective	Not Effective
We observe a difference in HbA1c	OK	error!
We don't observe a difference in HbA1c	error!	OK

Testing a hypothesis

- Let's think about these possibilities in terms of probabilities

Testing a Hypothesis

What we see (in our sample)	The "Truth" (unknown to us) Our text intervention: Works	 Doesn't Work
We observe a difference in HbA1c	Probability of deciding **there is an effect** when there really is one	Probability of deciding **there is an effect** when there really isn't one
We don't observe a difference in HbA1c	Probability of deciding **there is no effect**, when there really is one	Probability of deciding **there is no effect** when there really isn't one

Testing a Hypothesis

What we see (in our sample)	The "Truth" (unknown to us) Our text intervention: Works	 Doesn't Work
We observe a difference in HbA1c	Power = $1 - \beta$	Type I error $= \alpha$
We don't observe a difference in HbA1c	Type II error $= \beta$	$1 - \alpha$

Testing a Hypothesis

What we see (in our sample)	The "Truth" (unknown to us) Our text intervention: Works	 Doesn't Work
We observe a difference in HbA1c	Power = $1 - \beta$ Usually $\geq .80$	Type I error $= \alpha$ Usually $<.05$
We don't observe a difference in HbA1c	Type II error $= \beta$ Usually $< .20$	$1 - \alpha$ Usually $\geq .95$

Testing a Hypothesis

- Setting $\alpha = .05$ is traditional (and arbitrary)
  - i.e., we accept a 5/100 (or 1 in 20) chance of making type I error (i.e., deciding that there IS an effect when there really ISN'T)
- Setting power = .80, (i.e. $\beta = .20$) means we expect to have at least an 80% chance of detecting an effect when there really is one.

Testing a Hypothesis

- So… if we want to have 80% power and a 5% significance level how many patients do we need?

Sample Size (N) depends on

- Type I error = α
- Power = 1- β
- Effect size = Estimate of expected treatment effect, $\delta_{diff} = \delta_{tx} - \delta_{no\ tx}$
- Estimate of variability in treatment effect, σ
  - Will therapy affect everyone to the same degree or does the effect vary substantially, affecting some patients a lot and others very little?

Sample Size

- Some common sample size applications assume that your data are normally distributed

A simple sample size calculation:

$$2n = \frac{4(Z_{1-\alpha} + Z_{1-\beta})^2 \ \sigma^2}{\delta^2}$$

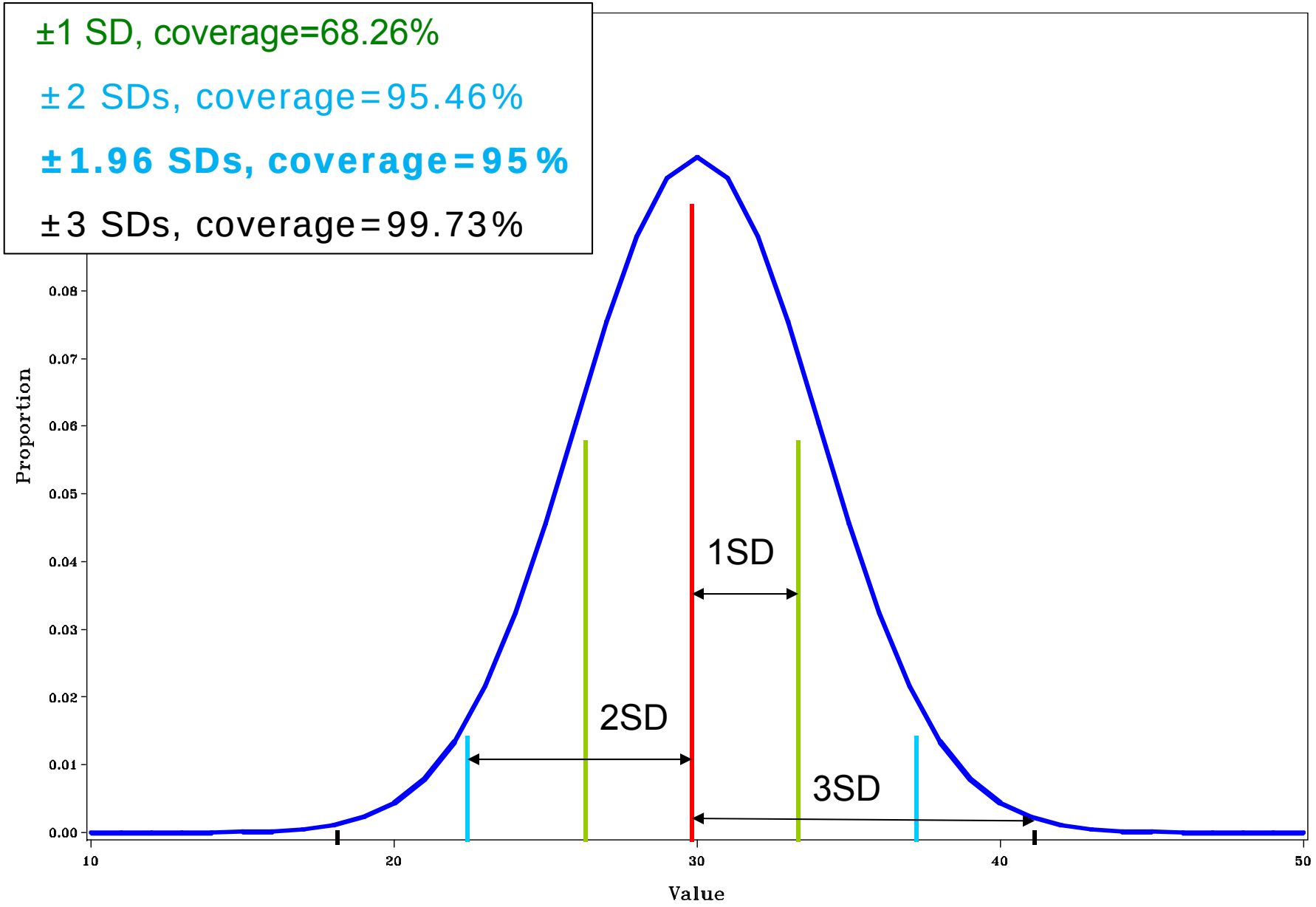
Normal Distribution

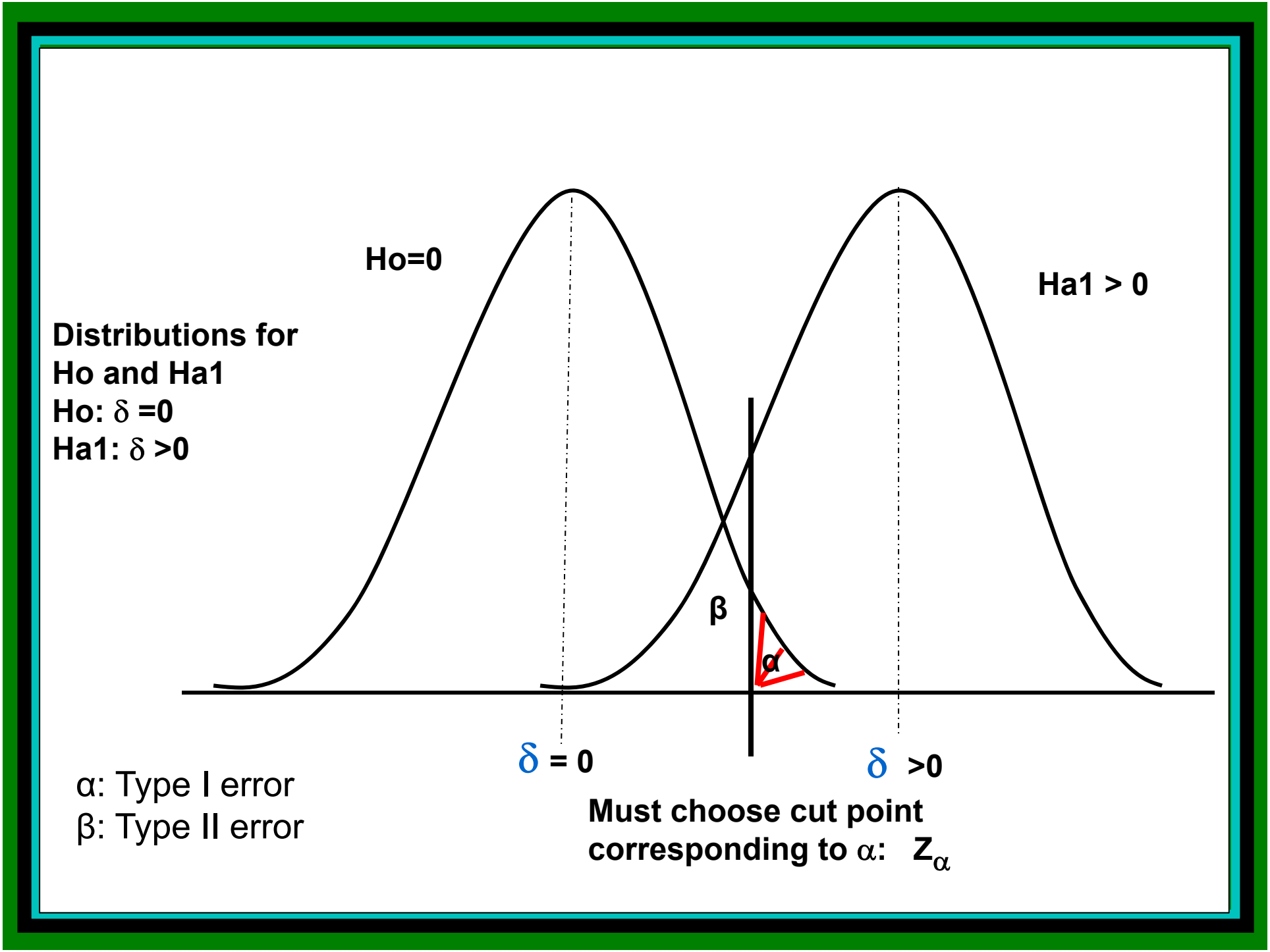
±1 SD, coverage=68.26%

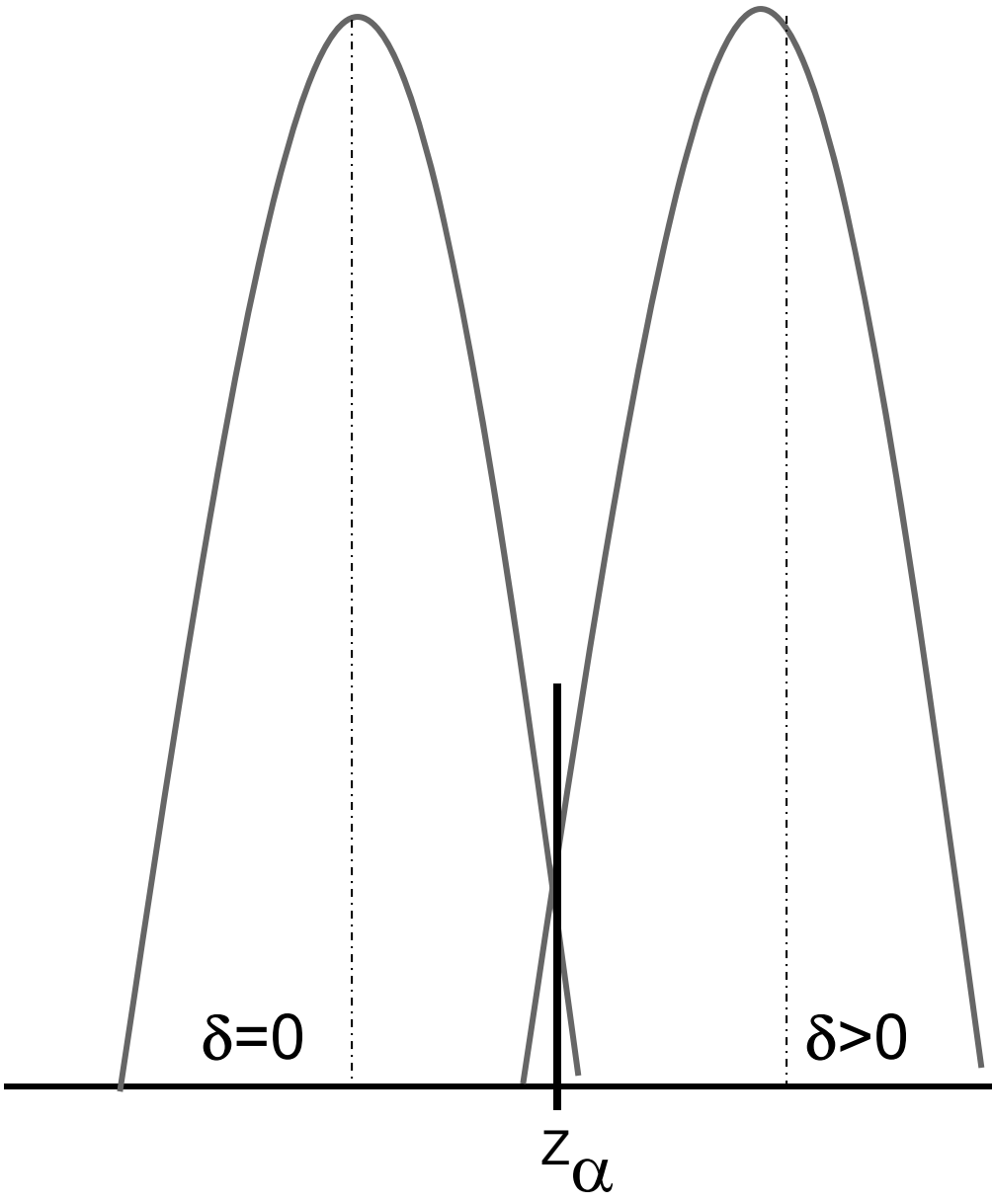
±2 SDs, coverage=95.46%

±1.96 SDs, coverage=95%

±3 SDs, coverage=99.73%




Distributions for
Ho and Ha1
Ho: δ =0
Ha1: δ >0
Ho=0
Ha1 > 0
β
α
δ = 0
δ >0
Must choose cut point
corresponding to α: Z_α
α: Type I error
β: Type II error



Increase in Sample Size
Tightens the Distributions
Decreases the Overlap

Both α and β are decreased

Trade-offs between sample size, power and effect size

- If we want to increase power, say from 80% to 90%, we must increase sample size
- If we want to detect a smaller effect size, say a 10% increase in survival instead of a 20% increase, we must increase sample size

Quiz

What is β?

- Type II error: The probability of failing to reject the null hypothesis when it is false

What is α?

- Type I error: The probability of rejecting the null hypothesis when it is true

What is 1-β?

- Power: the probability of accepting the alternative hypothsis when it is true.

Resources for power and sample size calculations

- http://davidmlane.com/hyperstat/power.html
- http://homepage.stat.uiowa.edu/~rlenth/Power/

Sampling Methods

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Sampling

- Methods for selecting subjects for study e.g., people, organizations, trees, etc.) from a population of interest .
- Often we want to generalize results of study to the larger population.

Sampling

- Theoretical population vs accessible population
- Usually not feasible to sample full population.
  => Sample some members of a theoretical population as representative of the entire population
- Multi-step process
  - Identify population of interest
  - Identify accessible population
  - Develop sampling frame
  - Draw sample
  - Contact/recruit subjects/participants

Types of sampling

- Convenience sampling = identify a group of people you can get to "conveniently“
  - Examples: hospital staff, market women, senior housing
  - No "formal" sampling frame is used
  - Not able to calculate confidence intervals
  - Useful for many purposes

- Probability sampling = random selection
  - SRS (‘simple random sample’)
  - Stratified sample
  - CS (‘cluster sample’)

Random Sampling

- Involves creation of a "Sampling frame" (list of members of group to be sampled).
- Assures equal probability of selection for all members of the population.
- Estimates will be "unbiased" (precise statistical meaning: average of multiple samples will have population mean)
- Note: random does not mean "haphazard"

Simple Random Sampling (SRS)

- SRS Process:
  - Develop sampling frame: List accessible population of N subjects from which n subjects will be drawn (e.g., all individuals/HHs).
  - Use random process, e.g. random number table, to generate "n" numbers between 1 and N.
  - Identify "n" individuals in sample corresponding to the "n" numbers generated.

- Examples: phonebook - random digit dialing.

Simple Random Sampling (SRS)

- Advantages:
  - Most basic form of sampling
  - The gold standard to which other methods are compared.
- Disadvantages:
  - All individuals/HH must be identified prior to sampling
  - May be unrealistic - time, money.
  - Selected individuals/HHs may be highly dispersed. Visiting each may be very time consuming

Stratified Random Sampling

- Divide population in homogeneous (mutually exclusive) subgroups (i.e., “strata”) and do SRS within each group
- Advantages
  - Enough cases from each strata to make meaningful inferences on key subgroups (e.g., small minority groups).
  - Generally have more statistical precision than SRS **if** the strata are homogeneous:

    => the variability within-groups is lower than the variability for the population as a whole.

    => smaller sample sizes
- Examples of common strata: age, race, gender, educational attainment level, socioeconomic status, rare traits, diseases or conditions

Cluster Sampling (CS)

- CS Process
  - Construct sampling frame using groups or clusters of individuals (or HHs) without identifying or listing each one.
    - Cluster = villages, towns, districts, urban blocks, etc.
  - List clusters
  - Take a random sample of the clusters
  - Obtain list of individuals/HHs only for those clusters selected in the sample
  - Sample a random sample of individuals/HHs within each selected cluster

Cluster Sampling (CS)

- Advantages:
  - Economy: listing costs/travel costs.
  - Feasibility: most countries will have lists of population by groups (villages, towns)
  - Allows a larger sample size than SRS due to decreased costs
    - for same resources (money, person-time), it's possible to gain greater precision with cluster sampling
- Disadvantages:
  - Estimate not as precise as SRS for the same sample size.
    - Design Effect = Variance Cluster Sampling/Variance SRS. Factor by which to increase CS sample size to obtain same precision as for SRS
- Example: WHO Expanded Programme on Immunization (EPI) sampling method.

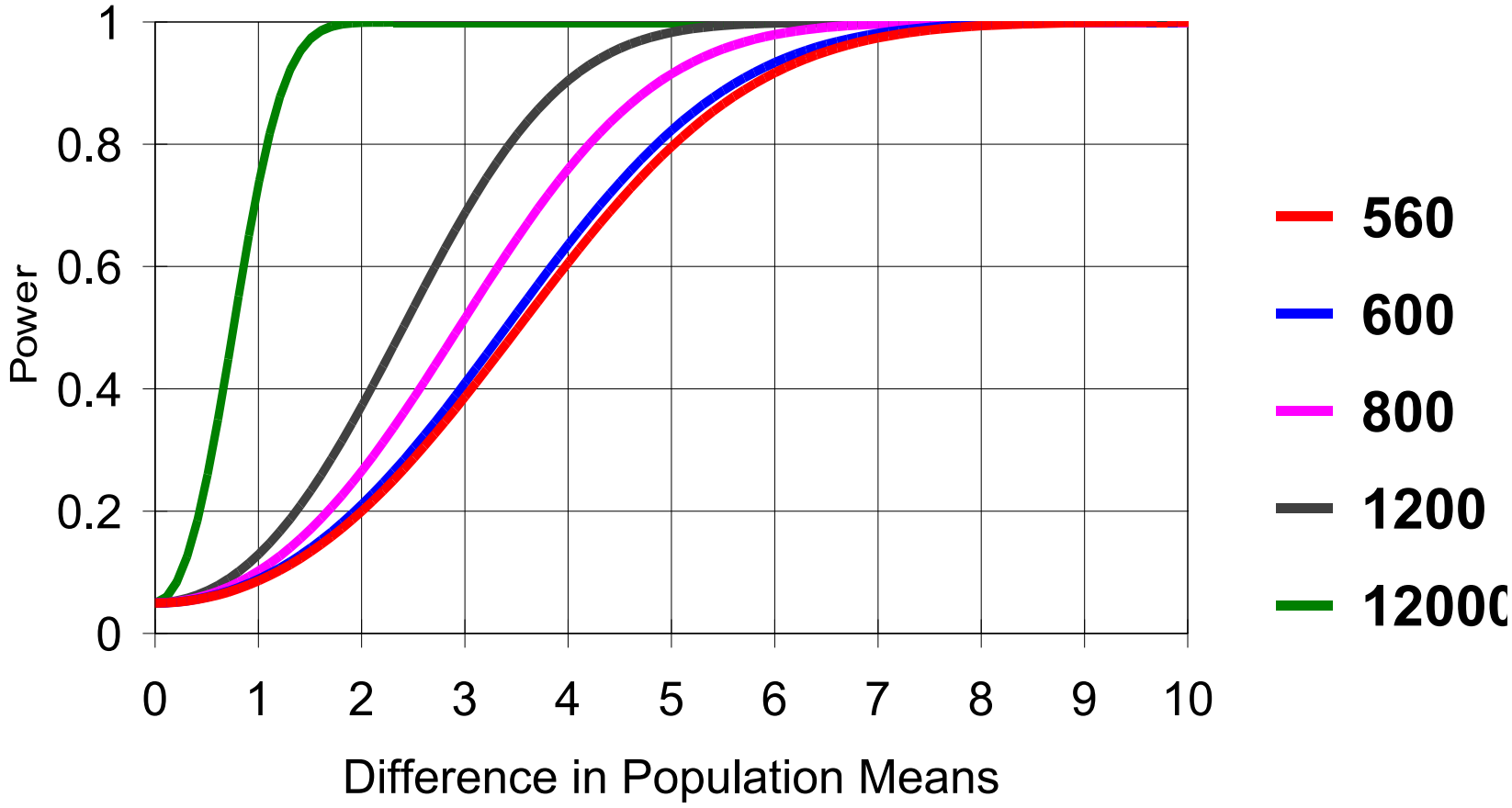
Cluster Sampling (CS)

- Considerations
  - Budget – transport costs can be high, esp. in rural areas
  - Travel/logistics
    - Define center/periphery of urban areas.
    - How to account for appartments vs houses – need to establish unambiguous methods, eliminate personal choice of researchers
    - Number of surveys in given area per day.
  - Supplies
  - Technical
    - Can estimate optimal cluster size if you know:
      - » transport costs to each cluster
      - » cost for interviewing each respondent
      - » Roh

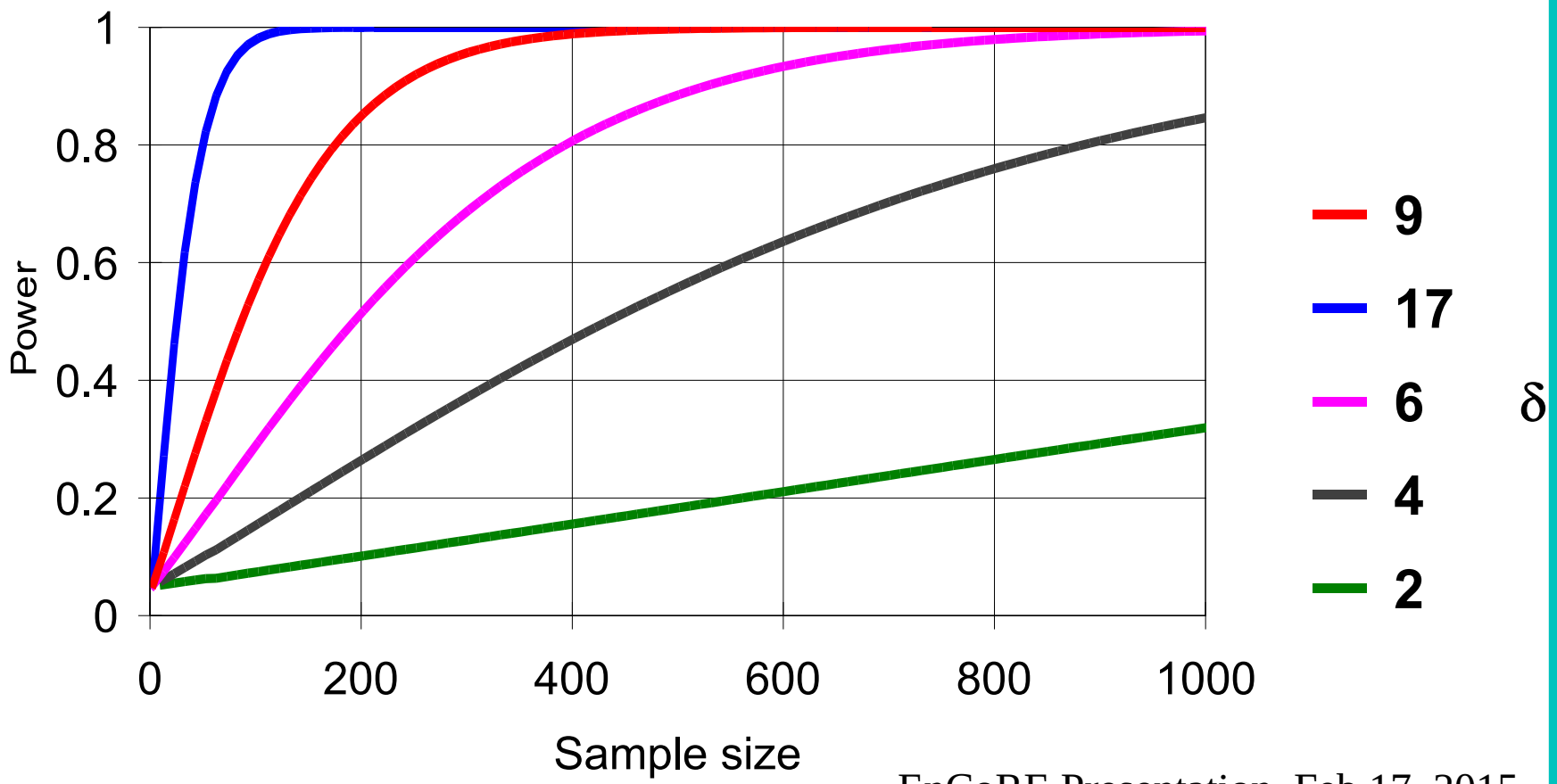
Questions?

Thank You!

How sample size affects the power to detect differences



How δ affects the relationship between power and sample size



How variability affects the relationship between power and sample size