Common Outcome Measures in Radiology

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Introductions
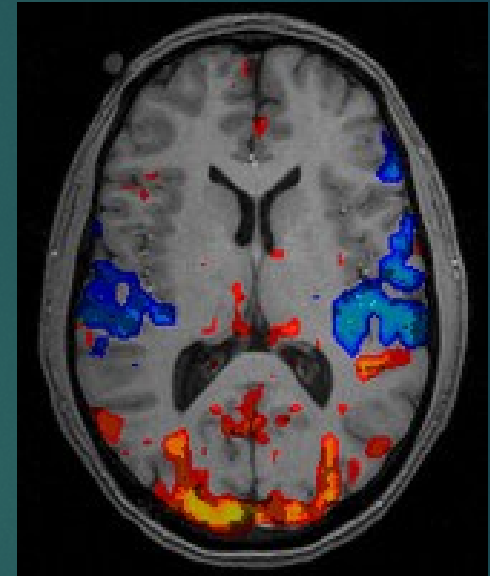
- Who we are

- What do biostatisticians do anyway?

- Why are you teaching this class?

Motivation

▶ Which imaging modalities are most helpful for diagnosis or assessment of treatment response?

▶ Which interventional radiology approach provides the best patient outcomes or fewest adverse events?

Overall learning objectives

- Identify common study designs used in medical imaging research
- Form a testable hypothesis from a clinical problem or question
- List the steps required to plan a research project
- Perform a literature review
- Identify safeguards for ethical human research
- Locate funding opportunities on the web
- List the steps required to submit a grant proposal
- List the steps required to execute a research study
- List the steps required to publish medical manuscripts

Outline for today's session

- Review common patient outcome measures
- Review common measures of diagnostic accuracy
- Break
- Review common experimental designs
- Describe common observational designs
- Questions

Format

- 10-15 minutes lecture blocks

- Interactive group worksheets

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▶ **Review common patient outcome measures**

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▶ Questions

Measurement scales

- Nominal data

Measurement scales

- Ordinal

Control   Doxil alone   RF alone   RF & Doxil

Measurement scales

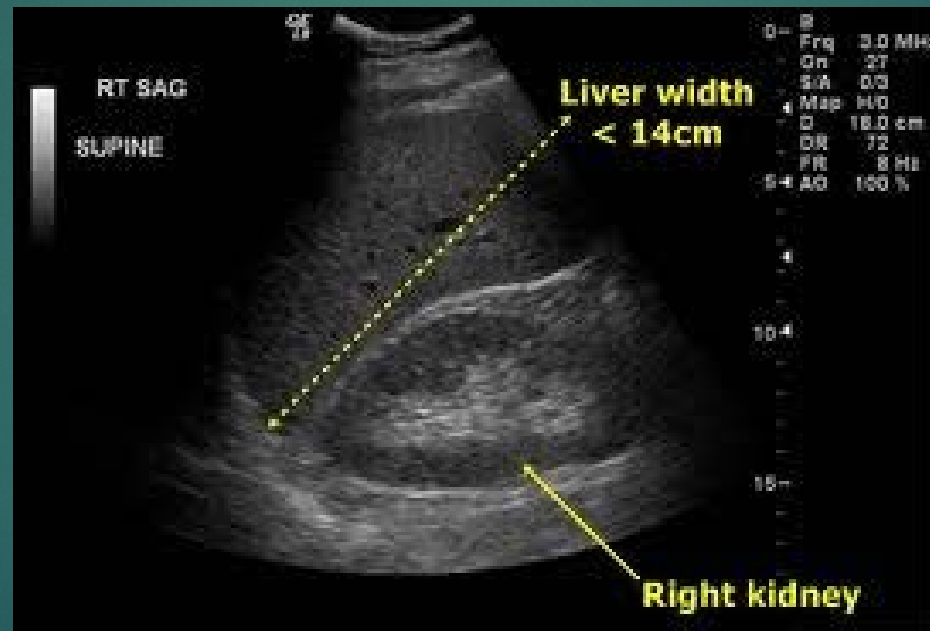- Interval Data

Measurement scales

- Ratio Data

Other ways to classify data

▶ Continuous values
  ▶ Cerebral blood flow

▶ Categorical values
  ▶ Tumor vs. Necrosis

▶ Time to event (censored data)
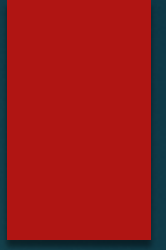 ▶ Patient survival

Patient outcomes in radiology

- Quality of life and disability scores
  - EDSS
- Image characteristics
  - SUVMax
- Anatomic characteristics
  - Hip anteversion
- Survival
  - Overall or progression-free
- Adverse events
  - Contrast reactions

Independence and Correlation

▶ Independence indicates that the occurrence of one event does not affect the probability of another

▶ When events are not independent, they are said to be **correlated**
  ▶ Multiple measurements taken on the same patient
  ▶ Measurements taken on patients from the same clinic or group

▶ *Correlation must be accounted for in the statistical analysis plan*

Worksheet #1

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▶ **Review common measures of diagnostic accuracy**

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Diagnostic accuracy

- Diagnostic tests
  - provide information about a patient's condition
  - guide treatment planning
  - Improve knowledge regarding disease mechanism and natural history

- **Diagnostic accuracy** is the ability of a test to discriminate between health and disease

Sensitivity

	D+	D-
T+	TP	FP
T-	FN	TN

- Sensitivity is the probability of a positive test given that the individual has the disease

$$sensitivity = \frac{TP}{TP + FN}$$

Specificity

	D+	D-
T+	TP	FP
T-	FN	TN

- Specificity is the probability of a negative test given that the individual does <u>not</u> have the disease

$$specificity = \frac{TN}{TN + FP}$$

Positive predictive value (PPV)

	D+	D-
T+	TP	FP
T-	FN	TN

▶ PPV is the probability that the disease is present, given that the test is positive

$$PPV = \frac{TP}{TP + FP}$$

Negative predictive value (NPV)

	D+	D-
T+	TP	FP
T-	FN	TN

▶ NPV is the probability that the disease is <u>not</u> present, given that the test is negative

$$PPV = \frac{TN}{TN + FN}$$

Problems with PPV and NPV

- Depend on the inherent accuracy of the test

- Depend on the prevalence of the disease in the population

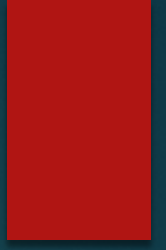- The same test will have **different PPV and NPV** values for different clinical populations

ROC curves

- **Receiver operator characteristic** curves summarize the inherent accuracy of a test

- Greater area under the curve (AUC) indicates a more accurate test

- An ROC curve along the 45° line indicates that the test provides no information about the condition
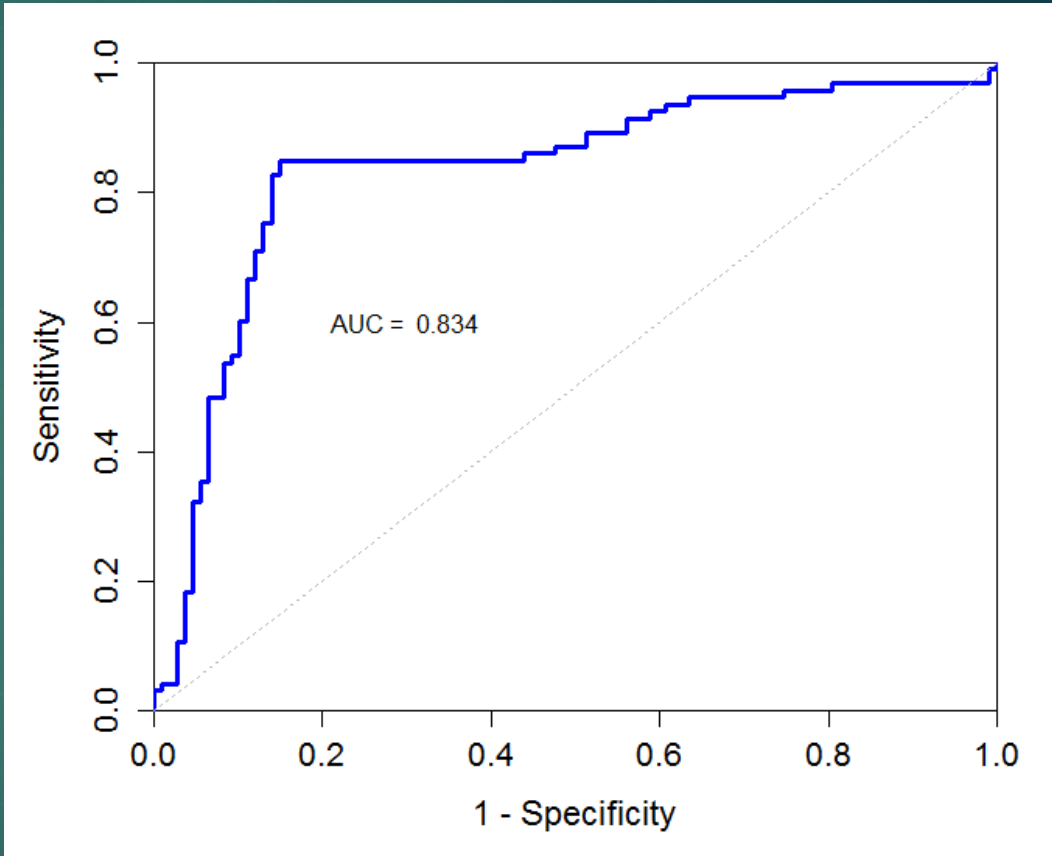
Worksheet #2

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Cross-sectional designs

- Measure an outcome at a single point in time
- Can be used to assess *prevalence* of a condition
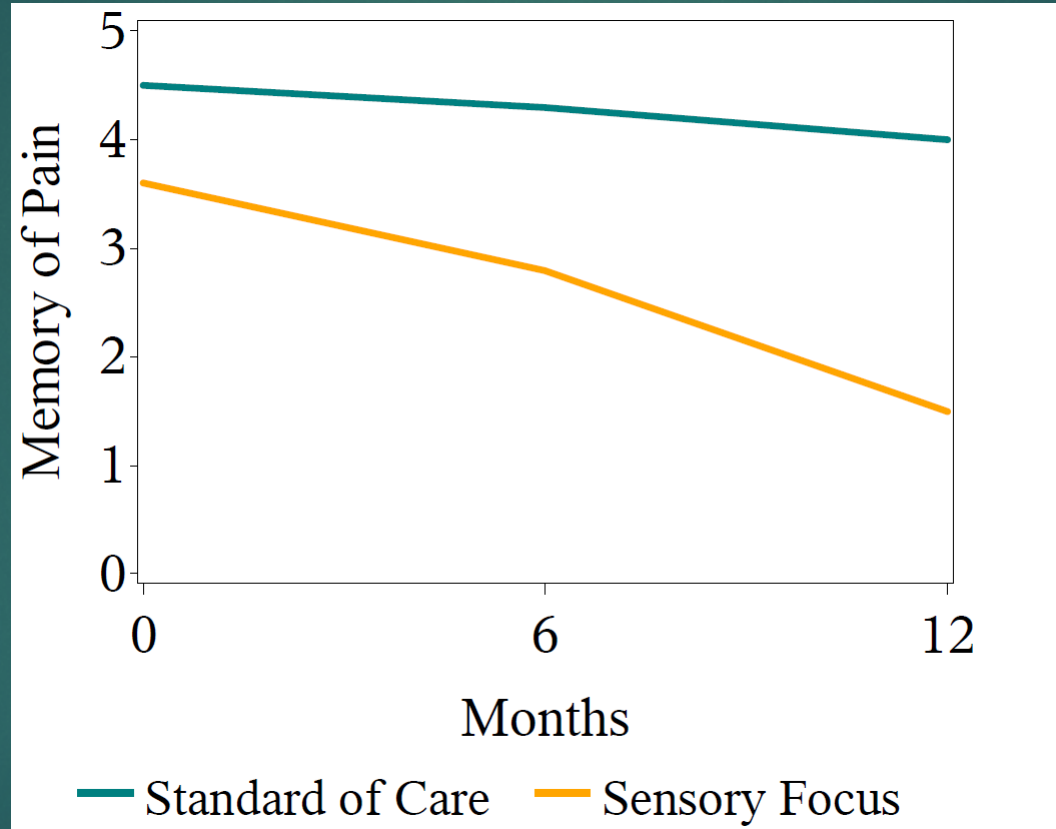- Can be used to test associations between two or more factors

Females	Measure hip anteversion
Males	Measure hip anteversion
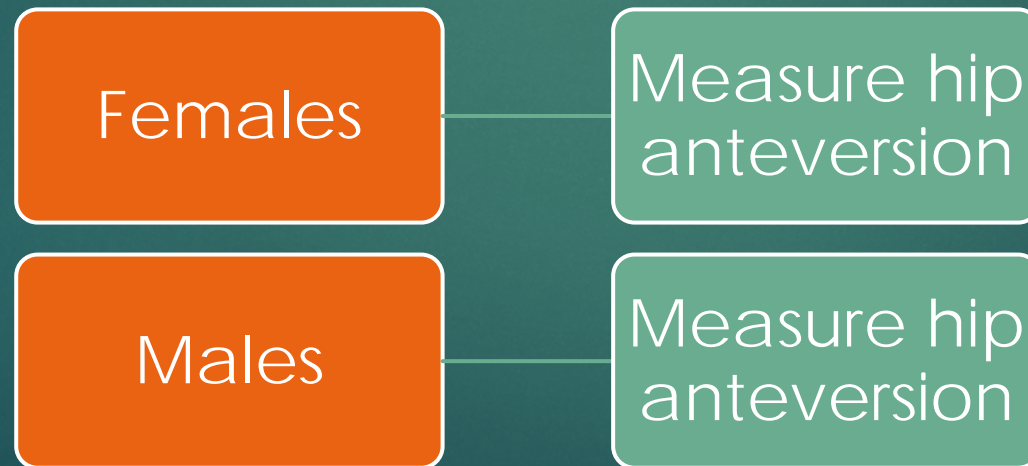
Longitudinal designs

▶ Outcomes are measured over time

▶ Measurements on a single participant are correlated

▶ Can be used to test hypotheses about time trends, treatment effects, and time by treatment *interactions*

Interactions

Multilevel designs

- Participants are organized into groups such as schools or clinics

- Multiple levels form a hierarchical structure

- Measurements on participants within a group are correlated

- Other terms: cluster-randomized trials, group-randomized trials
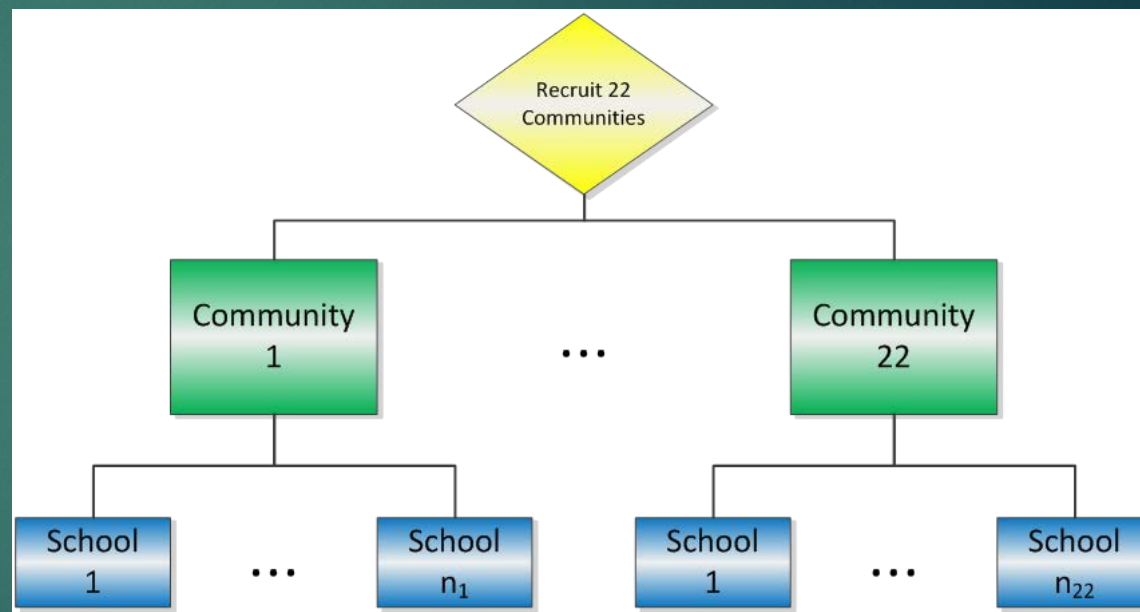
Survival studies

- Evaluate the association between participant characteristics and
  - Risk of an event (death, disease progression, etc.)
  - Time until event occurs

- The event may not occur for all participants during the study period
  - Partial information about time to event
  - Data are called *censored*
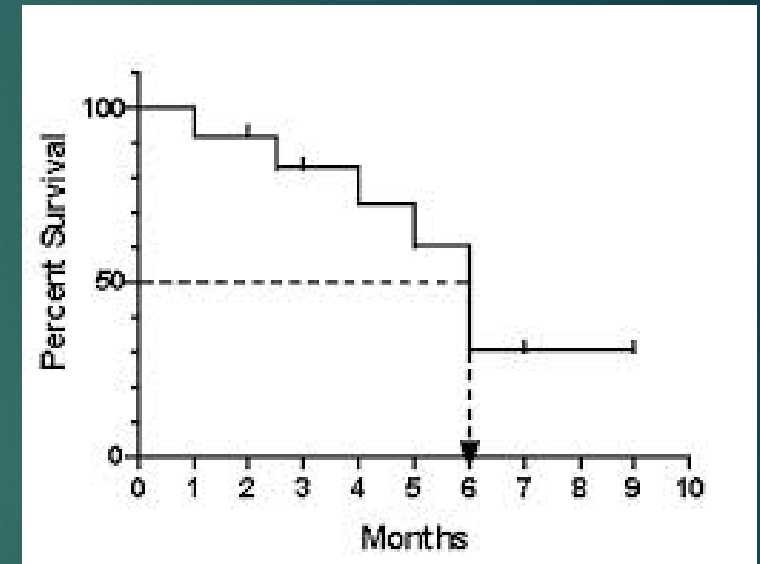
Randomized controlled trials

▶ Participants randomized to one or more experimental conditions

▶ Commonly used to establish efficacy of new therapies

▶ Can test superiority, non-inferiority, or equivalence among treatments

Participant → Randomization → Treatment A → Measure response

Randomization → Treatment B → Measure response
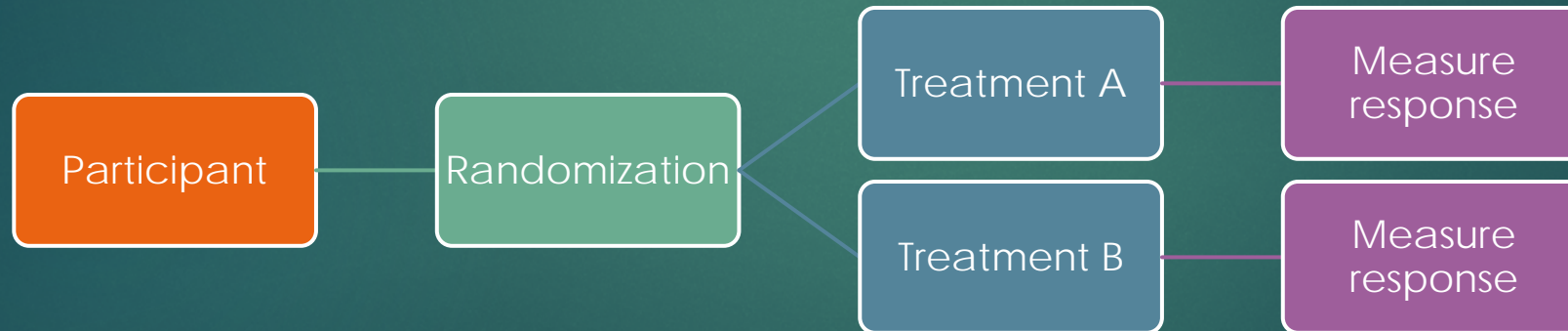
Interobserver and intraobserver reliability studies

- Assess agreement between multiple readers (interobserver) or multiple reads by the same reader (intraobserver).

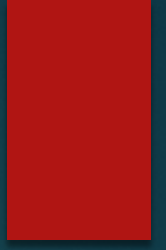|	⭐ Fully Crossed		Not Fully Crossed		
	Reader 1	Reader 2	Reader 1	Reader 2	
Case 1	X	X	X	X	
Case 2	X	X	X	X	
Case 3	X	X		X	
Case 4	X	X	X		
Case 5	X	X		X	

Diagnostic accuracy studies

▶ Participants are measured on both the *index test* and the *reference standard*

▶ Determines if a test can discriminate between individuals with and without the condition

▶ Determine if the test changes the likelihood of a patient having the condition

▶ Assess sensitivity, specificity, PPV, and NPV of a diagnostic test

	Index Test	Reference Standard
Participant 1	+	-
Participant 2	+	+
Participant 3	-	-
Participant 4	+	+

Worksheet #3

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Cohort studies

▶ Follow **exposed** and **non-exposed** individuals to see if a disease develops

▶ Used to evaluate the association between exposure and disease

▶ Prospective cohost studies follow individuals forward in time after exposure

▶ Retrospective cohort studies use historical data to determine exposure

Exposed		Not exposed	
Disease develops	Disease does not develop	Disease develops	Disease does not develop

Case-control studies

▶ Recruit individuals **with disease** and **without disease** and examine past exposures

▶ Used to evaluate the association between exposure and disease

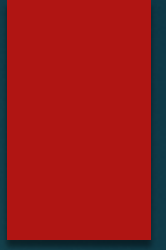▶ Care must be taken in selection of cases and controls

▶ Problems of recall bias

Exposed	Not exposed	Exposed	Not exposed

Do not have the disease

Have the disease

Imaging case series

▶ Collect a series of patient images for a target patient population

▶ Can be used to summarize

  ▶ Unexpected or unusual presentations of a disease

  ▶ Unexpected events when providing care to a patient

  ▶ New findings for emerging diseases.

▶ Low level of evidence

Worksheet #4

Questions