Oncology Biostatistics
What it is and why it is so important

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What it is

- **Biostatistics** or **biometry** is the application of **statistics** to a wide range of topics in **biology**
- **Oncology biostatistics** applies statistics to problems in cancer research to, for example,
  - Determine **how many patients** should be included in a clinical trial
  - Identify if there is **evidence** that new treatments **work better** than old treatments
  - Select the **most effective** dose of a drug
  - **Find genes** that cause cancer
Statistics

- Statistics is the art/science of **summarizing data**
- Better yet...summarizing data so that non-statisticians can understand it
- Scientific investigations usually involve collecting a lot of data.
- But, at the end of your trial, what you really want is a “punch-line:”
  - Did the new treatment work?
  - Are the two groups being compared the same or different?
  - Is the new method more precise than the old method?
- Statistical inference is the answer!
Oncology Biostatisticians: What we do

- We find the “signal” among the “noise”
- We answer the question “how strong is the signal?”
- We quantify uncertainty
At what stages of cancer research should biostatisticians get involved?

- **All of them!**
  - Laboratory
  - Translational
  - Clinical Phase I (dose finding)
  - Clinical Phase II (preliminary efficacy)
  - Clinical Phase III (comparing two treatments)
How can biostatistics help cancer research?

- **Planning:**
  - **Study design selection**
    - Placebo versus active treatment?
    - How many doses should we test?
    - How many patients should be treated at each dose?
    - What *kinds* of patients should be included?
  
  - **Choosing the number of patients**
    - Too small: cannot make a strong conclusion
    - Too big: waste of time, money, resources
How can biostatistics help cancer research?

- **Study conduct**
  - Early stopping considerations
  - Some studies require interim analyses to determine how to proceed ("adaptive" designs)
  - Data safety monitoring
How can biostatistics help cancer research?

- **Analysis: what is the strength of evidence?**
  - Did the new treatment work or not?
  - Which is the best dose level?
  - Is the target inhibited?
  - What is the prognosis of a new patient?
  - Which genes are associated with response to therapy?
Why is biostatistics so important?

- **Efficiency**
  - We can find the “optimal” number of patients (not too big, not too small)
  - We can determine the “optimal” design to answer the research question
  - We can use the most appropriate analysis to answer the research question
    - Sometimes this requires sophisticated methods
    - This is where our job can be most interesting
Why is biostatistics so important?

- **Clarity**
  - If study is conducted and analyzed appropriately, the interpretation of results is CLEAR and CONCISE
  - A good analysis will not leave the audience wondering ‘what if?’
Why is it so important?

- **Ethics**
  - Clinical studies must be done with the utmost concern for patients
  - Unethical to perform experiments that will not yield useful information
  - Unethical to include too many patients in trials
Why is it so important?

- **Funding!**
  - Scarcity of grant money
  - Biostatistics sections often “make or break” grants
  - Why?
    - Lots of interesting science out there
    - Well-developed biostatistics section shows
      - Strong collaboration
      - Well-conceived experiment
      - Clear consideration of possible outcomes
      - What the next step after the current study will be
Any questions?

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