



# Oncology Biostatistics

What it is and why it is so important

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# What it is

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- **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

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- 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

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- 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?

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- 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?

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- **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?

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## ○ **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?

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- **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?

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## ○ **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?

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## ○ **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?

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## ○ **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?

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## ○ **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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