SPEAKING TO A CLINICAL AUDIENCE

SOME EXPERIENCES AND SOME EXAMPLES

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ADVICE FROM FDA CLINICIANS

- At the FDA, I was charged with developing a basic biostatistics course for the medical reviewers
- I was given the following direction: NO GREEK LETTERS!
- They could handle α—they were used to that one—but no others!
- Key message: they don't want to get mired in the mathematical details, they want to understand the concepts
- Good advice for any presentation to a nonstatistical audience

WHAT ARE YOU GOING TO PRESENT TO A CLINICAL AUDIENCE?

◆ Probably not your dissertation work
◆ Probably not your latest results that you published in *JASA*
◆ Presentations to nonstatistical audiences are of three main types
  – Study design and analytical plan to collaborators
  – Presentation of study design and/or study results at a conference or other general meeting
  – Explanations of statistical methods at professional society meetings or local seminars
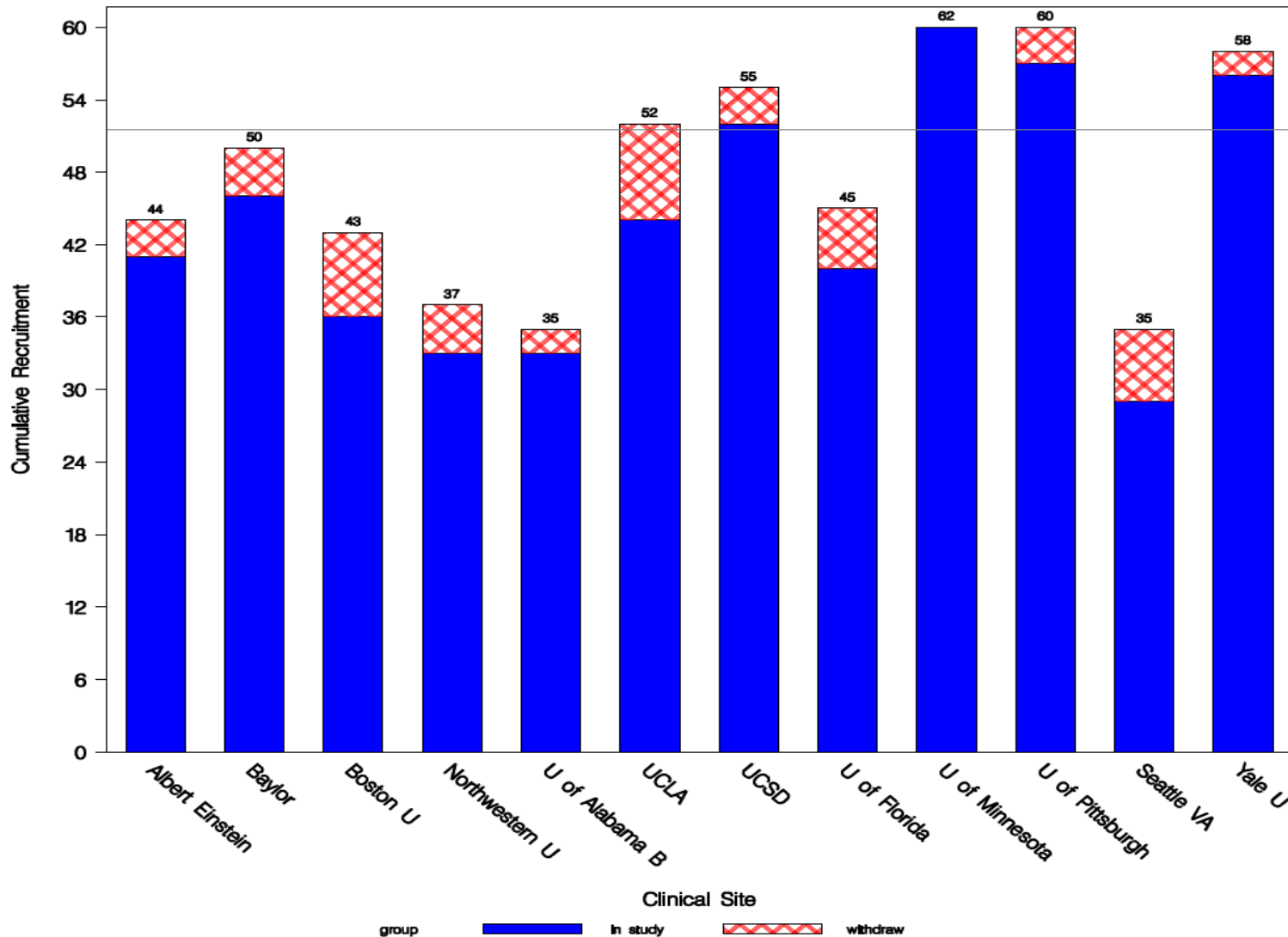
STUDY DESIGNS AND ANALYTICAL PLANS

- ◆ If you are working with a large team you may be asked to present study design and analysis at an investigator meeting
- ◆ Your collaborators have likely been involved in numerous previous studies—they will not freak out by terms like "mixed model," "Cox model," "logistic regression," etc
- ◆ If you are proposing a novel approach, acknowledge that and explain its advantages as conceptually as possible
- ◆ Be prepared for informed suggestions!
  - — Why are you planning on looking just at the difference between the first and last time point when we are collecting that measure at other times? Why not do a longitudinal analysis?
  - — Are you going to use some kind of multiplicity adjustment for the secondary endpoints?

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PRESENTING DATA
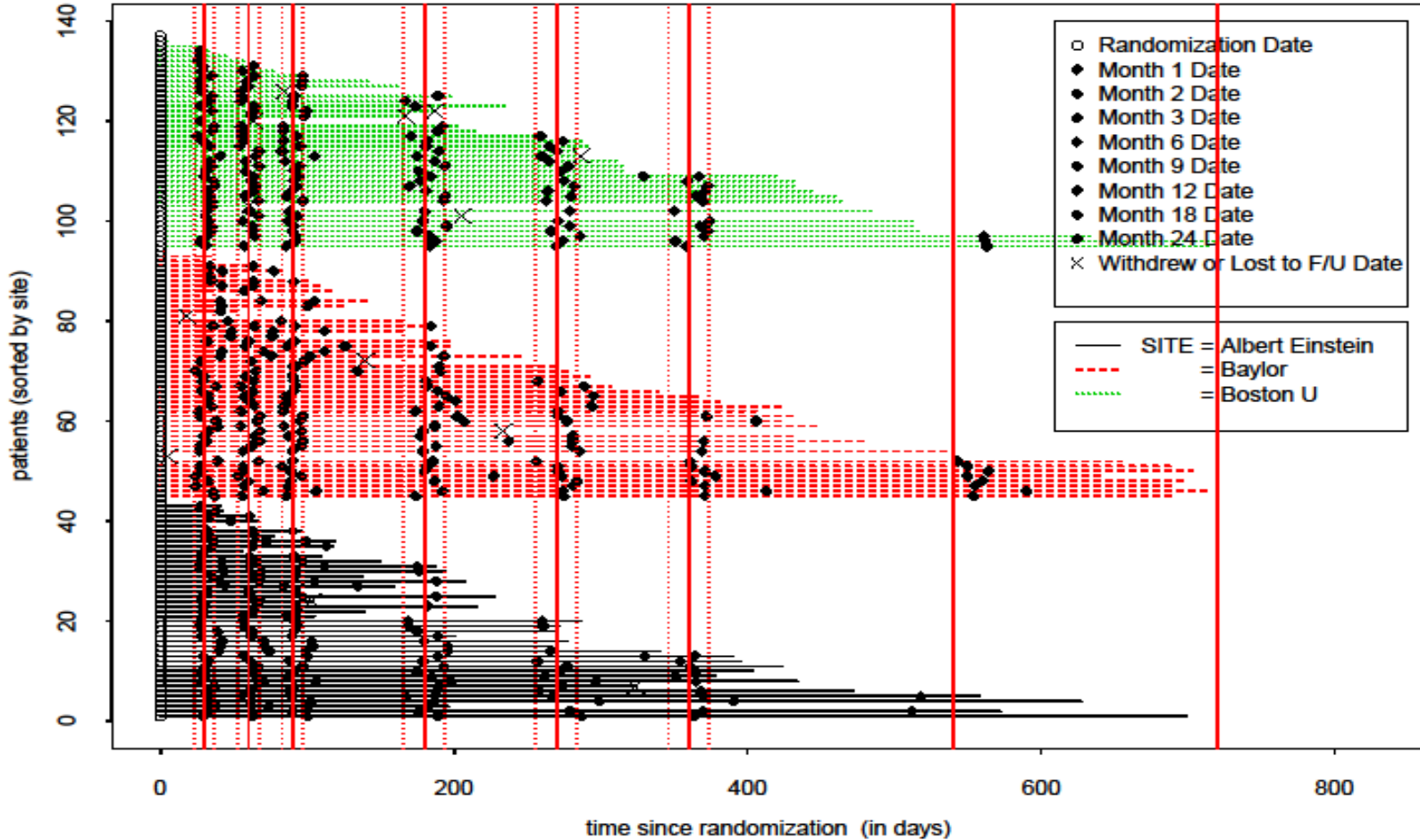
- Plots are usually better received than tables
- Physicians love bar charts!
- Keep material as simple as possible while still presenting the important findings
- If you present a table, make sure the entries can be read!
  - "I know you can't read this, but…"
  - Divide data up into multiple slides if necessary
  - Avoid tables with large numbers of cells
- Please never say, "we found no difference…" when presenting results that don't reach statistical significance
  - Physicians tend to do this—we need to train them not to

RANDOMIZATIONS AND WITHDRAWALS BY CLINICAL SITE

TTRIAL – Intervals Between Visits (a)

Legend:
- ○ Randomization Date
- ◆ Month 1 Date
- ◆ Month 2 Date
- ◆ Month 3 Date
- ◆ Month 6 Date
- ◆ Month 9 Date
- ◆ Month 12 Date
- ◆ Month 18 Date
- ● Month 24 Date
- × Withdrew or Lost to F/U Date

- SITE = Albert Einstein
- = Baylor
- = Boston U

x-axis: time since randomization (in days)
y-axis: patients (sorted by site)

TEACHING ABOUT METHODS

- ◆ Nonstatisticians will often tell you that the worst class they ever took was statistics
- ◆ Practicing scientists don't need (or want) to know statistical formulae or analytical details
- ◆ They need to know conceptually why a certain approach may be optimal in a given situation
- ◆ They need to understand pitfalls of common approaches, and ways to avoid them
  - — Why ignoring missing values, or using methods like "last observation carried forward" can yield unreliable results
  - — Why you can't simply compare number of events observed in different treatment groups when multiple events might be observed in a single participant

EXAMPLE: SAMPLE SIZE

- How to determine a sample size is often of interest
- Showing a formula is not needed—they can easily find a program to do the calculation

HERE IS WHAT THEY DON'T NEED (OR WANT) TO SEE

$$n = \frac{\left(c_1 \sqrt{2\,\overline{p}\,\overline{q}} + c_2 \sqrt{p_1 q_1 + p_2 q_2} \right)^2}{\left(p_2 - p_1 \right)^2}$$

c_1: 1.96, 1.64 c_2: 0.84, 1.28

WHAT THEY NEED TO KNOW ABOUT SAMPLE SIZE

- ◆ Sample size depends on the following:
  - — The size of the effect you want to document
  - — The risks of a false positive and a false negative you are willing to accept
  - — The variability of your outcome variable
- ◆ The size of the effect you want to document is the major driver of the required sample size
- ◆ It is often effective to present a table of sample sizes that would be needed to achieve high power for a range of plausible treatment effects
  - — This can demonstrate how changing the effect size and variability of outcome affects the sample size

KNOW YOUR AUDIENCE

- Some clinicians know a lot of statistics
- If you're working with a group like that, they may be fine with Greek letters, formulae, and more technical details
- If you are speaking to a large group to provide an overview of statistical methods, keep it as non-technical as possible
  - Focus on the concepts
  - Use simple graphics when possible
  - Be prepared to expand on technical issues when asked