Disclosures:

None relevant to this talk
Objectives

• Importance of the Specific Aims Page
• Hardest Parts
• Suggest a Structure
• Discuss Examples
Importance of the Specific Aims Page

- Many will read only this
Importance of the Specific Aims Page

- Many will read only this
- Most will read this first

You never get a second chance to make a first impression.

*Will Rogers*
Importance of the Specific Aims Page

• Many will read only this
• Most will read this first
• Returned to often
Hardest Parts

- Narrative
- Focus
narrative
/'nerədiiv/
noun
1. a spoken or written account of connected events; a story.
Elevator Pitch sentence structure:

FOR (target customer), WHO HAS (customer need), (product name) IS A (market category) THAT (one key benefit).

UNLIKE (competition), THE PRODUCT (unique differentiator).
Background

Challenge

Overarching goal

Specific Aims

Impact (significance innovation)
Focus

What’s missing?
What’s missing?
The big picture

Impact
And the back story

Detail
Depth of field
Suggest a Structure

• Disclaimers
Summary

- Specific aims page is critical
- Specific aims page is challenging
- Tell a story
- Wide focus
- Consider a template

https://goo.gl/WOYSIr
Specific Aims Page

Jordan Elm, PhD
Statistical Reviewer

- Approach/Significance
- Reads to Specific Aims Page
  - Understand Significance (brief paragraph)
  - Design (in a nutshell)
  - Consistency with Analyses
First things first …

• What’s the research question?
• Scientific, not statistical hypotheses
• Okay to give overarching hypothesis, but clearly state what you are testing/estimating in proposal
• State aims in the form of testable hypotheses.
  – What’s the population?
  – What are you comparing?
  – Response variable? At what timepoint?
What are you measuring?

• Specific Aims need to state the outcome/endpoint (what you are measuring, be specific).

• Avoid Jargon/Define constructs/Be succinct
  – “Slow the decline” of Parkinson’s / “Neuroprotective” (as measured by UPDRS change at 1 year)
  – “Functional Independence” (mRS ≤ 2)
Aims Consistent with Analysis

- Phase 3 easiest/Phase 2 hardest
  - E.g. Futility design
- Aim: To determine whether treatment A is as good as treatment B. (Non-inferiority)
- “Demonstrate safety” (vague/not testable)
- Disconnect implies poor collaboration with statistician (concern)
Exploratory Aims

• Lots of opinions-Pros/Cons
• All aims should have a power analysis and analysis plan
• By nature, this will have low rigor/reproducibility, power, etc ….