

NINDS Clinical Trials Methodology Course

Writing Specific Aims

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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

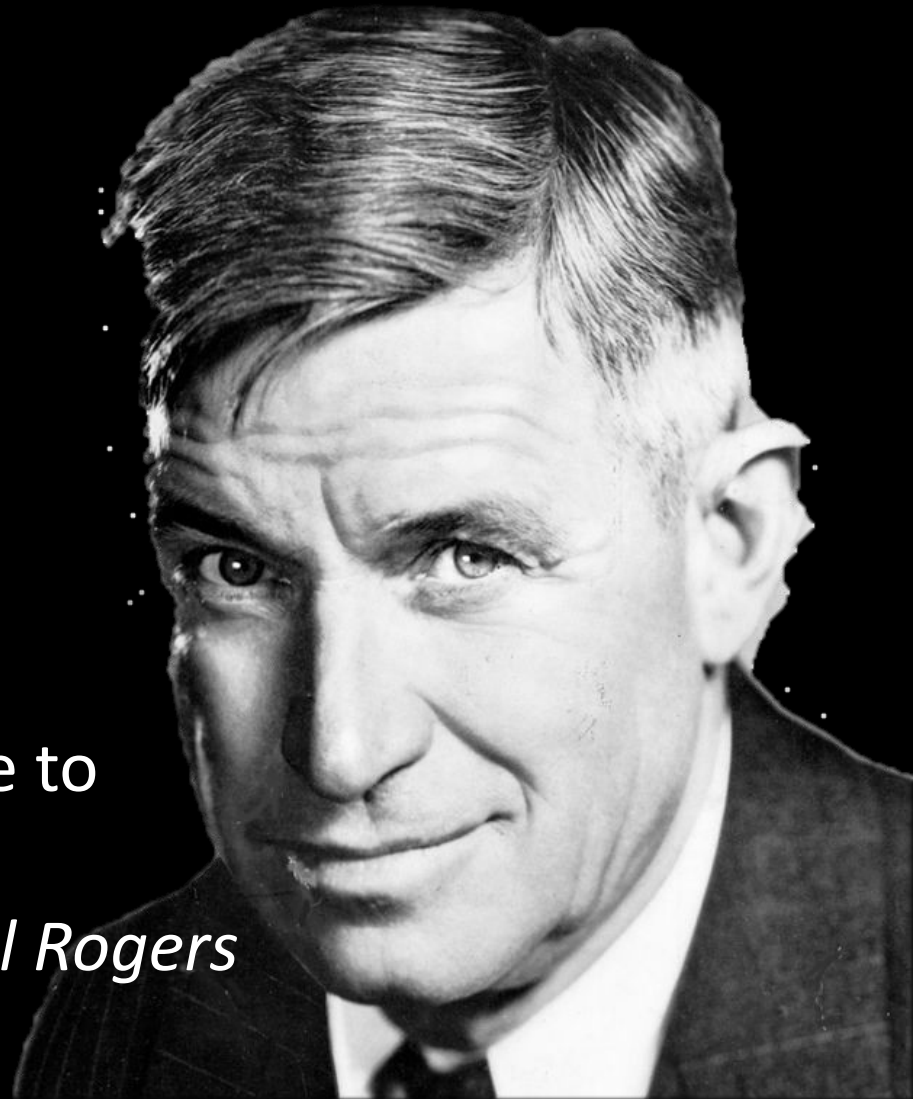


Importance of the Specific Aims Page

- 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

nar·ra·tive

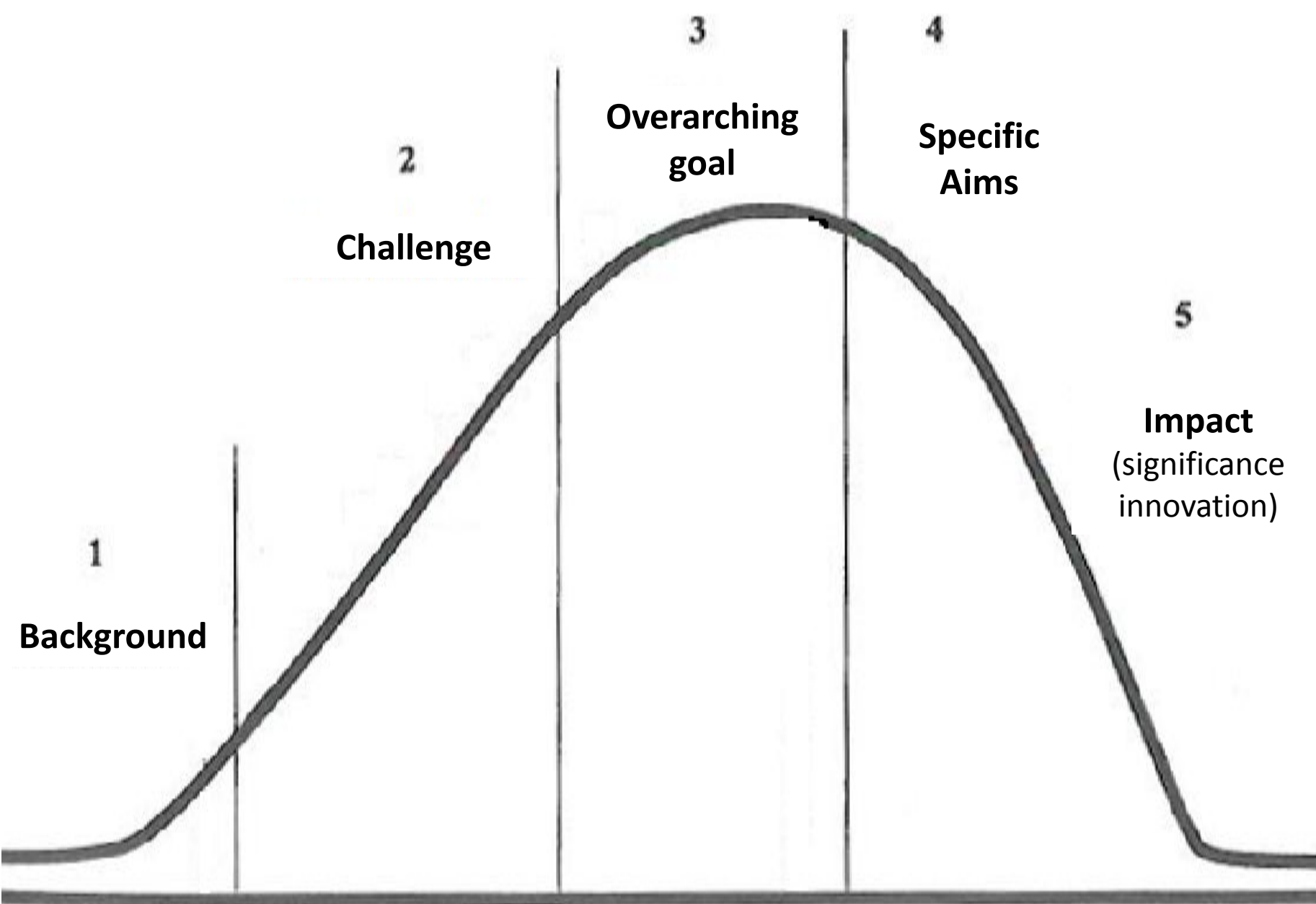
/'nerədiv/

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).



1

Background

2

Challenge

3

Overarching
goal

4

Specific
Aims

5

Impact
(significance
innovation)

What's missing?

Focus





What's missing?

The big
picture

Impact



And the back story

Detail



Depth of field



Suggest a Structure

- Disclaimers

Title

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Short Informative Title

Introduction and Background
Educate
Identify Knowledge Gaps and Needs

Specific Problem to be Solved
Overall Project Goal - Solution

Bulleted Specific Aims
Not too many
Conceptual

Briefly review
Significance + Innovation =
Impact

Summary

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

<https://goo.gl/WOYSlr>

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” ($mRS \leq 2$)

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