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## LAYOUT RECOMMENDATIONS:

Rather than several smaller images, we recommend you use **one image at large scale** for more memorable impact (see examples provided in the template).

**Keep text to a minimum.** Audience members are more likely to remember strong imagery supported by minimal text and your oral explanation.

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# Study Design

A statisticians point of view with focus  
on power and sample size

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DATE: March 3, 2022

PRESENTED BY: Emile Latour, MS, Associate Biostatistician, OHSU Knight Cancer Institute

# Acknowledgement

- Meike Niederhausen, PhD, Biostatistics & Design Program (BDP)
- Biostatistics, Epidemiology, & Research Design (BERD) Seminar, OCTRI Research Forum
  - [Slides](http://bit.ly/BERD-PSS-101) <http://bit.ly/BERD-PSS-101>
  - [Recording](https://echo360.org/media/c14ae529-27fe-4208-ae27-95c7c1adc928/public) <https://echo360.org/media/c14ae529-27fe-4208-ae27-95c7c1adc928/public>
- Upcoming Thursday March 31, 2022 over Zoom.
  - Register through Compass: "Power and Sample Size 101"

# About me

- Pension actuary for 16 years
- MS in Biostatistics from OHSU in 2017
- Associate Biostatistician with OHSU Knight Cancer Institute since 2016
- Long term support for OHSU Dermatology Department since 2018



# Goals

- Learn best practices in study design to aid in statistical analysis
- Understand statistical terms involved in study design:
  - Hypothesis tests (null and alternative),
  - Type I error rate,
  - Type II error rate and Power,
  - Effect size,
  - Sample size

# Goals

- Perform simple power and sample size calculations
  - Paired t-test (one sample)
  - 2 sample t-test
  - Two proportions test





It all starts with a  
research question



# The research question drives...

- Hypothesis
- The study design
  - Cohort, Case-control, cross-sectional, clinical trial, retrospective, prospective, longitudinal, etc.
- Data collection
- Statistical analysis

# Primary objectives / aims

- The stated principal purpose(s) of the study
- Expressed as a statement of purpose
  - General: efficacy, effectiveness, safety
  - Specific: dose-response, superiority, disease severity, effect on disease incidence, etc.
- “This study seeks to ...”
  - To answer, To investigate, To determine, To compare, To assess....
- Coupled with the research hypothesis

# Example from literature

- **Study:** Strober B, Mallya UG, Yang M, et al. Treatment Outcomes Associated With Dupilumab Use in Patients With Atopic Dermatitis: 1-Year Results From the RELIEVE-AD Study. *JAMA Dermatol.* 2022;158(2):142–150. doi:10.1001/jamadermatol.2021.4778
- **Research question:** What are the benefits of treatment with dupilumab in the clinical practice setting from the perspective of patients with moderate-to-severe atopic dermatitis?
- **Research hypothesis:** Disease control and quality of life in patients with atopic dermatitis (AD) will improve after the initiation of dupilumab treatment.
- **Objective:** To evaluate self-reported disease control and quality of life after initiating dupilumab treatment in patients with atopic dermatitis (AD) in the the clinical setting.

# Secondary objectives/aims

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# Power analysis for a new study



# Hypothesis and outcome

- Primary objective
  - To assess the change in quality of life in patients with atopic dermatitis (AD) one year after the initiation of dupilumab treatment
- Hypothesis:
  - Quality of life in patients with AD will improve one year after the initiation of dupilumab treatment
- Primary endpoint:
  - Change in Dermatology Life Quality Index (DLQI) from baseline to 12 months

# Study design

- Single group
- DLQI at baseline and 12-months (continuous, paired data)
- Statistical analysis plan:
  - Paired t-test

# What sample size is needed?

- To solve for sample size (n), we will need:
  - Alpha = 0.05
  - Power = 80.0%
  - Effect size = ??????

# Estimate for effect size

- Pilot or preliminary data
- Published literature
- Clinically or biologically meaningful difference

# Published literature

**Table 3. Patient-Reported Health-Related Quality of Life and Work and Activity Impairment**

Outcome	Mean (SD) [PMM range] <sup>a</sup>						
	Baseline (n = 699)	Month 1 (n = 632) <sup>b</sup>	Month 2 (n = 626) <sup>b</sup>	Month 3 (n = 596) <sup>b</sup>	Month 6 (n = 543) <sup>b</sup>	Month 9 (n = 477) <sup>b</sup>	Month 12 (n = 483) <sup>b</sup>
DLQI <sup>c</sup>							
Total score	14.4 (7.3)	5.9 (5.8) [5.6-6.7]	5.1 (5.5) [4.9-6.1]	4.8 (5.5) [4.4-6.5]	4.1 (5.0) [3.4-6.7]	3.8 (5.2) [2.9-7.8]	3.5 (4.9) [2.7-7.8]



[Link to paper](#) Strober, et al. Treatment Outcomes Associated With Dupilumab Use in Patients With Atopic Dermatitis: 1-Year Results From the RELIEVE-AD Study. *JAMA Dermatol.* 2022;158(2):142–150



- Minimally Important difference
- <https://www.karger.com/Article/Fulltext/365390>

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# Past talks

- Power and Sample Size 101, Mieke Niederhausen, PhD (February 2021)
  - [Slides](http://bit.ly/BERD-PSS-101) <http://bit.ly/BERD-PSS-101>
  - [Recording](https://echo360.org/media/c14ae529-27fe-4208-ae27-95c7c1adc928/public) <https://echo360.org/media/c14ae529-27fe-4208-ae27-95c7c1adc928/public>
- Planning a Study with Power and Sample Size Considerations in Mind, David Yanez, PhD (May 2019)
  - [Slides](https://www.ohsu.edu/sites/default/files/2019-12/PowerAndSampleSize_29MAY2019.pdf) [https://www.ohsu.edu/sites/default/files/2019-12/PowerAndSampleSize\\_29MAY2019.pdf](https://www.ohsu.edu/sites/default/files/2019-12/PowerAndSampleSize_29MAY2019.pdf)
  - [Recording](https://echo360.org/media/ee2a5565-1168-4941-82ea-d9eda4223281/public) <https://echo360.org/media/ee2a5565-1168-4941-82ea-d9eda4223281/public>
- Power and Sample Size for Clinical Trials: An Introduction, Yiyi Chen, PhD (February 2021)
  - [Slides](https://drive.google.com/file/d/1dK4ktxQa81SNjRM4z4JC8JXH8DTgcr8Z/view) <https://drive.google.com/file/d/1dK4ktxQa81SNjRM4z4JC8JXH8DTgcr8Z/view>
  - [Recording](https://echo360.org/media/c2197215-3c5f-4c9b-b3eb-3877566ad3da/public) <https://echo360.org/media/c2197215-3c5f-4c9b-b3eb-3877566ad3da/public>

# Resources

- PASS
  - [Training videos](https://www.ncss.com/videos/pass/training/) (short and good) <https://www.ncss.com/videos/pass/training/>
  - [Documentation](https://www.ncss.com/software/pass/pass-documentation/) <https://www.ncss.com/software/pass/pass-documentation/>
- [Biostats4You](https://biostats4you.umn.edu/resources), University of Minnesota
  - <https://biostats4you.umn.edu/resources>
  - “website was developed to serve medical and public health researchers and professionals who wish to learn more about biostatistics”
- Links I think are pretty good
  - [Sample size, power and effect size revisited: simplified and practical approaches in pre-clinical, clinical and laboratory studies](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7745163/#:~:text=When%20the%20sample%20size%20is,the%20power%20of%20the%20study) (Serdar et al.) <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7745163/#:~:text=When%20the%20sample%20size%20is,the%20power%20of%20the%20study>
  - [Understanding Statistical Power and Significance Testing](https://rpsychologist.com/d3/nhst/), an interactive visualization <https://rpsychologist.com/d3/nhst/>
  - [Power and Sample Size Applet](https://wbakerrobinson.shinyapps.io/Paired_T_test_shiny/) [https://wbakerrobinson.shinyapps.io/Paired\\_T\\_test\\_shiny/](https://wbakerrobinson.shinyapps.io/Paired_T_test_shiny/)
  - [The Relationship between Significance, Power, Sample Size & Effect Size](https://towardsdatascience.com/the-relationship-between-significance-power-sample-size-effect-size-899fcf95a76d) (K. Mysiak) <https://towardsdatascience.com/the-relationship-between-significance-power-sample-size-effect-size-899fcf95a76d>

# Software (free)

- Sample size calculators from UCSF
  - [Link](https://sample-size.net/calculator-finder/) <https://sample-size.net/calculator-finder/>
  - User friendly! Web based.
- G\*Power software
  - [Sample size determination and power analysis using the G\\*Power software](https://pubmed.ncbi.nlm.nih.gov/34325496/) (H. Kang)
  - <https://pubmed.ncbi.nlm.nih.gov/34325496/>
  - [Download](https://www.psychologie.hhu.de/arbeitsgruppen/allgemeine-psychologie-und-arbeitspsychologie/gpower.html) <https://www.psychologie.hhu.de/arbeitsgruppen/allgemeine-psychologie-und-arbeitspsychologie/gpower.html>
  - More involved. Requires download.
- CRAB (Cancer Research and Biostatistics) Statistical Tools
  - [Link](https://stattools.crab.org/) <https://stattools.crab.org/>
  - Web based.



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—Quotation Credit

“Lorem ipsum dolor sit  
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— A Very Wise Woman

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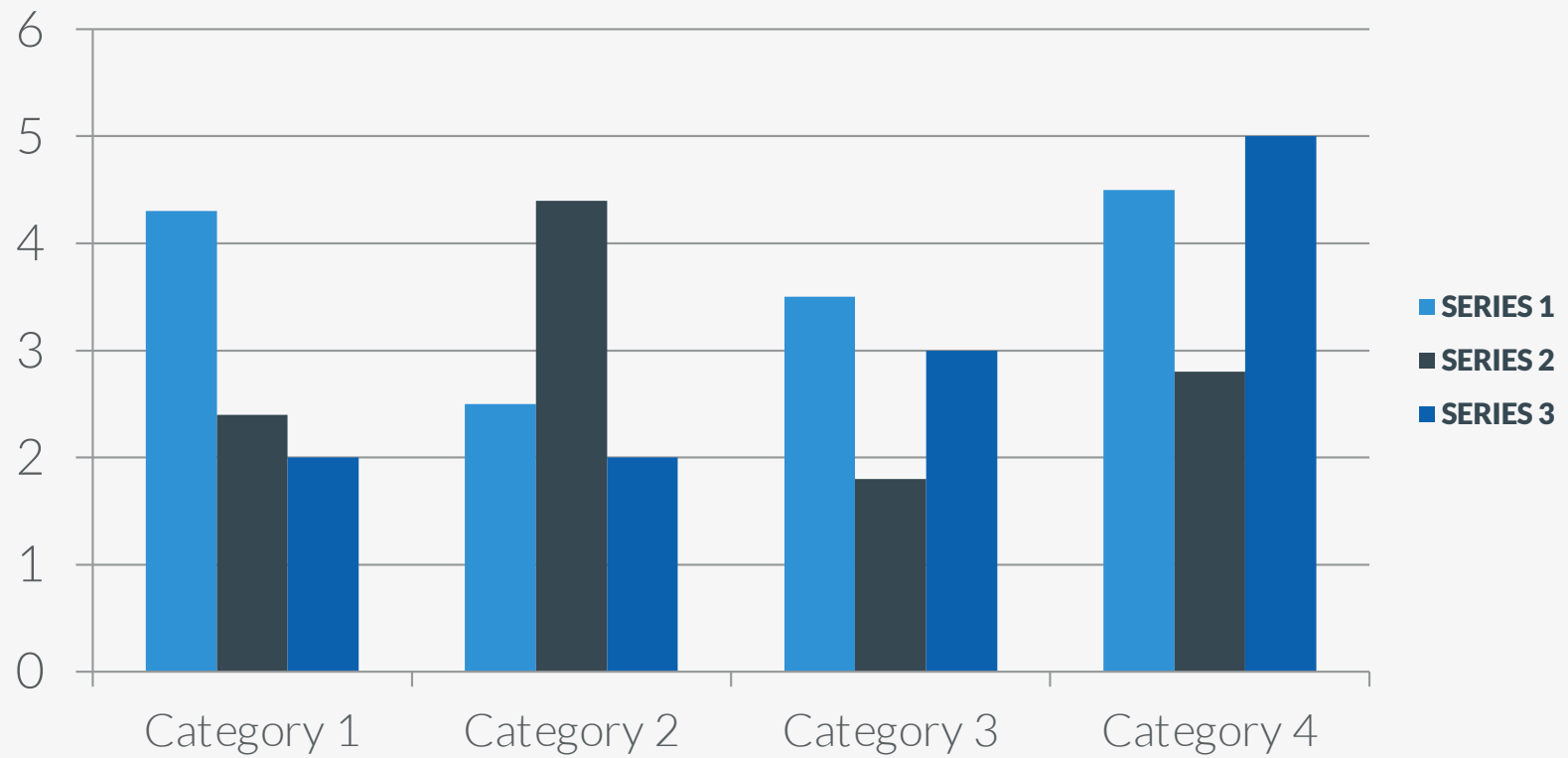
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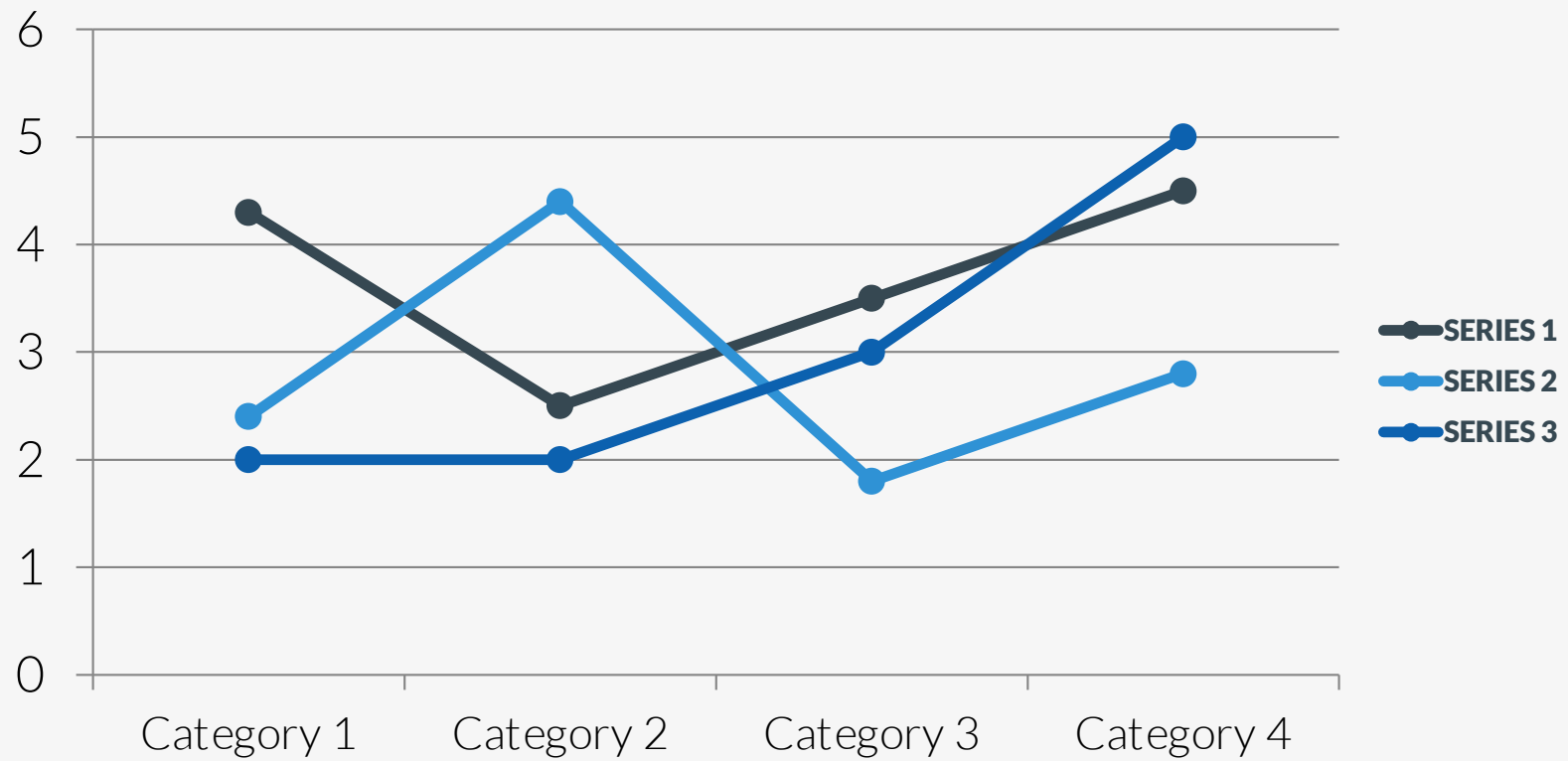
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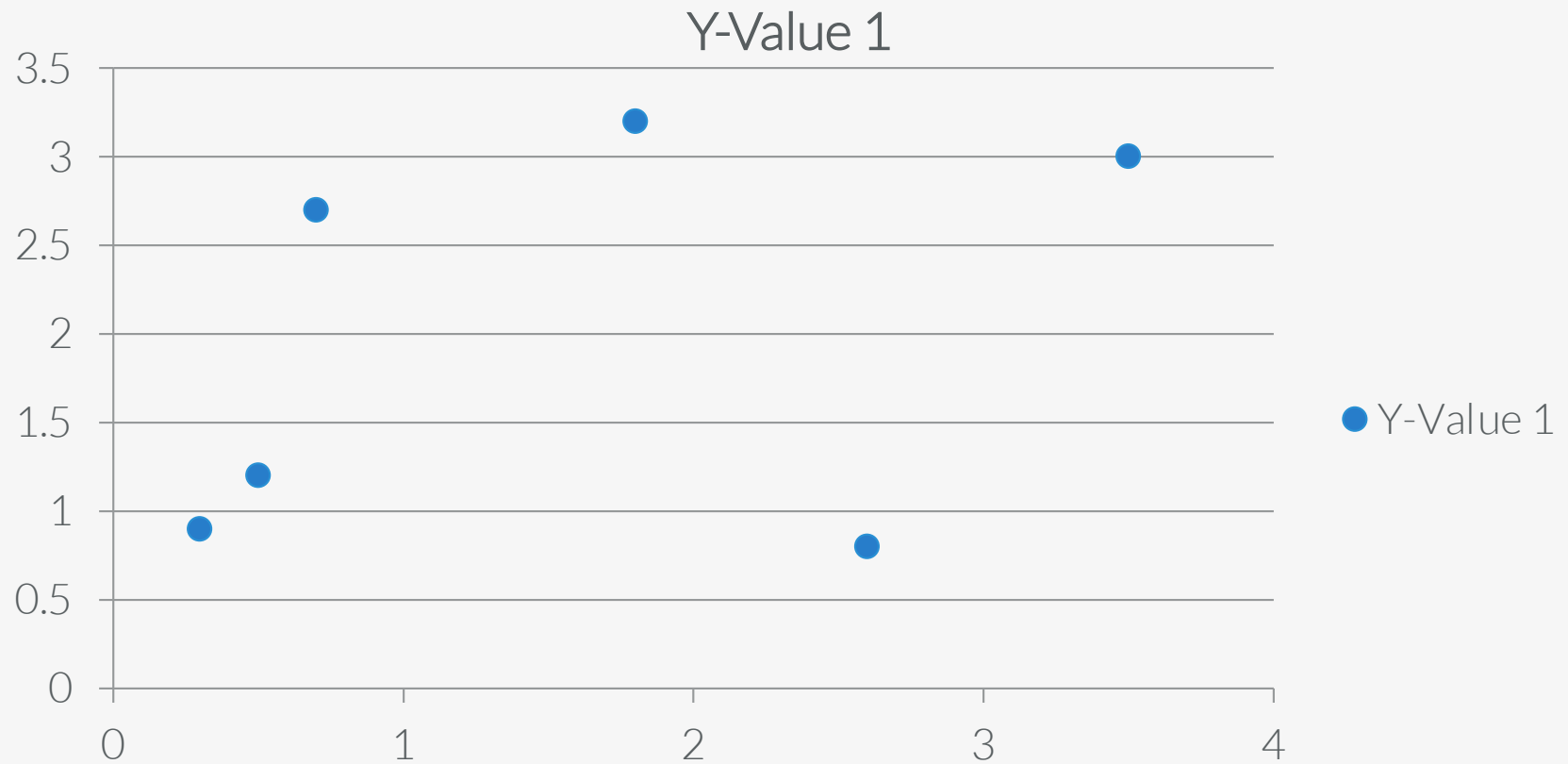
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perfecto.”

—Quotation Credit



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Thank You



Thank You