Biostatistics: Quick Overview

Power:
The ability of a **trial** to detect a statistically significant difference between groups when a difference truly exists.

P-value:
The probability that the observed difference between two groups for a **specific outcome** is due to chance only.

Level of significance (aka alpha):
The probability the investigator is willing to take that the results occurred due to chance alone.

Typically, the level of significance is set at 0.05 (5%) which would indicate that the investigators are willing to accept a 5% chance for a false positive result. If the p-value is less than alpha then the results are considered statistically significant.

Confidence interval:
A range of values in which the true value likely resides.

A 95% confidence interval means that if a trial is **repeated** several times from the same sample population then it would be expected that 95% of the confidence intervals would contain the true value for said measure (and 5% would not). For hazard ratios, if the confidence interval for a measurement includes the value of 1.00 then the difference cannot be considered statistically significant due to a hazards ratio of 1.00 indicating no difference between treatment groups.

Composite Endpoints
A combination of outcomes reported for a single measure of effect (e.g., cardiovascular death, myocardial infarction or stroke).

Each component should ideally occur at similar rates and have similar clinical significance to avoid distortion of the overall composite measure. A composite endpoint of cardiovascular death or minor bleeding would not be appropriate due to death being much more significant than minor bleeding.

Relative risk reduction (RRR):
The change in event rate of the active group relative to the control group.

RRR = 1 - (active/control)

Relative risk reduction is more commonly used when reporting treatment effect. However, it is subject to misinterpretation and overestimation of treatment effect. For example, if the event rate in group A was 10% and group B was 20% this would represent a 50% relative risk reduction but only a true treatment difference of 10%.

Absolute risk reduction (ARR):
The absolute change in event rates between two groups.

ARR = control event rate - active event rate

ARR is less commonly reported when reporting treatment effect.

Number Needed to Treat (NNT):
An estimate of how many patients would need to receive “Treatment A” to prevent one outcome compared to “Treatment B”.

NNT = 1/ARR
(must be reported as a whole integer, rounded up)

Number Needed to Harm (NNH):
An estimate of how many patients would need to receive “Treatment A” for one adverse outcome to occur compared to “Treatment B”.

NNH = 1/ARR
(must be reported as a whole integer, rounded down)

It is important to consider the time frame of the trial when interpreting NNT and NNH.

Note: It is only appropriate to report NNT/NNH for statistically significant differences.

Interpreting NNT and NNH:
While NNT and NNH are simple to calculate and appear straightforward to use, it is very important to remember that these values are **estimates** based on trial results used to help illustrate the magnitude of treatment effect in terms of patients instead of percentages.

A NNT value lower than a NNH value indicates that the benefit/risk ratio is favorable, however these calculations are based on average trial results from populations that may differ significantly from a specific patient.

Additionally, the clinical significance of each outcome must be considered (e.g., cardiovascular death vs hypotension).

Note: This is not intended to be a comprehensive review of biostatistics. Refer to the following for more thorough and comprehensive information on the subject.

• Malone PM, Witt BA, Malone MJ, Peterson DM. eds. Drug Information: A Guide for Pharmacists, Seventh edition. McGraw-Hill Education; 2022.
• Bryant PJ, Pace HA. The Pharmacist’s Guide to Evidence-Based Medicine for Clinical Decision Making. American Society of Health-System Pharmacists; 2008.