

Healthcare Information & Decision Equation: **Information** → **Decision** → **Action** → **Outcome**
 Is it true → Is it useful → Is it usable?

Meaningful Clinical Benefit is a combination of—

- **Clinically significant areas** (morbidity, mortality, symptom relief, emotional/physical functioning and health-related quality of life) +
- **Effect size** which means the difference in the size of outcomes between groups reported as measures of outcomes.

Measures of Outcomes measure the event outcome differences in the groups and should always be associated with the study time period. Most of these are measures of probability that an event will occur. **Synonyms for expressing effect size:** estimates of effect, point estimates.

- **Risk With and Without Treatment** is the number or percent of outcomes in each group (if number = 2 x 2 table data)
- **Absolute Risk Reduction (ARR)** is the percentage difference in outcomes between groups.
- **Number-needed-to-Treat (NNT)** is the reciprocal of the ARR and expresses the number of people needed to treat for 1 person to benefit over the comparator.
- **Relative Risk Reduction (RRR)** is the relative percentage difference in size between outcomes.
- **Relative Risk (RR)**, also known as Risk Ratio or Relative Risk Ratio, is the probability of the risk in the intervention group to the probability in the control group. Example: Probability of drawing an ace = 4/52.
- **Odds ratios (OR)** express the odds of an event occurring compared to not occurring and, therefore, cannot be as specific as probability measures. Example: Odds of drawing an ace = 4/48. Odds and RR are usually similar if the event rate is low, but compare both the odds ratio reduction and the RRR to check and see if the difference is clinically meaningful.
- Many of these measures can be used to express **harms** (e.g., Absolute Risk Increase or ARI, Number-needed-to-harm or NNH, etc.), prevention, screening, etc.

P-values: Assuming there truly is no difference between the groups studied, the P-value is a calculated probability of observing a difference as big as or bigger than the one you observed in a study based on compatibility with an assumed standard distribution. Problems include the P-value cannot tell you the chance the results are true or even how likely they are to be due to chance, you do not know if the null hypothesis is true or not, and you do not know if the sample is truly random and/or representative of the population.

- **Non-significant results** in a valid study arise either because it is true there is no difference between groups, it is a chance effect or there were insufficient people studied for the outcome to happen (e.g., the study was not sufficiently “powered”).
- **Trials stopped early** present a high risk of outcomes due to chance even if stopping rules are applied.
- **Multiple outcomes** and **multiple analyses points** increase the likelihood of chance effects as high as x the number.
- **A priori** should also be research questions, populations for analysis and outcomes for measurement to reduce risk of chance effects

Confidence Intervals (CIs) represent a range of statistically plausible results.

Example: ARI 1.3%, 95% CI (-0.21% to 2.8%), p=0.11.

Confidence intervals have some practical limitations similar to P-values. Although the CIs can project a range of results consistent with the study results, they cannot tell you the truth of the outcomes. CIs cannot replace the need to critically appraise the study. **Clinical significance** can be determined by whether values are wholly within or outside values judged clinically meaningful.

Applying CIs to Decide Meaningful Clinical Benefit Size: Hypothetical Endpoint—Reduction in Mortality

