

EBM Numbers: Statistics in Therapy

RATE NUMBERS

Event Rate

The number who experience an event. e.g., death as a proportion of the number of people in the population or ovulation in a PCOS trial.

Control Event Rate (CER)

The rate at which events occur in a control group. It may be represented by a percentage (say 10%) or as a proportion (0.1).

Experimental Event Rate (EER)

The rate at which events occur in the experimental group.

Incidence

The percent of the population that will develop disease during an interval (the incidence of diabetes is 0.2% per year, referring only to new cases).

Prevalence

The probability of disease in the entire population at any point in time (i.e. 2% of the U.S. population has diabetes).

STATS

P-value

A measure of probability that a difference between groups during an experiment happened by chance. For example, a p-value of .01 means there is a 1 in 100 chance the result occurred by chance. The lower the p-value, the more likely it is that the difference between groups was caused by treatment assuming that there are no confounders or biases in the study.

Confidence Intervals

The results can tell you both the magnitude of effect and the precision. A confidence interval combines the statistical stuff and the clinical stuff to provide you with an idea of how precise the results are. Data is usually presented as a 95% confidence interval, meaning that if the study is repeated multiple times, 95% of the studies will have the true result within that range. When you look at confidence intervals, see how wide they are. A narrow or tight confidence interval represents a precise estimate. These are usually found in studies with a large number of participants.

Intention to Treat

A method by which participants are analyzed in the group to which they were initially randomized, whether or not they completed the intervention of that group. A patient randomized to the treatment group should be analyzed based on how we intended to treat them, even if they never took the treatment. This may seem to defy all logic, however, imagine the bias created if you let researchers pick and choose who they got to analyze at the end of the trial. An ITT analysis is the most conservative way to look at the results. The alternative is often called a "Per Protocol Analysis" where you analyse only those people who actually did what they were initially randomized to do.

RELATIVE TYPE NUMBERS

Relative risk/risk ratio (RR), Relative risk reduction (RRR)

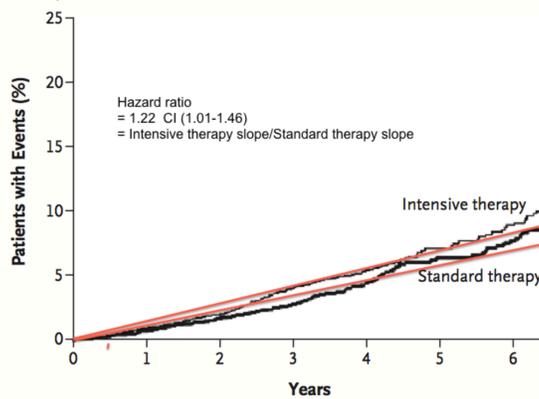
There can also be relative increases as well

A ratio of two probabilities (%) at one point in time - treatment/control. For example if the event rates in two groups were 8% (treatment) and 10% (control) then the RR = 8/10 or 0.8. If there was no difference between the groups the RR would be 1. An RR of 0.8 can also be expressed as a relative risk reduction (RRR). In this case 0.8 is 20% less than 1 (no difference) so the RRR would be 20%.

Hazards ratio (HR)

Similar to RR except it is a ratio of two hazard rates (slopes) over a time period in contrast to a relative risk, which is a ratio of two probabilities at one specific point in time. The HR would be the ratio of the 2 slopes (redlines) in the figure below. An HR and RR are often very similar.

B Death from Any Cause



No. at Risk

| | | | | | | | |
|-------------------|------|------|------|------|------|-----|-----|
| Intensive therapy | 5128 | 4972 | 4803 | 3250 | 1748 | 523 | 506 |
| Standard therapy | 5123 | 4971 | 4700 | 3180 | 1642 | 499 | 480 |

Odds ratio (OR)

A ratio of two odds (25:1) and is typically used in case-control studies when the incidence is not known. OR is a reasonable estimate of the RR if a disease is “rare” <~15% but treating an OR as if it were an accurate estimate of the RR will typically overestimate both the likely benefits and harms of treatment.

ABSOLUTE TYPE NUMBERS

Absolute Risk Difference (ARD) or Absolute Risk Reduction (ARR)

There can also be absolute increases as well

This is the difference between two event rates; it represents the difference between event rates AND depends on the baseline risk. NB: The lower the event rate in the control group; the larger the difference between the RRR and ARD.

Number Needed to Treat (NNT) or Numbers Needed to Harm (NNH)

The number of patients who would have to receive the treatment in order for one of them to benefit or be harmed. *Easily* calculated from the risk difference by dividing

the absolute risk difference (%) into 100. The NNT/NNH is a concise, clinically relevant overview of the effect of the intervention.

FORMULAS – best if you can just do it in your head instead of trying to remember these formulas but start with this

| | Outcome Event (e.g. MI) | | |
|--------------------|-------------------------|-----------|--------------|
| | YES | NO | TOTAL |
| Experimental group | a | b | a+b |
| Control group | c | d | c+d |

Control event rate (CER) = $c/c+d$

Experimental event rate (EER) = $a/a+b$

Relative Risk (RR) = $EER/CER = (a/a+b) / (c/c+d)$

Relative Risk Reduction (RRR) = $EER - CER/CER$

Risk Difference (RD) = Absolute Risk Reduction (ARR) = $EER - CER$

Number Needed to Treat (NNT) = $1/RD = 1/ARR$