Treatment Effect

Concepts and Glossary

Randomization (or random allocation)

Method analogous to tossing a coin to assign patients to treatment groups (the experimental treatment is assigned if the coin lands "heads" and a conventional, "control" or "placebo" treatment is given if the coin lands "tails").

Randomized controlled clinical trial (RCT)

A group of patients is randomized into an experimental group and a control group. These groups are followed up for the variables/outcomes of interest.

Intention-to-treat analysis

A method of analysis for randomized trials in which all patients randomly assigned to one of the treatments are analyzed together, regardless of whether or not they completed or received that treatment.

Event rate

- The proportion of patients in a group in whom the event is observed. Thus, if out of 100 patients, the event is observed in 27, the event rate is 0.27.

- Control event rate (CER) and experimental event rate (EER) are used to refer to this in control and experimental groups of patients, respectively

Treatment effects(一)

The evidence-based journals (*Evidence Based Medicine* and *ACP Journal Club)* have achieved consensus on some terms they use to describe both the good and bad effects of therapy.

Treatment effects(二)

- *When the experimental treatment reduces the probability of a bad outcome*

- *When the experimental treatment increases the probability of a good outcome*

- *When the experimental treatment increase the probability of a bad outcome*

Insulin therapy example

- *worsening diabetic retinopathy*
  - *several years of intensive insulin therapy reduced the proportion of patients with worsening retinopathy to 13% from 38%*

- *satisfactory hemoglobin A1c levels*
  - *several years of intensive insulin therapy raised the proportion of patients with satisfactory hemoglobin A1c levels to 60% from about 30%*

- *Increased episodes of hypoglycemia*
  - *several years of intensive insulin therapy increased the proportion of patients with at least one episode of symptomatic hypoglycemia to 57% from 23%*

worsening diabetic retinopathy

- **RRR (relative risk reduction).** The proportional reduction in rates of bad outcomes between experimental and control participants in a trial, calculated as |EER – CER|/CER, In the case of worsening diabetic retinopathy, |EER – CER|/CER = |13% – 38%|/38% = 66%.

- **ARR (absolute risk reduction).** The absolute arithmetic difference in rates of bad outcomes between experimental and control participants in a trial, calculated as |EER – CER|, In this case, |EER – CER| =|13% – 38%| = 25%.

- **NNT (number needed to treat).** The number of patients who need to be treated to achieve one additional favorable outcome, calculated as 1/ARR. In this case, 1/ARR = 1/25% = 4.

satisfactory hemoglobin A1c levels

- **RBI (relative benefit increase).** The proportional increase in rates of good outcomes between experimental and control patients in a trial, calculated as $|EER - CER|/CER$. In the case of satisfactory hemoglobin A1c levels, $|EER - CER|/CER = |60\% - 30\%|/30\% = 100\%$.

- **ABI (absolute benefit increase).** The absolute arithmetic difference in rates of good outcomes between experimental and control patients in a trial, calculated as $|EER - CER|$. In the case of satisfactory hemoglobin A1c levels, $|EER - CER| = |60\% - 30\%| = 30\%$.

- **NNT (number needed to treat).** The number of patients who need to be treated to achieve one additional good outcome, calculated as $1/ARR$. In this case, $1/ARR = 1/30\% = 3$

increase episodes of hypoglycemia

- **RRI (relative risk increase).** The proportional increase in rates of bad outcomes between experimental and control patients in a trial, calculated as |EER − CER|/CER. In the case of hypoglycemic episodes, |EER − CER|/CER = |57% − 23%|/23% = 148%. (RRI is also used in assessing the impact of "risk factors" for disease.)
- **ARI (absolute risk increase).** The absolute arithmetic difference in rates of bad outcomes between
- experimental and control patients in a trial, calculated as |EER − CER|. In the case of hypoglycemic episodes, |EER − CER| = |57% − 23%| = 34%. (ARI is also used in assessing the impact of "risk factors" for disease.)
- **NNH (number needed to harm).** The number of patients who, if they received the experimental treatment, would lead to one additional patient being harmed, compared with patients who received the control treatment, calculated as 1/ARR. In this case, 1/ARR = 1/34% = 3