

治療效果的文獻評讀



Critical Analysis of Therapy

“治療效果”的評讀分析

- **Are the results valid ? (效度如何)**
 - Randomization – demographic table I
 - Blindedness
 - Follow Up -- Drop rate, intention-to-treat analysis
- **What are the results ? (效果強度如果)**
 - ARR (NNT), RRR, CI
- **Will the patients help me with my individual patient ? (可以應用到我的病人嗎)**
 - Outcomes
 - Benefits vs harms

Effect of Therapy (治療效果)

	一年的死亡人數	一年的存活人數
接受某治療	300	700
不接受某治療	800	200

實驗組事件發生率 (EER) = $300 / (300+700) = 30\%$

對照組事件發生率 (CER) = $800 / (800+200) = 80\%$

風險比 (Risk Ratio, RR) = $EER / CER = 0.3 / 0.8 = 0.375$

絕對危險性降低度 (ARR) = $CER - EER = 80\% - 30\% = 50\%$

相對風險性降低度 (RRR) = $(CER - EER) / CER = (80\% - 30\%) / 80\% = 62.5\%$

EER: Experimental event rate

CER: Control event rate

ARR: Absolute risk reduction

RRR: Relative risk reduction

相對風險性降低度 (RRR)與 絕對危險性降低度 (ARR)的比較

對照組的風險 CER	實驗組的風險 EER	相對風險性降低度 RRR	絕對危險性降低度 ARR
70%	35%	50%	35%
7%	3.5%	50%	3.5%
0.7%	0.35%	50%	0.35%

- 相對風險性降低度 (RRR)無法呈現實際風險降低程度，亦沒有考慮起始風險
- 絕對危險性降低度 (ARR)更準確表示治療效果，但亦不容易體會兩組的差別

Number Needed to Treat (NNT)

“益一需治數”

“益一需治數”：爲了預防一個不良結果
或減少一人死亡所需治療的病人數

例如：治療五人可減少一人死亡 **VS** 治療兩千人可減少一人死亡

$$\text{NNT} = 1 / \text{ARR} \text{ or } 100 / \text{ARR} (\%)$$

Confidence Intervals

信賴區間

Mean of the results
eg mortality reduction

45% (CI: 40% - 50%)

The range that includes
the true relative risk
reduction 95% of the time

If we were to conduct the same study an infinite number of times, we can expect that 95% of the time the mean reduction in mortality will fall somewhere between 40% and 50%

Confidence Intervals

45% (CI: 1% - 99%)

**Very small
sample size**

**Wide confidence
interval**

Need to take into consideration the characteristics of your specific patient and determine if your patient is more likely to fall closer to the lower end of the interval or higher end

Confidence Intervals

45% (CI: -2% - 53%)

1. Wide interval – small sample
2. Cross 0 – not statistically significant

- **Results can be clinically significant without being statistically significant**
- **A negative CI tells us that the results were opposite the expected outcome**
- **A mean probability of 45% reduction in mortality and no greater than 53%**
- **For some subset of the population studied, there is a 2% chance of and increase in Mortality (Clinically significant, but not statistically)**

Critical Appraisal of Therapy Study

“治療研究”的評析

- **Are the results of the trial valid (效度如何) ?**
 - Was the assignment of patients to treatment randomised (是隨機分配嗎) ?
 - Were the groups similar at the start of the trial (試驗開始時兩組條件是否相似) ?
 - Aside from the allocated treatment, were groups treated equally (兩組其他治療條件一樣) ?
 - Were all patients who entered the trial accounted for and were they analysed in the groups to which they were randomised (所有進入試驗者皆列入統計，並依所分配的組別計算) ?
 - Were measures objective or were the patients and clinicians were blinded (結果的測量客觀，受試者及醫師都不知道所接受的治療為何) ?
- **What were the results (結果為何) ?**
 - How large was the treatment effect (治療效果有多大) ?
 - How precise was the estimate of the treatment effect (治療效果的預測多準確) ?
- **Will the results help me in my patient care (適用於我的病人嗎) ?**

Was the assignment of patients to treatment randomised 是隨機分配嗎？

最理想狀況為何？	何處找到相關訊息？
理想狀況是以中央電腦隨機分配，尤其是跨中心的研究 較小型臨床試驗也可以“獨立人士”執行隨機分配	“研究方法”應敘述受試者如何做分配，是否隱藏式隨機分配

是

否

不清楚

評論： _____

Were the groups similar at the start of the trial 試驗開始時兩組條件是否相似？

最理想狀況為何？	何處找到相關訊息？
如果隨機分配的方式及過程無誤，實驗組與對照組應該相似 應有統計比較兩組是否有差異	“研究結果”的 table 1 比較兩組之間可能影響研究結果的基本條件及變異因子的差別，如年齡、性別、致病危險因子等

是

否

不清楚

評論： _____

Aside from the allocated treatment, were groups treated equally
兩組其他治療條件一樣？

最理想狀況為何？	何處找到相關訊息？
除了要研究的治療方式或檢驗外，兩組病人所接受的其他治療都一樣	“研究方法”中所敘述的追蹤計劃，允許的追加治療 “研究結果”中兩組實際接受各項額外治療的種類與比例

是

否

不清楚

評論： _____

Were all patients who entered the trial accounted for and were they analysed in the groups to which they were randomised

所有進入試驗者皆列入統計，並依所分配的組別計算？

最理想狀況為何？	何處找到相關訊息？
<p>追蹤過程中病人流失越少越好，流失率應低於20%</p> <p>達到研究結果的病人數太少時，即使流失率低，研究結論仍可能有偏差</p> <p>統計分析時應按照病人隨機分配的組別計算(Intention-to-treat analysis)</p>	<p>“研究結果”中應說明多少病人接受隨機分配 (table I)，多少病人進入統計分析。病人流失的數目、原因</p>

是

否

不清楚

評論： _____

Were measures objective or were the patients and clinicians were blinded

結果的測量客觀，受試者及醫師都不知道所接受的治療為何？

最理想狀況為何？	何處找到相關訊息？
<p>雙盲試驗：研究者與受試者皆不知道受試者所接受的治療為何</p> <p>結果評估的客觀性：結果的判斷若是客觀的（如死亡）較無爭議，但若結果的判斷是主觀的（如症狀、功能、能力）則評估者就不能知道病人是對照組或治療組</p>	<p>“研究方法”應說明對照組如何做偽裝治療，如給予外觀一樣的安慰劑</p> <p>“研究方法”應說明如何做結果評估，評估者是否知道病人接受的治療為何</p>

是

否

不清楚

評論： _____

How large was the treatment effect

治療效果有多大？

某一研究追蹤二年，對照組死亡率**15%**，治療組死亡率**10%**，結果的呈現方式有：

呈現方式	代表的意義
Relative Risk (相對風險) RR = 0.10 / 0.15 = 0.67	治療組發生風險相對於對照組的倍數。RR=1兩組無差別，RR<1治療可降低風險，RR>1治療會增加風險 RR<1表示治療可降低死亡的風險
Absolute Risk Reduction (絕對危險性降低度) ARR = 0.15 - 0.10 = 0.05 or 5%	治療組與對照組發生風險的絕對差異 治療的益處是降低5%的死亡率
Relative Risk Reduction (相對風險性降低度) RRR = 0.05 / 0.15 = 0.33 or 33% Or RRR = 1 - 0.67 = 0.33 or 33%	相對於對照組，治療組降低風險的比率 (最常見的呈現方式) 相對於對照組，治療可以降低死亡的的機率33%
Number Needed to Treat (益一需治數) NNT = 1 / ARR = 1 / 0.05 = 20	要預防一位不良結果發生所必需治療的病人數 必需治療20位病人2年才能預防1人死亡

How precise was the estimate of the treatment effect

治療效果的預測多準確？

- The true risk of the outcome in the population is not known
- The best we can do is to estimate the true risk based on the sample of patients in the trial
- We can gauge how close this estimate is to the true value by looking at the confidence intervals (CI)
- Narrow CI represents a precise reflection of the population value
- The CI also provides us with information about the statistical significance of the result
 - If the value corresponding to no effect falls outside the 95% CI then the result is statistically significant at the 0.05 level
 - If the CI includes the value corresponding to no effect then the results are not statistically significant

Will the Results Help Me in Caring for My Patients ?

- Are the people in the study like my patient ?
 - Age
 - General state of health
 - Type and severity of disease process
 - Time in the course of the disease
- Did the study cover all aspects of problem ?
 - eg treatment effect on symptom relief, quality of life, mortality etc
- Is the treatment feasible in my setting ?
- Will the potential benefits of treatment outweigh the potential harms of treatment for my patients ?
- Does it suggest a clear and useful plan of action ?
 - Help to clarify a patient's prognosis
 - Suggest a useful plan to improve patient's state of health