# Hur stor är behandlingseffekten? Om olika sätt att jämföra risker.

Per Liv

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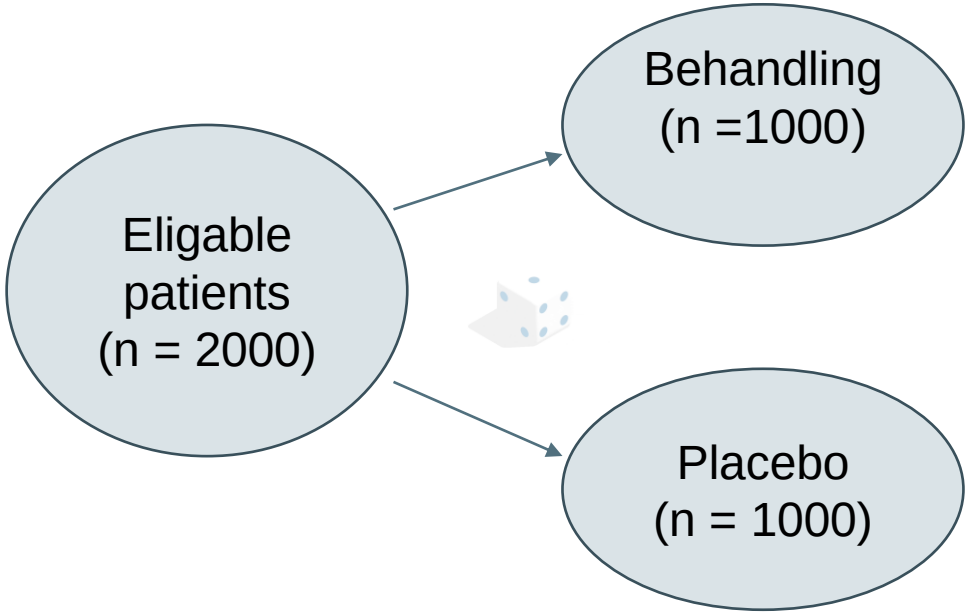
Antag att vi vill studera om en ny behandling kan minska risken för insjuknande jämfört med placebo

Vi randomiserar 2000 patienter till behandling eller placebo

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# Resultat

**Behandlingsgrupp:**
N = 1000

Antal insjuknade: 200

**Risk: 20%**

**Kontrollgrupp:**
N = 1000

Antal insjuknade: 300

**Risk: 30%**

## Sååå…hur bra är vår nya behandling?

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# Hur bra är vår nya behandling?

| Behandlingsgrupp | Kontrollgrupp |
|---|---|
| 20% | 30% |

Relativ risk: 20 / 30 = 0.66

Oddskvot: (20/80) / (30/70)  = 0.58

Riskskillnad: 30% - 20% = 10 procentenheter

# Vilket effektmått är bäst?

- Givetvis: vi vill kunna motivera nyttan med behandling
- Beskriva heterogenitet i behandlingseffekt
  - Hur mycket varierar behandlingseffekten mellan olika patientgrupper
- Om vi skattar behandlingseffekt på en patientgrupp, är den då överförbar (transportable) till en annan patientgrupp?

| Behandlingsgrupp | Kontrollgrupp |
|---|---|
| 20% | 30% |

Låt oss testa vår nya behandling på en population där kontrollgruppens risk bara är 9%
Hur stor risk förväntar vi oss i behandlingsgruppen?

Relativ risk: 9 *(2/3) **= 6%**

Oddskvot: lös ekvationen: x/(100-x) / (9/91) = 0.58 **= 5.4%**

Riskskillnad: 9 - 10 **= - 1% ??????**

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**Ingen behandling: 30% risk**

**Under behandling: 20% risk**

RR = 0.667

OR = 0.583

**Ingen behandling: 3% risk**

**Under behandling: 2% risk**

RR = 0.667

OR = 0.660

Ju högre risker, desto mer avviker oddskvoten från den relativa risken (om relativa risken hålls konstant )

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Letters

## Odds ratios should be avoided when events are common

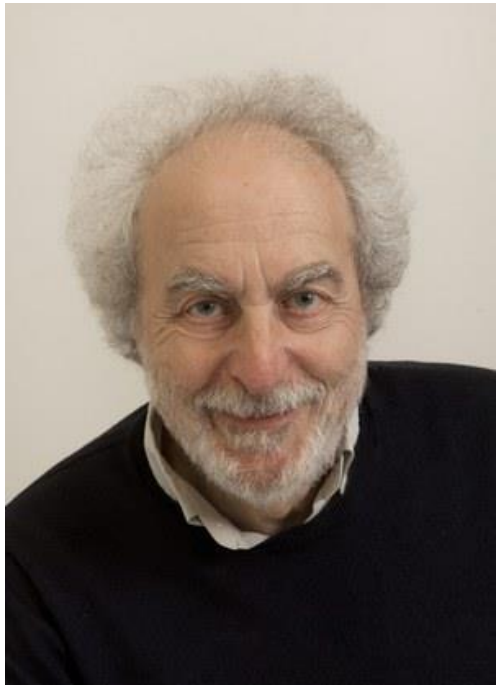
*BMJ* 1998 ; 317 doi: https://doi-org.proxy.ub.umu.se/10.1136/bmj.317.7168.1318 (Published 07 November 1998)
Cite this as: *BMJ* 1998;317:1318



This article has corrections. Please see:
Odds ratios should be avoided when events are common - December 05, 1998
Odds ratios should be avoided when events are common - November 21, 1998

*Douglas G Altman, Director, Jonathon J Deeks, Statistician, David L Sackett, Professor*

Author affiliations



EDITOR—A news item stated that "a review article written by authors with affiliations to the tobacco industry is 88 times more likely to conclude that passive smoking is not harmful than if the review was written by authors with no connection to the tobacco industry."[1] We are concerned that readers may have interpreted this huge effect at face value. The proportions being compared (which were not given in the news item) were 29/31 (94%) and 10/75 (13%). The relative risk here is 7, which indicates a strong association but is an order of magnitude lower than the reported odds ratio of 88.[2] This value is correct but is seriously misleading if presented or interpreted as meaning that the relative risk that affiliated authors would draw favourable conclusions was 88, as it was in this news item.

The odds ratio is valuable in case-control studies where events are usually rare and the relative risk cannot validly be estimated directly. In prospective studies interpretation of the odds ratio as an approximation to the relative risk becomes unreliable when events are common, and thus its use for prospective studies, especially randomised trials and systematic reviews, has been criticised.[3] [4] The distortion is especially large when the event rate is high in only one group, as in this example. The odds ratio should not be interpreted as an approximate relative risk unless the events are rare in both groups (say, less than 20-30%).

https://www.bmj.com/content/317/7168/1318.1

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# Dom fick ett svar av statistiker Stephen Senn

## Rare Distinction and Common Fallacy

Altman, Deeks and Sackett1 claim that the odds ratio should be avoided except when events are rare because the odds ratio becomes unreliable as an approximation to the relative risk when events are common. The correct advice, however, is the opposite of what they give.
The odds ratio and not the relative risk is the gold standard. The relative risk is only acceptable when events are rare, when it becomes adequate as an approximation to the odds ratio. The odds ratio is invariant to the arbitrary decision of deciding whether we concentrate on
the relative risk of dying or the relative risk of living, being the product of the two.It is only when the latter is nearly equal to one that using the former alone is acceptable.

How many statisticians, in examining their medical students, would give them full marks if in carrying out a test of association on a two by two table they worked only with the discrepancy between observed and
expected for the two cells corresponding to 'events' rather than for all four? However, this is the exact analogy of working with relative risk rather than odds ratios. It gives an approximately correct answer when 'events' are rare relative to 'non-events' but it is generally and philosophically unsound.

1 Douglas G Altman, Jonathon J Deeks, and David L Sackett Odds ratios should be avoided when events are common BMJ 1998; 317: 1318

**Competing interests:** No competing interests

**10 May 1999**

**Stephen Senn**
Professor of pharmaceutical health statistics
*University College London*

"The correct advice, however, is the opposite of what they give.
The odds ratio and not the relative risk is the gold standard. The relative risk is only acceptable when events are rare, when it becomes adequate as an approximation to the odds ratio. "

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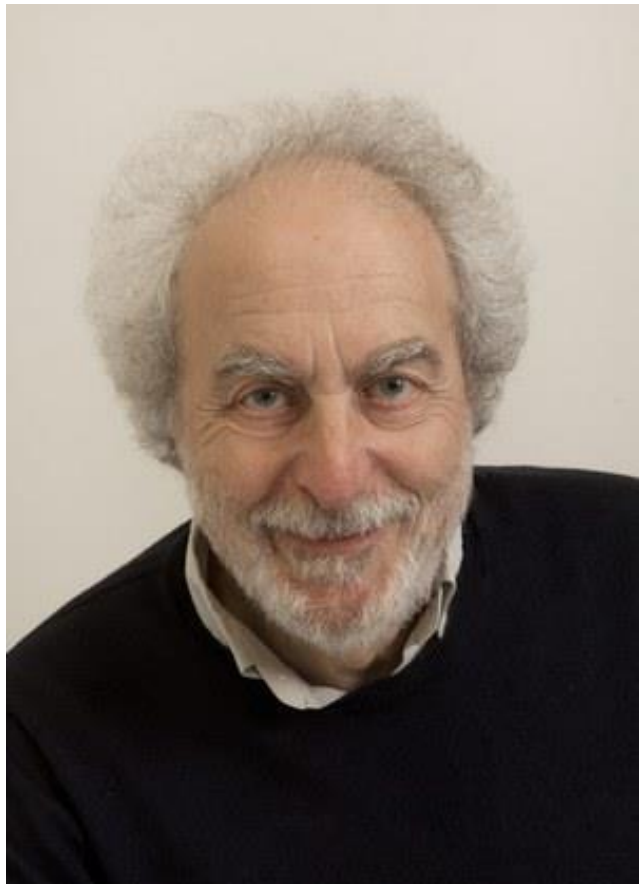
Stephen Senn

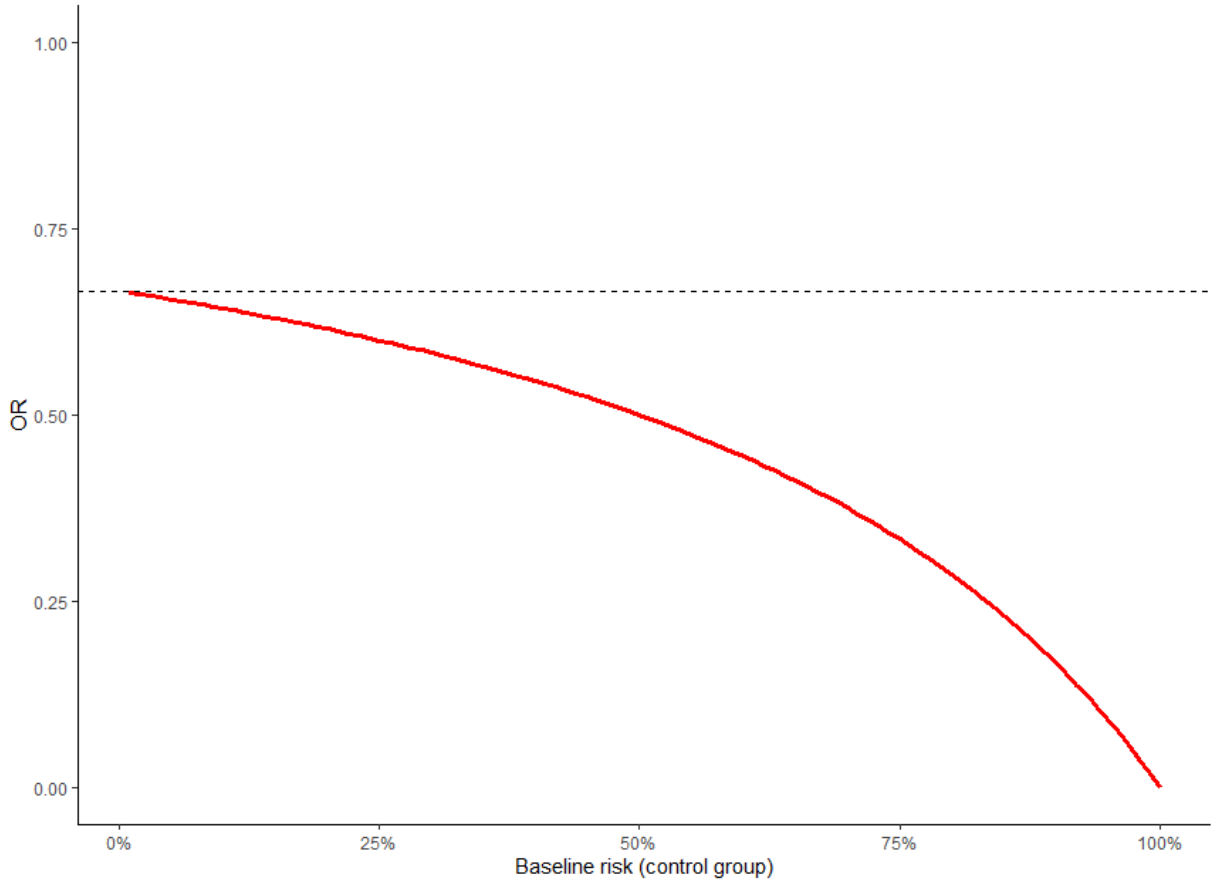
Doug Altman



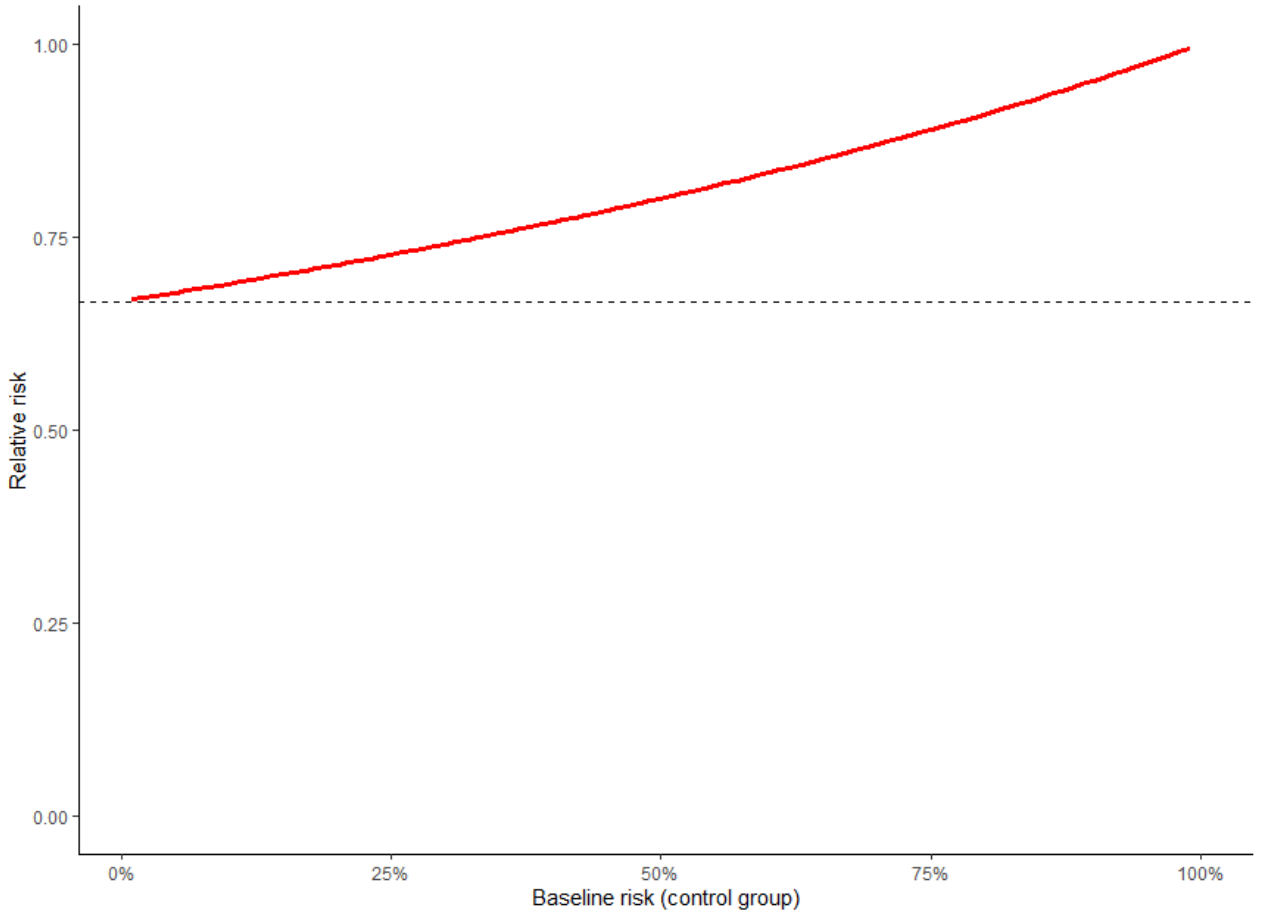
# Två statistiska giganter – två diametralt motsatta slutsatser

## Hur är det möjligt?

## Om vi antar att relativa risken är konstant: oddskvoten blir bara lägre




## Om vi antar att oddskvoten är konstant: relativa risken blir bara större och större

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# Ur ”Statistical issues in Drug Development” av Stephen Senn

Ur ”Glossory”:

Epidemiologist - one who think that the odds ratio is an approximation of relative risk opposed to a statistician who knows the opposite”

# Vanliga argument för/emot relativ risk

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- ”Enklare att förstå”

-

- Kan ge leda till ”omöjliga” sannolikheter vid beräkning
  - Om kontrollgruppens risk är 50% och den relativa risken är 3 kommer risken under behandling predikteras till 150%

Ger olika svar beroende på om vi räknar sjuka eller friska

- Asymmetrisk

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# Antag att vi gör en intervention för att öka andelen mammor som ammar

**Kontrollgruppen**

Andel ammande : 80%

**Interventionsgrupp**

- Andel ammande : 90%

"Liten effekt" → RR: 90/80 = 1.12

"Stor effekt" → OR: (90/10)/(80/20) = 2.25

**Men vad händer om vi räknar andel som INTE ammar:**

"Stor effekt" → RR: 10/20 = 0.50

"Stor effekt" → OR: (10/90)/(20/80) = 0.44 *(= 1 / 2.25)*

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# Switch ratio

Mendel C Sheps (1913 – 1973)

Publicerade 1958 en artikel med titeln

”Shall we count the living or the dead”.

Sheps, Mindel C. "Shall we count the living or the dead?." *New England Journal of Medicine* 259.25 (1958): 1210-1214.

# Shall we count the living or the dead?

Anders Huitfeldt[1,2], Matthew P. Fox[3,4], Rhian M. Daniel[5], Asbjørn Hróbjartsson[1,2], and Eleanor J. Murray[3]

Preprint: https://arxiv.org/abs/2106.06316

https://www.youtube.com/watch?v=gS1tSeXO_Kk

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# Shall we count the living or the dead?

Anders Huitfeldt[1,2], Matthew P. Fox[3,4], Rhian M. Daniel[5], Asbjørn Hróbjartsson[1,2], and Eleanor J. Murray[3]

Switch ratio:

När behandlingen minskar risken:  räkna ”döda” (den vanlig relativa risken, RR-)

Om behandlingen ökar risken: räkna ”dom levande” (RR+)

# Risk ratio, odds ratio, risk difference... Which causal measure is easier to generalize?

Bénédicte Colnet * Julie Josse† Gaël Varoquaux‡ Erwan Scornet §

May 3, 2023

**Abstract**

There are many measures to report so-called treatment or causal effect: absolute difference, ratio, odds ratio, number needed to treat, and so on. The choice of a measure, *eg* absolute versus relative, is often debated because it leads to different appreciations of the same phenomenon; but it also implies different heterogeneity of treatment effect. In addition some measures – but not all – have appealing properties such as collapsibility, matching the intuition of a population summary. We review common measures and their pros and cons typically brought forward. Doing so, we clarify notions of collapsibility and treatment effect heterogeneity, unifying different existing definitions. Our main contribution is to propose to reverse the thinking: rather than starting from the measure, we start from a non-parametric generative model of the outcome. Depending on the nature of the outcome, some causal measures disentangle treatment modulations from baseline risk. Therefore, our analysis outlines an understanding of what heterogeneity and homogeneity of treatment effect mean, not through the lens of the measure, but through the lens of the covariates. Our goal is the generalization of causal measures. We show that different sets of covariates are needed to generalize an effect to a different target population depending on *(i)* the causal measure of interest, *(ii)* the nature of the outcome, and *(iii)* the generalization's method itself (generalizing either conditional outcome or local effects).

*Keywords:* Standardization; Transportability; Collapsibility; Treatment effect modifier; Clinical trials.

https://arxiv.org/abs/2303.16008

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# Vanliga argument för/emot oddskvot

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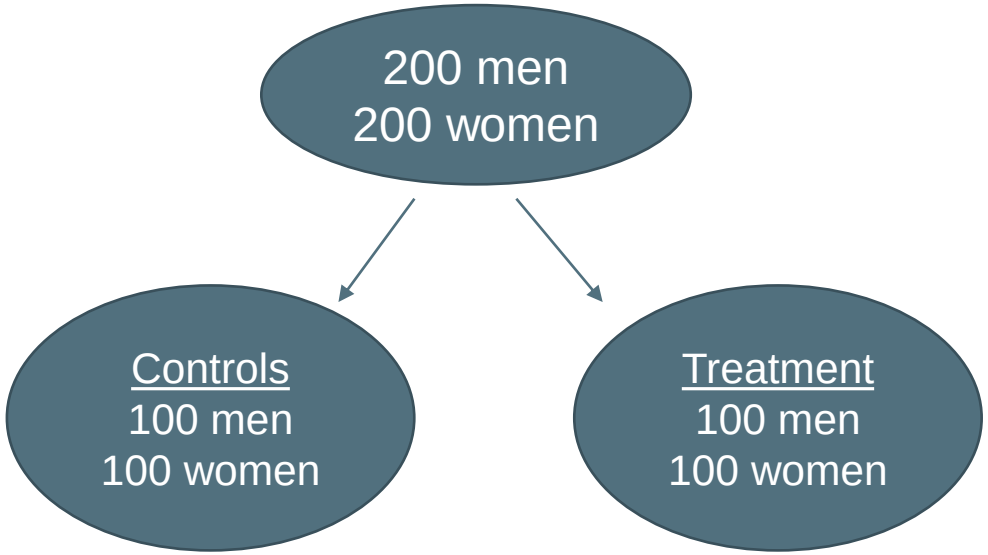
- Symmetrisk
- ”T.om sportfånar förstår odds”
- Ökad överförbarhet tack vare inte beroende av baslinjerisken (diskuteras)
- ”Logistisk regression”

-

- Svårare att tolka
- Icke-kollapsbar

# Icke-kollapsbarhet




| Men | | |
|---|---|---|
| | Sick | Healthy |
| Treatment | 90 | 10 |
| Control | 95 | 5 |

Odds ratio: 0.47

| Women | | |
|---|---|---|
| | Sick | Healthy |
| Treatment | 5 | 95 |
| Control | 10 | 90 |

Odds ratio: 0.47

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Subject-specific effect

| Men | | |
|---|---|---|
| | Sick | Healthy |
| Treatment | 90 | 10 |
| Control | 95 | 5 |

Odds ratio: 0.47

| Women | | |
|---|---|---|
| | Sick | Healthy |
| Treatment | 5 | 95 |
| Control | 10 | 90 |

Odds ratio: 0.47

Kollaps av data

Population-averaged effect

| Collapsed data | | |
|---|---|---|
| | Sick | Healthy |
| Treatment | 95 | 105 |
| Control | 105 | 95 |

Odds ratio: 0.82

Samma egenskap har hazardkvoten

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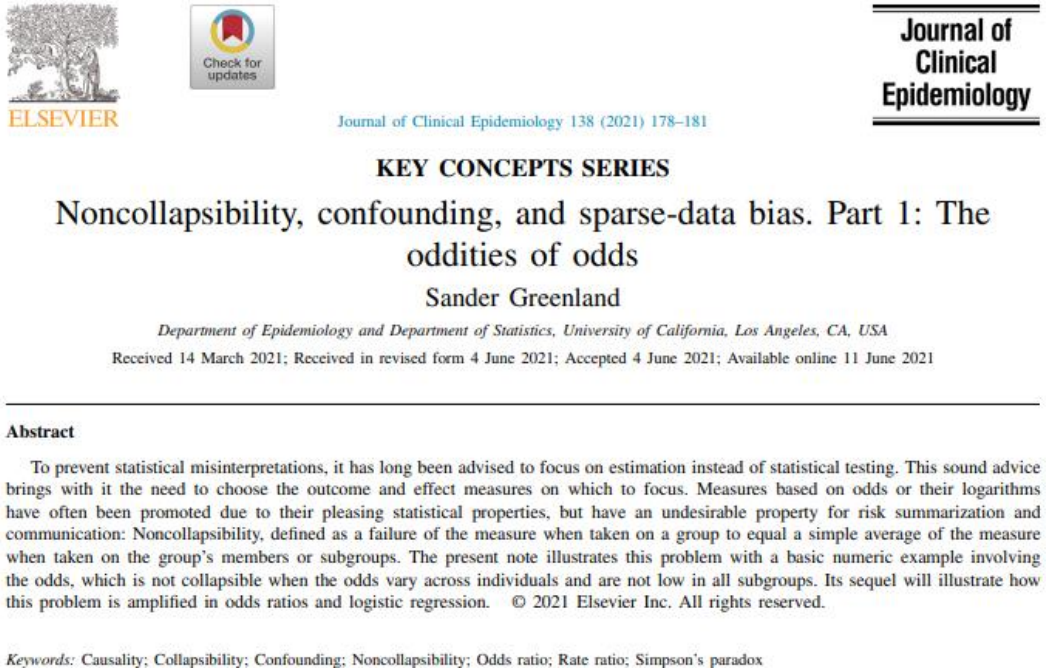

Journal of Clinical Epidemiology 138 (2021) 178–181

Journal of Clinical Epidemiology

KEY CONCEPTS SERIES

# Noncollapsibility, confounding, and sparse-data bias. Part 1: The oddities of odds

Sander Greenland

*Department of Epidemiology and Department of Statistics, University of California, Los Angeles, CA, USA*

Received 14 March 2021; Received in revised form 4 June 2021; Accepted 4 June 2021; Available online 11 June 2021

**Abstract**

To prevent statistical misinterpretations, it has long been advised to focus on estimation instead of statistical testing. This sound advice brings with it the need to choose the outcome and effect measures on which to focus. Measures based on odds or their logarithms have often been promoted due to their pleasing statistical properties, but have an undesirable property for risk summarization and communication: Noncollapsibility, defined as a failure of the measure when taken on a group to equal a simple average of the measure when taken on the group's members or subgroups. The present note illustrates this problem with a basic numeric example involving the odds, which is not collapsible when the odds vary across individuals and are not low in all subgroups. Its sequel will illustrate how this problem is amplified in odds ratios and logistic regression. 

*Keywords:* Causality; Collapsibility; Confounding; Noncollapsibility; Odds ratio; Rate ratio; Simpson's paradox

"It is hoped that this pair of papers will aid readers in approaching more detailed analyses of the distinction and the reasons for preferring measures comparing proportions (risks) rather than odds whenever the odds do not approximate the risks"

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# Oddskvot och baslinjerisk

| | Fall | Icke-fall | Risk |
|---|---|---|---|
| Exponerade | A | B | A/(A+B) |
| Icke-exponerade | C | D | C/(C+D) |

$$Oddskvot = \frac{A/(A+B)}{1 - A/(A+B)} \; / \; \frac{C/(C+D)}{1 - C/(C+D)} = \ldots\ldots = \frac{AD}{CB}$$

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# Fall-kontrollstudier

Fallkontroll börjar med att identifiera ett antal fall: A och C

| | Fall | Kontroller |
|---|---|---|
| Exponerade | A | |
| Icke-exonerade | C | |

En stor begränsning med fall-kontrollstudier är att vi observerar “fel” antal controller (icke-fall) pga godtycklig samplingfrekvens av dessa

Låt oss säga att *g* är hur mycket "undersamplade" icke-fallen är
T.ex. g = 0.01 innebär att en kohortstudie med motsvarande antal fall skulle innehållit 100 gånger så många "icke-cases"

| | Fall | Kontroller |
|---|---|---|
| Exponerade | A | gB |
| Icke-exponerade | C | gD |

**Vi kan inte räkna ut absoluta risker att bli ett case eftersom vi inte känner *g***

Risk bland exponerade = A/(A+gB) BIAS!
Risk bland icke-exponerade = C/(C+gD) BIAS!

| | Fall | Kontroller |
|---|---|---|
| Exponerade | A | gB |
| Icke-exponerade | C | gD |

| | Fall | Kontroller |
|---|---|---|
| Exponerade | A | gB |
| Icke-exponerade | C | gD |

Riskskillnad = $\frac{A}{A+gB} - \frac{C}{C+gD}$ **BIAS!**

Relativ risk = $\frac{A}{A+gB} / \frac{C}{C+gD}$ **BIAS!**

Oddskvot = $\frac{AgD}{CgB} = \frac{AD}{CB}$ **!!!!!!! Samma som för en kohort**

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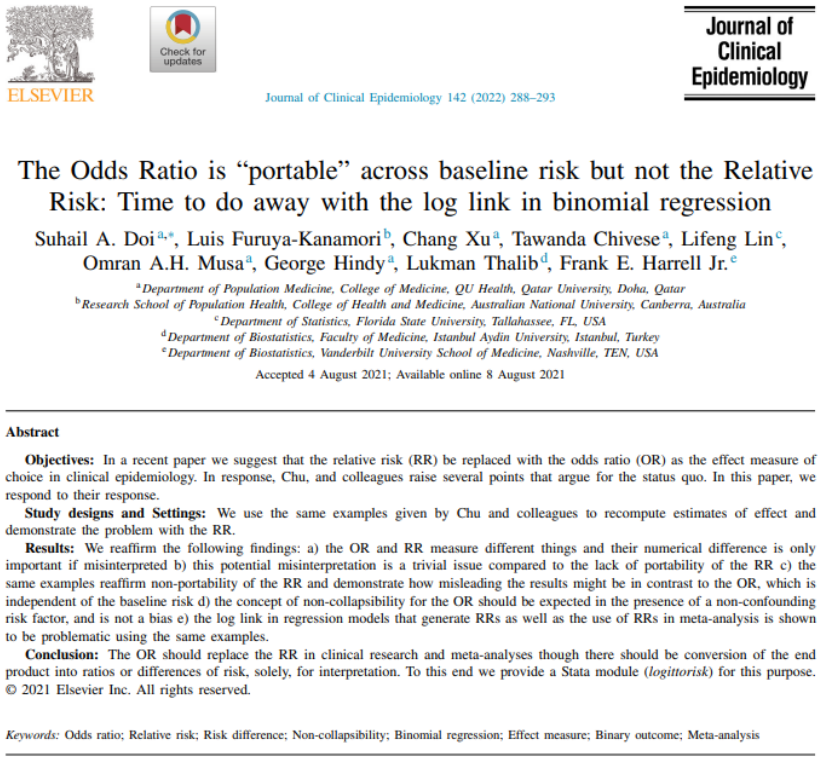
# Finns det empiriska belägg för att oddskvoten är mer överförbar?



ELSEVIER

Journal of Clinical Epidemiology 142 (2022) 288–293

Journal of Clinical Epidemiology

## The Odds Ratio is "portable" across baseline risk but not the Relative Risk: Time to do away with the log link in binomial regression

Suhail A. Doi[a,*], Luis Furuya-Kanamori[b], Chang Xu[a], Tawanda Chivese[a], Lifeng Lin[c], Omran A.H. Musa[a], George Hindy[a], Lukman Thalib[d], Frank E. Harrell Jr.[e]

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Accepted 4 August 2021; Available online 8 August 2021

**Abstract**

**Objectives:** In a recent paper we suggest that the relative risk (RR) be replaced with the odds ratio (OR) as the effect measure of choice in clinical epidemiology. In response, Chu, and colleagues raise several points that argue for the status quo. In this paper, we respond to their response.

**Study designs and Settings:** We use the same examples given by Chu and colleagues to recompute estimates of effect and demonstrate the problem with the RR.

**Results:** We reaffirm the following findings: a) the OR and RR measure different things and their numerical difference is only important if misinterpreted b) this potential misinterpretation is a trivial issue compared to the lack of portability of the RR c) the same examples reaffirm non-portability of the RR and demonstrate how misleading the results might be in contrast to the OR, which is independent of the baseline risk d) the concept of non-collapsibility for the OR should be expected in the presence of a non-confounding risk factor, and is not a bias e) the log link in regression models that generate RRs as well as the use of RRs in meta-analysis is shown to be problematic using the same examples.

**Conclusion:** The OR should replace the RR in clinical research and meta-analyses though there should be conversion of the end product into ratios or differences of risk, solely, for interpretation. To this end we provide a Stata module (*logittorisk*) for this purpose.


*Keywords:* Odds ratio; Relative risk; Risk difference; Non-collapsibility; Binomial regression; Effect measure; Binary outcome; Meta-analysis

https://www.sciencedirect.com/science/article/pii/S0895435621002419?via%3Dihub

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ELSEVIER

Journal of Clinical Epidemiology 142 (2022) 294–304

Journal of Clinical Epidemiology

# Controversy and Debate : Questionable utility of the relative risk in clinical research: Paper 4 :Odds Ratios are far from "portable" — A call to use realistic models for effect variation in meta-analysis

Mengli Xiao[a], Haitao Chu[a,*], Stephen R. Cole[b], Yong Chen[c], Richard F. MacLehose[d], David B. Richardson[c], Sander Greenland[e]

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Received 1 June 2021; Accepted 4 August 2021; Available online 11 August 2021

**Abstract**

**Objective:** Recently Doi et al. argued that risk ratios should be replaced with odds ratios in clinical research. We disagreed, and empirically documented the lack of portability of odds ratios, while Doi et al. defended their position. In this response we highlight important errors in their position.

**Study design and setting:** We counter Doi et al.'s arguments by further examining the correlations of odds ratios, and risk ratios, with baseline risks in 20,198 meta-analyses from the Cochrane Database of Systematic Reviews.

**Results:** Doi et al.'s claim that odds ratios are portable is invalid because 1) their reasoning is circular: they assume a model under which the odds ratio is constant and show that under such a model the odds ratio is portable; 2) the method they advocate to convert odds ratios to risk ratios is biased; 3) their empirical example is readily-refuted by counter-examples of meta-analyses in which the risk ratio is portable but the odds ratio isn't; and 4) they fail to consider the causal determinants of meta-analytic inclusion criteria: Doi et al. mistakenly claim that variation in odds ratios with different baseline risks in meta-analyses is due to collider bias. Empirical comparison between the correlations of odds ratios, and risk ratios, with baseline risks show that the portability of odds ratios and risk ratios varies across settings.

**Conclusion:** The suggestion to replace risk ratios with odds ratios is based on circular reasoning and a confusion of mathematical and empirical results. It is especially misleading for meta-analyses and clinical guidance. Neither the odds ratio nor the risk ratio is universally portable. To address this lack of portability, we reinforce our suggestion to report variation in effect measures *conditioning* on modifying factors such as baseline risk; understanding such variation is essential to patient-centered practice. 

*Keywords:* Baseline risk; Clinical guidance; Cochrane Database of Systematic Reviews; Correlation; Meta-analysis; Odds ratio; Risk ratio

"Conclusion: The suggestion to replace risk ratios with odds ratios is based on circular reasoning and a confusion of mathematical and empirical results. It is especially misleading for meta-analyses and clinical guidance…."

https://www.jclinepi.com/article/S0895-4356(21)00242-0/fulltext

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HHS Public Access

Author manuscript

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*J Clin Epidemiol*. 2022 February ; 142: 280–287. doi:10.1016/j.jclinepi.2021.08.004.

# Is OR "portable" in meta-analysis? Time to consider bivariate generalized linear mixed model

**Mengli Xiao**[a], **Yong Chen**[b], **Stephen Cole**[c], **Richard MacLehose**[d], **David Richardson**[c], **Haitao Chu**[a,*]

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**Study designs and settings:** We summarized the Spearman's rank correlation coefficient between study-specific OR and the baseline risk in 40,243 meta-analyses from the Cochrane Database of Systematic Reviews (CDSR)

**Conclusions**: "Replacing RR or RD with OR is currently unadvisable in clinical trials and meta-analyses. It is possible that no effect measure is "portable" in a meta-analysis"

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8842816/

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# Vanliga argument för/emot absolut riskskillnad

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- ”lätt att förstå”
- Numbers needed to treat

-

- Helt frånkopplad från baslinjerisken
  - Risk för dålig överförbarhet
- Kan också ge leda till ”omöjliga” sannolikheter vid beräkning

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Open access Protocol

BMJ Open

# SALpingectomy for STERilisation (SALSTER): study protocol for a Swedish multicentre register-based randomised controlled trial

Leonidas Magarakis ,[1] Annika Idahl,[2] Karin Sundfeldt,[1,3] Per Liv,[4] Mathias Pålsson,[1] Annika Strandell[1,3]

**To cite:** Magarakis L, Idahl A, Sundfeldt K, *et al*. SALpingectomy for STERilisation (SALSTER): study protocol for a Swedish multicentre register-based randomised

**ABSTRACT**

**Introduction** Salpingectomy is currently suggested as an alternative to tubal ligation for sterilisation. Precursor lesions of ovarian carcinoma can be found in the fallopian tubes; thus, salpingectomy could possibly reduce the

**STRENGTHS AND LIMITATIONS OF THIS STUDY**

⇒ The register-based randomised controlled trial combines the advantages of two study designs: the randomised trial with unbiased allocation to minimise

Non-inferiority-marginal för komplikationer på 10 pp.

Studien utvärderar om riskskillnaden är mindre än 10 pp

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10481827/

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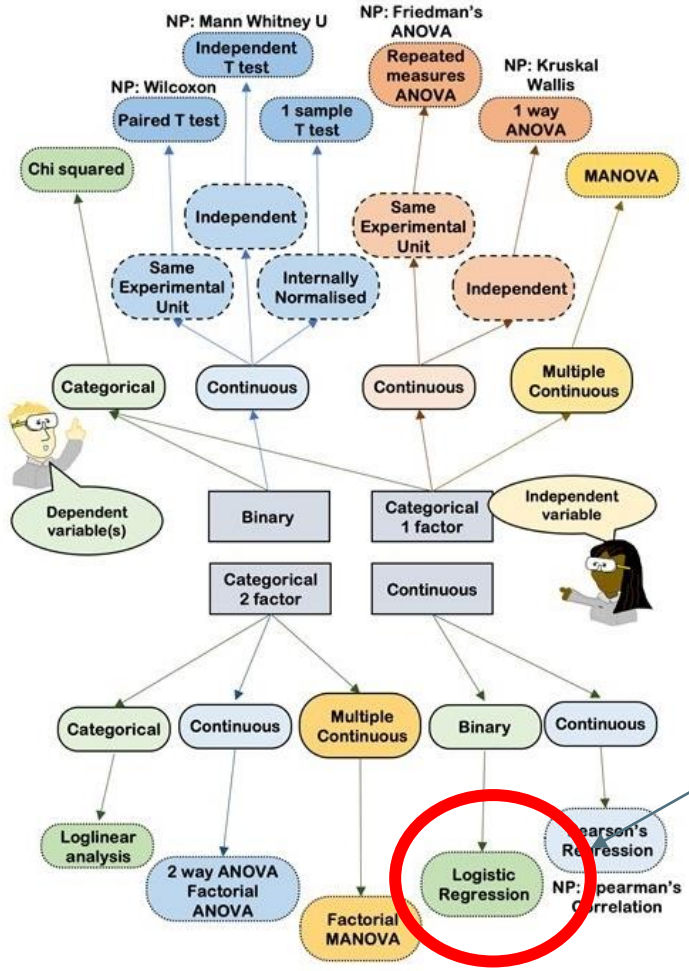
# Hur skatttar vi konfidensintervallen för effektmått?




Logistisk regression är vad som tenderas att läras ut som enda model vid binära utfall

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# Hur skatttar vi konfidensintervallen för effektmått?

- Oddskvot – logistisk regression – fungerar väldigt ofta

- Relativ risk – flera val
  - Robust poissonregression
  - Log-binomial,…mm
  - T.om. logistisk regression, med lite beräkningsjobb

- Riskskillnad – flera val
  - Binomialregression – ger svaret direkt, men modell som ofta passar data dåligt
  - Poisson, logistisk logistisk regression mm…, med lite beräkningsjobb

# Evaluating Public Health Interventions: 6. Modeling Ratios or Differences? Let the Data Tell Us

We provide an overview of the relative merits of ratio measures (relative risks, risk ratios, and rate ratios) compared with difference measures (risk and rate differences). We discuss evidence that the multiplicative model often fits the data well, so that rarely are interactions with other risk factors for the outcome observed when one uses a logistic, relative risk, or Cox regression model to estimate the intervention effect.

As a consequence, additive models, which estimate the risk or rate difference, will often exhibit interactions. Under these circumstances, absolute measures of ef-

*Donna Spiegelman, MS, ScD, and Tyler J. VanderWeele, PhD*

In part one of this two-part commentary, the sixth in this series, we provide an overview of the considerations involved in the choice of the intervention effect estimator, primarily but not exclusively focusing on the relative merits of ratio measures (relative risks, risk ratios, or rate ratios), compared with difference measures (risk or rate differences). These terms are defined in the box on the next page.

Nearly all studies we are aware of in population health, including public health evaluations, are epidemiological design principle has made it possible for an enormous amount of information about risk factors for most common diseases to have been obtained over the past 35 years.

The logistic regression model became quite popular in population sciences because it is very stable numerically and may give odds ratio parameter estimates that quite closely approximate the risk ratio when exponentiated. As is well established, the odds ratio is not a parameter

important examples in which the logistic approximation has led us astray have been given.[8,9] The rare disease assumption is obviated when rates are the measure of disease frequency in cohort studies and in incidence-density, or risk-set-sampled case–control studies. The appendix (available as a supplement to the online version of this article at http://www.ajph.org) contains a more in-depth overview of these points.

"In summary, it is best to estimate intervention effects on the scale that best fits the data, which seems very often to be the multiplicative scale."

"As a consequence, additive models, which estimate the risk or rate difference, will often exhibit interactions."

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5463222/

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# Numbers needed to treat

- NNT = 1 / riskskillnad

- Antal patienter du behöver behandla för att förhindra ett utfall
  - Om NNT är 1/0.01 = 100, behöver vi behandla 100 patienter för att förhindra en hjärt-kärlhändelse

# Problem med numbers-needed-to-treat

- Konfidensintervall
  - Singularitet vid noll
  - Svårt att konstruera
  - Ofta breda
- Man tappar referensen till baslinjerisken
- Ofta missförstådd
- Tidsaspekt glöms ofta bort: ju längre behandling, desto lägre NNT

# Sammanfattning

- Rapportera båda absoluta och relativa mått på risk
- Var försiktig med att jämföra behandlingseffekter med olika baslinjerisker, ofta beror slutsatsen på effektmåttets skala
- Håll isär begreppen risk och odds
- I valet av bästa effektstorlek råder ingen konsensus.

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