A NOTE ABOUT THE R COEFFICIENT, THE INTRACLASS CORRELATION COEFFICIENT AND THEIR ASSOCIATION WITH OUTCOME PREVALENCE

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Current developments in cluster randomised trials and stepped wedge designs
London, November 21, 2018

Plan

Analyse a cluster randomized trials $\longrightarrow$ Report the ICC[1]

[1] Campbell MK et al., *British Medical Journal*, 328(7441):702–708, 2004

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- k: number of clusters
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$\rho = \text{cor}(X_{ij}, X_{ij'})$ for all $i = 1, 2, ..., k$ and $j \neq j'$

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$$\rho = \frac{\text{P}(X_{ij}=1|X_{ij'}=1)-\pi}{1-\pi}$$

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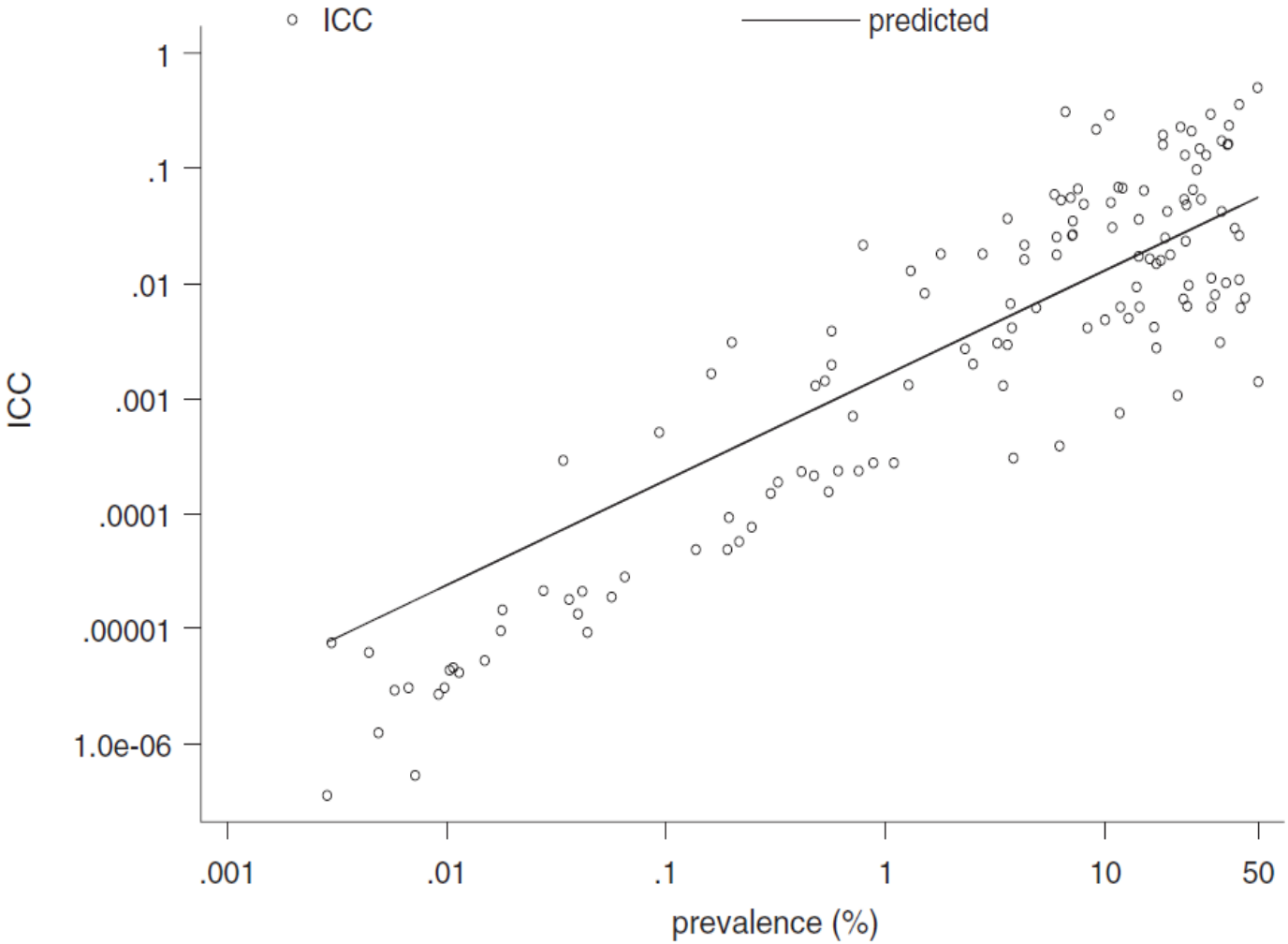
Binary outcome → Association between the outcome prevalence and the ICC[3]



[3]Gulliford M et al., *Journal of clinical epidemiology*, 58(3):246–251, 2005

Plan

- As defined by Rosner[4]:

$$\mathrm{P}(X_{ij} = 1 | X_{ij'} = 1) = R\pi, \quad j, j' = 1, 2 \text{ and } j \neq j'$$

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- $R = 1 + \frac{\rho(1-\pi)}{\pi}$

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- R is less influenced by the outcome prevalence than the ICC[6]
- Illustration with an example

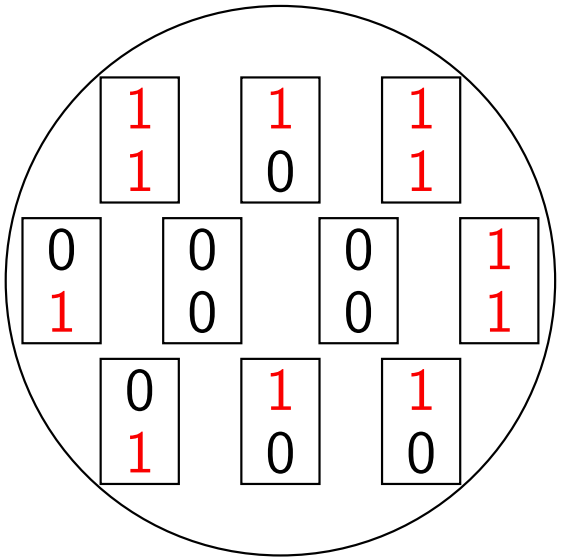
[6] Crespi et al., *Clinical Trials*, 8(6):687–698, 2011

Plan

To explore the relation between the ICC, the R coefficient and the outcome prevalence.

1. Through mathematical developments
2. Through a simulation study

Plan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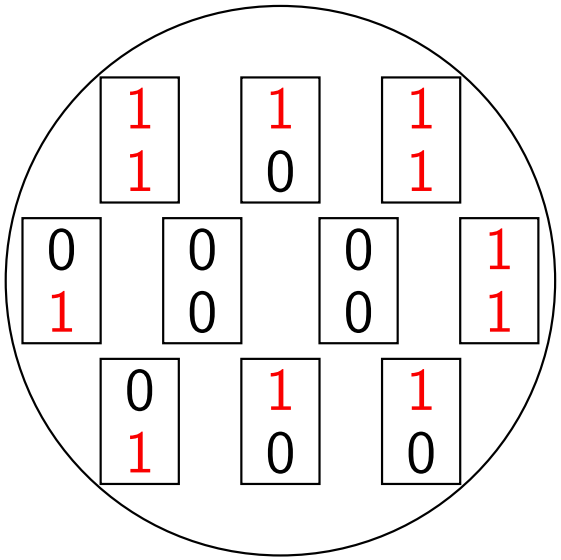
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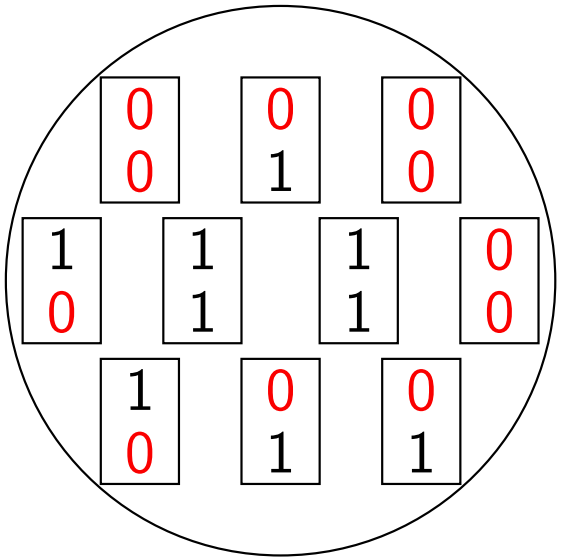
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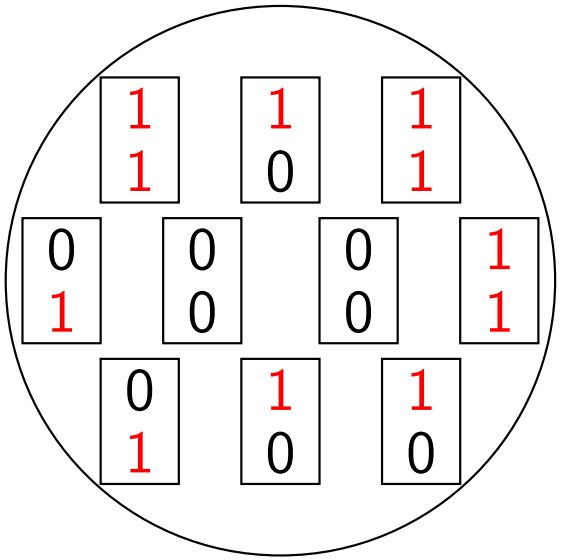
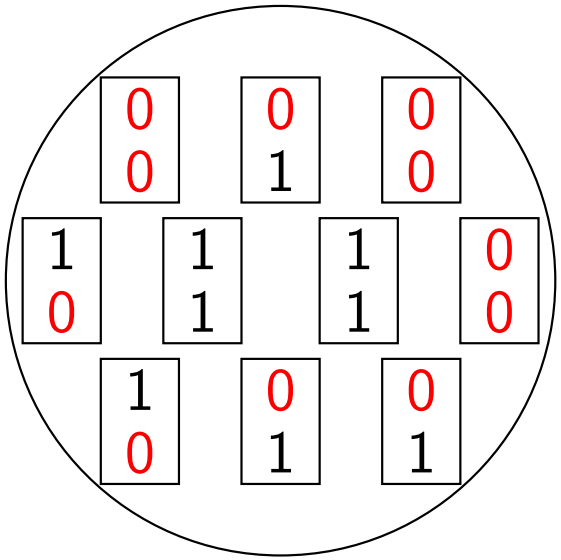
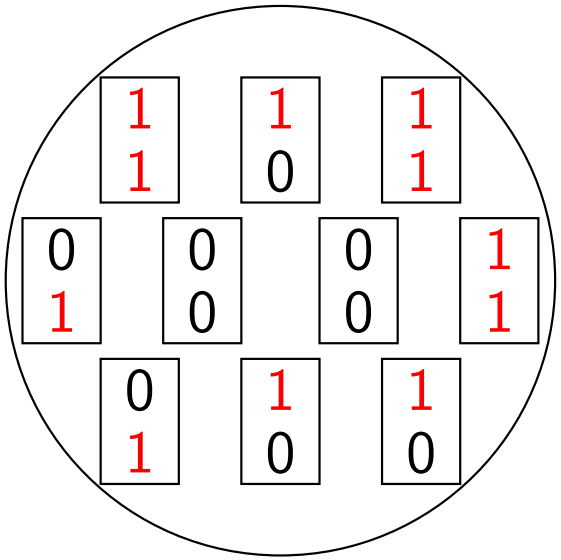


Prevalence=$\widehat{\pi}$

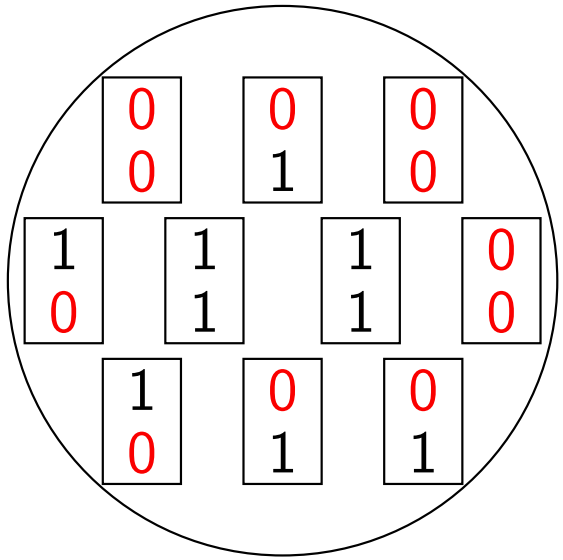


Prevalence=$1 - \widehat{\pi}$



Prevalence$=\widehat{\pi}$

ICC$=\widehat{\rho}$



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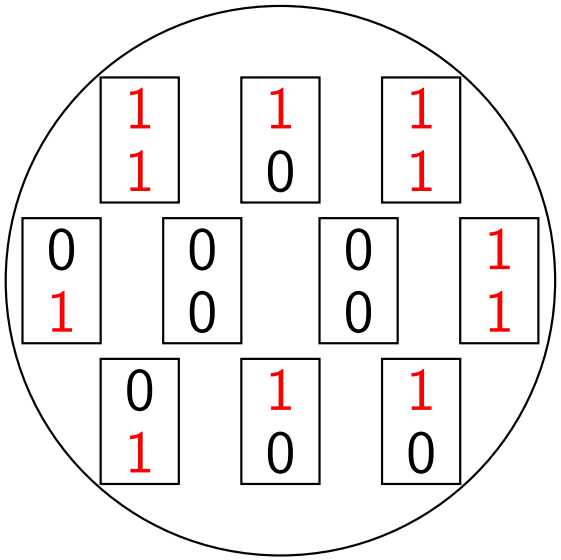
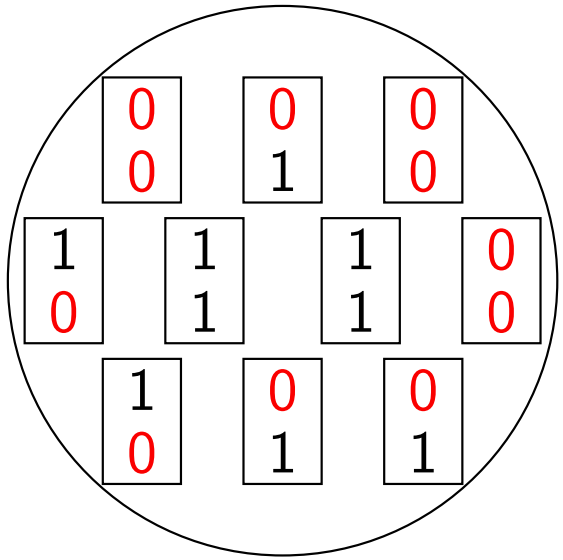


Prevalence=$\widehat{\pi}$

ICC=$\widehat{\rho}$

R coefficient=$\widehat{R}_1$

$\widehat{R}_1 \neq \widehat{R}_2$



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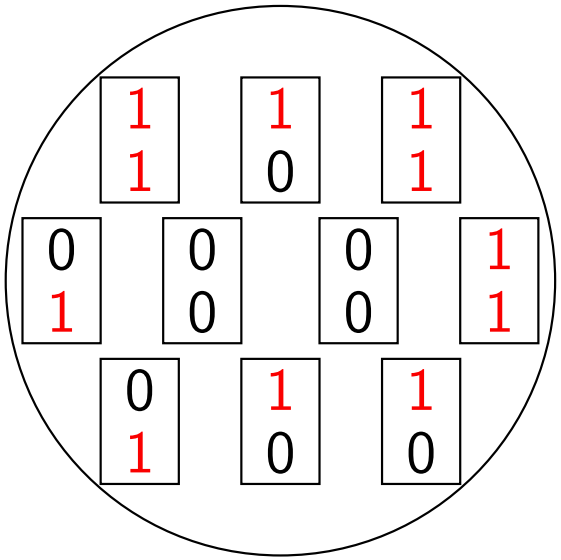

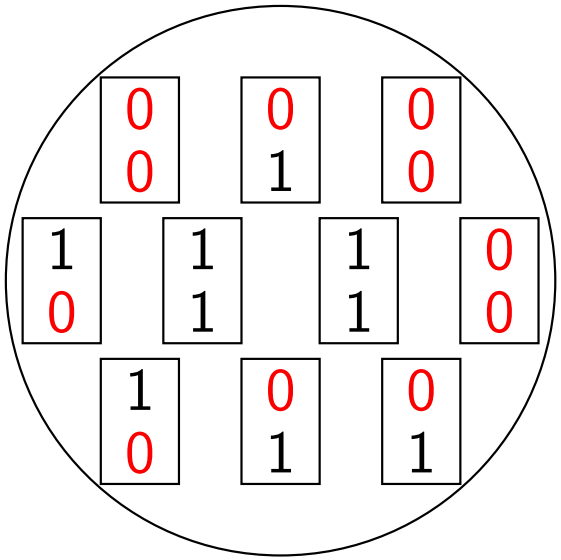

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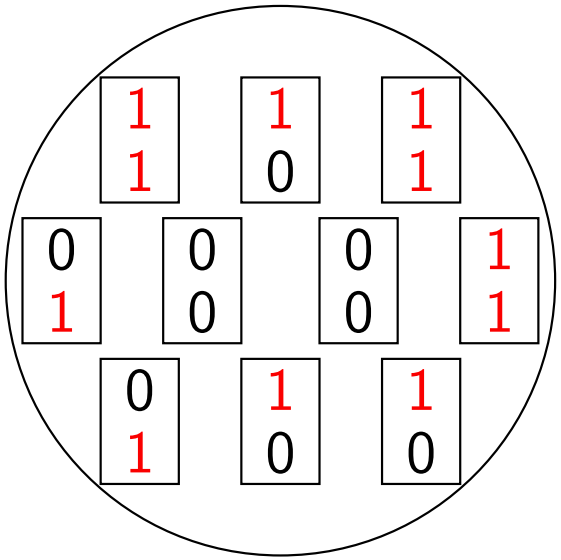
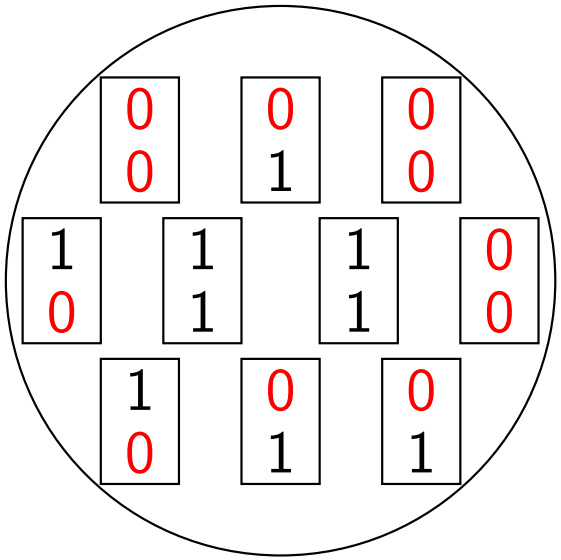
Asymmetry of R around the 0.5 prevalence value

R depends on prevalence



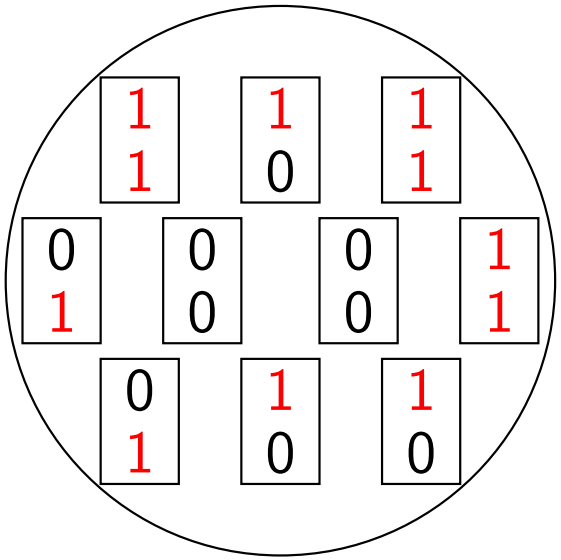
50 clusters

$\widehat{\pi}_1 = \mathbf{0.35}$



50 clusters

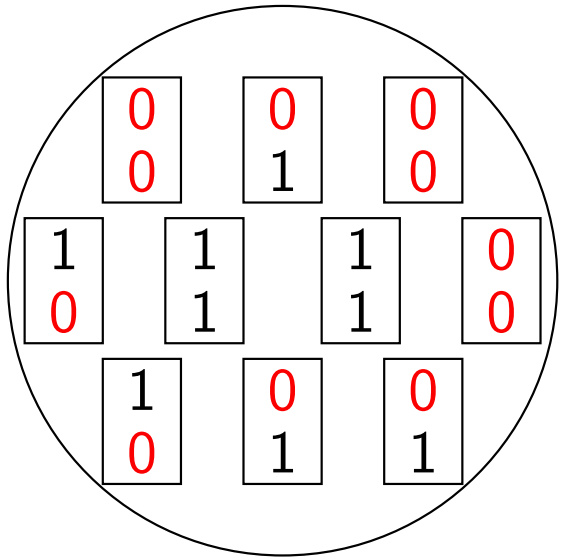
$\widehat{\pi}_2 = \mathbf{0.65}$



50 clusters

$\widehat{\pi}_1 = 0.35$
$\widehat{\rho}_1 = 0.78$
$\widehat{R}_1 = 2.45$

$\widehat{\rho}_1 = \widehat{\rho}_1$
$\widehat{R}_1 \neq \widehat{R}_2$



50 clusters

$\widehat{\pi}_2 = 0.65$
$\widehat{\rho}_2 = 0.78$
$\widehat{R}_2 = 1.42$

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- Vary the threshold of dichotomization $\rightarrow$ vary prevalence with a fixed underlying clustering
- Generate 10 000 datasets
- Many scenarios : $\begin{cases} \text{Fixed/variable cluster sizes} \\ \text{Various underlying ICCs} \\ \text{Various numbers of cluster} \end{cases}$

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ICC = 0.3

20 clusters: mean = 25, variance = 225

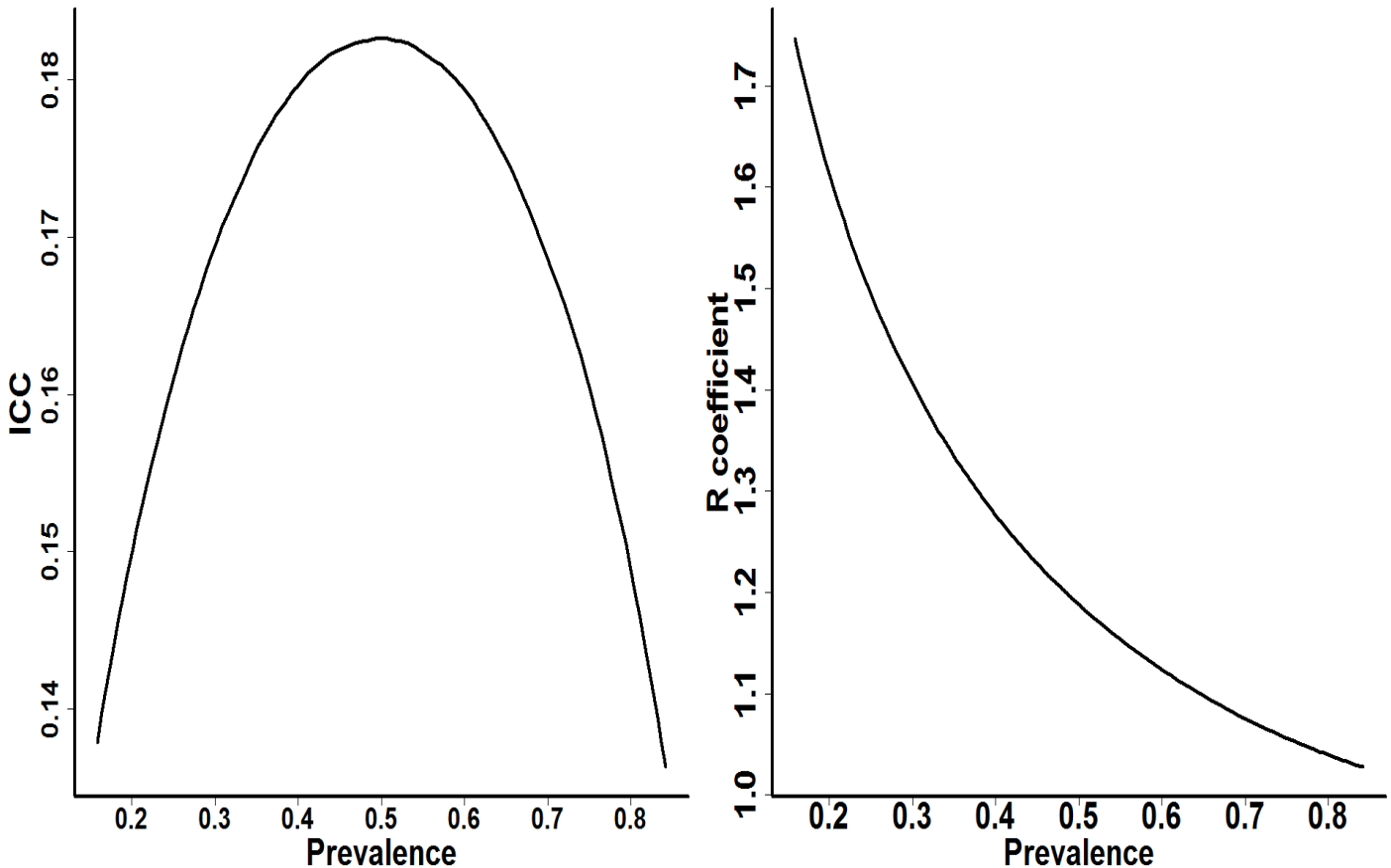


Figure: Mean ICC and R coefficient estimates for binary outcomes as a function of estimated outcome prevalence.

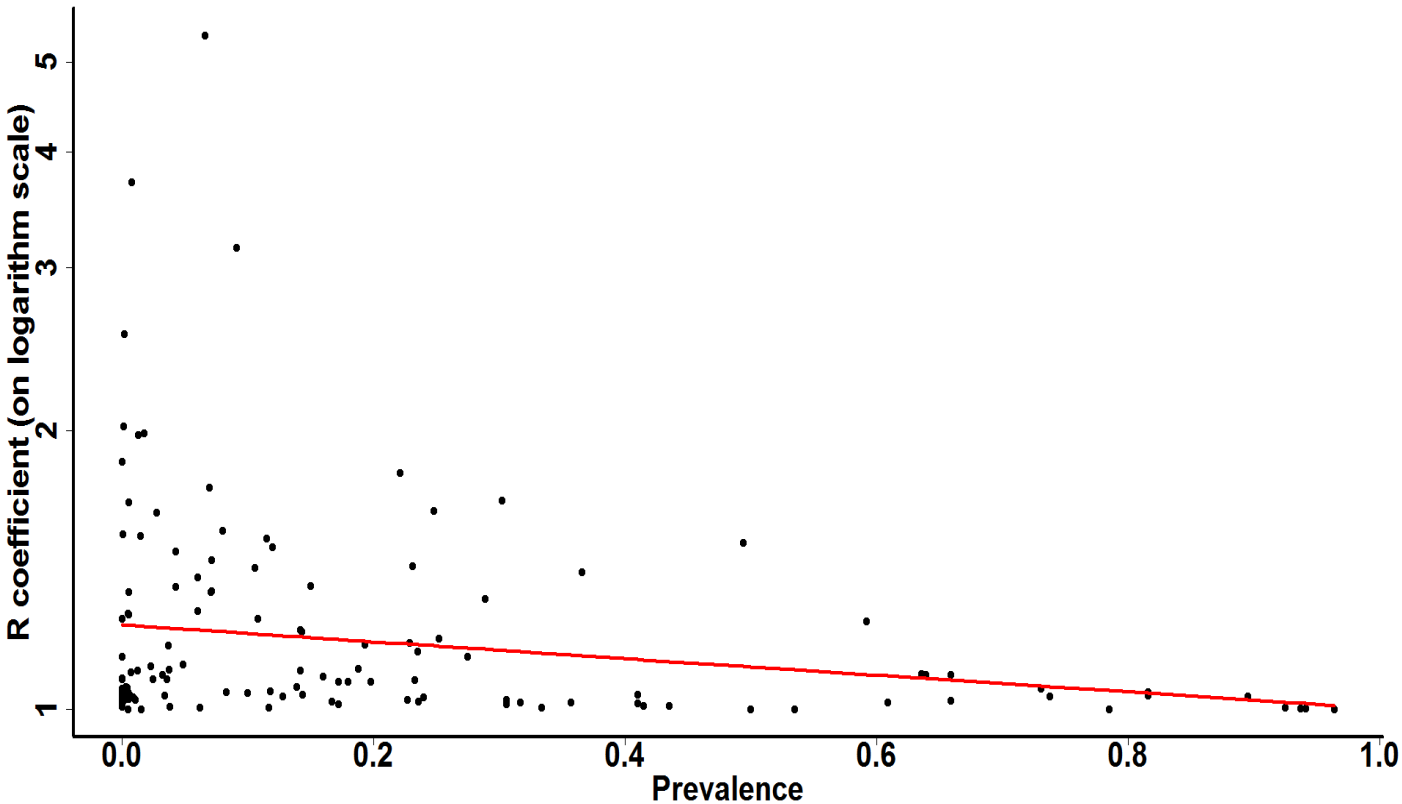
Plan

Data

- Data obtained from a Health Technology Assessment (HTA) review[7]
- 139 ICCs and prevalences for analysis

[7] Ukoumunne O. et al., *Health Technology Assessment*, 3(5):iii–92, 1999

Results



Association between R and prevalence with data from the Health Technology Assessment review.
The estimated slope coefficient was -0.208 and $p = 0.018$.

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- Clusters of size greater than 2: Simulations $\rightarrow$ R depends on prevalence

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- Clusters of size greater than 2: Simulations $\rightarrow$ R depends on prevalence
- As for the ICC, R coefficient depends on the outcome prevalence
- R coefficient is not the appropriate coefficient
- Future works needed

THANK YOU FOR YOUR ATTENTION