

Staircase cluster randomised trial designs

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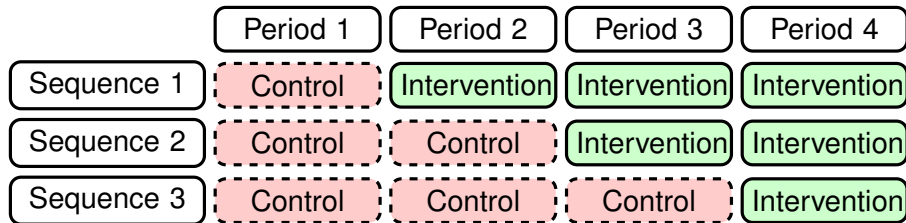
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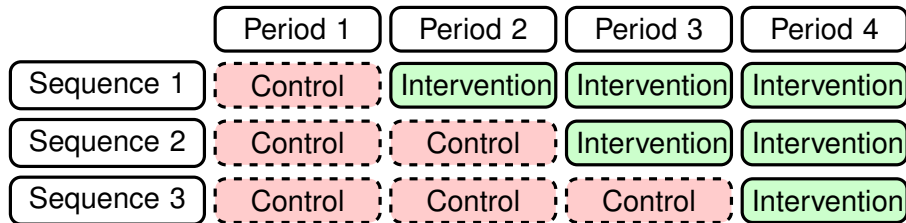


MONASH University

The usual stepped wedge



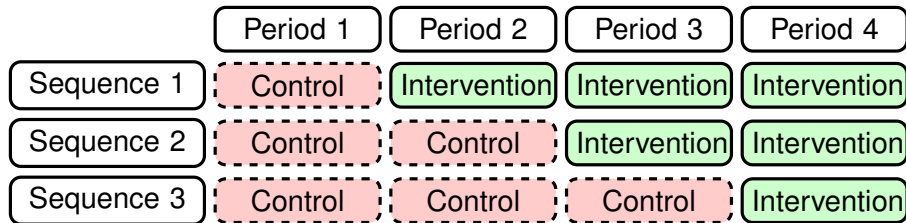
The usual stepped wedge



Stepped wedge designs are wonderful!

- All clusters know they will receive the intervention (eventually...);
- Useful when interventions cannot be undone or will be rolled out anyway.

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BUT...

What's wrong with the stepped wedge?

Stepped wedge designs can be burdensome and expensive.

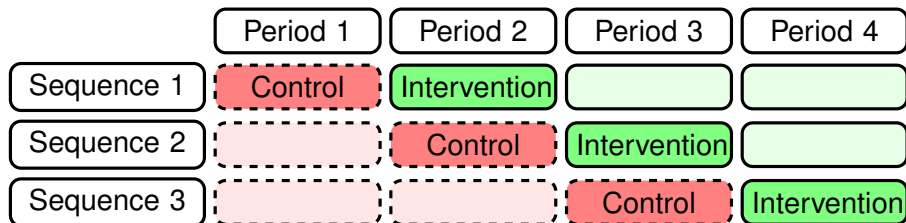
- Clusters must contribute data for a long time;
- As must patients, if followed up for multiple periods.
- So some important interventions may never even be tested!

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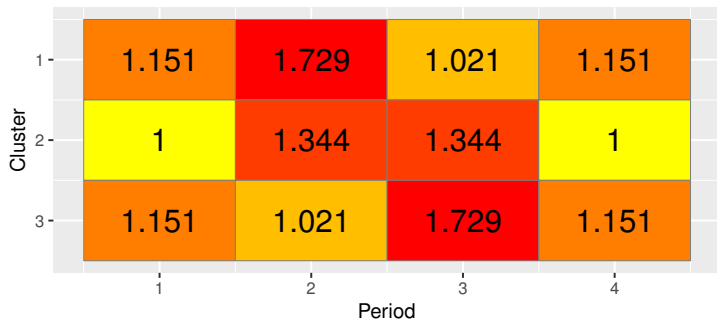
- Clusters must contribute data for a long time;
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Staircase designs: a cheaper, less burdensome alternative!



Why the staircase?

Consider how much “information” each sequence-period cell contributes to the estimation of the treatment effect¹

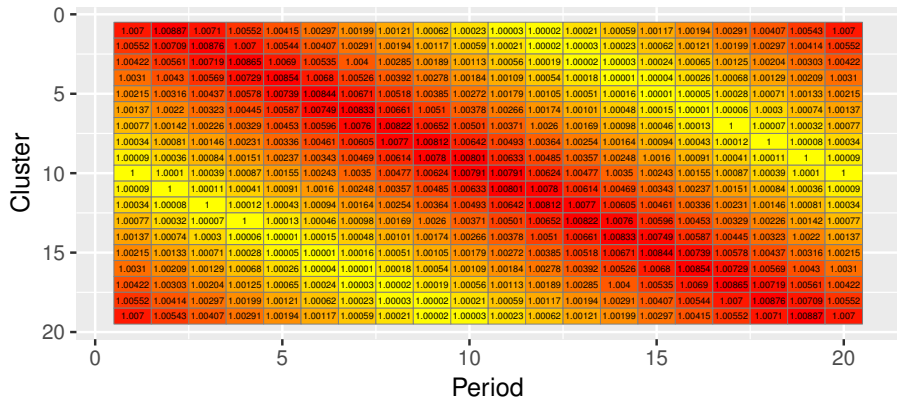


3-sequence stepped wedge

- The larger the number/redder the colour, the more information.
- Some cells contribute no information!

¹Kasza & Forbes, Information content of stepped wedge cluster-period cells, *Biometrics*, 2019

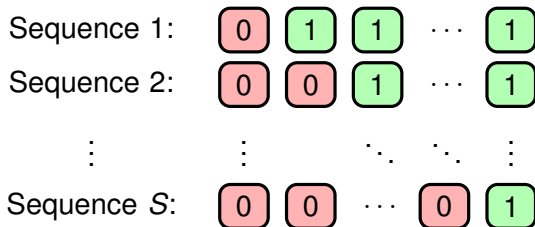
Information content of SW sequence-period cells



19-sequence stepped wedge

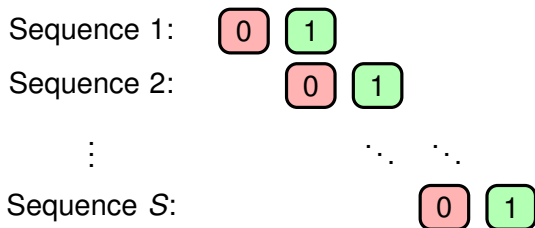
Staircases embedded in stepped wedges

- Each stepped wedge contains a staircase...



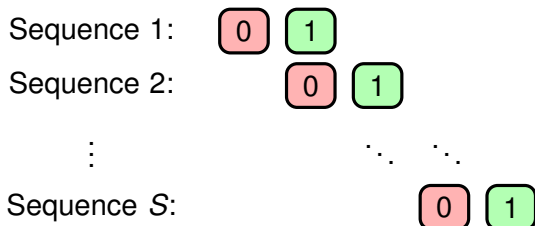
Staircases embedded in stepped wedges

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Staircases embedded in stepped wedges

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How much information do we lose when we only consider the staircase?

Staircases versus stepped wedges

$var(\hat{\theta})$: variance of the treatment effect estimator

$$\frac{var(\hat{\theta})_{SC}}{var(\hat{\theta})_{SW}} = \frac{\text{Staircase design}}{\text{Stepped wedge design}}$$

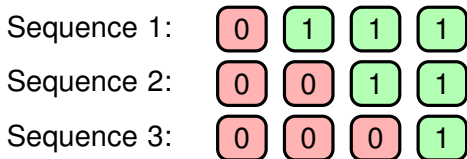
- Continuous outcomes, 100 subjects in each cluster in each period.
- For two subjects in the same cluster:

$$corr(\text{measured in same period}) = \rho$$

$$corr(\text{measured in different periods}) = r\rho$$

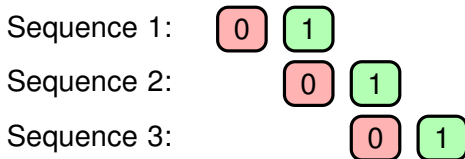
- Within-period intra-cluster correlation $\rho = 0.005, \dots, 0.50$;
- cluster auto-correlation $r = 0.50, \dots, 0.99$.

3-sequence SW and SC



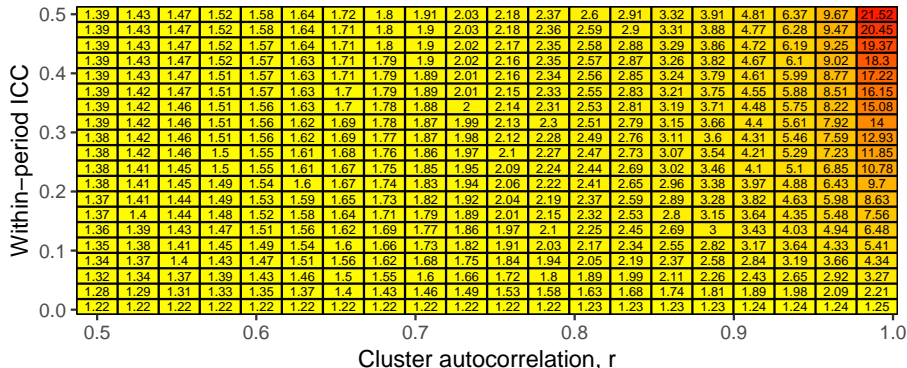
Stepped wedge: 12 cluster-period cells
Staircase: 6 cluster-period cells.

3-sequence SW and SC

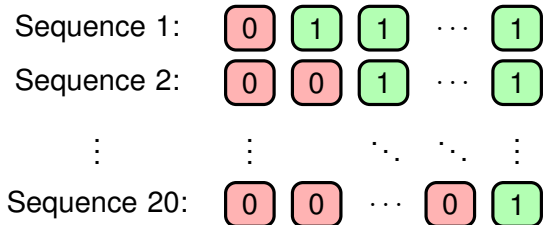


Stepped wedge: 12 cluster-period cells
Staircase: 6 cluster-period cells.

3 sequences, constant between-period ICC



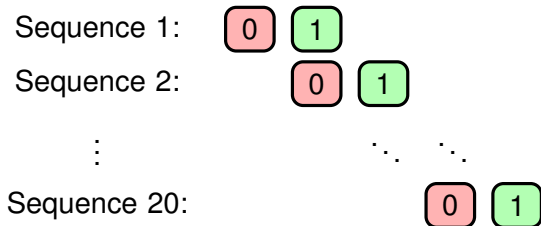
20-sequence SW and SC



Stepped wedge: 420 cluster-period cells

Staircase: 40 cluster-period cells.

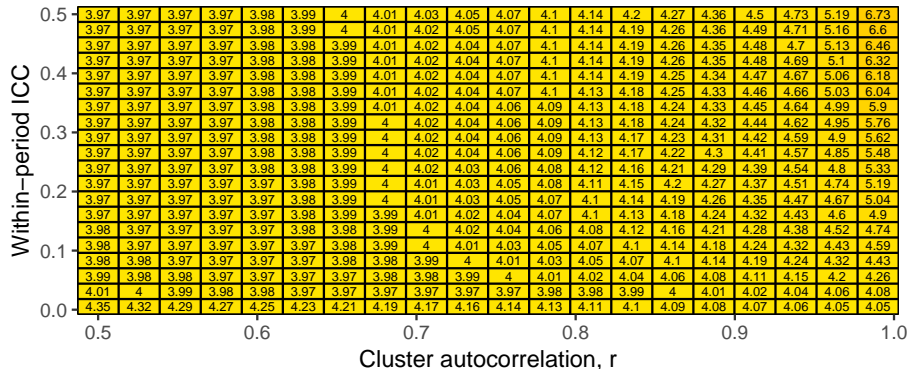
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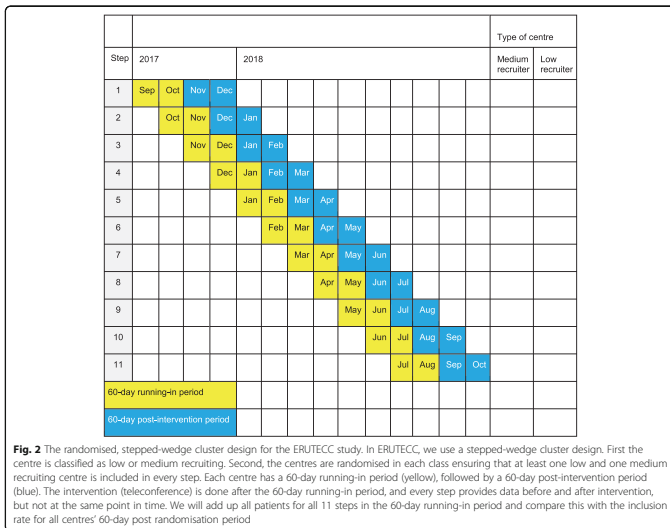
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Staircase vs. Stepped wedge

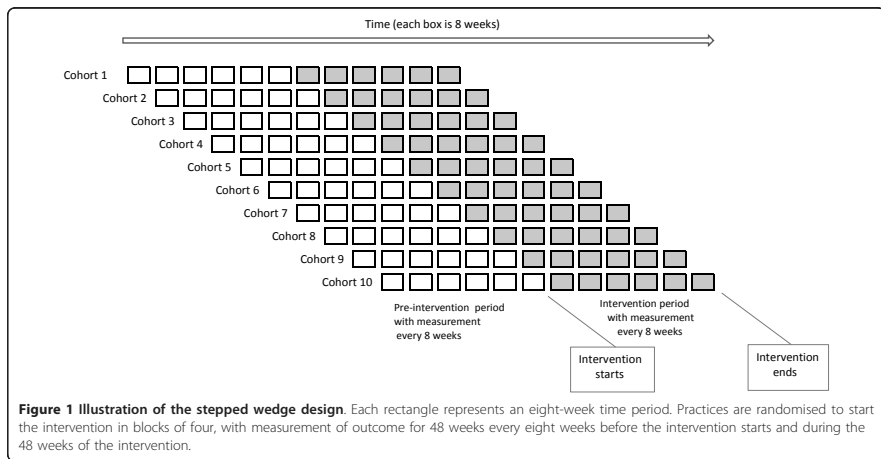
- Can lose a lot of information about the treatment effect
 - Why? Stepped wedge “hot spots”.
- Loss can be out of proportion to the reduction in the number of measurements.
 - 3 sequence design:
50% of the measurements, up to $20\times$ increase in variance.
 - 20 sequence design:
10% of the measurements, but only up to $7\times$ increase.

Staircase designs in practice?



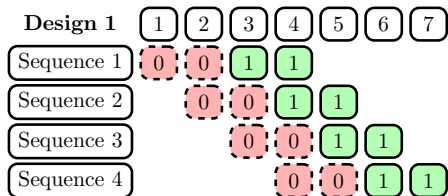
Lundström et al, Enhancing Recruitment Using Teleconference and Commitment Contract (ERUTECC): study protocol for a randomised, stepped-wedge cluster trial within the EFFECTS trial. *Trials*. 2018.

Another staircase design...

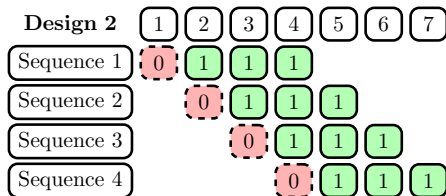
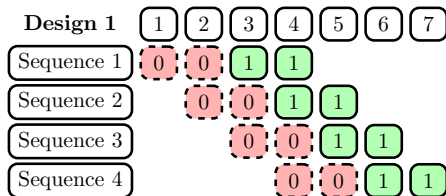


Dreischulte et al, A cluster randomised stepped wedge trial to evaluate the effectiveness of a multifaceted information technology-based intervention in reducing high-risk prescribing of non-steroidal anti-inflammatory drugs and antiplatelets in primary medical care: The DQIP study protocol. *Trials*, 2012.

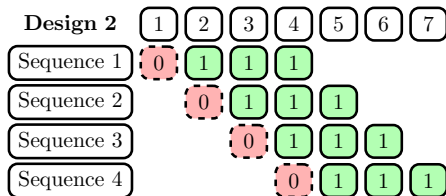
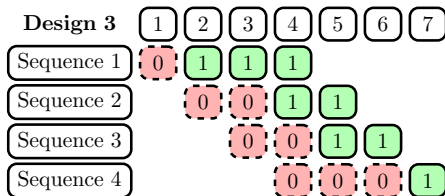
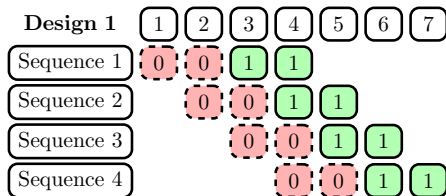
Which “staircase-type” design is most efficient?



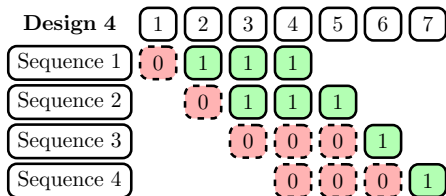
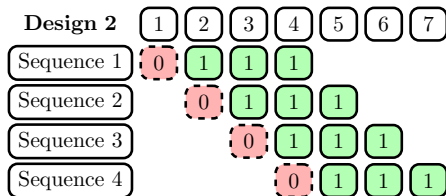
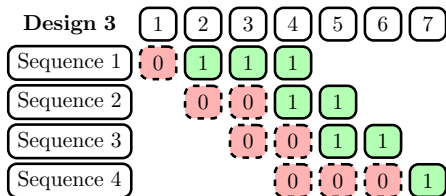
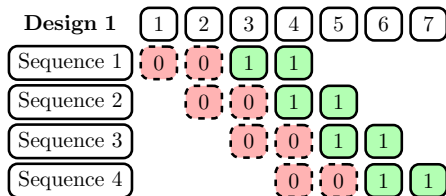
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Which staircase design is most efficient?

$var(\hat{\theta})$: variance of the treatment effect estimator

Calculate this for each of the 4 designs.

- Continuous outcomes, 100 subjects in each cluster in each period.
- Within-cluster correlation structure:
 - **Constant correlations:** for two subjects in the same cluster

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- **Decaying correlations:** for two subjects in the same cluster

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$$corr(\text{measured in period } t \text{ and } s) = r^{|t-s|}\rho$$

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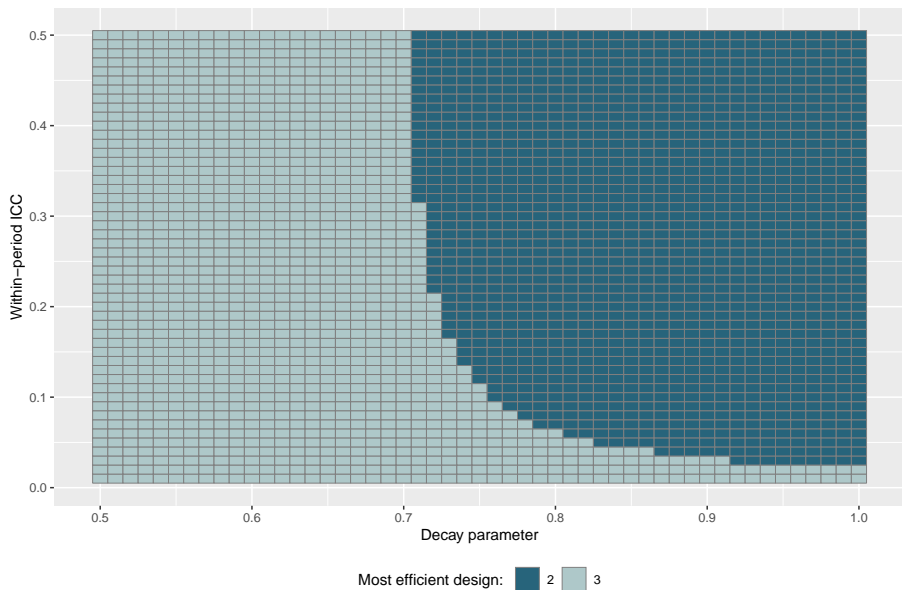
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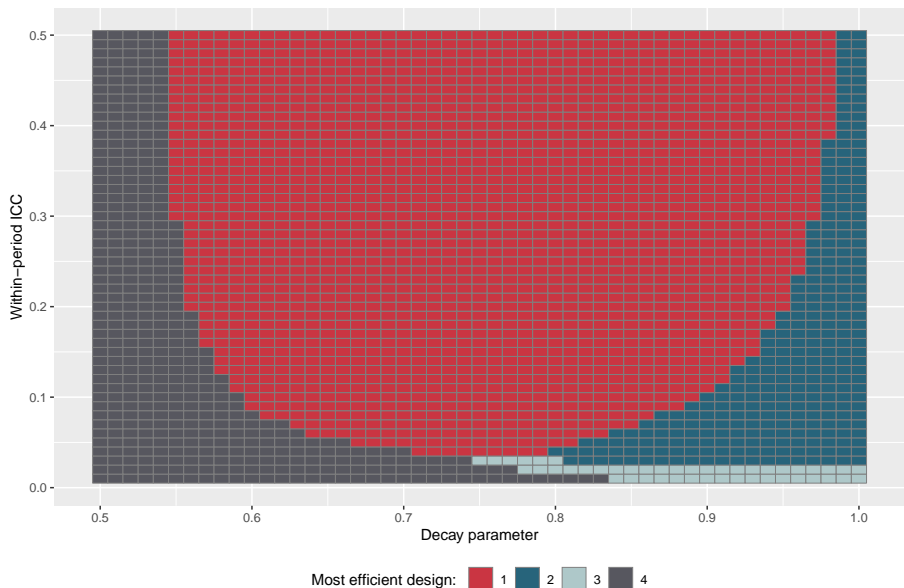
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Most efficient design; constant correlations



Most efficient design; decaying correlations



Staircase designs: take home messages

- Staircase designs:
 - Less efficient than stepped wedge designs;
 - But far less burdensome for participating clusters.
- Researchers **are already using** staircase designs!
 - Despite minimal research into their properties.
 - Tendency for *multiple* pre- and post-switch periods.
 - **BUT** a balanced number of pre- and post-switch periods is not always best!

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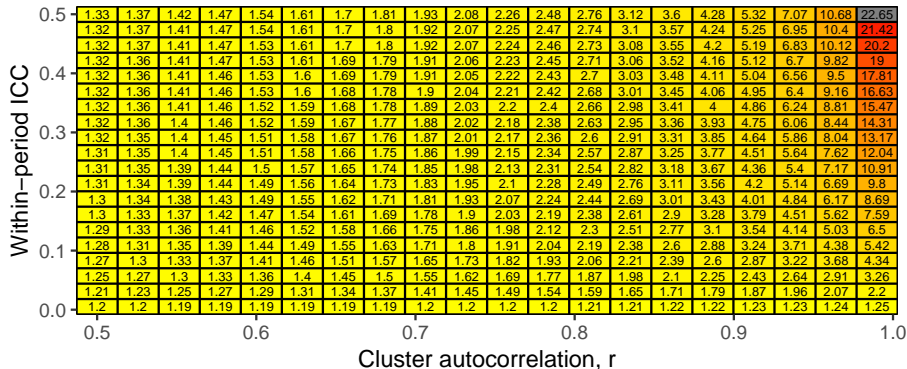
Explore our results at:

<https://monash-biostat.shinyapps.io/StaircaseDesign/>

Future work:

- When is it possible to re-organise the observations in a stepped wedge design to come up with a **more efficient staircase**?
- Are there alternative incomplete stepped wedge designs that are better choices?

3 sequences, decaying between-period ICC



20 sequences, decaying between-period ICC

