The value of observational causal inference for medical decision making

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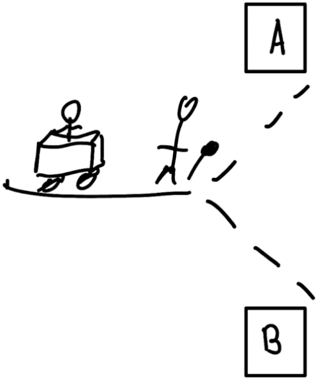
Medical decisions optimize for patient outcomes under interventions

Medical decisions are interventions (treatments) that change the outcome of a patient

Medical decisions optimize for patient outcomes under interventions

Need expected outcome under intervention (prediction under intervention)



Medical decisions optimize for patient outcomes under interventions

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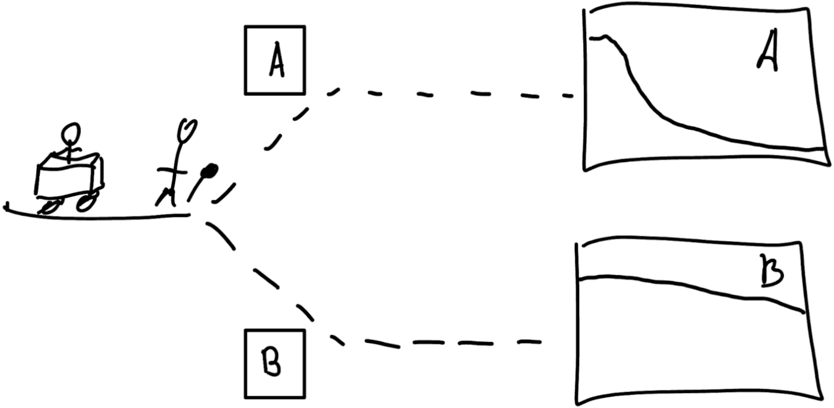

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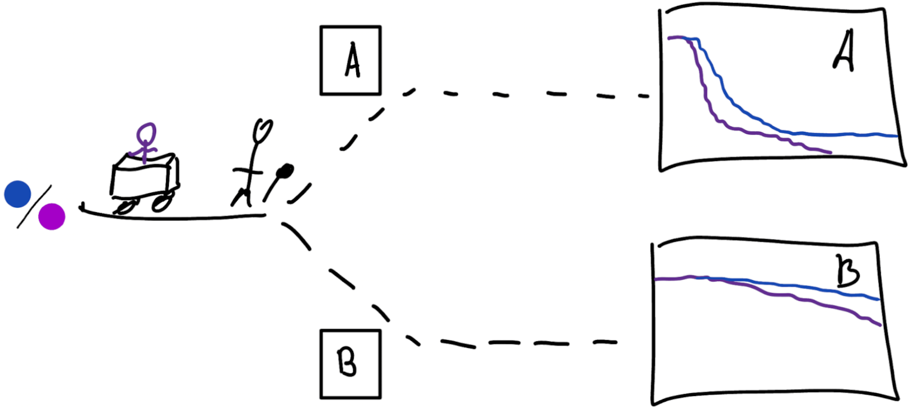

How to estimate treatment effects?

Treatment effect estimation requires unconfoundedness

Which generally does not hold in observational data

$\boxed{\text{concurrent vs sequential}}$

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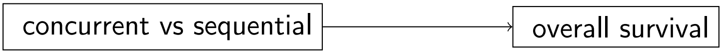

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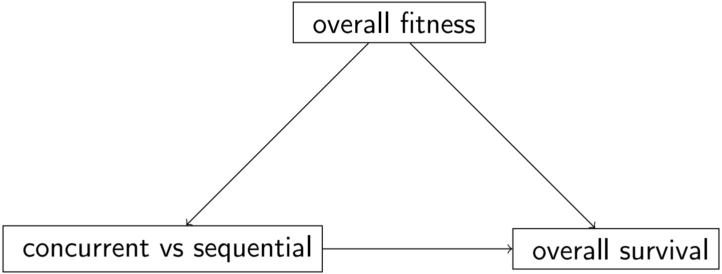

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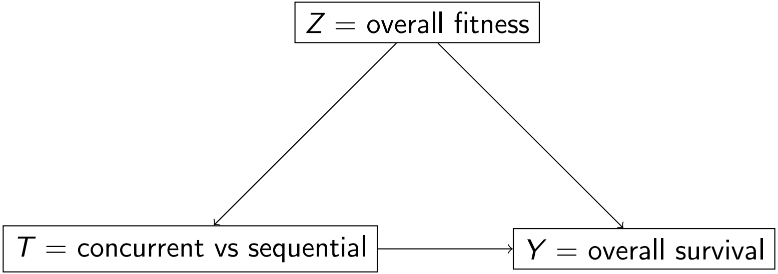

$$Y|do(T) \neq Y|T$$

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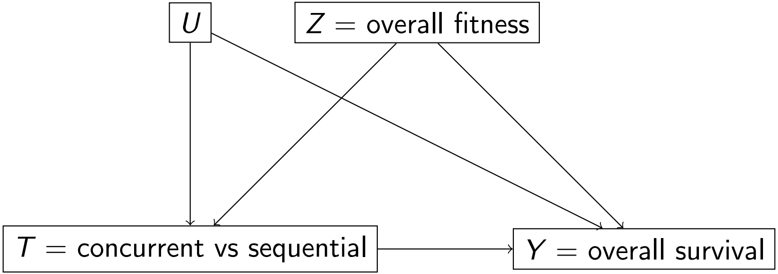

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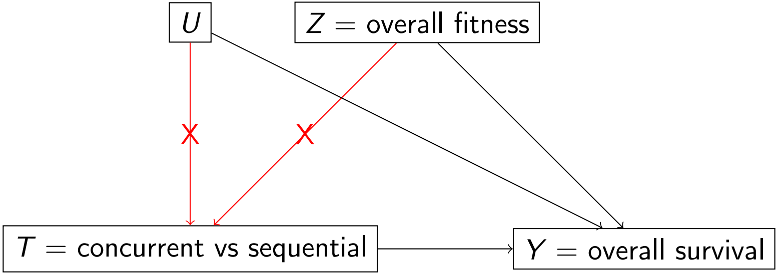
Randomized controlled trials gaurantee unconfoundedness

Thus allow for causal inference



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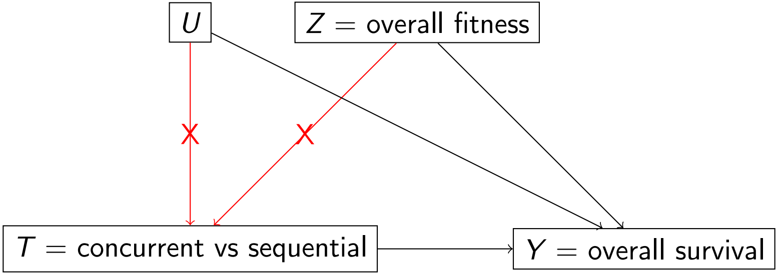

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\text{Conditional Average Treatment Effect} \quad & \mathbb{E}\, Y|do(T=A), X=x - Y|do(T=B), X=x
\end{aligned}$$

Randomized controlled trials can estimate any interventional parameter

But generally only measure average treatment effect *often on a relative scale*

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Problems with RCTs for medical decision making

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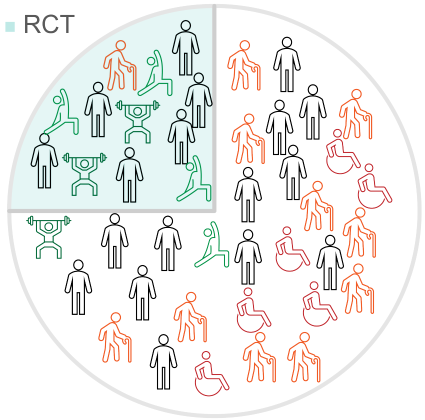
RCTs are conducted in non-random samples of the population so transportability assumptions are needed



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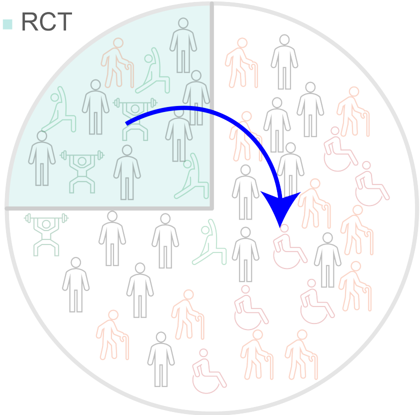


e.g. transportability of risk ratio (RR):

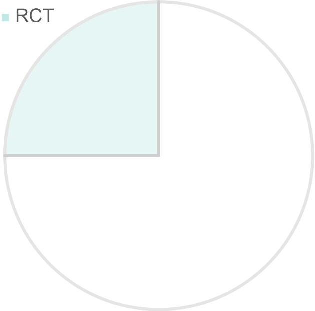
$$RR(\text{pop.} = \text{RCT}) = RR(\text{pop.} = \text{clinical})$$

Trading one unverifiable assumption (unconfoundedness) for another (transportability)

Problems with RCTs for medical decision making

Suspected treatment effect heterogeneity, but RCT too small to measure and lack of equipoise

- RCT establishes average treatment effect



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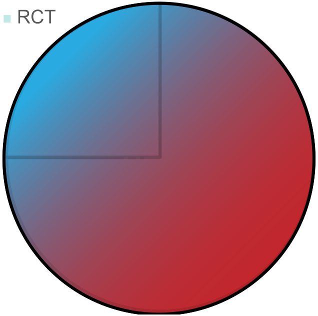

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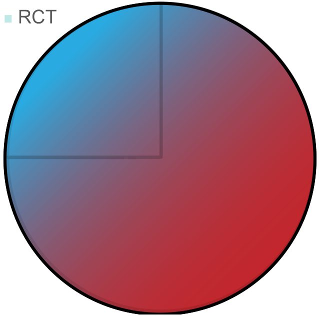

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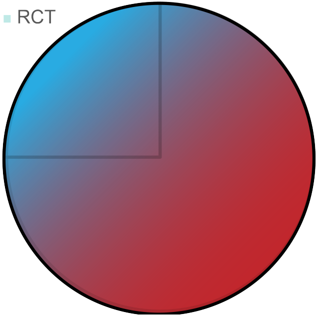

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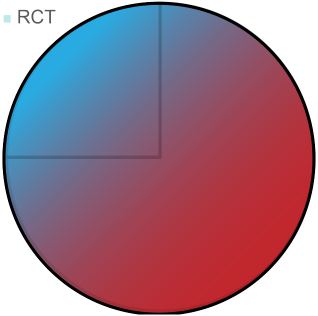

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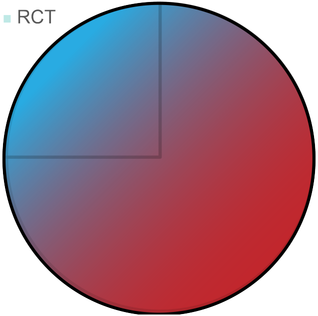
- Need preliminary evidence from observational studies (W. A. C. v. Amsterdam et al. 2022)



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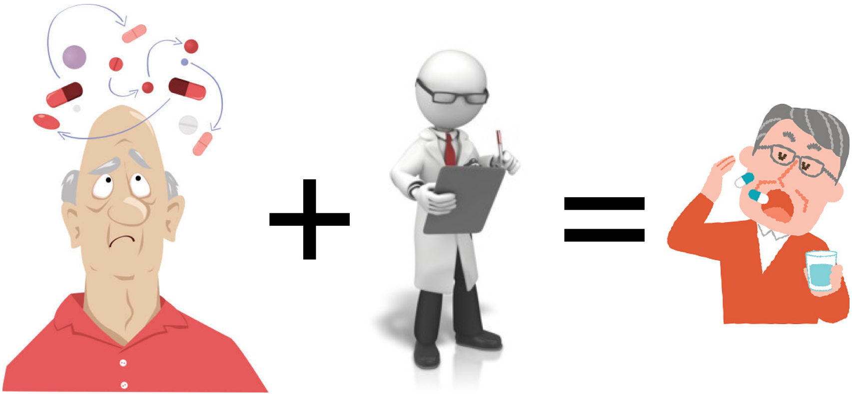

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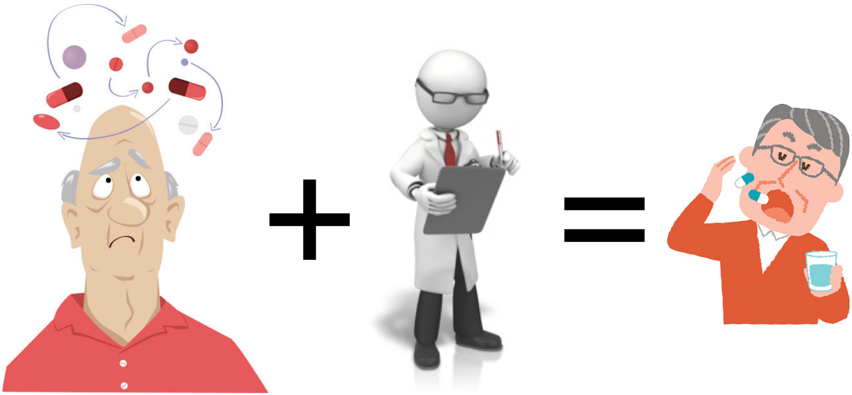

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- Trading one unverifiable assumption (unconfoundedness) for another (consistency)

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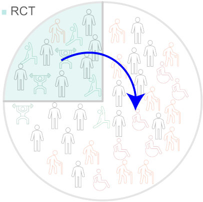
RCTs solve confounding but have their own limitations



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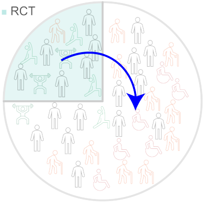

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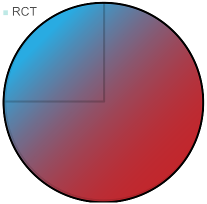

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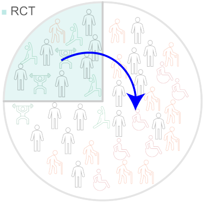

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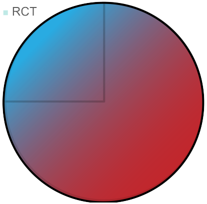

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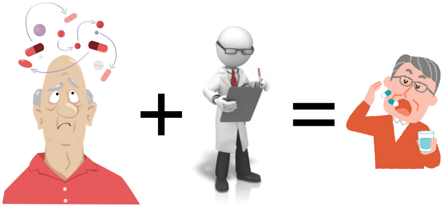

Consistency

How can observational causal inference help medical decision making?

How observational causal inference can support medical decision making

Constant relative treatment effect + varying untreated risk = varying treatment effect

- Transportability assumption needed: most often on *relative scale*

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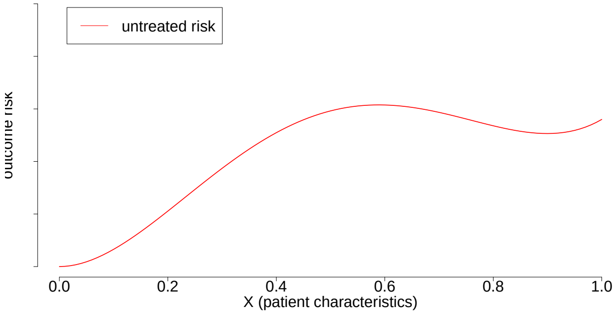

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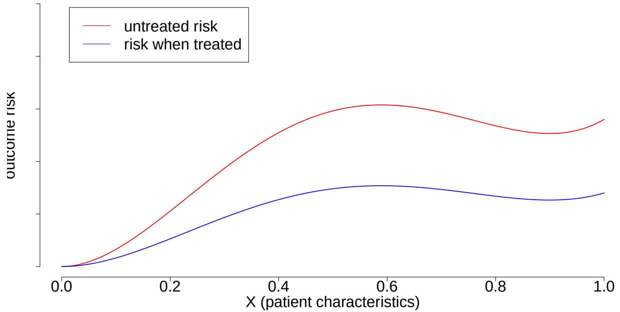

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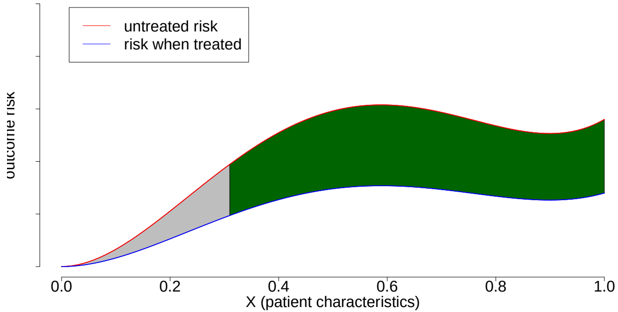

How observational causal inference can support medical decision making

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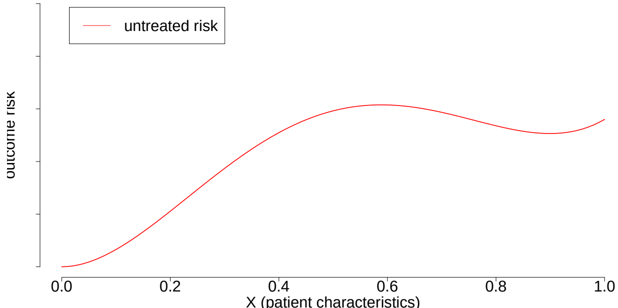

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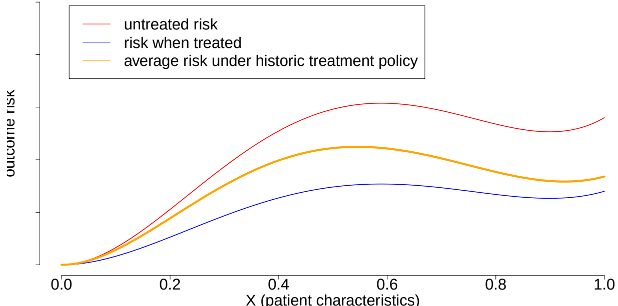

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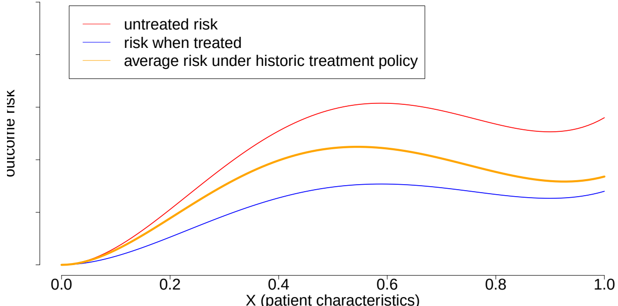

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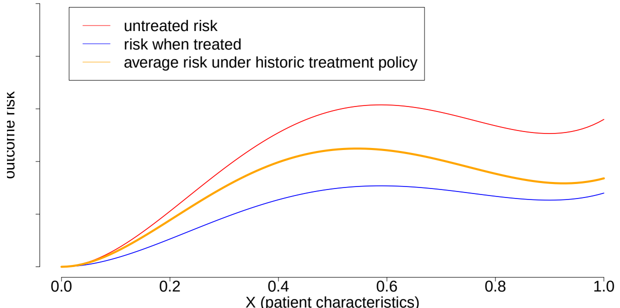
- Need better 'causal' methods (Amsterdam et al 2023)



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- Observational data may improve statistical efficiency of RCTs
  - RCT and observational data share* outcome mechanism
  - Need less randomized patients when using both data sources (Rosenman et al. 2020; Ilse et al. 2023)

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Observational Causal Inference and RCTs interplay

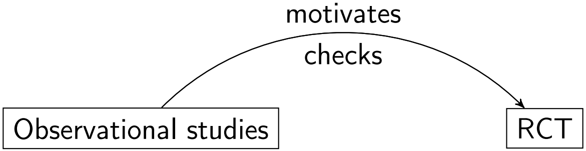
Observational studies

RCT

Observational Causal Inference and RCTs interplay

$$\begin{tikzcd}
\boxed{\text{Observational studies}} \arrow[rr, bend left, "\text{motivates}"] & & \boxed{\text{RCT}}
\end{tikzcd}$$

Observational Causal Inference and RCTs interplay



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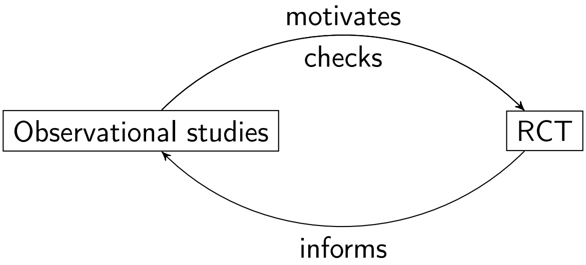
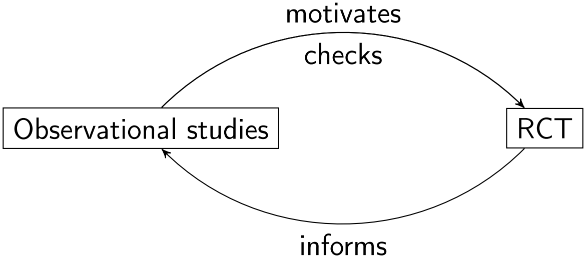


Observational causal inference and RCTs for medical decision making



Must not abandon RCTs,
observational causal inference can be of great value for medical decision making

References

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