The KIDS RESEARCH INSTITUTE AUSTRALIA

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How significant is (statistical) significance?

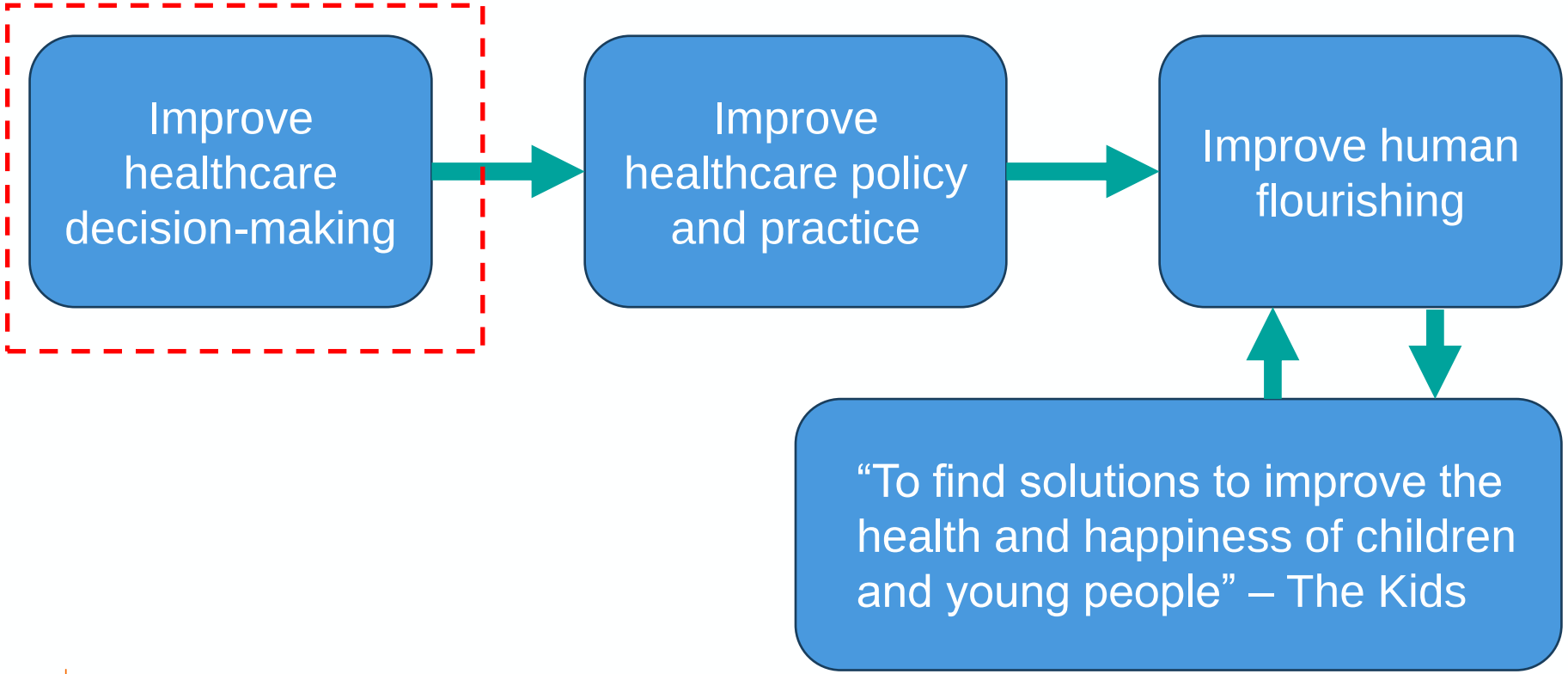
You’re in luck!

This is more like a philosophy of science talk

My intention is to provoke thought (and hopefully discussion)

(You may still prefer to nap, be my guest)

Why do medical research? (My philosophy)



Important concepts: How can I be wrong?

- Research Question: “Does the drug cure the disease?”
- There are four possible scenarios
- Statistical significance = Type One Error tolerance

		Does the drug cure the disease?	
		Yes	No
Did I declare that the drug cures the disease?	Yes	Power	Type One Error
	No	Type Two Error	

Medical research through a statistical lens

- A scientific hypothesis is distilled into a statistical hypothesis test
- E.g., **A**: treatment has **no** effect vs **B**: treatment has a **positive** effect
- Scientists (humans) want to make a declaration: **A** is true, or **B** is true
  - This is a **truth claim**. It may be wrong. E.g., type one and two errors
  - “Power” a study to detect a “clinically meaningful effect” with a sufficiently large “sample size”
  - They crave a significant p-value to declare the treatment works

Does this even make sense?

- This approach originated in crop trials almost a century ago
  - Does it make sense for medical research? (Think of the flow chart)
- Does a significant p-value for a single (primary) outcome influence decision-making? No! (Or well, it shouldn’t)
- A decision-maker will consider multiple outcomes (e.g., efficacy and toxicity) and cares about the size of the effects (and their uncertainty)!

The role of science in the pursuit of truth

- Admittedly, I am a truth supremacist (it is my quasi-religion)
- “Science” is a tool we can use to uncover truths about the universe
- Therefore, it is **good** to do science to better understand phenomena
- It will lead to improved healthcare decision-making and human flourishing
- But! We have finite resources!
- We should pursue the **right** amount of truth to the **right** questions

How?

- Bayesian decision-theory and health economics
- Specify the decision-making process **explicitly**
- Collect data to reduce parameter uncertainty to reduce decision uncertainty
- Stop collecting data once the cost of data collection exceeds the incremental value to the decision-making process
- Our research should **directly** inform healthcare decision-making

What am I supposed to do now? I still need to do my research!

Statistical significance is no longer significant?!

Dammit, I should have taken that nap when he offered!

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*This was an 8-minute talk: terms and conditions apply