

My Hardest Lessons Learned About Clinical Trials

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NINDS Clinical Trials Methodology Course

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Ask the right questions

Anticipate regrets

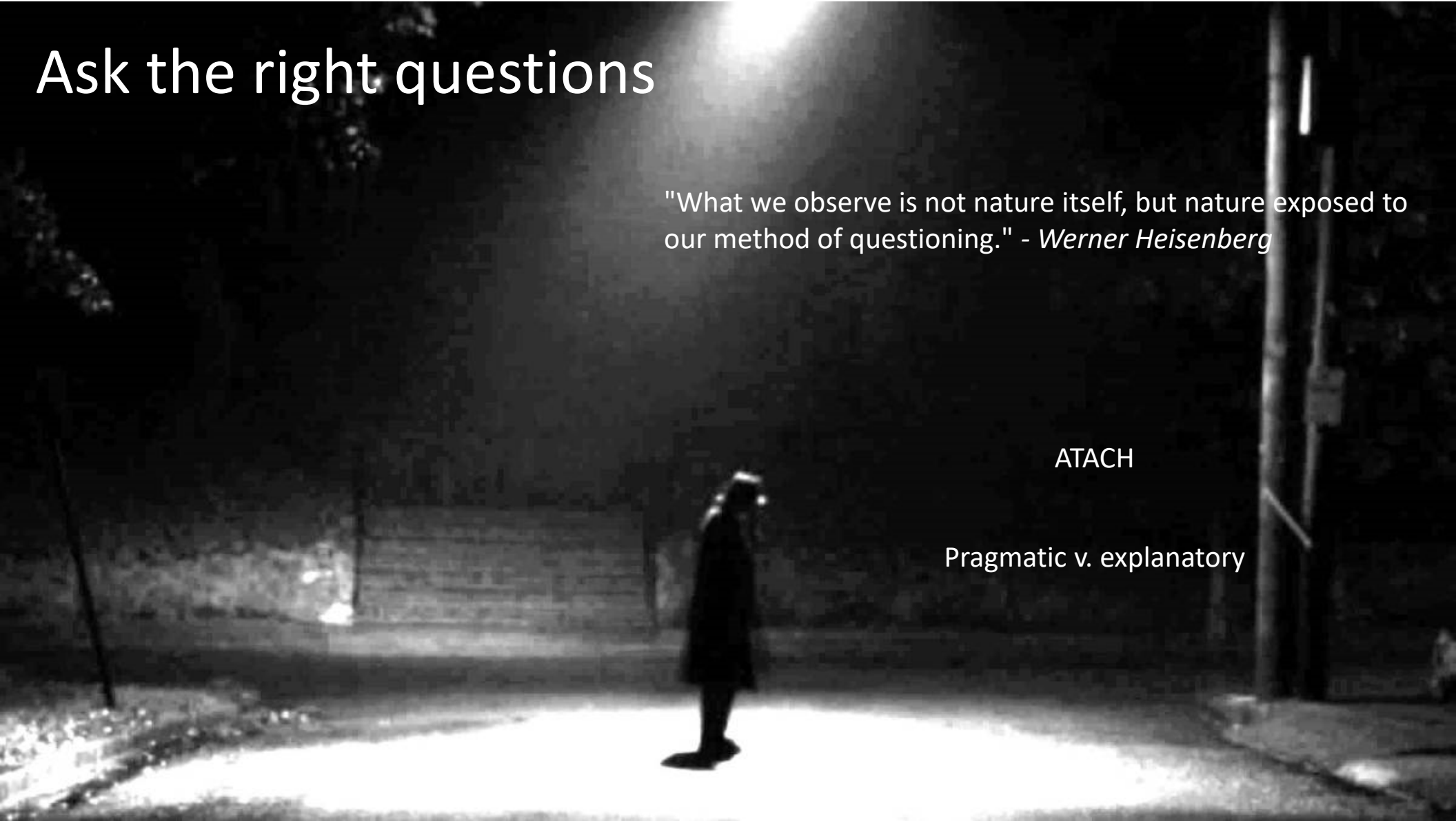
Bidirectional translation

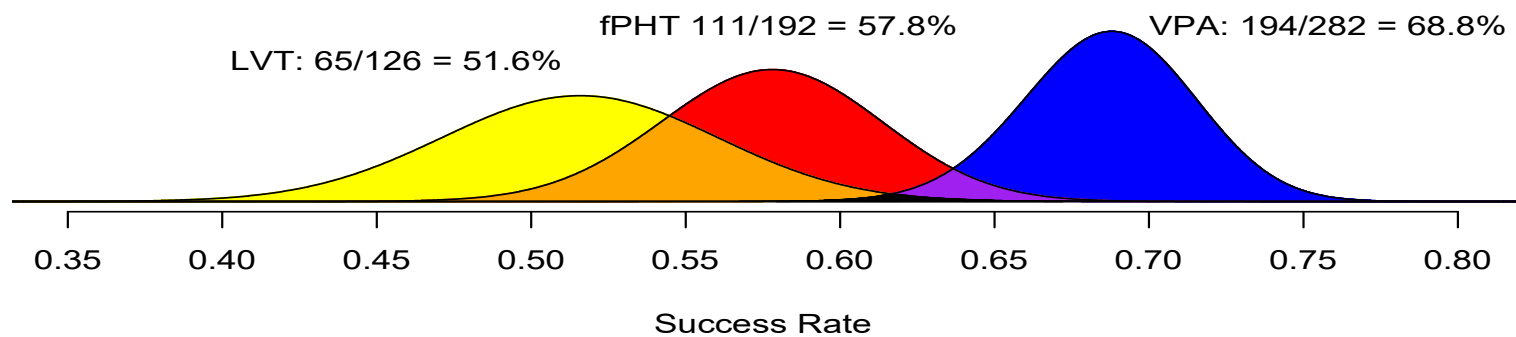
Ask the right questions

"What we observe is not nature itself, but nature exposed to our method of questioning." - *Werner Heisenberg*

ATACH

Pragmatic v. explanatory





Treatment	Observed	%	95% CI	Pr(Best)	Pr(Worst)
LVT	65/126	51.6%	(.429, .601)	0.0005	0.862
fPHT	111/192	57.8%	(.507, .646)	0.007	0.138
VPA	194/282	68.8%	(.632, .739)	0.992	0.0005

Difference	Observed	95% CI	Pairwise Comparison
VPA – fPHT	0.110	(0.022, 0.197)	Pr(VPA>fPHT) = 0.993
VPA – LVT	0.172	(0.069, 0.272)	Pr(VPA>LVT) > 0.999
fPHT - LVT	0.062	(-0.049, 0.172)	Pr(fPHT>LVT) = 0.862

Anticipate regrets

Optimism

WITH ALL THIS MANURE
THERE'S GOT TO BE
A PONY IN HERE
SOMEWHERE!





Irrational Exuberance

I've got a rule in mind that can describe a series of numbers. I will tell you two numbers in the series, then you will ask me about sequential numbers, and I'll tell you whether or not they fit the rule. We'll see how many numbers you need to check to discover the rule. Ok?

Two and then four.

Yes.

Yes.



Ok.

Eight?

Sixteen?



I know the rule. It is the powers of 2.

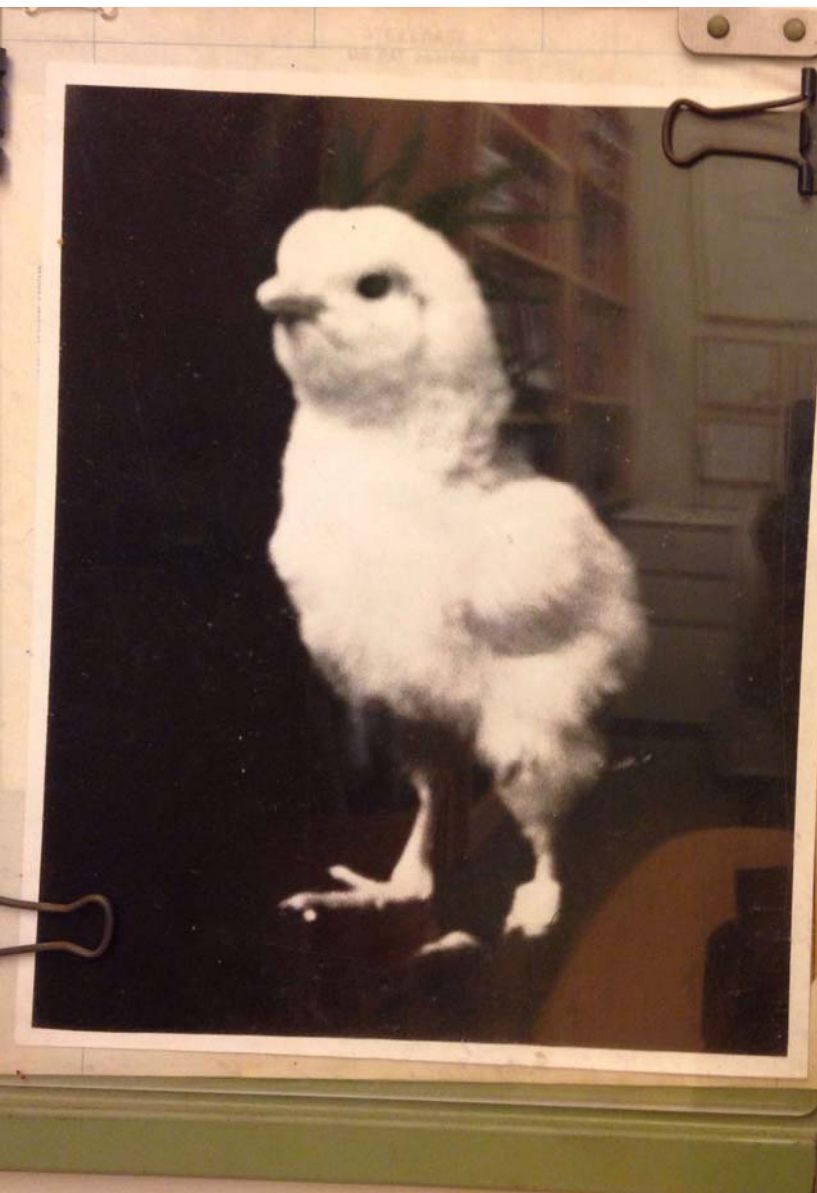
Actually, no, the rule is any sequentially increasing number.

Anticipate regrets

Adaptive Trials

Better use of Learning
Phase trials





Better translation



Ask the right questions

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