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Oncology
Special Interest Group

ISSUE PANEL

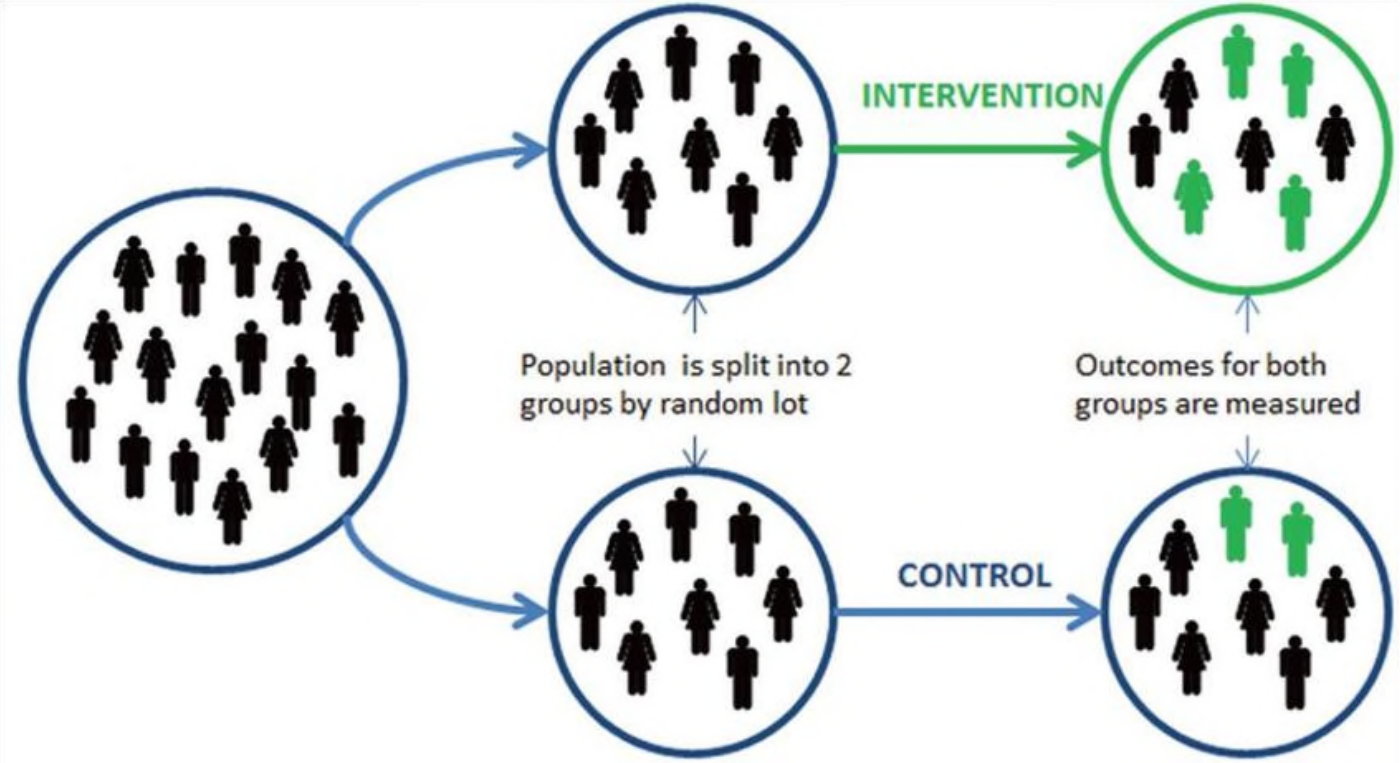
Clinical Evidence for Health Technology Assessment in Oncology, Are We Going Backwards? Where Are We Going With Single-arm Trials?

A patient perspective

Anne-Pierre Pickaert

Tuesday, 8 November 2022 | 10:15-11:15

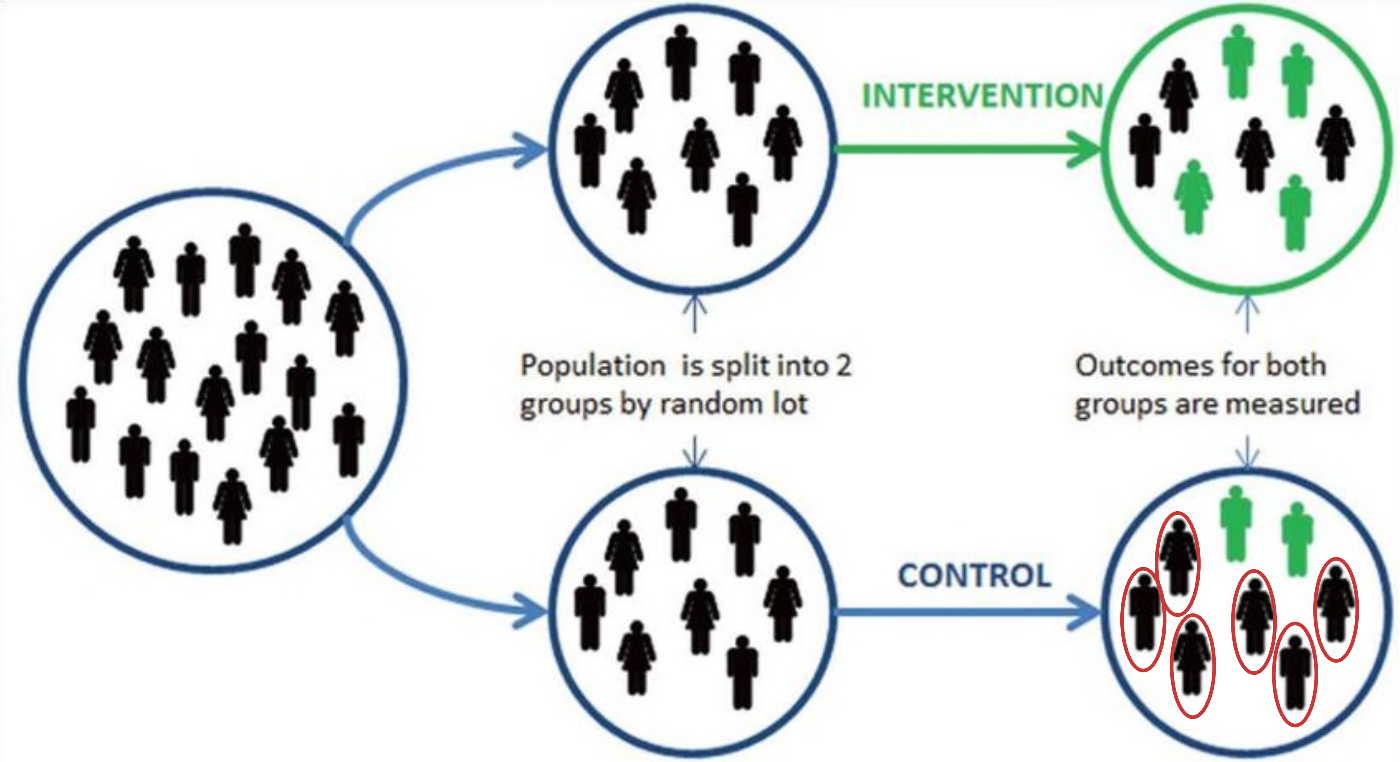
Randomised controlled trials



Survived

Illustration: https://www.researchgate.net/figure/The-basic-design-of-a-randomised-controlled-trial-adapted-from-Haynes-Service-Goldacre_fig2_318153807

Randomised controlled trials



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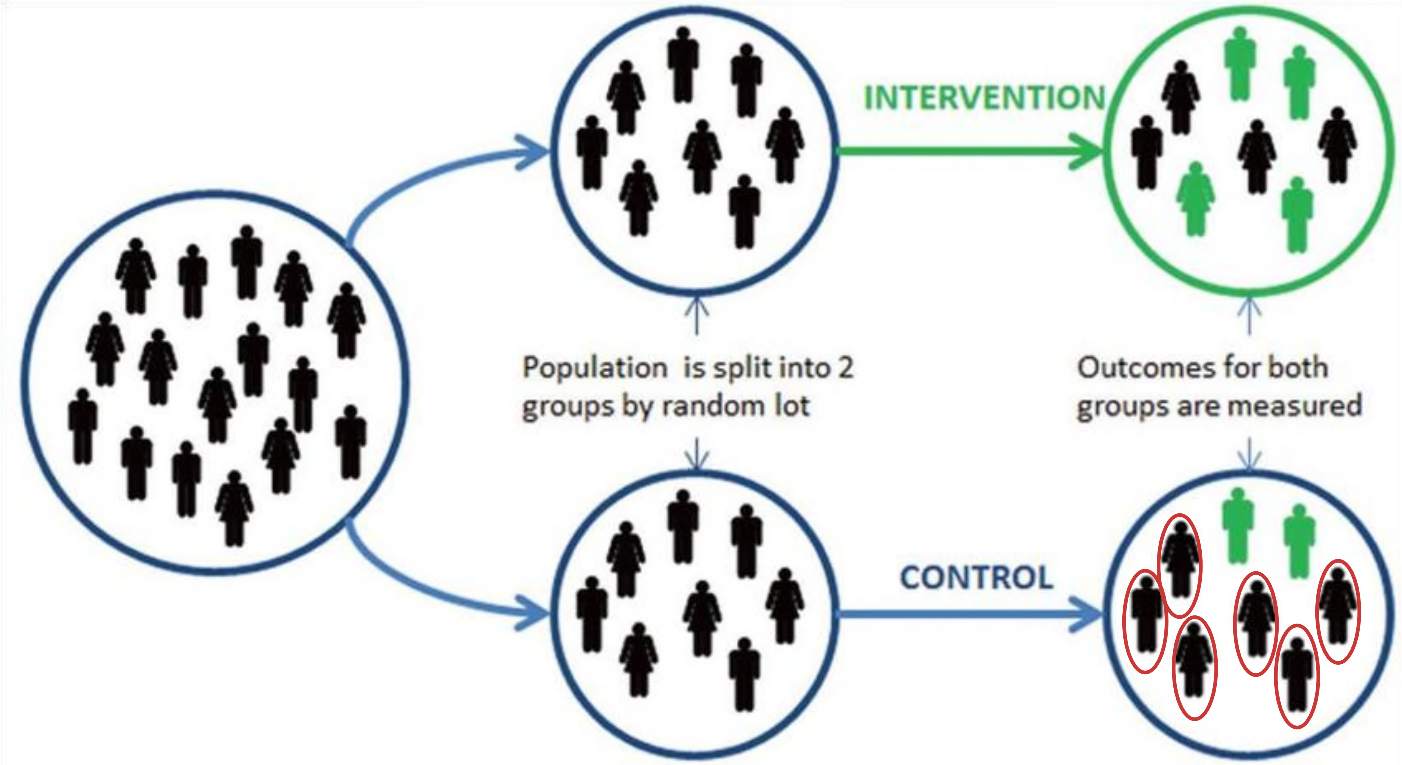
Patients prefer non-randomised controlled trials



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Patients prefer non-randomised controlled trials

- Some RCTs are unethical when the control arm isn’t of any help

Illustration: https://sloanreview.mit.edu/article/ethics-or-compliance-in-a-crisis/

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- Cancer patients do not enter a clinical trial in the hope of receiving placebo + standard of cancer

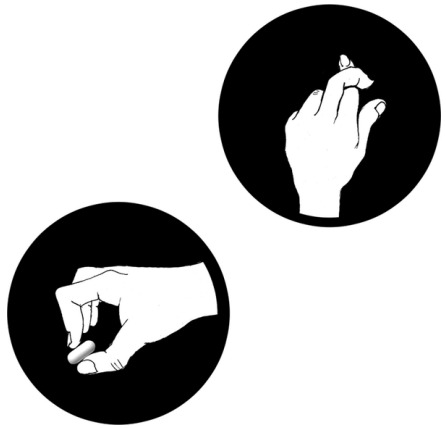

Illustration:
https://journals.plos.org/plosmedicine/article?id=10.1371/journal.pmed.0020262

Patients prefer non-randomised controlled trials

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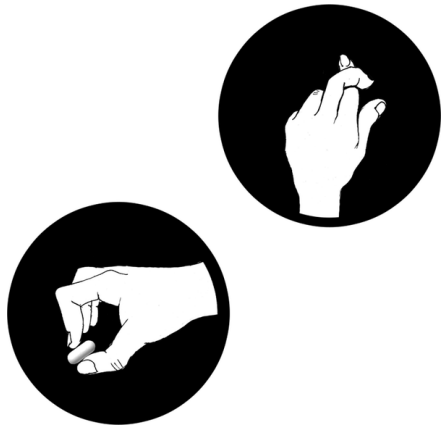

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- Most patients are altruistic.

Illustration: https://www.genesisdx.com/news-forum/blog/help-others/

Patients prefer non-randomised controlled trials

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- Most patients are altruistic. But they also make sure they look after themselves before helping others.

Illustration: https://uxdesign.cc/make-sure-your-own-project-will-have-stellar-ux-before-helping-others-1cca43af71ba

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- Cancer patients enter a trial to receive a treatment that would otherwise not be available to them
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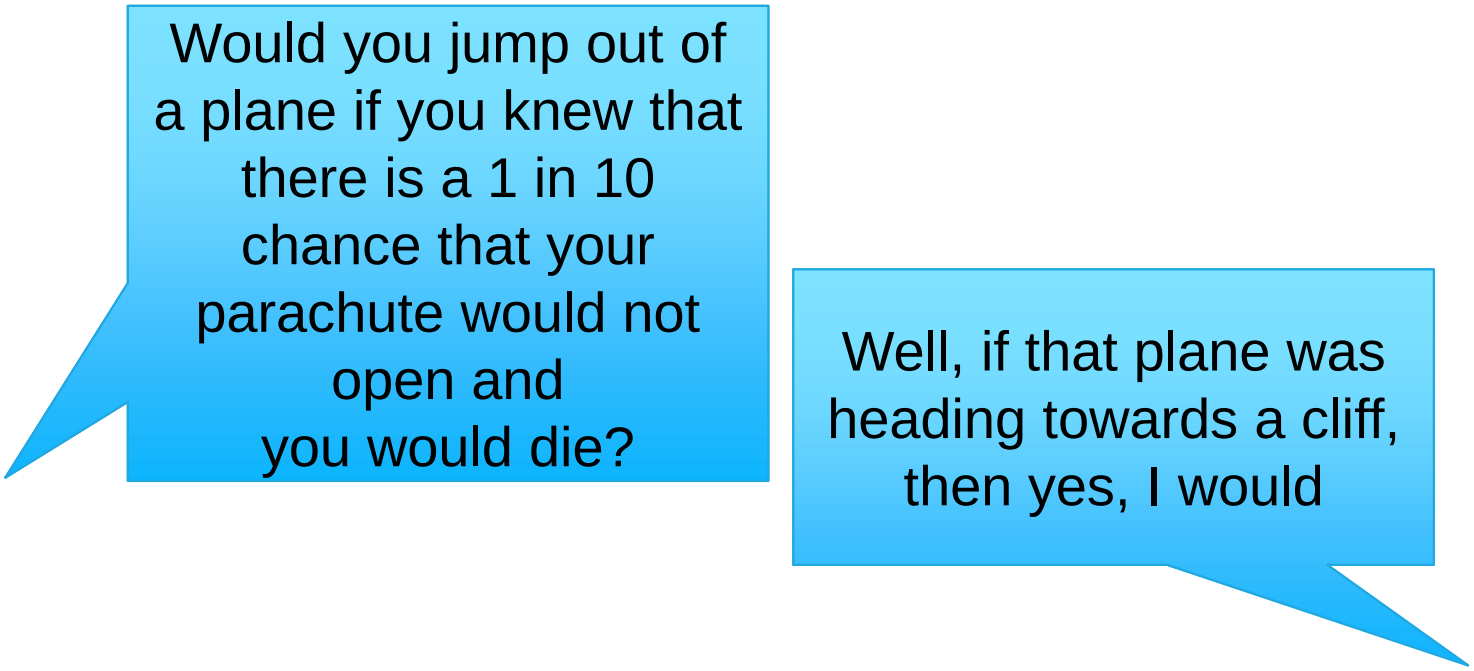
The experimental treatment may be their only source of hope.

Illustration: http://www.starsofhope.org/website/

Would you jump out of a plane if you knew that there is a 1 in 10 chance that your parachute would not open and you would die?

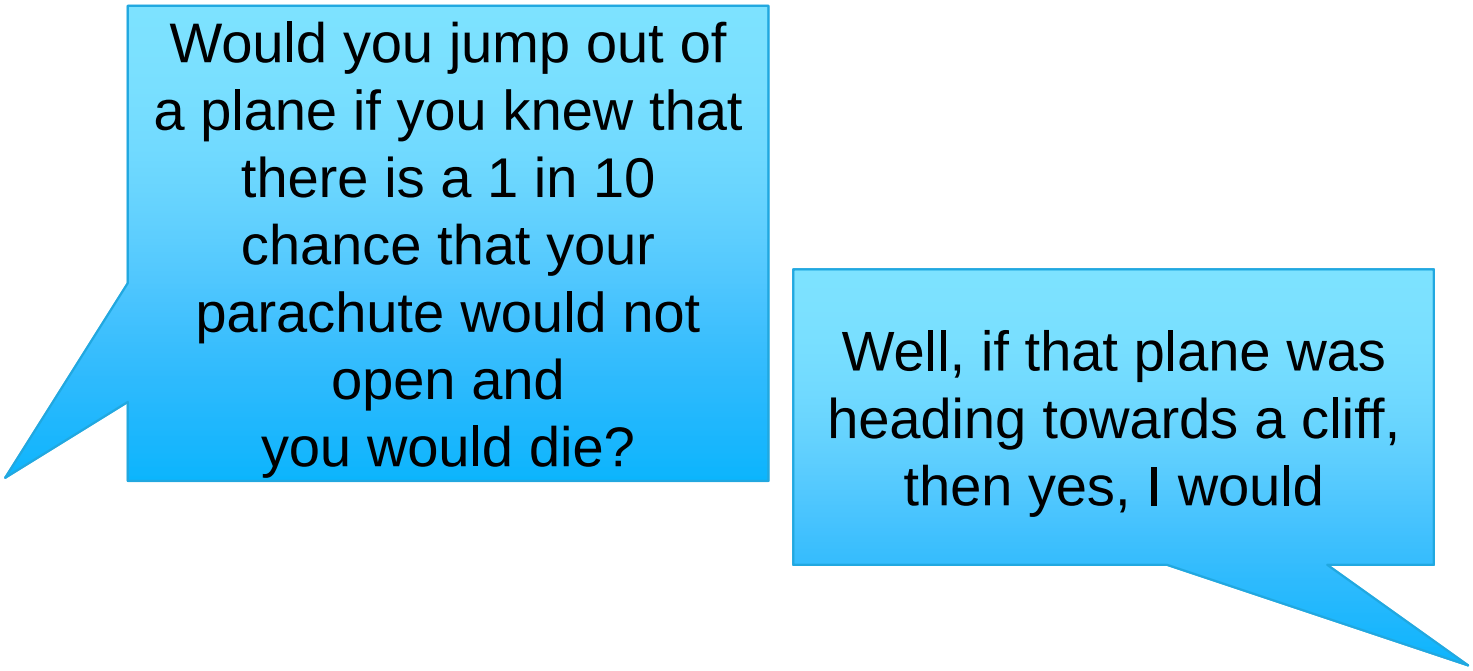
Would you jump out of a plane if you knew that there is a 1 in 10 chance that your parachute would not open and you would die?

Well, if that plane was heading towards a cliff, then yes, I would



EMA workshop on single-arm trials in oncology. Presentation - Clinical trial design- the patient standpoint (Bettina Ryll). 2016. https://www.ema.europa.eu/en/events/workshop-single-arm-trials-oncology

What is the risk of not taking risks?



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What is the risk of not taking risks?

Would you jump out of a plane if you knew that there is a 1 in 10 chance that your parachute would not open and you would die?

How certain are we that the plane is going to crash?

Well, if that plane was heading towards a cliff, then yes, I would

Not all trial participants prefer to be in the experimental arm

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Involving patients early in trial design optimises relevant outcomes & reduces some uncertainties

Illustration: https://edhub.ama-assn.org/steps-forward/module/2702513

Involving patients early in trial design optimises relevant outcomes & reduces some uncertainties

- Choosing patient-relevant outcomes
- Defining realistic in- and exclusion criteria
- Setting optimum trial duration
- Helping define the ratio of potential benefit and burden of trial participation

Illustration: https://edhub.ama-assn.org/steps-forward/module/2702513

Balancing ethics & the need for evaluating comparative effectiveness

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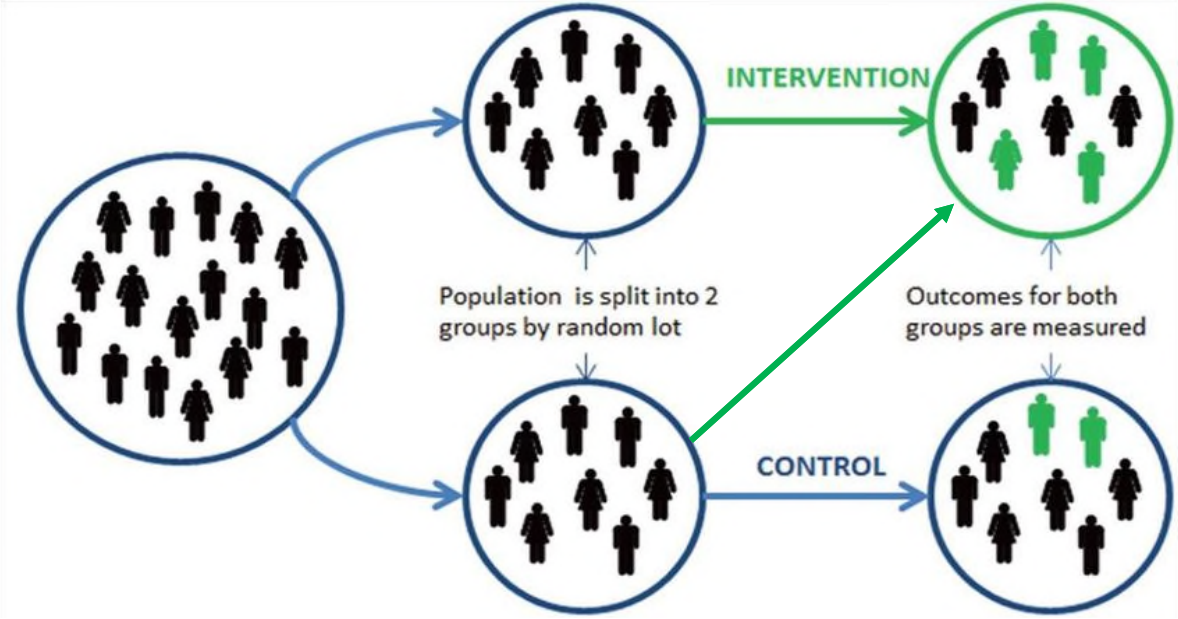
Patient-friendly RCTs: Allow cross-over.



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Balancing ethics & the need for evaluating comparative effectiveness

Patient-friendly RCTs: Reduce placebo groups

- Share placebo data over trials or use natural history data as comparator in single arm trials
- Collect patient data in a registry, before drug trials are in the pipeline.

Illustration: https://www.share4rare.org/news/what-patient-registry

Balancing ethics & the need for evaluating comparative effectiveness

People over profit

Beating competitors to launch as 1st in class or 1st in indication should **NOT** be the key decision-making criteria to chosing a single arm trial design.

Illustration: https://technical.ly/civic-news/climate-crisis-sustainability-corporate-response/