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

Illustration: https://www.researchgate.net/figure/The-basic-design-of-a-randomised-controlled-trial-adapted-from-Haynes-Service-Goldacre_fig2_318153807
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Illustration: https://journals.plos.org/plosmedicine/article?id=10.1371/journal.pmed.0020262
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Illustration: https://www.genesisdx.com/news-forum/blog/help-others/
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The experimental treatment may be their only source of hope.

Illustration: http://www.starsofhope.org/website/
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How certain are we that the plane is going to crash?

Not all trial participants prefer to be in the experimental arm.

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

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