

Breakout session on pragmatic trials – a HTA perspective

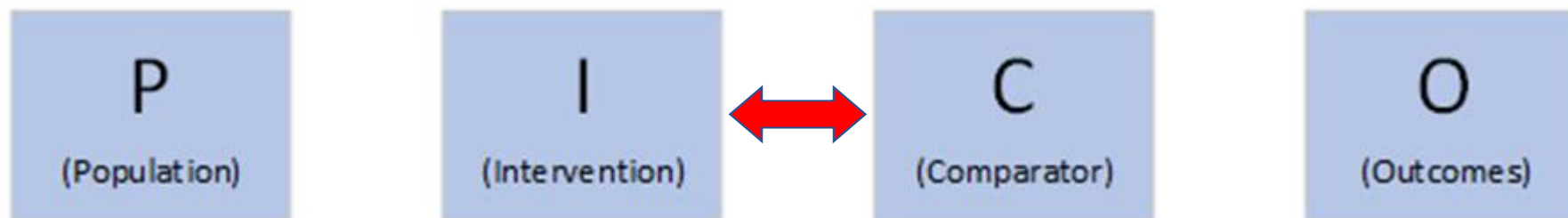
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ACT EU PA08 multi-stakeholder workshop on methodology guidance:
collaborating across the regulatory network

23 November 2023

Context of health technology assessment

- Our question: comparative effectiveness (is there any difference between a new intervention and the current standard of care/best available treatment?)
- => inform treatment decisions, inform pricing and reimbursement decisions



- We often lack data to answer our question (on comparative effects, on patients we are treating in routine care, on endpoints that are relevant)
- We need more and other studies to enable a description of comparative effectiveness

Why pragmatic trials?

- We need more (randomised) comparative trials
- We need data on comparative effects in patients we are treating in routine care
 - (limited narrow populations not covering patients treated in routine practice are not a consequence of randomisation but of decisions on inclusion criteria)
 - Caveat for broad populations: in case of heterogenous populations, assessment of effect modification by patient characteristics required
- We need faster and less expensive trials => randomised registry-based pragmatic trials

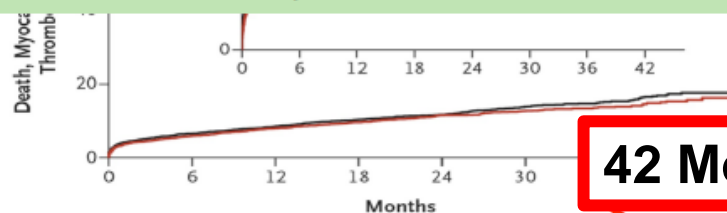
R-RCT vs. RCT

STEMI Thrombectomy Story (SWEDEHEART-Register)

TASTE (R-RCT)



500,000 €



42 Monate

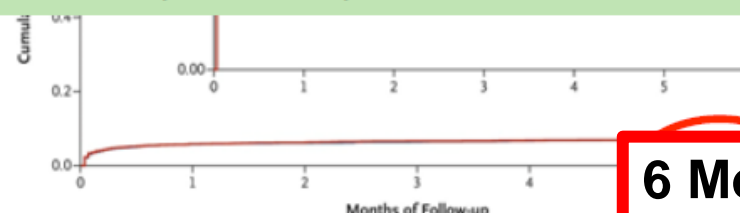
No. at Risk	0	6	12	18	24	30	36	42
PCI+TA	3623	3404	3328	2821	2180	1505	864	184
PCI only	3621	3386	3315	2796	2200	1494	862	190

1st patient: June 2010
30 centers
33 months to full enrollment
7,244 patients

TOTAL (traditionell RCT)



15,000,000 €



6 Monate

No. at Risk	0	1	2	3	4	5	6
Thrombectomy	5033	4734	4696	4678	4662	4647	4628
PCI alone	5030	4727	4688	4666	4653	4642	4618

1st patient: August 2010
87 centers
48 months to full enrollment
10,732 patients

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