

# Towards More Efficient Clinical Trials with Adaptive Designs

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13 October 2025



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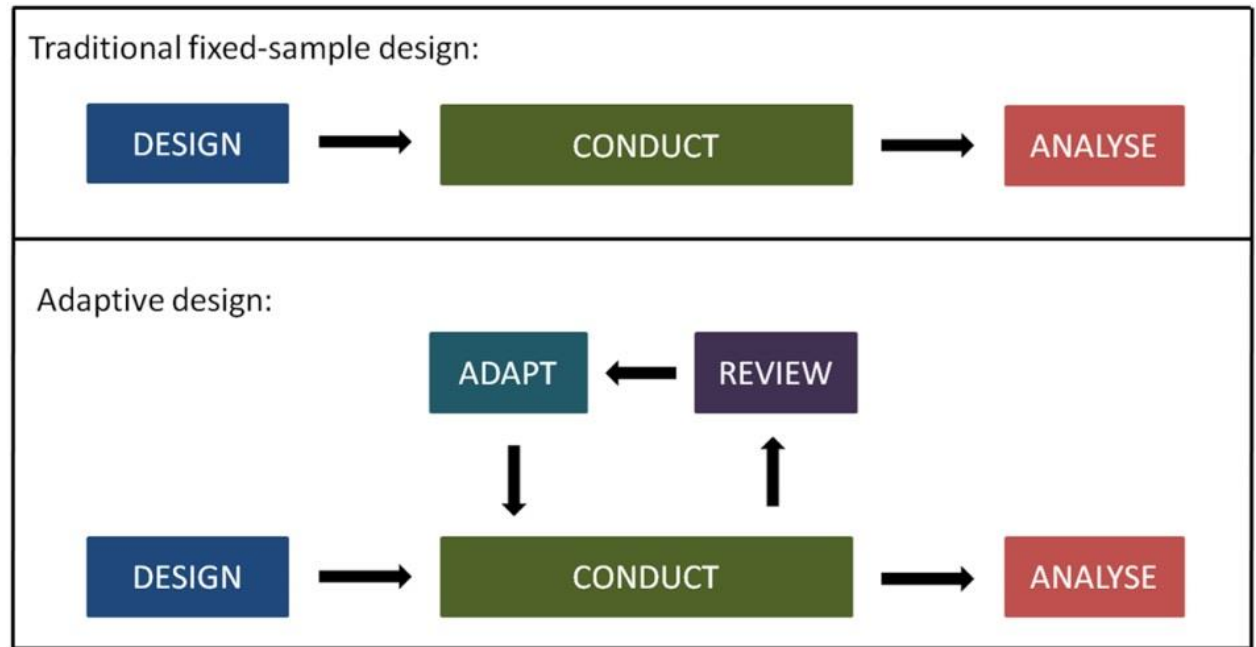
# To adapt or not to adapt?

Being adaptive is a useful thing...

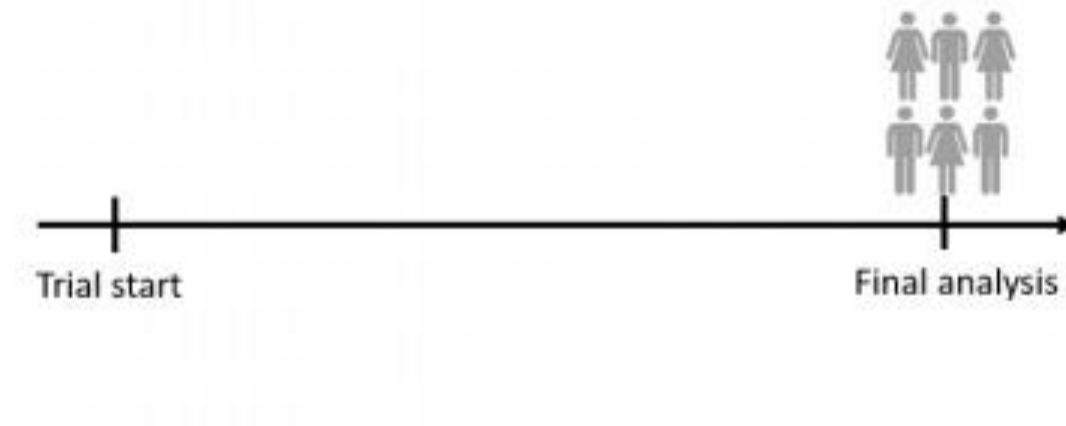


# To adapt or not to adapt?

Being adaptive is a useful thing...



# Fixed vs. adaptive designs

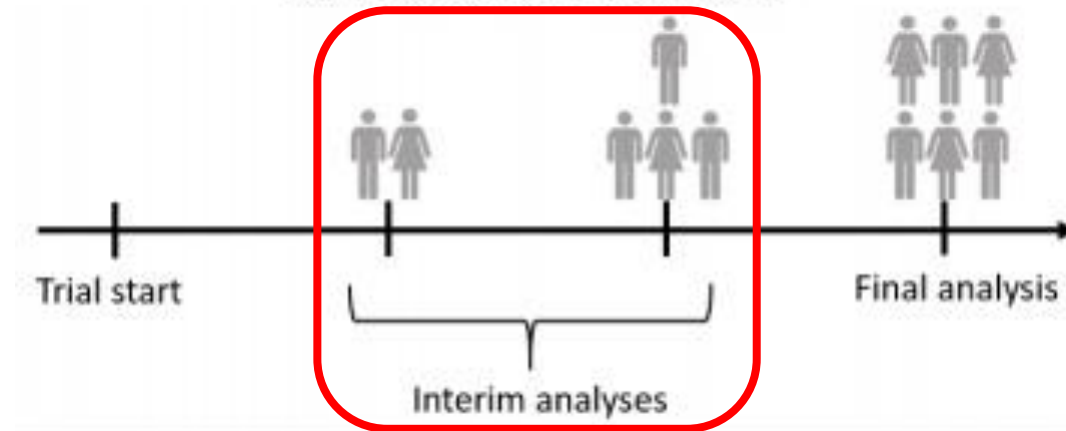


(a) Fixed design analysis plan.

# Fixed vs. adaptive designs

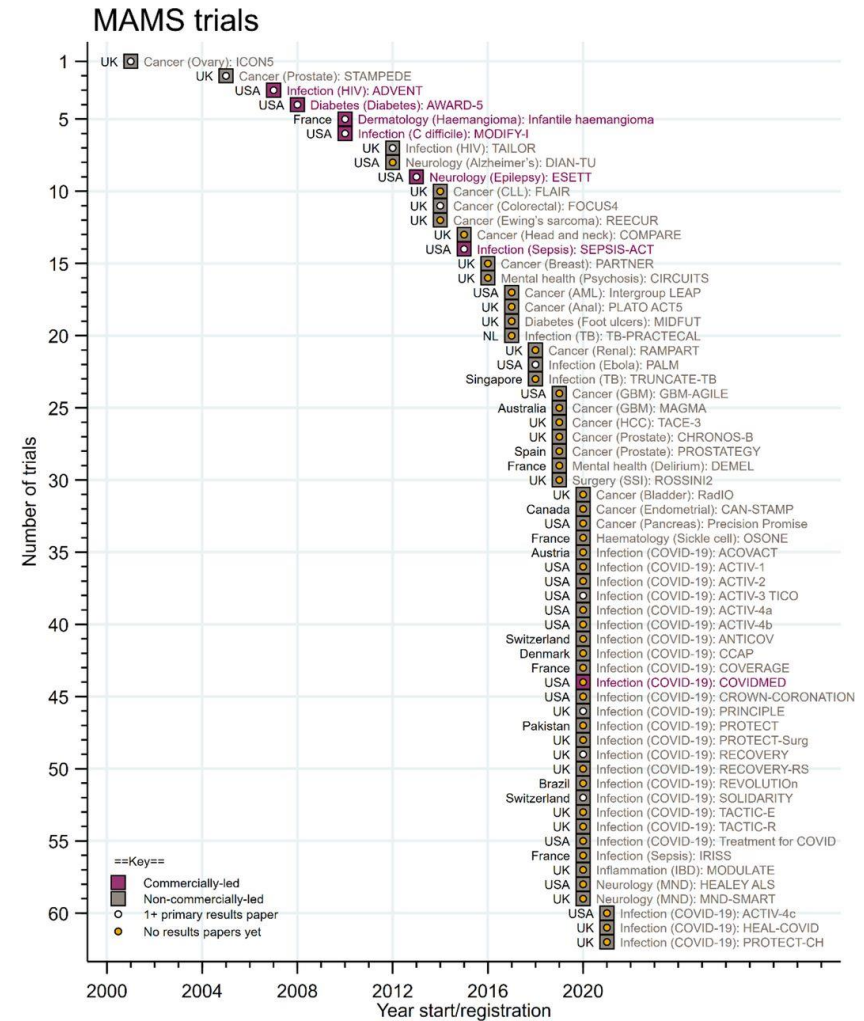
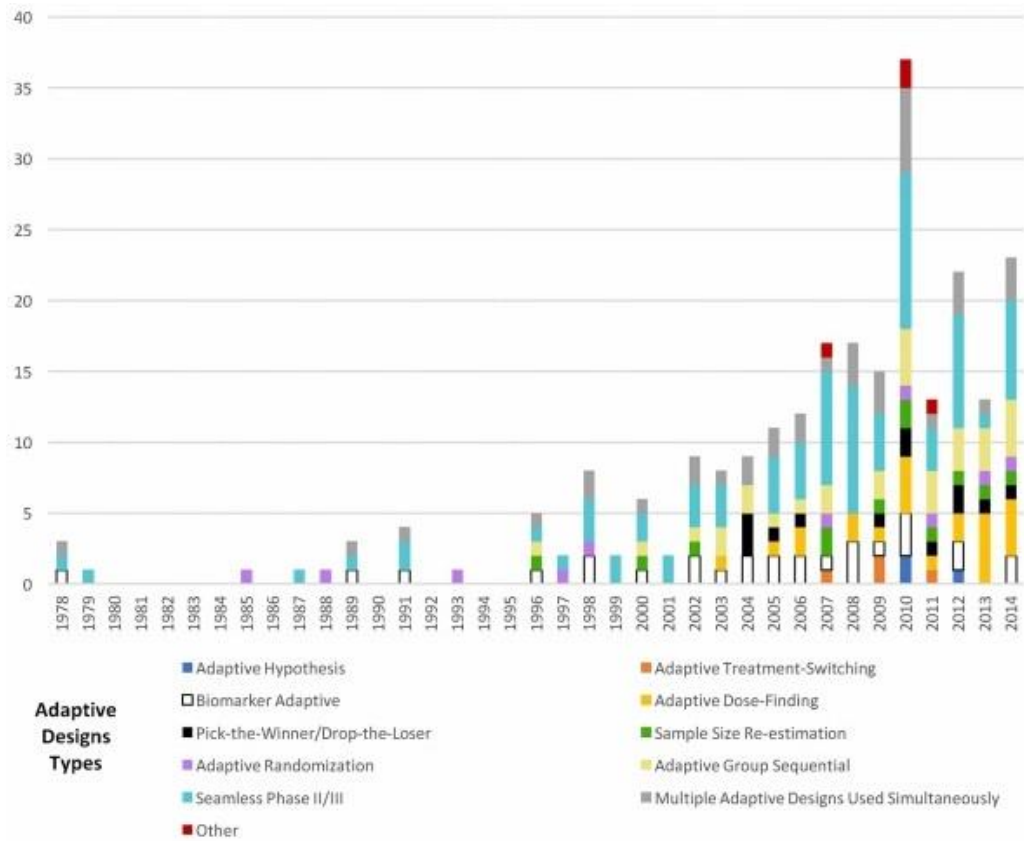


(a) Fixed design analysis plan.



(b) Adaptive design analysis plan.

# The trials they are a-daptive



# What is an adaptive design?

A clinical trial design

that offers **pre-planned** opportunities

to use accumulating trial data

to **modify aspects** of an ongoing trial

while **preserving the validity and integrity** of that trial.

→ includes **group-sequential** and **Bayesian adaptive** methods

→ excludes **'fully flexible'** designs

# Planning to be flexible

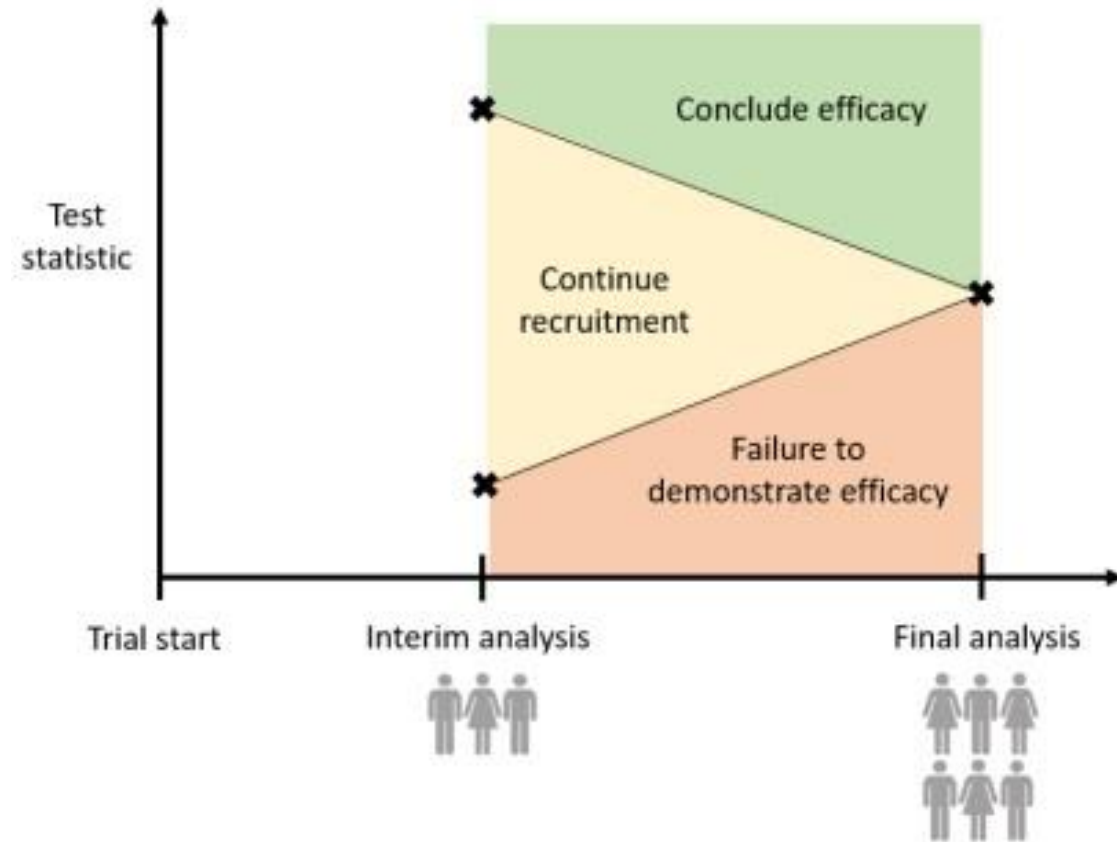


# Design type 1: Group-sequential

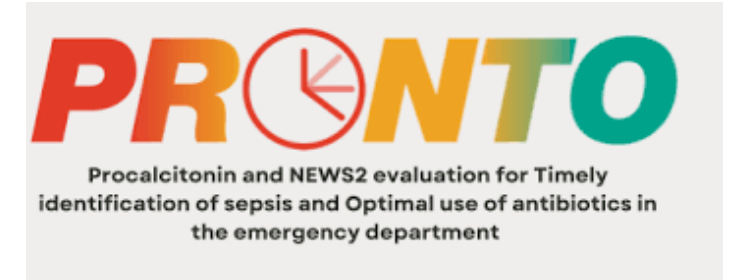
**Goal** efficient use of patients, time, and money

**Problem** ignoring clear evidence of futility or efficacy is suboptimal

**Idea** early stopping for futility or efficacy (or safety)



# Example: PRONTO trial



Adults presenting to the ED with **suspected sepsis** (n=7676)

Addition of **procalcitonin testing** to NEWS2 scoring to stratify risk

Co-primary endpoints:

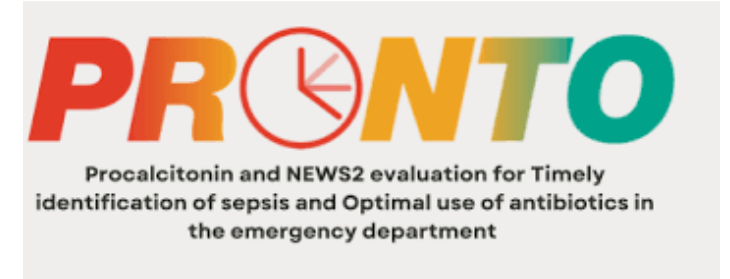
- 1) Initiation of IV antibiotics at 3h (**effectiveness** outcome, tested for superiority)
- 2) 28-day mortality (**safety** outcome, tested for non-inferiority)

PCT and NEWS2 as adjunct for risk stratification aligned to NEWS2 and standard care.

STRATIFY RISK

	Procalcitonin		
NEWS2	<0.5	0.5-1.9	≥2
<5	low	low	medium
5-6	low*	medium	high
≥7	medium**	high	high

# Example 1: PRONTO trial



Using O'Brien-Fleming **group-sequential stopping** rules to ensure overall type I error rate control

Early stopping for **effectiveness**:

- Stop if the interim analysis indicates both **superiority** for the **effectiveness** endpoint and **non-inferiority** for the **safety** endpoint, or
- Stop if the interim analysis indicates **superiority** for the **safety** endpoint

Early stopping for **futility**:

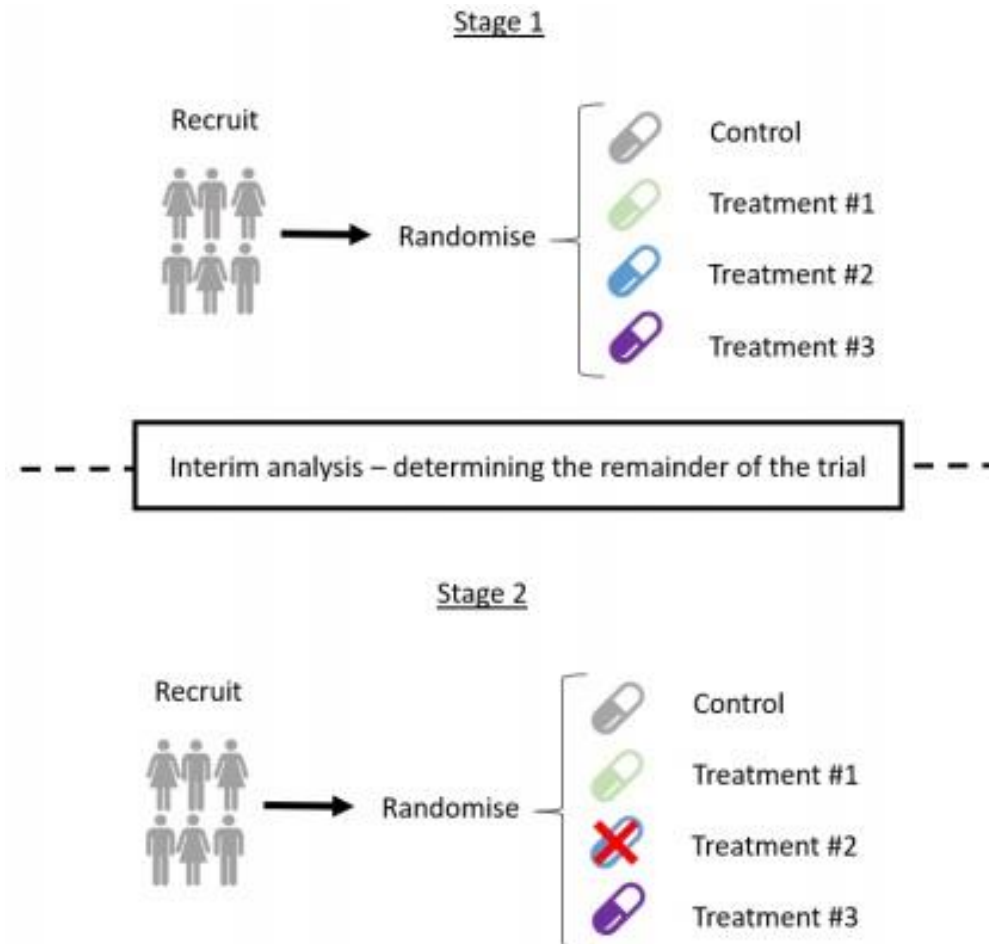
- Stop if the interim analysis indicates **futility** for **both** endpoints

# Design type 2: Multi-arm multi-stage

**Goal** compare different experimental interventions vs. a reference

**Problem** running multiple controlled trials is inefficient

**Idea** start off with several intervention arms and then drop/select/add

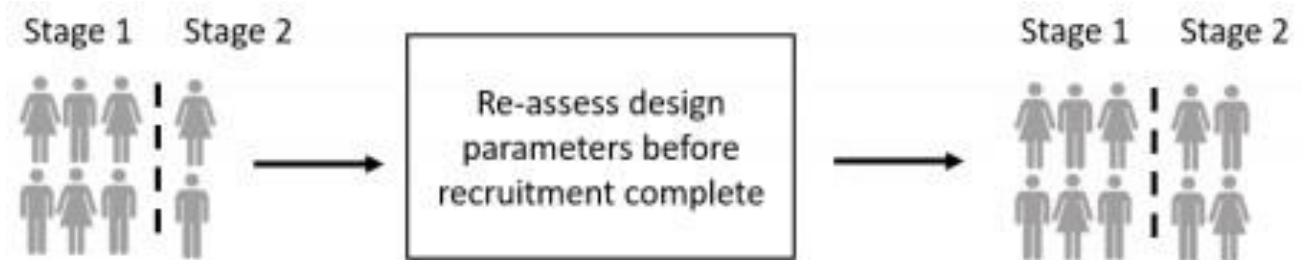


# Design type 3: Sample size reassessment

**Goal** achieve desired statistical power (e.g. 90%)

**Problem** sample size calculation is often based on vague assumptions

**Idea** get better sample size estimate from interim data

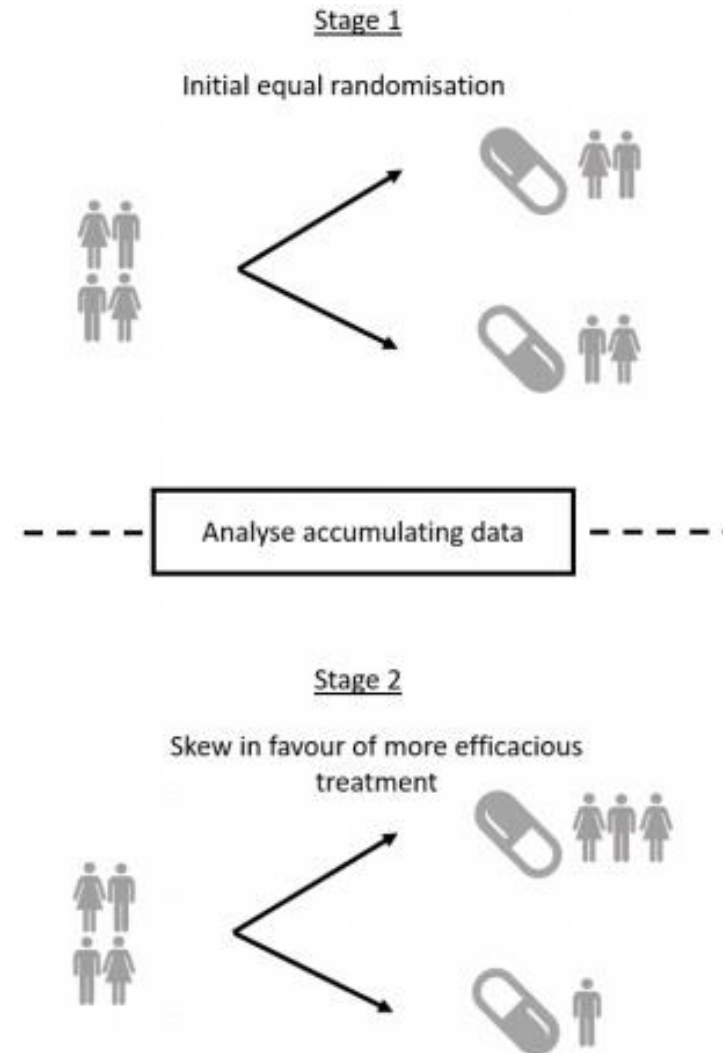


# Design type 4: Adaptive randomisation

**Goal** compare different interventions

**Problem** subjecting patients to inferior interventions is unethical

**Idea** shift randomisation ratio towards more promising intervention arm

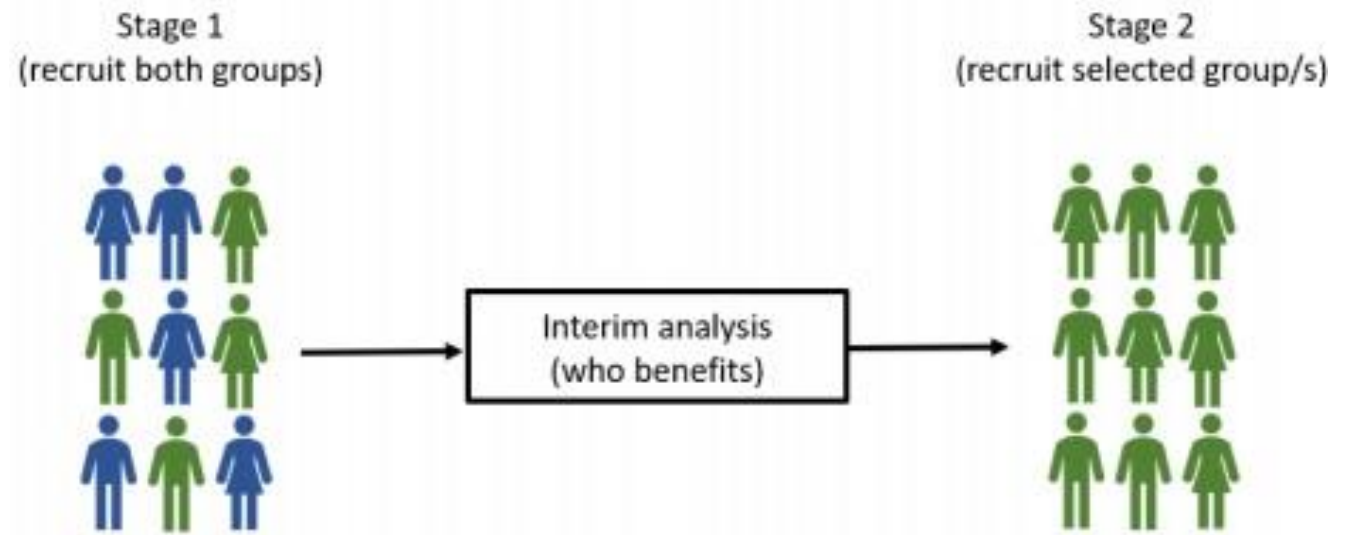


# Design type 5: Population enrichment

**Goal** focus on patients who benefit most from a treatment

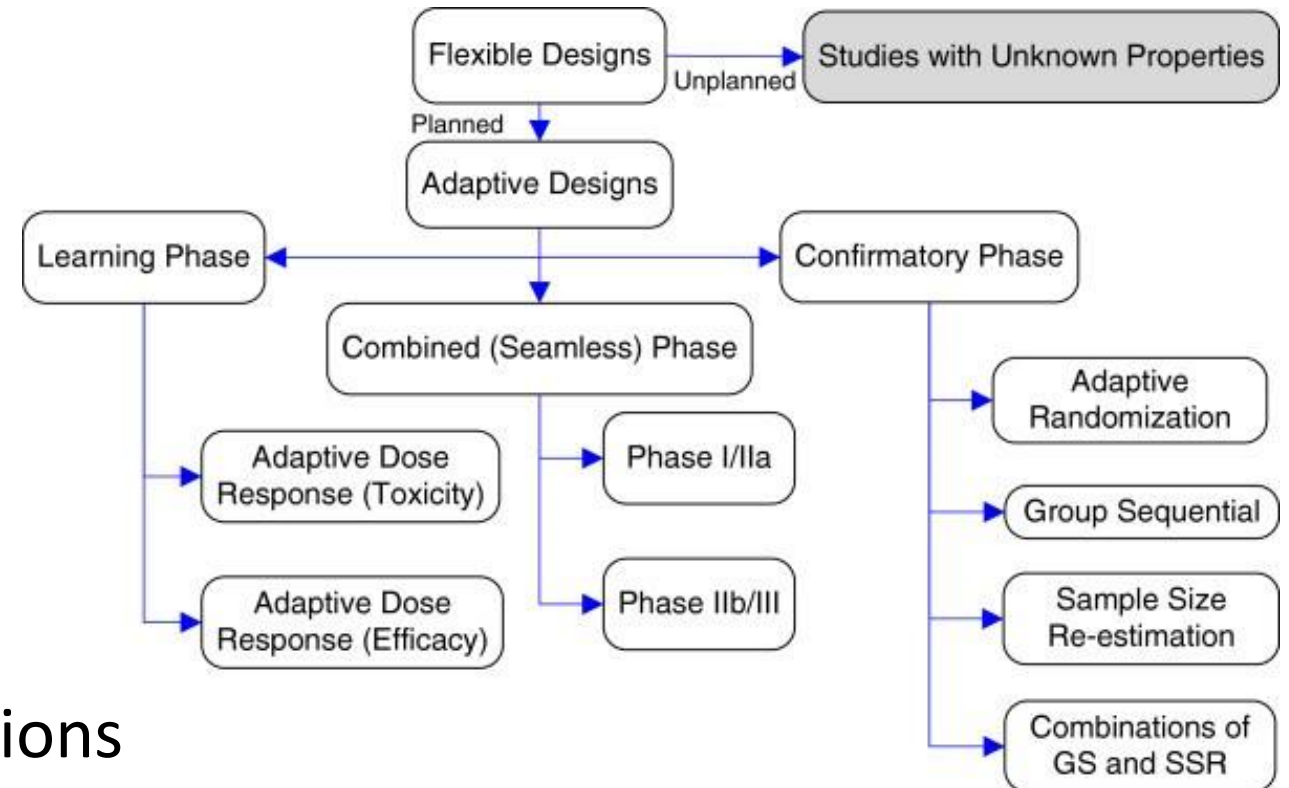
**Problem** not all patients might benefit equally

**Idea** target patients who are most likely to benefit

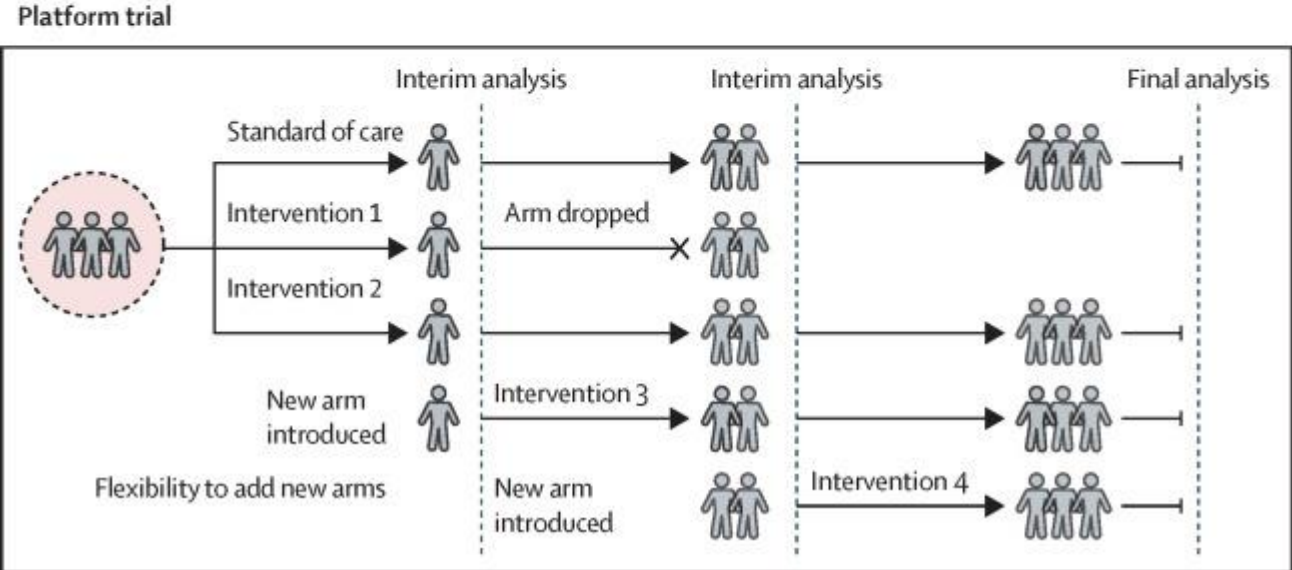
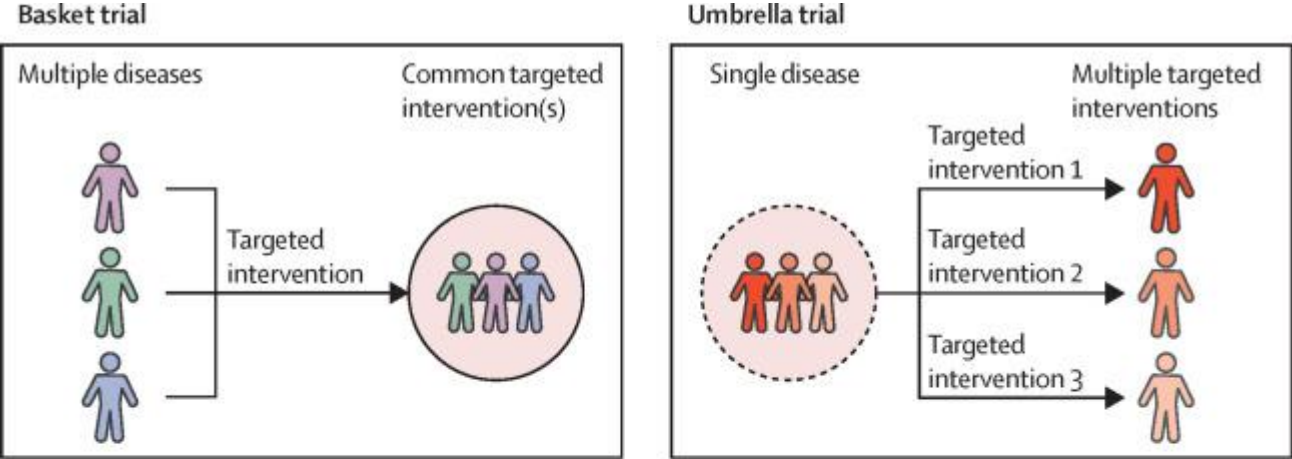


... and many more

- Adaptive dose escalation
- Adaptive dose ranging
- Biomarker-adaptive
- Adaptive treatment switching
- Adaptive hypotheses
- Seamless phase I/II or II/III
- Combinations of several adaptations
- Bayesian adaptive



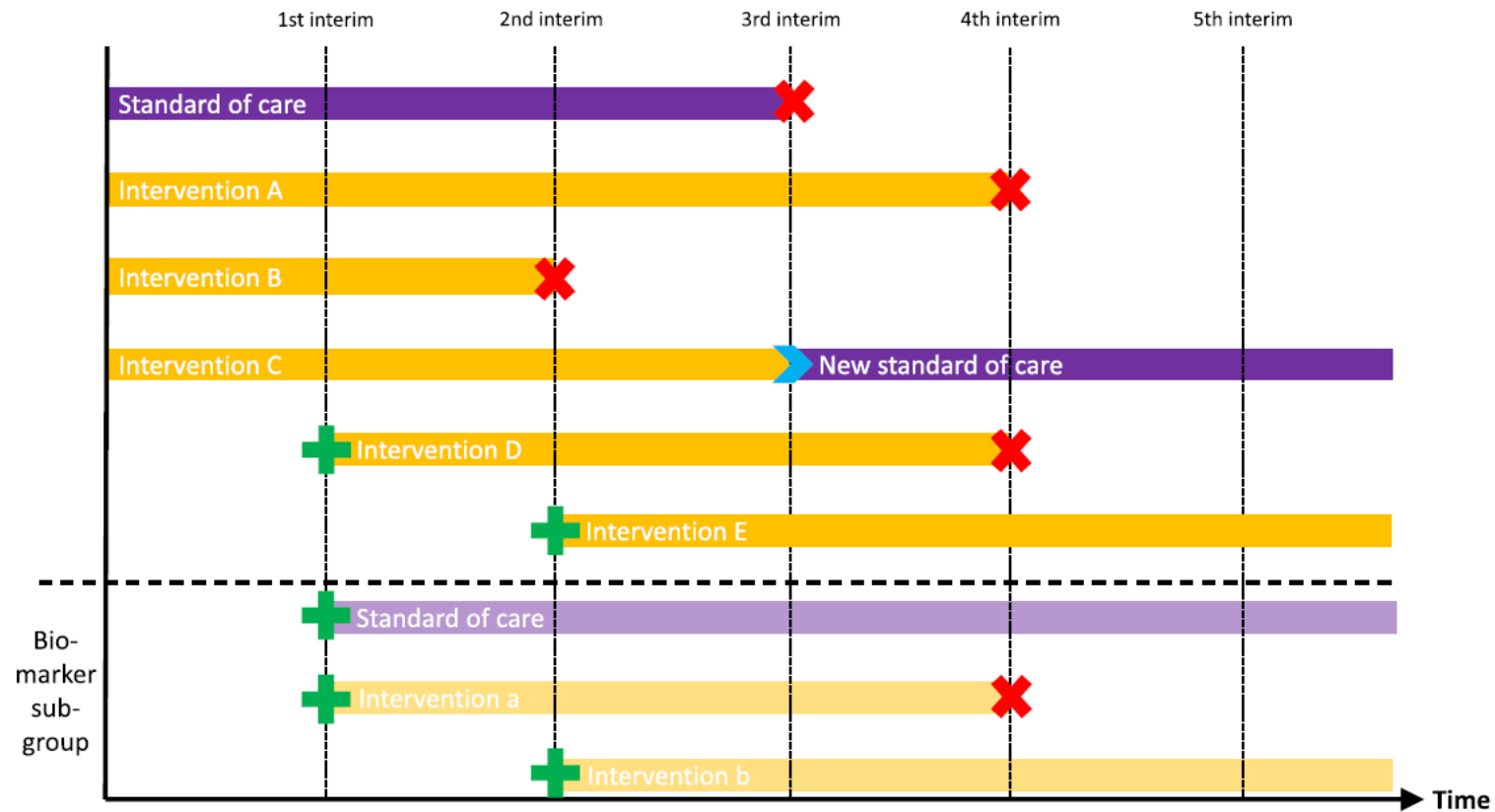
# Master protocol designs



**Table 1. Types of Master Protocols.**

Type of Trial	Objective
Umbrella	To study multiple targeted therapies in the context of a single disease
Basket	To study a single targeted therapy in the context of multiple diseases or disease subtypes
Platform	To study multiple targeted therapies in the context of a single disease in a perpetual manner, with therapies allowed to enter or leave the platform on the basis of a decision algorithm

# Platform designs: MAMS on steroids



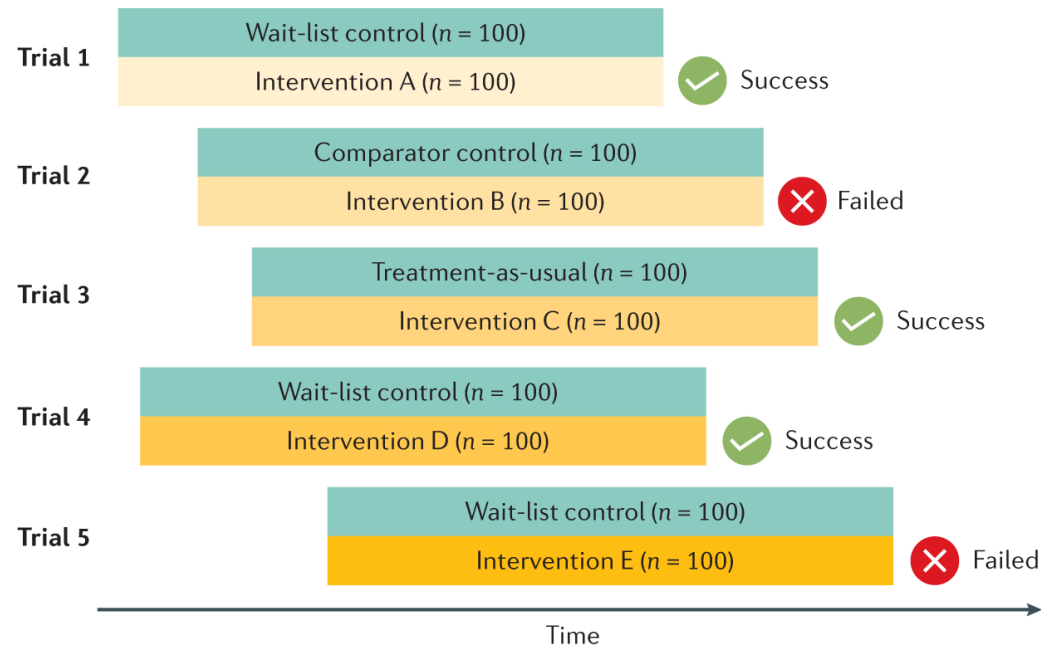
# The efficiency of trial platforms



“The way we carry out clinical trials is like building a new stadium for every football game.”

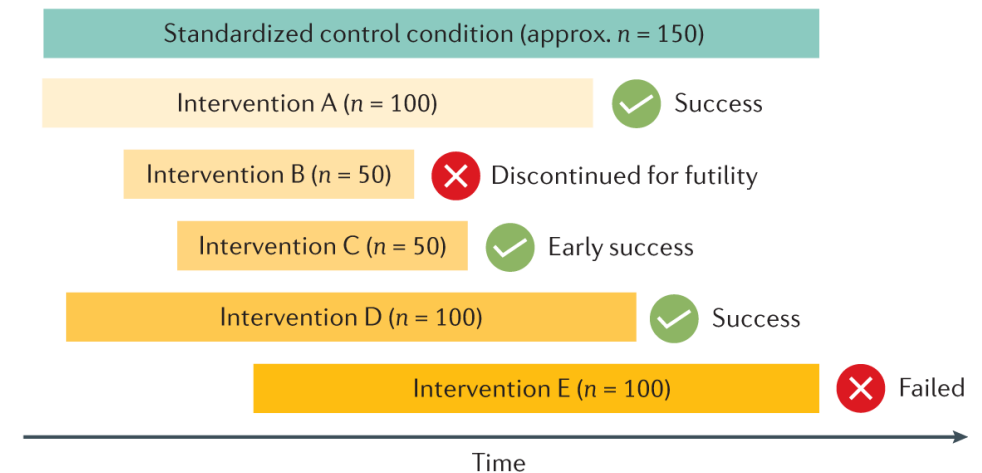
## a Individual randomized controlled trials

Overall sample size:  $N = 1,000$



## b Platform trial

Overall sample size: approx.  $N = 550$



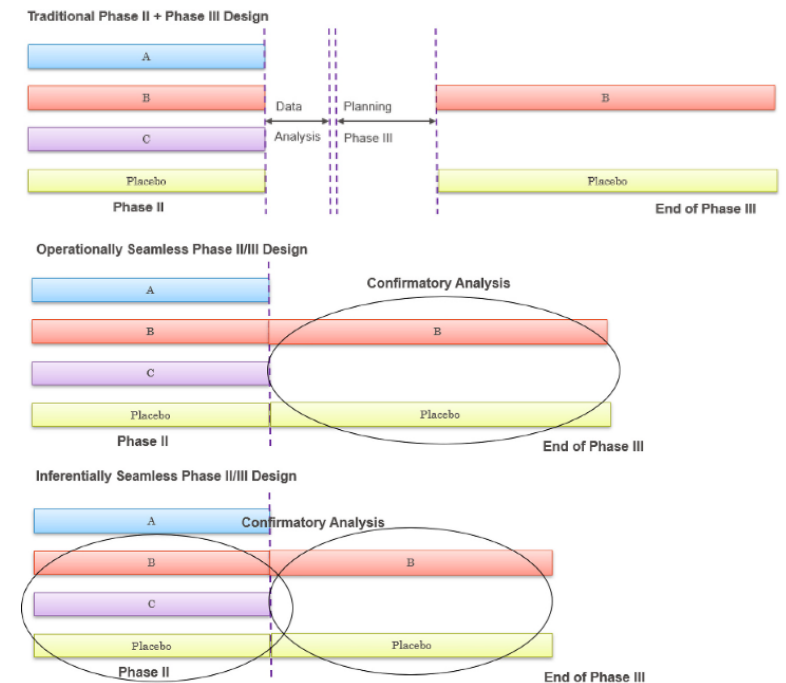
# Efficiencies of adaptive designs

Ideally, adaptive designs are **statistically** and **operationally efficient**

Example: seamless phase II/III designs

Potential benefits:

- Saving **resources** (time, money, patients)
- Fewer allocated to **control**
- Fewer allocated to **inferior treatments**
- Results available **sooner**
- Single **protocol** (trial set-up, approvals, infrastructure)
- Head-to-head **comparisons**



# Efficiencies of adaptive designs

Efficiency gains can be (partially) offset by **complexity**

- More time and expertise needed for design and analysis

Potential risks of:

- Losing **statistical efficiency** e.g. when follow-up is long compared to recruitment period
- Losing **operational efficiency** e.g. due to added complexity
- Compromising **integrity and validity** e.g. due to biased results

# When adaptive designs are less useful

Types of adaptive features	What it does at the interim stages	Potential Limitations					
		Impact of Long-term primary outcomes <sup>a,b</sup>  (Potential loss in predicted "theoretical" efficiency <sup>a</sup> )	Missing important secondary information	Operational complexity	Additional cost/resources to build adequate infrastructure	Methodological challenges (complex designs require specialist training)	Communication to enable understanding of the workings of the adaptive features
Sample size re-estimation  (often 2 stages)	Adjust sample size to ensure adequate power						
Population enrichment  (often 2 stages)	Concentrate on participants more likely to experience treatment benefit						
Stopping early for futility	Drop futile arms						
Stopping early for efficacy	Declare promising arm(s) as effective						
Biomarker adaptive	Adapt based on biomarker information						
Outcome-adaptive randomisation  (often ≥3 stages)	Adjust allocation ratio to place more participants in better-performing treatments						

**Table 1 | Overview of practical considerations for adaptive trials in health services or implementation research**

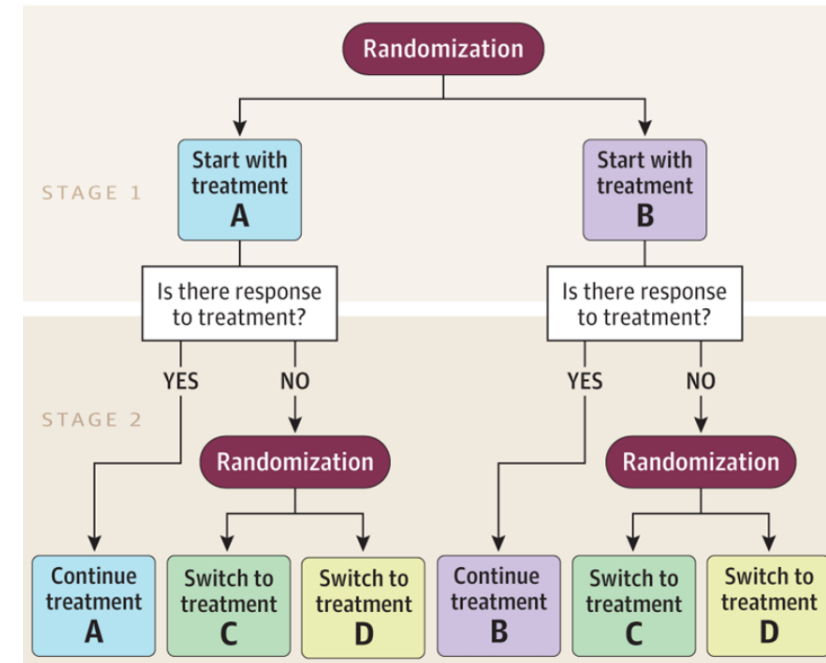
Trial characteristics	Considerations in adaptive trials
Interventions appropriate for testing	<ul style="list-style-type: none"> <li>▶ Individual intervention components should be easily described</li> <li>▶ Interventions should be able to be allocated to study participants over different time periods</li> <li>▶ Multicomponent interventions can be studied if components are tested across multiple arms</li> </ul>
Eligibility criteria, enrolment procedures, and allocation probabilities	<ul style="list-style-type: none"> <li>▶ Narrow down recruitment of participants to those most likely to benefit in subsequent trial stages</li> <li>▶ Address covariate balance by adjusting sample composition to improve balance</li> </ul>
Choice of outcomes and interim analyses	<ul style="list-style-type: none"> <li>▶ Primary outcome for adaptation is ideally measured quickly</li> <li>▶ Outcome must be rapidly retrievable from underlying data sources</li> <li>▶ Multiple interim analyses typically done, which can be modified based on follow-up</li> </ul>
Required sample size and length of follow-up	<ul style="list-style-type: none"> <li>▶ Follow-up time may be until enough outcome data points are measured, rather than an a priori window</li> <li>▶ Using long-term outcomes for adaptation could greatly extend the length of the trial</li> <li>▶ Substantial missing data or loss-to-follow-up issues may lengthen necessary follow-up time</li> </ul>

High Medium Low

# Adaptive designs vs. adaptive interventions

Table 1 Table of terms and concepts related to “adaptive design”<sup>a</sup>

Term	Design	Actor (who)	When	Goal	Example in literature
Adaptation	1 Adaptation of intervention as an implementation research design	Implementation scientist	Before an implementation trial	Alter an effective intervention prior to studying its implementation	Trauma-focused CBT (14)
	2 Adaptation of intervention as an effectiveness intervention design	Effectiveness scientist	Before an effectiveness trial	Alter an intervention prior to studying its effectiveness	SMI Life Goals (36)
	3 Adaptation of an intervention as an implementation strategy	Implementation practitioner	During implementation	Improve implementation	Getting To Outcomes (10, 11), Replicating Effective Programs (39)
Adaptive intervention	4 Adaptive intervention design	Clinician	During intervention	Improve patient outcomes	Stepped care (32)
Adaptive implementation	5 Adaptive implementation strategy	Implementation practitioner	During implementation	Improve implementation	ASIC (36, 40, 58), Re-Engage (35), ADEPT (59, 60), ROCC (62)
Adaptive trial	6 Adaptive intervention trial design	Effectiveness scientist	During an effectiveness trial	Improve intervention effectiveness study efficiency or ethics	ESETT (31)
	7 Adaptive implementation trial design	Implementation scientist	During an implementation trial	Improve implementation study efficiency or ethics	PEPReC (22)
Adaptive iterative design	8 Iterative intervention effectiveness research	Effectiveness scientist	During a pilot effectiveness trial	Ensure feasibility and acceptability of intervention	PARAMEDIC2 (50)
	9 Iterative implementation research	Implementation scientist	During a pilot implementation trial	Ensure feasibility and acceptability of implementation strategy	FRAME-IS (45)



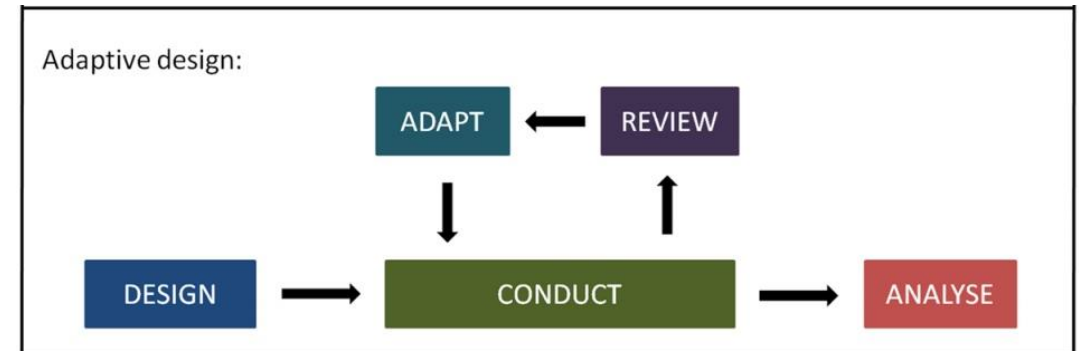
Sequential multiple assignment randomised trial (SMART)

**Not an adaptive design!**

# Adaptive designs vs. adaptive interventions

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When you come to a fork in the road...



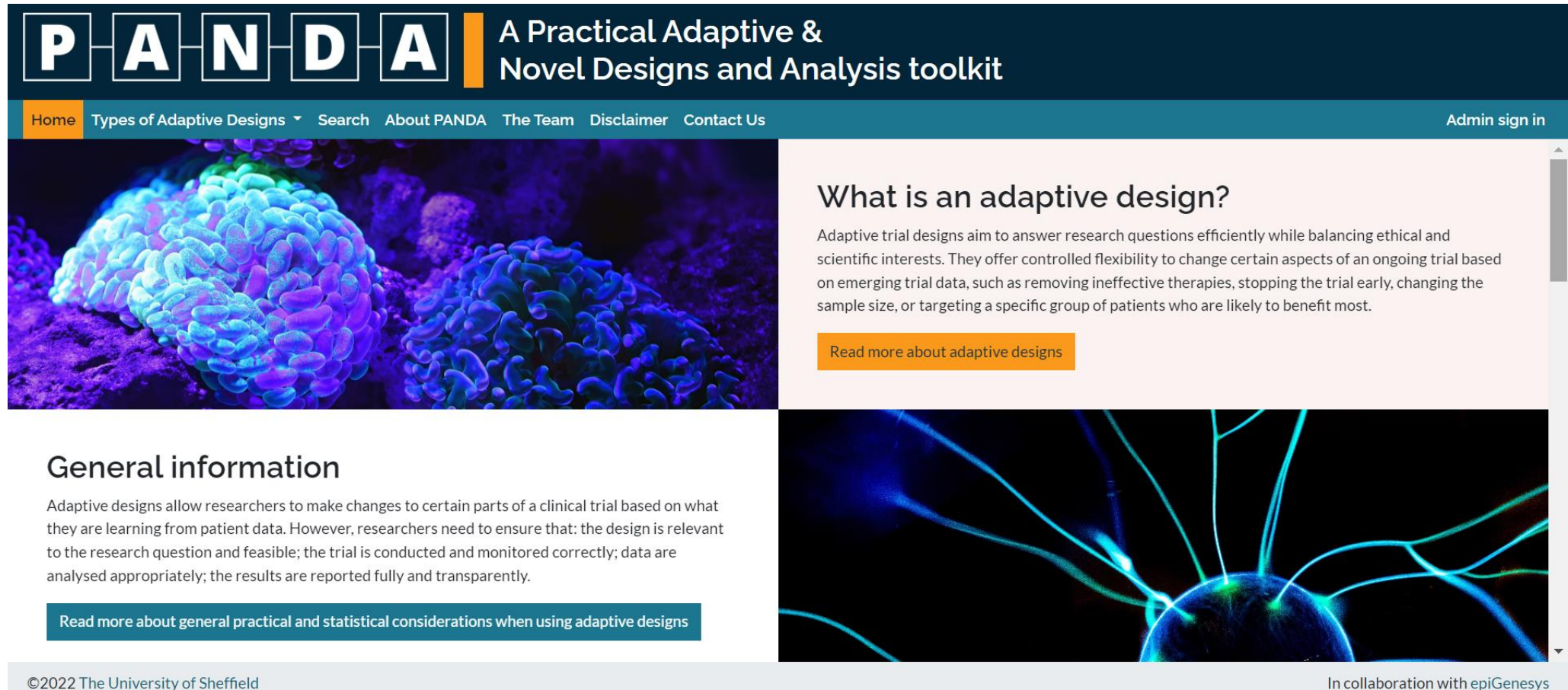
**... take it!**

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# PANDA

<https://panda.shef.ac.uk>



The screenshot shows the PANDA website homepage. At the top, the word "PANDA" is displayed in large, white, outlined letters on a dark blue background. To its right, the text "A Practical Adaptive & Novel Designs and Analysis toolkit" is written in white. Below this is a navigation bar with links: "Home" (highlighted in orange), "Types of Adaptive Designs" (with a dropdown arrow), "Search", "About PANDA", "The Team", "Disclaimer", and "Contact Us". On the far right of the navigation bar is a link for "Admin sign in".

The main content area is split into two columns. The left column features a large image of glowing, colorful biological cells. Below the image is the heading "General information" and a paragraph of text: "Adaptive designs allow researchers to make changes to certain parts of a clinical trial based on what they are learning from patient data. However, researchers need to ensure that: the design is relevant to the research question and feasible; the trial is conducted and monitored correctly; data are analysed appropriately; the results are reported fully and transparently." Below this text is a blue button with white text: "Read more about general practical and statistical considerations when using adaptive designs".

The right column has a heading "What is an adaptive design?" followed by a paragraph: "Adaptive trial designs aim to answer research questions efficiently while balancing ethical and scientific interests. They offer controlled flexibility to change certain aspects of an ongoing trial based on emerging trial data, such as removing ineffective therapies, stopping the trial early, changing the sample size, or targeting a specific group of patients who are likely to benefit most." Below this text is an orange button with white text: "Read more about adaptive designs".

At the bottom of the page, there is a footer with the text "©2022 The University of Sheffield" on the left and "In collaboration with epiGenesys" on the right.

# PANDA

<https://panda.shef.ac.uk>

PANDA **Types of Adaptive Designs** Search About PANDA The Team Disclaimer Contact Us

Planning and design

- Appropriateness
- Design concepts
- Trial phases covered by MAMS des...
- Prospective case studies
- Underpinning statistical methods
- How are decision rules defined?
- Ethical considerations
- Tips on explaining the design to...
- Other considerations
- Some limitations and challenges
- Examples of how MAMS trials are ...

Conduct

Analysis

Resources

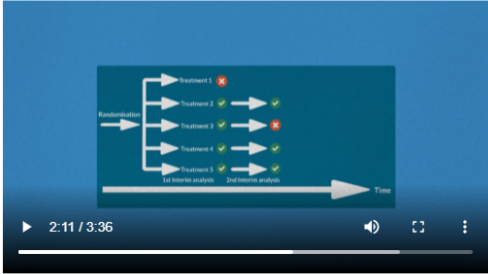
## Multi-Arm Multi-Stage (MAMS)

### Overview

In summary:

- Multi-arm multi-stage (MAMS) trials evaluate several active treatments (e.g. different regimens or doses/schedules of the same treatment or completely different treatments) in a single trial;
- Treatment arms that show promise on the basis of accrued outcome data are retained for further testing, whilst those unlikely to show benefit are dropped;
- The criteria for selecting or dropping arms at interim analyses should be pre-specified;
- MAMS designs are useful where multiple competing treatments need to be reduced to a small number of most promising ones.

Video



2:11 / 3:36

Concept behind the MAMS design

PANDA **Types of Adaptive Designs** Search About PANDA The Team Disclaimer Contact Us

Multi-Arm Multi-Stage (MAMS)

Overview

Planning and design

Conduct

Analysis

Resources

Statistical software

Classification scenario	Achieved sample size	Savings in sample size compared to:			
		2 separate 2-arm fixed trial designs (1:1 randomisation) (n=368)	2 separate 2-arm fixed trial designs (2:1 randomisation) (n=420)	3-arm 2-stage (MAMS) maximum sample size (n=255)	3-arm fixed trial design with Dunnett test (n=354)
Expected sample size (if both interventions are ineffective)	164	204	256	91	190
Expected sample size (if both interventions are effective)	251	117	169	165	103
Actual sample size					
If both arms are stopped at stage 1	116	252	304	139	230
If one arm is stopped at stage 1	221	147	199	34	133
If no arm is stopped at stage 1	255	113	165	0	99

Sample size savings.

Stata code:

```
// install packages
ssc install nstagebin
ssc install nstagebinopt

// to read help files
chelp nstagebinopt
chelp nstagebin

// exploring 3-arm and 2-stage designs:
Click this hyperlink to access the full code on github

// final selected design
nstagebin, nstage(2) accrate(8 8) alpha(0.27 0.014) ///
power(0.96 0.92) arms(3 3) theta0(0) ///
theta1(-0.20) ctrlp(0.3) fu(0.92) ltfu(0) tunit(4) ///
aratio(0.5) probs ess extrat(0) seed(25)
```

# Further reading

Pallmann et al. *BMC Medicine* (2018) 16:29  
<https://doi.org/10.1186/s12916-018-1017-7>

BMC Medicine

CORRESPONDENCE

Open Access

## Adaptive designs in clinical trials: why use them, and how to run and report them



Philip Pallmann<sup>1\*</sup>, Alun W. Bedding<sup>2</sup>, Babak Choodari-Oskoei<sup>3</sup>, Munyaradzi Dimairo<sup>4</sup>, Laura Flight<sup>5</sup>, Lisa V. Hampson<sup>1,6</sup>, Jane Holmes<sup>7</sup>, Adrian P. Mander<sup>8</sup>, Lang'oo Odondi<sup>7</sup>, Matthew R. Sydes<sup>3</sup>, Sofia S. Villar<sup>3</sup>, James M. S. Wason<sup>8,9</sup>, Christopher J. Weir<sup>10</sup>, Graham M. Wheeler<sup>8,11</sup>, Christina Yap<sup>12</sup> and Thomas Jaki<sup>1</sup>

*The NEW ENGLAND JOURNAL of MEDICINE*

REVIEW ARTICLE

**THE CHANGING FACE OF CLINICAL TRIALS**

Jeffrey M. Drazen, M.D., David P. Harrington, Ph.D., John J.V. McMurray, M.D., James H. Ware, Ph.D., and Janet Woodcock, M.D., *Editors*

## Adaptive Designs for Clinical Trials

Deepak L. Bhatt, M.D., M.P.H., and Cyrus Mehta, Ph.D.

Burnett et al. *BMC Medicine* (2020) 18:352  
<https://doi.org/10.1186/s12916-020-01808-2>

BMC Medicine

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## Adding flexibility to clinical trial designs: an example-based guide to the practical use of adaptive designs



Thomas Burnett<sup>1\*</sup>, Pavel Mozgunov<sup>1</sup>, Philip Pallmann<sup>2</sup>, Sofia S. Villar<sup>3</sup>, Graham M. Wheeler<sup>4</sup> and Thomas Jaki<sup>1,3</sup>

RESEARCH METHODS AND REPORTING

## Key design considerations for adaptive clinical trials: a primer for clinicians

Kristian Thorlund,<sup>1,2</sup> Jonas Haggstrom,<sup>2</sup> Jay JH Park,<sup>1</sup> Edward J Mills<sup>1,2</sup>

OPINION

Open Access

## When to keep it simple – adaptive designs are not always useful



James M. S. Wason<sup>1,2\*</sup>, Peter Brocklehurst<sup>3</sup> and Christina Yap<sup>4,5</sup>

# Further reading

Love et al. *Trials* (2022) 23:757  
<https://doi.org/10.1186/s13063-022-06680-4>

Trials

METHODOLOGY

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## Practical guidance for running late-phase platform protocols for clinical trials: lessons from experienced UK clinical trials units



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REVIEW ARTICLE

THE CHANGING FACE OF CLINICAL TRIALS

Jeffrey M. Drazen, M.D., David P. Harrington, Ph.D., John J.V. McMurray, M.D., James H. Ware, Ph.D., and Janet Woodcock, M.D., Editors

## Master Protocols to Study Multiple Therapies, Multiple Diseases, or Both

Janet Woodcock, M.D., and Lisa M. LaVange, Ph.D.

Morrell et al. *Trials* (2019) 20:297  
<https://doi.org/10.1186/s13063-019-3377-5>

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COMMENTARY

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## Mind the gap? The platform trial as a working environment



Liz Morrell<sup>1\*</sup>, Joshua Hordern<sup>2</sup>, Louise Brown<sup>3</sup>, Matthew R. Sydes<sup>3</sup>, Claire L. Amos<sup>3</sup>, Richard S. Kaplan<sup>3</sup>, Mahesh K. B. Parmar<sup>3</sup> and Timothy S. Maughan<sup>4,5</sup>



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REVIEW

## An overview of platform trials with a checklist for clinical readers

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METHODOLOGY

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## This is a platform alteration: a trial management perspective on the operational aspects of adaptive and platform and umbrella protocols



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## Changing platforms without stopping the train: experiences of data management and data management systems when adapting platform protocols by adding and closing comparisons



Dominic Hague<sup>1,2†</sup>, Stephen Townsend<sup>1,2</sup>, Lindsey Masters<sup>1,2</sup>, Mary Rauchenberger<sup>1,2</sup>, Nadine Van Looy<sup>1,2</sup>, Carlos Diaz-Montana<sup>1,2</sup>, Melissa Gannon<sup>1,2</sup>, Nicholas James<sup>3</sup>, Tim Maughan<sup>5</sup>, Mahesh K. B. Parmar<sup>1,2</sup>, Louise Brown<sup>1,2</sup>, Matthew R. Sydes<sup>1,2</sup> and for the STAMPEDE and FOCUS4 investigators

Questions?

