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What's it going to take to get your study started? Pilot and feasibility studies

Sabine Braat

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research Hub (MISCH)

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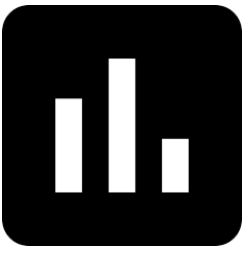
BIostatISTICIAN
Vanessa Pac Soo



BIostatISTICIAN
Hannah Johns



BIostatISTICIAN
Karen Lamb



Do you have experience with pilot and feasibility studies?

- ☐ Yes
- ☐ No
- ☐ Not sure

EDITORIAL

Open Access

Pilot and feasibility studies come of age!

Gillian A Lancaster

Abstract

This editorial introduces the new, online, open-access journal *Pilot and Feasibility Studies*. The journal considers manuscripts on any aspect of the design and analysis of pilot and feasibility studies, as well as protocols for pilot and feasibility studies, and discussions and reviews of methodological issues around the planning and reporting of such studies. These studies are generally carried out in preparation for future large-scale definitive randomised controlled trials or observational studies and address key issues of uncertainty. Objectives for conducting pilot and feasibility studies therefore differ from those of the future large-scale study and should be clearly expressed. The journal provides a dedicated place for publication of this important work as well as a forum for discussion of methodological issues that will lead to increased scientific rigour in this area.



What we all want...

The NEW ENGLAND JOURNAL of MEDICINE

2018

ORIGINAL ARTICLE

Restrictive versus Liberal Fluid Therapy for Major Abdominal Surgery

P.S. Myles, R. Bellomo, T. Corcoran, A. Forbes, P. Peyton, D. Story, C. Christophi, K. Leslie, S. McGuinness, R. Parke, J. Serpell, M.T.V. Chan, T. Painter, S. McCluskey, G. Minto, and S. Wallace, for the Australian and New Zealand College of Anaesthetists Clinical Trials Network and the Australian and New Zealand Intensive Care Society Clinical Trials Group*

RESEARCH



OPEN ACCESS

BMJ 2018

Preoperative physiotherapy for the prevention of respiratory complications after upper abdominal surgery: pragmatic, double blinded, multicentre randomised controlled trial

Ilanthe Boden,^{1,2} Elizabeth H Skinner,^{2,3} Laura Browning,^{2,3} Julie Reeve,^{4,5} Lesley Anderson,⁵ Cat Hill,⁶ Iain K Robertson,^{7,8} David Story,⁹ Linda Denehy^{10,11}

But maybe start with this...

Downloaded from <http://bmjopen.bmj.com/> on May 25, 2017 - Published by group.bmj.com

Open Access

Protocol

BMJ Open Restrictive versus liberal fluid therapy in major abdominal surgery (RELIEF): rationale and design for a multicentre randomised trial

Kemp et al. *Pilot and Feasibility Studies* (2018) 4:16
DOI 10.1186/s40814-017-0157-4

Pilot and Feasibility Studies

RESEARCH

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A pilot randomised clinical trial of physiotherapy (manual therapy, exercise, and education) for early-onset hip osteoarthritis post-hip arthroscopy



Definitions of a pilot study

All types of research

- Clinical trials
- Observational studies
- Public health
- Health services
- Basic science
- Implementation research

Definition*

A trial study carried out before a research design is finalised to assist in defining the research question or to test the feasibility, reliability and validity of the proposed study design

A smaller version of a study is carried out before the actual investigation is done. Researchers use information gathered in pilot studies to refine or modify the research methodology for a study and to develop large-scale studies

A small scale study conducted to test the plan and method of a research study.

A small study carried out before a large-scale study to try out a procedure or to test a principle

An experimental use of a treatment in a small group of patients to learn if it will be effective and safe on a broad scale

The initial study examining a new method or treatment

A small study often done to assist the preparation of a larger, more comprehensive investigation.

Small, preliminary test or trial run of an intervention, or of an evaluation activity such as an instrument or sampling procedure. The results of the pilot are used to improve the program or evaluation procedure being piloted before it is used on a larger scale.

Uses of pilot and feasibility studies

- The goal of pilot work is **not to test hypotheses** about the effects of an intervention, but rather, to assess the feasibility/acceptability of an approach to be used in a larger scale study.
- Not answering the question “Does this intervention work?” Instead gather information to help answer “**Can I do this?**”.
- Usually intended to inform a definitive randomised controlled trial or observational study.

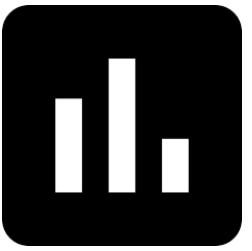
Pilot and feasibility studies

- While funding may be sought for pilot or feasibility studies, it is important to be clear on what these studies are seeking to determine and why a fully powered study cannot be conducted at this stage.
- A well-designed pilot and feasibility study should improve the quality of the final study.
- Importantly, a pilot study is not a study which is too small to provide an answer to the research question.

Why bother?

- **Reduce uncertainty** to improve the chances of the main study producing valuable evidence.
- Give advance warning of where a main study could fail.
- Help to **avoid wasting resources and time** on larger studies that are unlikely to answer the intended research question.
- Provides **feasibility for grant applications** for the future trial.





Is the following a feasibility or pilot outcome?

Question 1

Proportion of participants completing the study

- ☐ Yes
- ☐ No
- ☐ Not sure

Question 2

Estimate of the risk of the outcome event for sample size calculation of future trial

- ☐ Yes
- ☐ No
- ☐ Not sure

Feasibility study

A **feasibility study** asks **whether something can be done**, should we proceed with it, and if so, how. They may also be used to **estimate important parameters** that are needed to design a larger study.

Can I recruit my target population?

Number screened per month; number enrolled per month; average time delay from screening to enrollment; average time to enroll enough participants to form classes (group-based interventions)

Can I randomize my target population?

Proportion of eligible screens who enroll; proportion of enrolled who attend at least one session

Can I keep participants in the study?

Treatment-specific retention rates for study measures; reasons for dropouts

Will participants do what they are asked to do?

Treatment-specific adherence rates to study protocol (in-person session attendance, homework, home sessions, etc.); treatment-specific competence measures



Feasibility study

A **feasibility study** asks **whether something can be done**, should we proceed with it, and if so, how. They may also be used to **estimate important parameters** that are needed to design a larger study.

Can the treatment(s) be delivered per protocol?	Treatment-specific fidelity rates
Are the assessments too burdensome?	Proportion of planned assessments that are completed; duration of assessment visits; reasons for dropouts
Are the treatment conditions acceptable to participants?	Acceptability ratings; qualitative assessments; reasons for dropouts; treatment-specific preference ratings (pre- and postintervention)
Are the treatment conditions credible?	Treatment-specific expectation of benefit ratings



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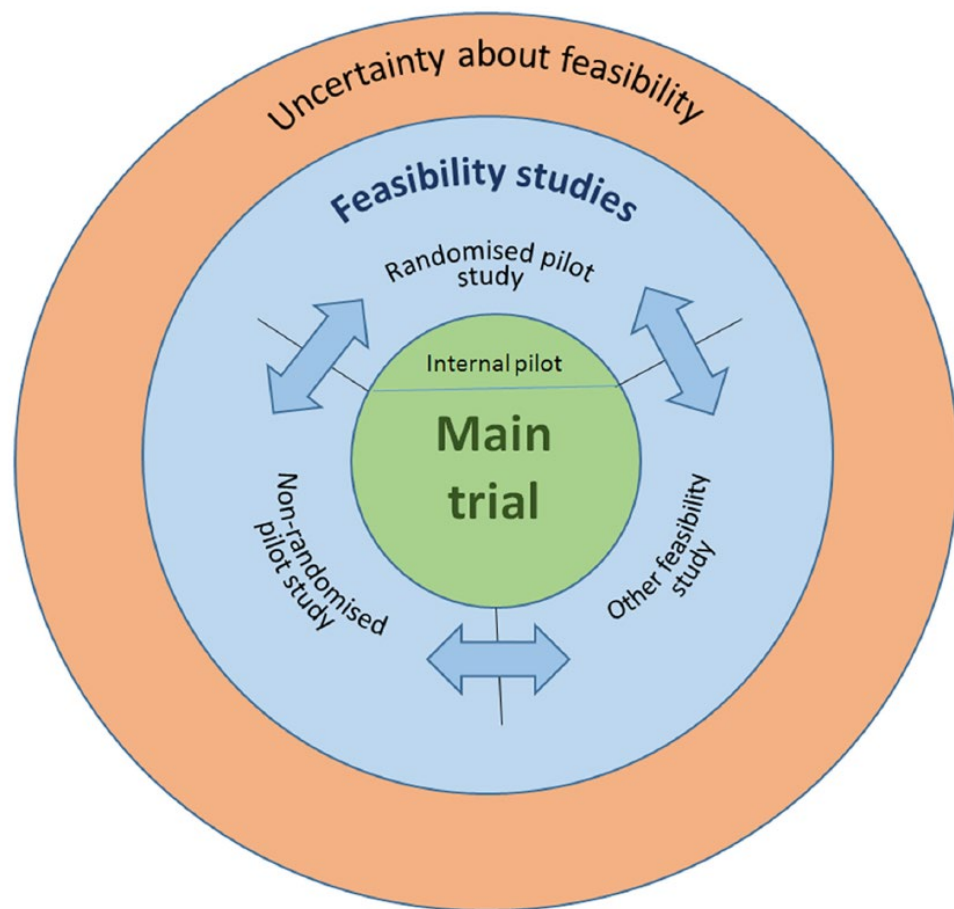
Pilot study



A **pilot study** are a type of feasibility study whereby in a pilot study a **future study**, or part of a future study, is **conducted on a smaller scale**.

- **Internal** pilot study:
 - Part of the larger study (e.g. run-in), data is include in the analysis of the larger trial.
 - To provide a check on the adequacy of the sample size calculation.
- **External** pilot study:
 - Independent from the larger study, data does not contribute to main trial.
 - To test all aspects of the integrity of a study protocol and feasibility of the intervention and of the trial design.

Pilot or Feasibility?



- Pilot studies are a subset of feasibility studies.
- **Pilot** study tests all aspects of the future protocol as a whole and how the components of the protocol work together and can be:
 - Randomised
 - Non-randomised
- **Feasibility** study that is not a pilot study might test various subsets of the future protocol but not the protocol as a whole, typically do not implement the intervention.

Study design of a pilot study

Should a pilot be randomised?

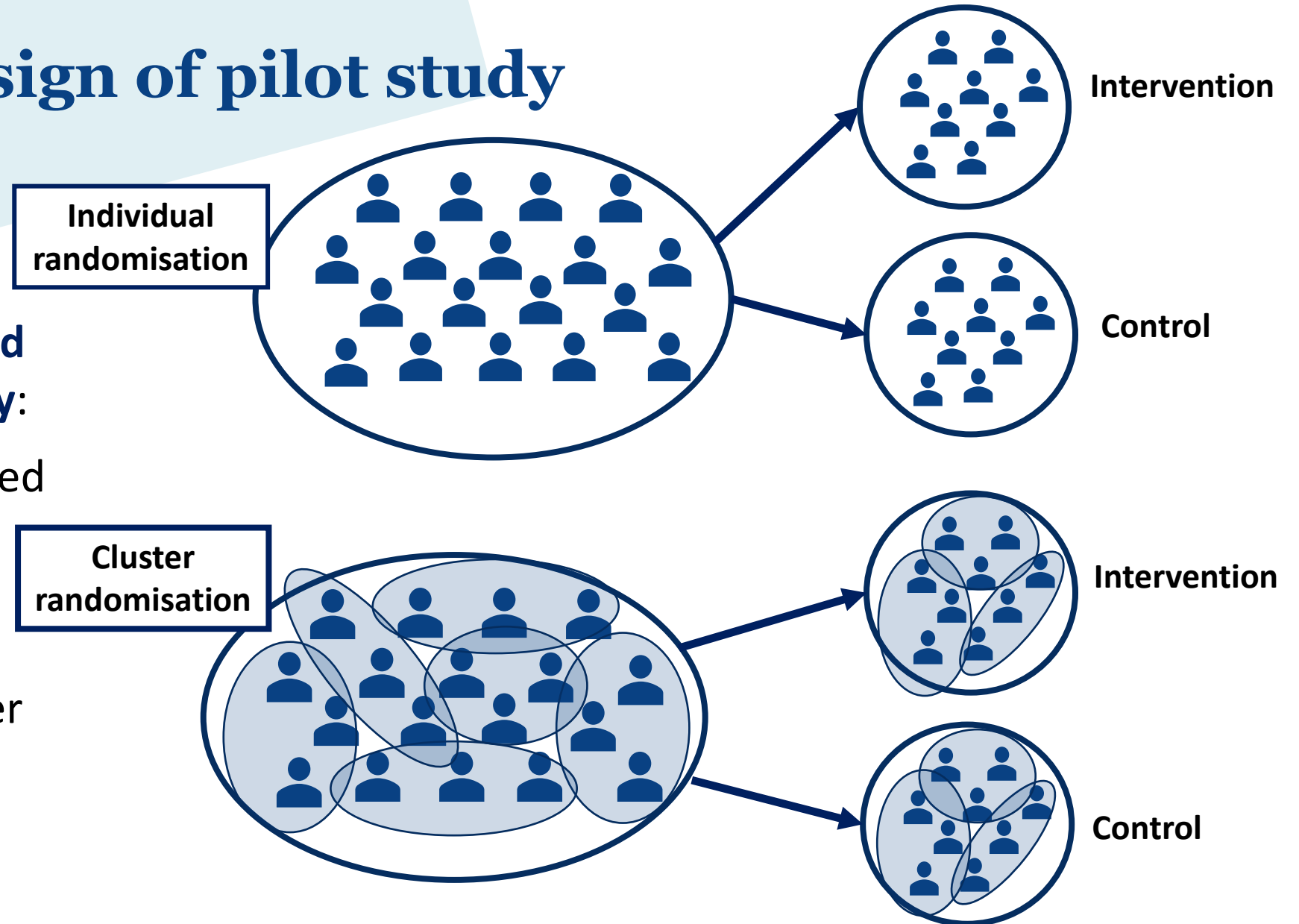
- **Randomised:**
 - Future randomised design on a smaller scale.
 - To check that the study processes (e.g. recruitment, randomisation, intervention, follow-up assessments) all run smoothly.
- **Non-randomised:**
 - Similar aims to randomised but without randomisation of the participants.
 - May give misleading data on recruitment, retention, ...

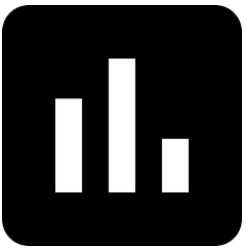


Study design of pilot study

Generally should be
**similar to the anticipated
design of the main study:**

- Individually randomised
- Cross-over
- Cluster randomised
- Stepped wedge cluster
- ...





**What is a sufficient sample size for
a two-group individually randomised pilot and feasibility study?**

- 12 participants per group
- 30 participants per group
- 50 participants per group
- As many as my funds allow
- As many as I can recruit
- Don't know, need to talk to a statistician

Sample size – what NOT to do



“The throughput of the clinic is around 50 patients a year, of whom 10% may refuse to take part in the study. Therefore over the 2 years of the study, the sample size will be 90 patients.”

- This is valuable information to include in your protocol and helps highlight the **feasibility** of the study being undertaken.
- It does **NOT** mean your study will be **able to achieve its objectives**.
- A sample size calculation is still preferred.
- If the sample size is too small to obtain precise results, you may want to extend the study length or collaborate with other centres.

Sample size – Feasibility outcome

Feasibility study objective:

To estimate the proportion of participants completing the study

- Relate the sample size to the width of the (two-sided) 95% confidence interval.
- For example:

“With a sample size of 120, we will be able to estimate a follow-up proportion of 90% to within a 95% confidence interval of +/- 5% (i.e. 95% CI 85% to 95%)”

Sample size – Feasibility outcome

Feasibility study objective:

To estimate the proportion of participants completing the study

Sample size	95% confidence interval
30	79% to 100%
60	82% to 98%
90	84% to 96%
120	85% to 95%

“With a sample size of 120, we will be able to estimate a follow-up proportion of 90% to within a 95% confidence interval of +/- 5% (i.e. 95% CI 85% to 95%)”

Sample size – Pilot outcome

Pilot study objective:

Estimate of the risk of the outcome event for sample size calculation of future trial

Teare et al. *Trials* 2014, **15**:264
<http://www.trialsjournal.com/content/15/1/264>



RESEARCH

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Sample size requirements to estimate key design parameters from external pilot randomised controlled trials: a simulation study

M Dawn Teare*, Munyaradzi Dimairo, Neil Shephard, Alex Hayman, Amy Whitehead and Stephen J Walters

- At least 70 subjects (35 per group) when estimating the standard deviation for a continuous outcome.
- At least 60 subjects (120 in total) when estimating the event rate in an intervention group.

Sample size



- Pilot and feasibility studies are not designed to test effectiveness of intervention, therefore power-based sample size calculations may not be relevant.
- All studies should have a sample size justification. Not all studies however need to have sample size calculation.
- Sample size approaches depend on the objective.
- **Statisticians can assist with exploring the sample size for your pilot and feasibility study.**

Success criteria and study outcome

Criteria for success:

- State the criteria for success or feasibility.
- Should be based on key feasibility aims.

For example:

- **Objective:** To estimate the proportion of participants completing the study.
- **Criterion for success:** Feasible if complete follow-up for at least 85% of the participants.

Outcome:

- Stop – main study not feasible.
- Continue with modifications.
- Continue without modifications.
- Continue without modifications but monitor closely.



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Example 1 – Pilot and feasibility study

Patients: Post-hip arthroscopy surgery patients
Intervention: Physiotherapy
Control: Health-education
Outcome: Several feasibility outcomes
Time: 12 weeks follow-up
study design: Randomised controlled trial in one hospital (Australia)

Objective: To evaluate the feasibility of a randomised controlled trial investigating a targeted physiotherapy intervention for early-onset hip osteoarthritis post-hip arthroscopy.

Outcomes: Willingness to participate, adherence, follow-up.

Example 1 – Pilot and feasibility study

Feasibility outcomes – **quantitative**:

- 17/48 (35%) eligible patients randomised.
- 100% adherence to intervention.
- No losses to follow-up.

Feasibility outcomes – **qualitative**:

*“Feedback from **participants and physiotherapists** indicated that future studies would be improved through **additional supervision of exercise programs to enhance compliance and motivation**, as well as offering a **wider range of appointment times to fit with patients’ work and family responsibilities.**”*

Example 2 – Pilot and feasibility study

Patients: Patients ≥ 40 years elective or expedited abdominal or intrathoracic surgery
Intervention: Sugammadex
Control: Neostigmine
Outcome: Several feasibility outcomes
Time: 3-months follow-up
study design: Randomised controlled trial in two hospitals (Australia and Hong Kong)

Objective: To determine the feasibility of a large international randomised controlled trial of sugammadex, neostigmine and postoperative pulmonary complications in adult patients having abdominal and intrathoracic surgery.

Outcomes: Recruitment, crossover, completeness, acceptability, and workload.

Example 2 – Pilot and feasibility study



- Recruitment of 120/150 (80%) (95% confidence interval [CI] 73 to 86%).
- No crossover 115/117 (98%) patients who received reversal (95% CI 94 to 100%).
- The protocol was acceptable or highly acceptable to the anaesthetist in 108/116 (93%) cases (95% CI 87 to 97%; missing = 4).
- Four patients of the 120 (3.3%) patients were lost to follow-up at 3 months (95% CI 0.9 to 8.3%).
- Case report forms were complete at 3 months for all remaining patients.
- The median time to complete trial processes was 3.5 h (range 2.5–4.5 h).
- Trial coordinators reported no barriers to trial processes.

Example 3 – Feasibility study

Patients: Multimorbid hospitalized subjects with community-acquired pneumonia
Intervention: None
Control: None
Outcome: Recruitment, patient reported outcomes (EQ-5D-5L, LLFDI, CAP-Sym)
Time: 90-day follow-up
study design: Prospective observational study in one hospital (Australia)

Objective: To investigate the feasibility and limitations of three credible PROM instruments in a representative hospitalized cohort to identify potential barriers to routine application.

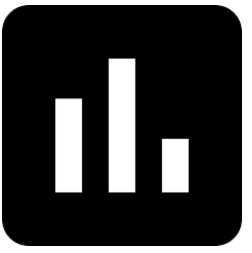
Outcomes: Willingness to participate, missing data, ease of use, floor/ceiling effects.

Example 3 – Feasibility study

- Of the 82 CAP patients admitted during the recruitment period, **24 (29%) were unable to participate** most commonly due to either limited English (n = 17, 21%) and/or cognitive impairment (n = 12, 15%), and **a further 14 (17%) declined to participate**.
- All 44 participants successfully completed required inpatient assessments, with **10 (23%) and 17 (39%) lost to follow-up at 30 and 90 days**, respectively.

Table 2 Patient-reported outcome measure completion time and missing data by instrument and mode of collection

		EQ-5D	LLFDI	CAP-Sym 18
Completion time (minutes) (median, [IQR])	Admission face-to-face (n = 44)	3 [2–5]	10 [6–11]	4 [2.5–5]
	30-day phone (n = 34)	3 [2–5]	10 [7–13]	3 [2–5]
Missing data (n/N * (%))	Instrument level	Face-to-face	0/87 (0%)	0/44 (0%)
		Phone	20/78 (25.6%)	20/78 (25.6%)
		Mail	12/20 (60.0%)	14/20 (70.0%)
	Item level: No. of items with > 5% missing values	Face-to-face	0/6 (0%)	0/18 (0%)
		Phone	0/6 (0%)	0/18 (0%)
		Mail	0/6 (0%)	0/18 (0%)



A pilot and feasibility study can be run on a shoestring

- ☐ Yes
- ☐ No
- ☐ Maybe

Misconceptions of pilot and feasibility studies

- **Does not require much funding**
 - Funding should account for hospital staff, database, trial manager, statisticians, ...
- **Can be conducted by a student/intern**
 - While a good learning experience for a student/intern, the required resources may be more than available (e.g. preparing all required paper work).
- **Can be completed quickly**
 - While the sample size may be small, the timeframe may be longer than anticipated (e.g. slow recruitment).
- **Is a single site study**
 - Single-site may be easier to conduct but less generalisable to the main trial.
 - Multi-site may allow to test the feasibility of running a larger multi-site trial.

Misuses of pilot studies

- **Attempting to assess safety/tolerability of a treatment**
 - Due to small sample size, generally cannot provide useful information in particular when the safety concerns are rare.
 - If safety concerns are observed, report 95% confidence intervals alongside.
- **Seeking to provide a preliminary test of the research hypothesis**
 - Due to small sample size, uncertainty around result.
 - Also uncertainty around the intervention, may differ from that in the larger study.
- **Estimating treatment effect sizes for power calculations of the larger scale study**
 - Due to small sample size, estimate is generally unstable.

Misuses of pilot studies



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Pilot study treatment effect size small:

Type II error or False negative result

Potential consequences:

- May conclude that the intervention does not work and not pursue future study.
- If proceed to future study, then sample size may be large based on small effect.
- Large sample size of future study may make it difficult to find funding.

Pilot study treatment effect size large:

Type I error or False positive result

Potential consequences:

- If proceed to future study, then sample size may be small based on large effect.
- Small sample size of future study may make it difficult to find clinically relevant effect.

Reporting guideline

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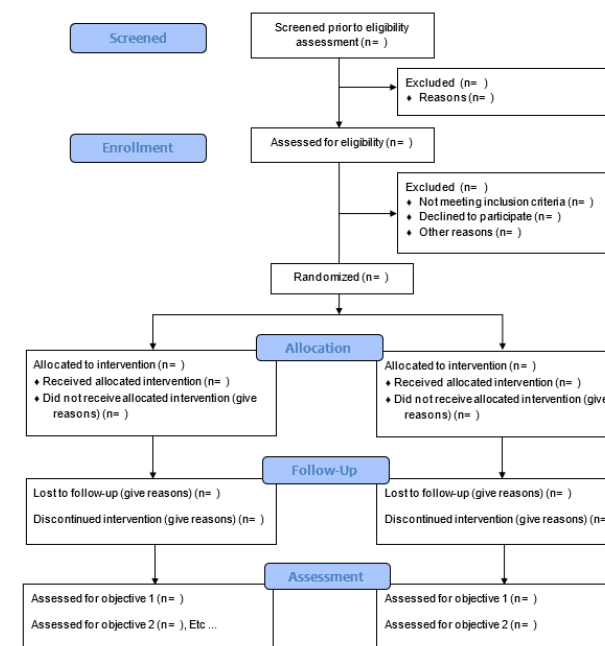
CONSORT 2010 statement: extension to randomised pilot and feasibility trials

Sandra M. Eldridge^{1*}, Claire L. Chan¹, Michael J. Campbell², Christine M. Bond³, Sally Hopewell⁴, Lehana Thabane⁵, Gillian A. Lancaster⁶ and on behalf of the PAFS consensus group



CONSORT 2010 checklist of information to include when reporting a pilot or feasibility trial*

Section/Topic	Item No	Checklist item	Reported on page No
Title and abstract			
	1a	Identification as a pilot or feasibility randomised trial in the title	
	1b	Structured summary of pilot trial design, methods, results, and conclusions (for specific guidance see CONSORT abstract extension for pilot trials)	
Introduction			
Background and objectives	2a	Scientific background and explanation of rationale for future definitive trial, and reasons for randomised pilot trial	
	2b	Specific objectives or research questions for pilot trial	
Methods			
Trial design	3a	Description of pilot trial design (such as parallel, factorial) including allocation ratio	
	3b	Important changes to methods after pilot trial commencement (such as eligibility criteria), with reasons	
Participants	4a	Eligibility criteria for participants	
	4b	Settings and locations where the data were collected	
	4c	How participants were identified and consented	
Interventions	5	The interventions for each group with sufficient details to allow replication, including how and when they were actually administered	
Outcomes	6a	Completely defined prespecified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed	
	6b	Any changes to pilot trial assessments or measurements after the pilot trial commenced, with reasons	
	6c	If applicable, prespecified criteria used to judge whether, or how, to proceed with future definitive trial	
Sample size	7a	Rationale for numbers in the pilot trial	
	7b	When applicable, explanation of any interim analyses and stopping guidelines	
Randomisation:			
Sequence generation	8a	Method used to generate the random allocation sequence	
	8b	Type of randomisation(s); details of any restriction (such as blocking and block size)	
Allocation concealment mechanism	9	Mechanism used to implement the random allocation sequence (such as sequentially numbered containers), describing any steps taken to conceal the sequence until interventions were assigned	




Publishing

- Results of a pilot study can be published.
- Publishing is important because:
 - Provide information about feasibility to the wider research community.
 - Avoid duplication by someone else
 - Ethical and scientific obligation.
- Emphasis is often wrongly placed on statistical significance.
- Should be explicit mentioning that aim is feasibility to inform future studies.




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Research | 30 March 2022

Restrictive Fluid Administration vs. Standard of Care in Emergency Department Sepsis Patients (REFACED Sepsis)—protocol for a multicenter, randomized, clinical, proof-of-concept trial

Marie Kristine Jessen, Lars Wiuff Andersen, Marie-Louise Holm Thomsen, Peter Kristensen, Wazhma Hayeri, Ranva Espegård Hassel, Anders Perner, Jens Aage Kølsen Petersen and Hans Kirkegaard

Study Protocol | 29 March 2022

Aims and scope

Pilot and Feasibility Studies encompasses all aspects of the design, conduct and reporting of pilot and feasibility studies in biomedicine. The journal publishes research articles that are intended to directly influence future clinical trials or large scale observational studies, as well as protocols, commentaries and methodology articles. The journal also ensures that the results of all well-conducted, peer-reviewed, pilot and feasibility studies are published, regardless of outcome or significance of findings.

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Implementation science and practice

We are pleased to announce the thematic series Implementation Science and Practice has been published. Guest edited by Professor Dr. Michael A. G. Huisman.

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Aims and scope

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Speed

97 days to first decision for all manuscripts
141 days to first decision for reviewed manuscripts only
251 days from submission to acceptance
22 days from acceptance to publication


Example

Leslie et al. *Pilot and Feasibility Studies* (2021) 7:200
<https://doi.org/10.1186/s40814-021-00942-9> Pilot and Feasibility Studies

RESEARCH Open Access

Sugammadex, neostigmine and postoperative pulmonary complications: an international randomised feasibility and pilot trial

Kate Leslie^{1,2*}, Matthew T. V. Chan³, Jai N. Darvall^{1,2}, Anurika P. De Silva^{4,5}, Sabine Braat^{4,5}, Nancy J. Devlin⁶, Philip J. Peyton^{1,7}, Jade Radnor⁸, Carmen K. M. Lam⁹, Sofia Sidiropoulos^{1,7} and David A. Story^{1,7}



Day 1

- Submitted

Day 12

- Accepted

Day 23

- Published

Conclusions

Feasibility and pilot studies:

- Enhance research design.
- Avoid wasting time and money.
- Short term, publishable research
 - Positive or negative
- Good for Honours / Masters.
- Good for smaller grant funding.





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Next MACH webinar

11th May

Statistics for your grant applications

Prof. Julie Simpson, director of MISCH

Thank you

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