Beyond the CONSORT extension for pilot trials: guideline, planning, abstracts and protocol

ICTMC workshop
Sunday 6 October 2019



Welcome and introduction to workshop

Sandra Eldridge

The team

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Time	Minutes	Topic	Lead
09.00 – 09.10	10	Welcome and introduction to workshop	Sandra
09.10 – 09.30	20	**Small Group:** participants introduce own pilot and feasibility study	Sandra
09.30 – 09.45	15	Framework for defining pilot and feasibility studies	Claire
09.45 – 10.05	20	**Small Group:** participants discuss how own examples fit within framework	Claire
10.05 – 10.20	15	Objectives of pilot and feasibility studies	Gill
10.20 – 10.35	15	CONSORT extension for pilot and feasibility trials – overview of checklist items	Christine
10.35 – 10.55	20	**Small Group:** focusing on participants' examples and how different parts of the CONSORT extension would work for different trials	Christine / Gill
10.55 – 11.15	20	COFFEE BREAK	
11.15 – 11.30	15	Sample size, progression criteria and analysis	Sandra
11.30 – 11.50	20	**Small Group:** focusing on participants' examples and how different parts of the CONSORT extension would work for different trials	Sandra
11.50 – 12.05	15	Guidance on flow diagrams and writing abstracts	Sally
12.05 – 12.35	30	**Group Exercise:** using the CONSORT extension to assess completeness of pilot trial reporting	Sally
12.35 – 12.50	15	Guideline on planning pilot and feasibility studies and writing study protocols	Lehana
12.50 – 13.00	10	Future plans and close	Lehana

Small group: participants introduce own pilot and feasibility study

Sandra Eldridge

Framework for defining pilot and feasibility studies

Claire Chan

Background

Issues:

1. Large and growing number of studies in the literature called feasibility or pilot studies
2. Terms pilot and feasibility (and other terms eg exploratory, preliminary, small…) used inconsistently
3. Evidence that poorly conducted
4. Evidence that poorly reported **and** difficult to get published

Aims of team

Work on studies prior to randomised controlled trials…

Initial:

- To provide reporting guidelines for pilot and feasibility studies (two checklists)

(UK National Institute for Health Research mutually exclusive definitions)

Eventual:

- To develop a conceptual framework for pilot and feasibility studies
- To provide a CONSORT extension for pilot trials

Conflicting ideas amongst funders, the literature and research community

Terminology – literature & funders

Arain et al. (2010) 'feasibility' studies had **slightly different characteristics** from those described as 'pilot'

Thabane et al. (2010) common idea from health websites of conducting a preliminary study "*a pilot study **is synonymous with** a feasibility study intended to guide the planning of a large scale investigation*"

Feasibility studies and pilot studies are different

NHS National Institute for Health Research

Pilot studies: "***a smaller scale version** of the main study used to test whether the components of the main study can all work together…"*

*Feasibility studies: "are pieces of research done before a main study in order…. **to answer the question "Can this study be done?"**….. used to estimate important parameters ….needed to design the main study……"*

Pilot studies and all other types of feasibility studies under one umbrella

MRC Medical Research Council

*"A pilot study **need not be a 'scale model'** of the planned main stage evaluation, but **should address the main uncertainties** that have been identified in the development work."*

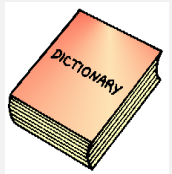
Terminology – research community and dictionary

"….. study was both feasibility and pilot study"

"Well nobody uses the definitions so it doesn't seem to matter, also there are many more terms used"

"The definitions are taken from the funders so how can you change them?"

Pilot study: A small-scale experiment or set of observations undertaken to decide **how and whether to launch a full-scale project**

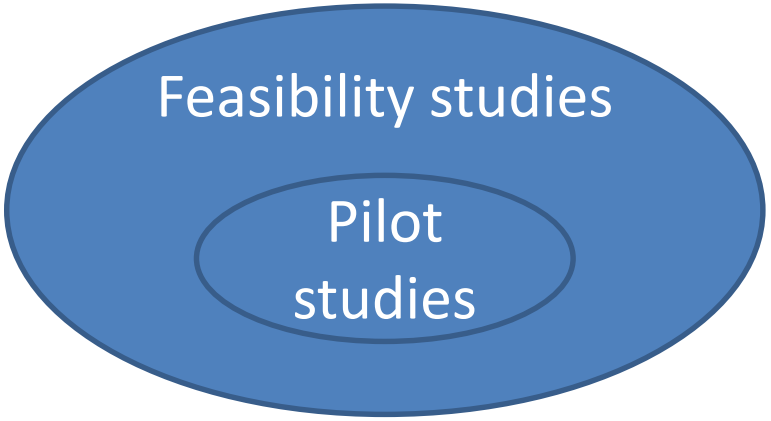


Feasibility study: Looks at the viability of an idea with an emphasis on identifying potential problems and attempts to answer one main question: **will the idea work and should we proceed with it**

Our definitions

RCT?
Intervention?

- **A feasibility study** asks whether **something** can be done, should we proceed with it, and if so, how.
- **A pilot study** asks the same questions but also has a specific design feature: in a pilot study a future study, or part of a future study, is conducted on a smaller scale.
- ***Corollary***: all pilot studies are feasibility studies but not all feasibility studies are pilot studies



Examples

To **assess feasibility** of RCT of management of reduced fetal movement (Heazell et al. BMC Preg Childbirth 2013)

- Recruitment , retention, accep
  prevalence of poor perinatal c

Randomised pilot study

To **pilot an intervention** to avoid the use of syringes and contamination of materials amongst injecting drug users (Colon et al. AIDS Behav.

- Adoption of each of four com
- Whether pre-post changes in
  intervention merited further testing

Non - randomised pilot study

To **determine feasibility** of RCT comparing operative with non-operative trea
impingement surgery (Pa

- Surgeon and patient opinion

Feasibility study, not a pilot study

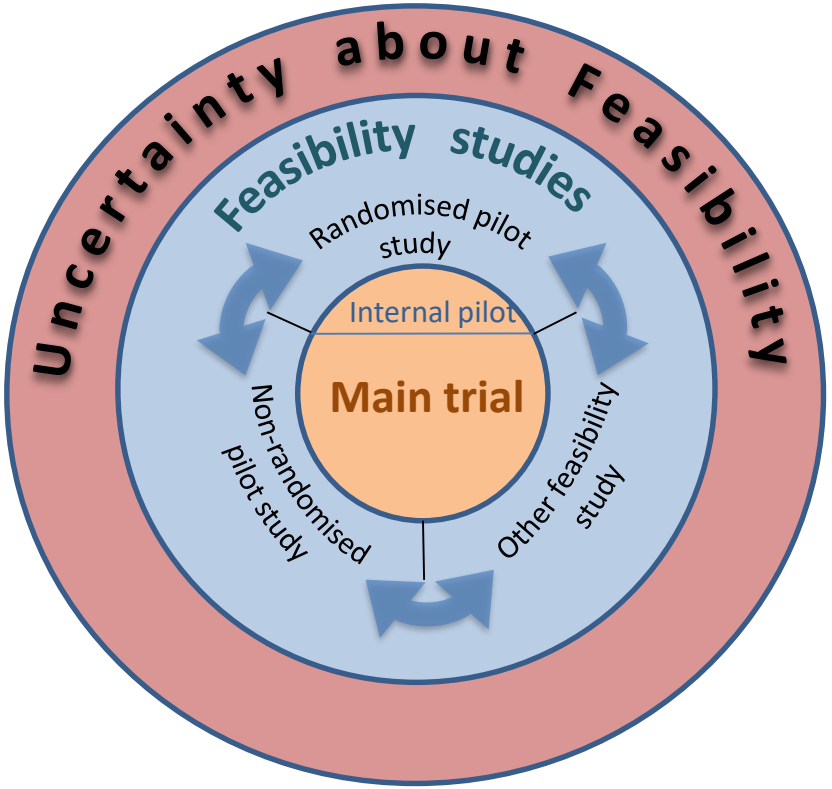
Conceptual framework

Focus on blue part

Feasibility overarching concept

Three distinct types of feasibility study

Non-linear development

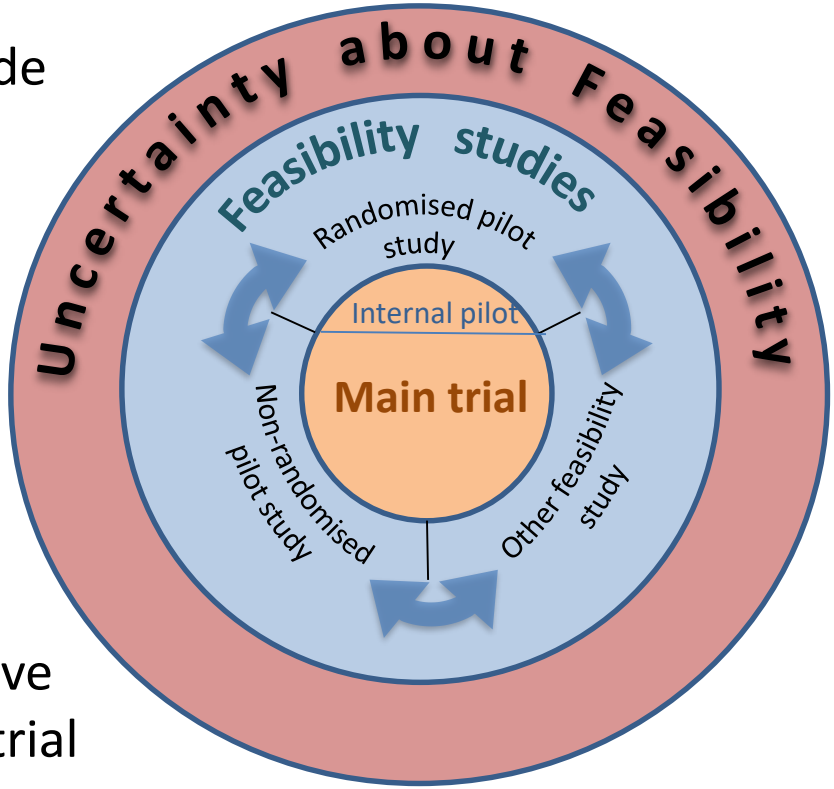


Conceptual framework

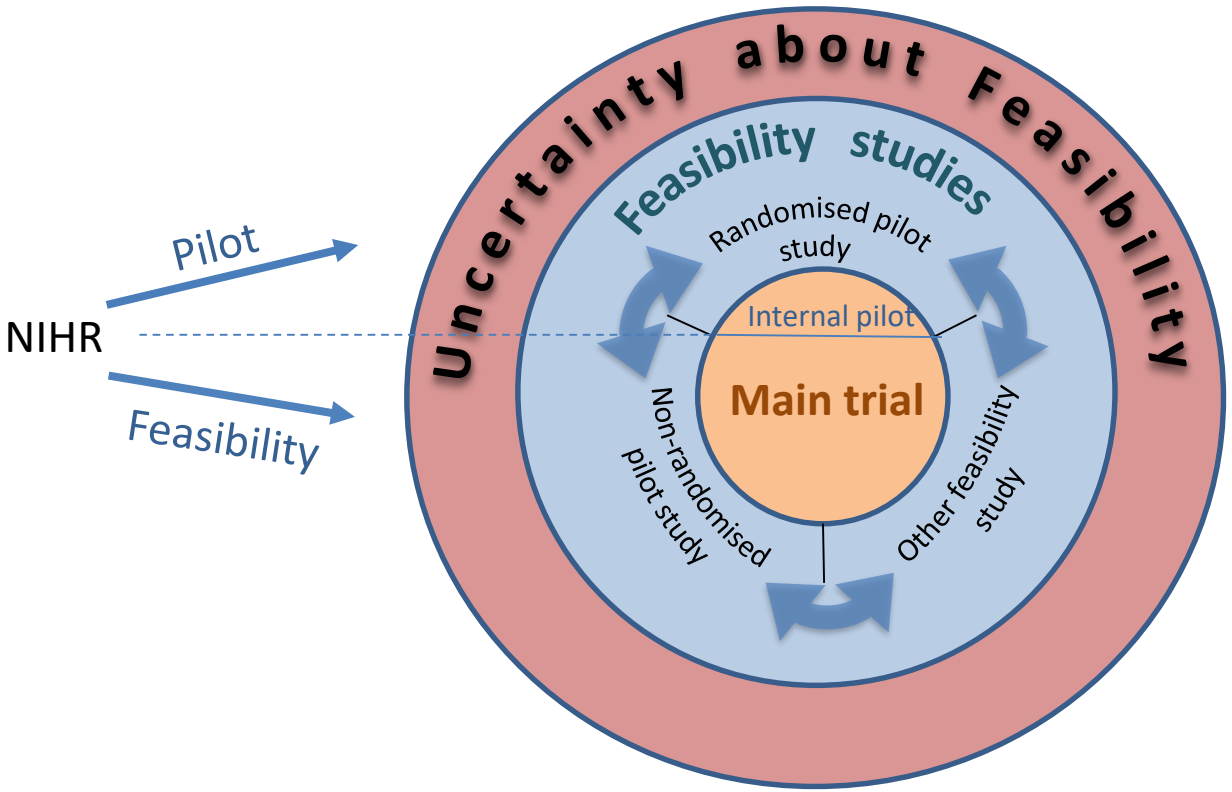
Start at the outside with uncertainty

Choose most appropriate feasibility study

Continue with feasibility studies until ready to move inwards to main trial



Conceptual framework



MRC: "Pilot study need not be a 'scale model' of the planned main-stage evaluation, but should address the main uncertainties that have been identified in the development work"

PLOS ONE

RESEARCH ARTICLE

Defining Feasibility and Pilot Studies in Preparation for Randomised Controlled Trials: Development of a Conceptual Framework

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Small group: participants discuss how own examples fit within framework

Claire Chan

Objectives of pilot and feasibility studies

Gill Lancaster

Main uncertainties in future trial?

Design	Population	Setting & Recruitment
Intervention	Outcomes	Stopping rules
Randomisation type	Allocation concealment	Randomisation implementation
Blinding	Similarity of interventions	Statistical methods

(broadly following CONSORT statement items)

Empirically from a review in 2004

(Lancaster et al, JECP 2004)

i. Test integrity of study protocol
ii. Sample size calculation inputs
iii. Pilot data collection forms/questionnaires
    - Prepare and plan data collection and monitoring
iv. Acceptability of the intervention
    - Develop and test implementation and delivery of the intervention
    - Train staff in delivery and assessment
v. Selection of most appropriate outcome measures (endpoints)
vi. Recruitment and consent rates
vii. Randomisation procedure

Example 1 - Lifestyle referral assessment in an acute cardiology setting protocol

(Hill et al. Trials 2013)

"The main aim ……to assess the **feasibility of conducting a definitive trial** in terms of recruitment, use and acceptability of the intervention, follow-up at 3 and 6 months, and data collection methods.

….to establish **suitable procedures** for delivering the intervention and conducting assessments and procedures for ensuring recruitment and retention in the study.

……the **acceptability of the assessment tool** to patients in an acute cardiology setting as well as **patients' experiences of making lifestyle changes** in order to develop effective recruitment and retention strategies."

Example 2 - Nail bed INJury Assessment Pilot (NINJA-P) protocol

(Jain et al. Pilot and Feasibility Studies 2015)

Feasibility measures are as follows:

- Number of potentially eligible children
- Number of patient/parents and guardian's approached to take part in the study
- Proportion of children for whom consent was sought which took part in the study
- Proportion of children who received the allocated treatment and reasons for any non-compliance
- Proportion of participants with a valid response at each follow-up point (for 4-month time point both overall and only by method of follow-up)

Patient-centred outcome measures are as follows:

- Presence of post-operative complications at 2 weeks and 30 days
- Cosmetic appearance of the nail at 4 months
- Level of pain experienced by child at 1st dressing change at 2 weeks

Example 2 - Nail bed INJury Assessment Pilot (NINJA-P) protocol

(Jain et al. Pilot and Feasibility Studies 2015)

Secondary study objectives are to inform the design and conduct of the main trial:

- Identify any conflicts or areas of concern for the research pathway compared with the existing standard clinical pathway
- Assess suitability of outcome measures for children in this setting
- Quantify event proportion and variability data to help inform a sample size calculation for main study

Remember: methods of analysis - should address each feasibility objective (primary and secondary)

Feasibility eg. recruitment, adherence

Patient-centred eg. data collection

Examples of necessary external pilots

FEMUR – thinking of **randomising primary care groups** (in the 1990s) to see if a whole systems approach could reduce falls in older people

UK BEAM – thinking of **cluster randomising** and recruiting back pain patients from general practices (the clusters) after randomisation

PreDove – thinking of randomising general practices, to evaluate an intervention to reduce depression amongst **victims of domestic violence**

COMQUOL – thinking of randomising in **secure mental health wards**

Example 3 – UK BEAM Trial

(Farrin et al Clinical Trials 2005)

- UK Back Pain, Exercise, Active management and Manipulation trial
- To test the integrity of the study protocol using a series of sub-studies
- Planned as cluster randomised trial
- 3 treatments – active management (practice level); spinal manipulation and exercise (patient level) – 3 x 2 x 2 factorial design
- Qualitative and quantitative pilot work
  - Views, acceptability and needs of support staff
  - Sample size, staff training, data collection processes, treatment delivery

Example 3 – UK BEAM Trial

(Farrin et al Clinical Trials 2005)

Findings:

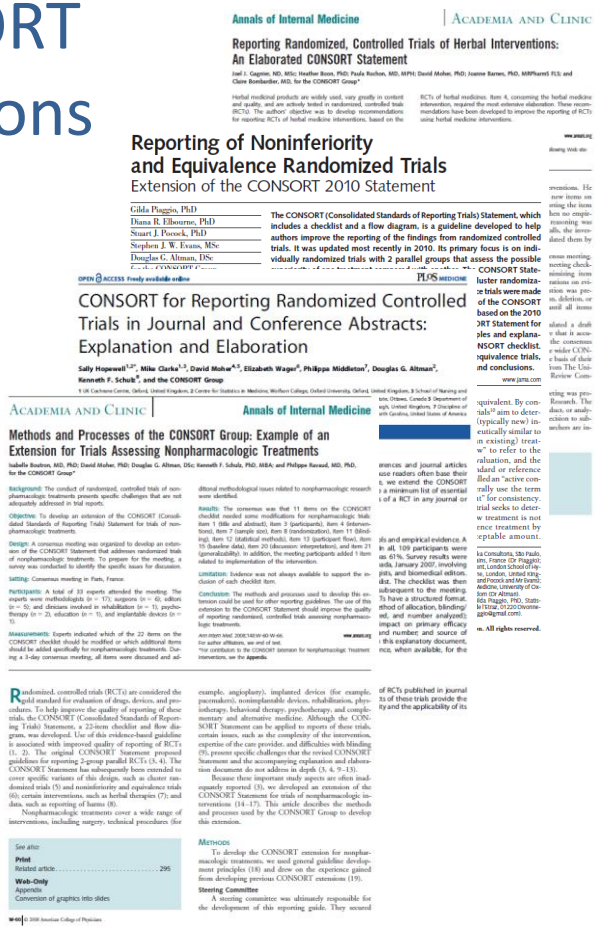
- Majority of methods were successful but highlighted where changes were needed
- Problem with differential recruitment between practices
- Twice as many recruited to intervention arm (active management) than control
- Less severe back pain, less depression, higher education, more in full-time work in intervention group than control at baseline

  ➔ changed to non-clustered design

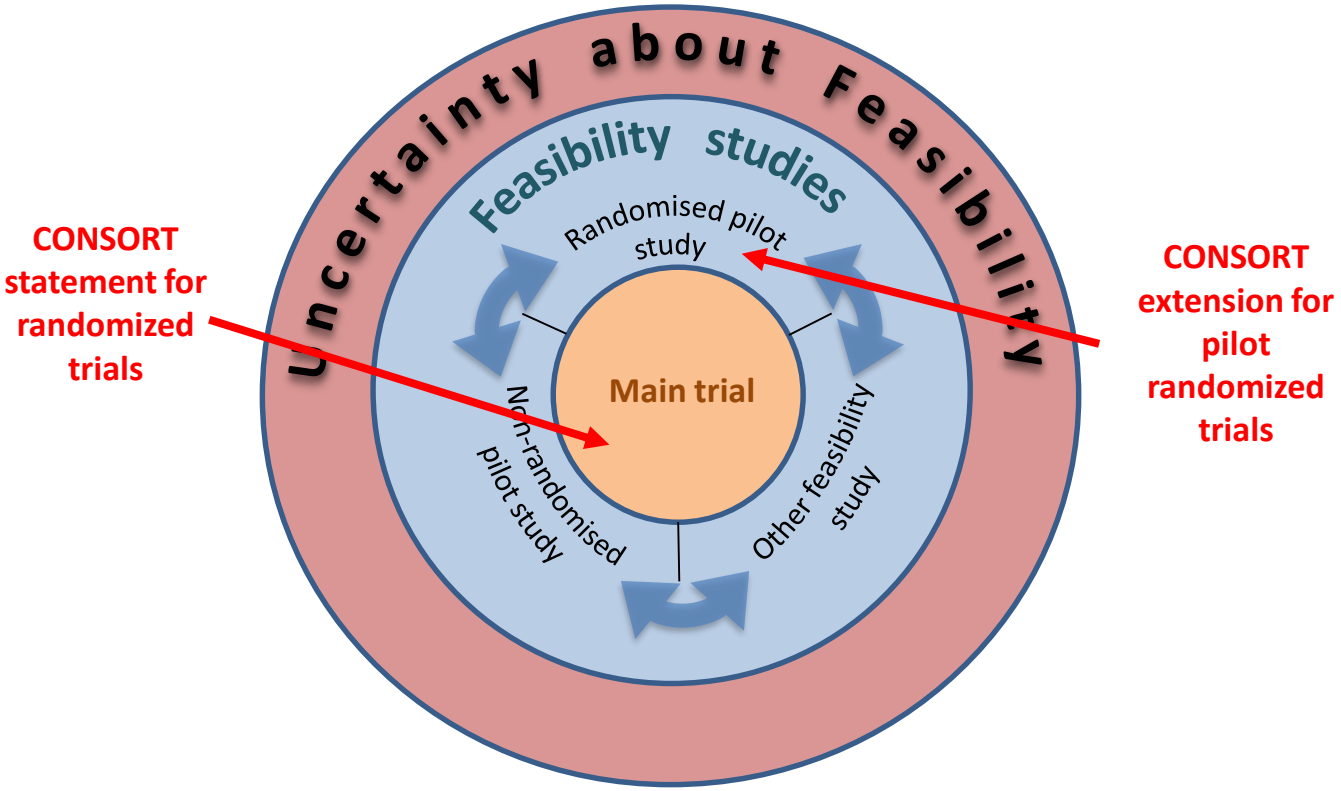
CONSORT extension for pilot and feasibility trials – overview of checklist items

Christine Bond

CONSORT extensions



Need for a further Consort extension?



Checklist development

Review of literature	Consort adaptation	Delphi exercise
Stakeholder consensus meeting	Iterative review and refinement	New and adapted items

CONSORT extension for randomized pilot and feasibility trials

Checklist applies to:

- Randomized trials conducted in preparation for a future definitive trial of effectiveness or efficacy
- Primary aim: feasibility of the future definitive trial
- No restrictions on terminology used to describe the preparatory trial
- No restrictions on the design of either trial
- Doesn't apply to internal pilot studies.

RESEARCH METHODS AND REPORTING

OPEN ACCESS

CrossMark

CONSORT 2010 statement: extension to randomised pilot and feasibility trials

Sandra M Eldridge,[1] Claire L Chan,[1] Michael J Campbell,[2] Christine M Bond,[3] Sally Hopewell,[4] Lehana Thabane,[5] Gillian A Lancaster[6] on behalf of the PAFS consensus group

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The Consolidated Standards of Reporting Trials (CONSORT) statement is a guideline designed to improve the transparency and quality of the reporting of randomised controlled trials (RCTs). In this article we present

Consequently, although much of the information to be reported in these trials is similar to those in randomised controlled trials (RCTs) assessing effectiveness and efficacy, there are some key differences in the type of

Section/topic and item No	Standard checklist item	Extension for pilot trials
Title and abstract		
1a	Identification as a randomised trial in the title	Identification as a pilot or feasibility randomised trial in the title
1b	Structured summary of trial design, methods, results, and conclusions (for specific guidance see CONSORT for abstracts)	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	Scientific background and explanation of rationale for future definitive trial, and reasons for randomised pilot trial
2b	Specific objectives or hypotheses	Specific objectives or research questions for pilot trial
Methods		
Trial design:		
3a	Description of trial design (such as parallel, factorial) including allocation ratio	Description of pilot trial design (such as parallel, factorial) including allocation ratio
3b	Important changes to methods after trial commencement (such as eligibility criteria), with reasons	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	

Item 2b: Specific objective or research question for pilot trial

- Item 2b
- *Standard CONSORT item*: specific objectives or hypotheses
- *Extension for pilot trials*: specific objectives or research questions for pilot trial
- *Example 1 (listing objectives as primary and secondary)*

"In this feasibility trial, the research aim was to explore trial design, staff and resident acceptability of the interventions and outcome measures and to provide data to estimate the parameters required to design a definitive RCT . . . The primary objectives of the trial were as follows:

1. To assess how many care homes accepted the invitation to participate in research.
2. To determine whether the eligibility criteria for care home residents were too open or too restrictive by estimating feasible eligibility and recruitment rate.
3. To assess retention of care homes and residents by estimating 3 and 6-month follow-up rates.

- *Explanation*

Although many aspects of feasibility may be related to each other, an articulation of specific objectives enables readers to understand the main areas of uncertainty to be addressed in the pilot trial and provides a working structure for presenting the methods and results in relation to these objectives. In addition, a comprehensive list of objectives enables other researchers to learn from and adopt similar approaches in their own studies.

It might be beneficial to separate the objectives into primary objectives (often those on which decisions about progressing to a future definitive RCT may be made) and secondary objectives, as in example 1, where feasibility objectives are primary and questions related to patient centred outcomes are treated as secondary.

Outcomes:		
6a	Completely defined pre-specified primary and secondary outcome measures, including how and when they were assessed	Completely defined pre-specified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed
6b	Any changes to trial outcomes after the trial commenced, with reasons	Any changes to pilot trial assessments or measurements after the pilot trial commenced, with reasons
6c		If applicable, pre-specified criteria used to judge whether, or how, to proceed with future definitive trial
Sample size:		
7a	How sample size was determined	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; details of any restriction (such as blocking and block size)	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	
Implementation:		
10	Who generated the random allocation sequence, who enrolled participants, and who assigned participants to interventions	

Item 6c: Outcomes – if applicable, pre-specified criteria to judge whether to proceed with future definitive trial

- Item 6c
- *Extension for pilot trials*: if applicable, prespecified criteria used to judge whether, or how, to proceed with future definitive trial
- *Example*

"Feasibility (delivery) and acceptability (uptake) of the DECISION+ program were the main outcome measures of this pilot trial. Investigators had established a priori threshold for specific feasibility and acceptability criteria. These were the following: (a) the proportion of contacted FMGs [Family medicine groups] participating in the pilot study would be 50% or greater, (b) the proportion of recruited family physicians participating in all three workshops would be 70% or greater, (c) the mean level of satisfaction from family physicians regarding the workshops would be 65% or greater, and (d) the proportion of missing data in each completed questionnaire would be less than 10%."[34]

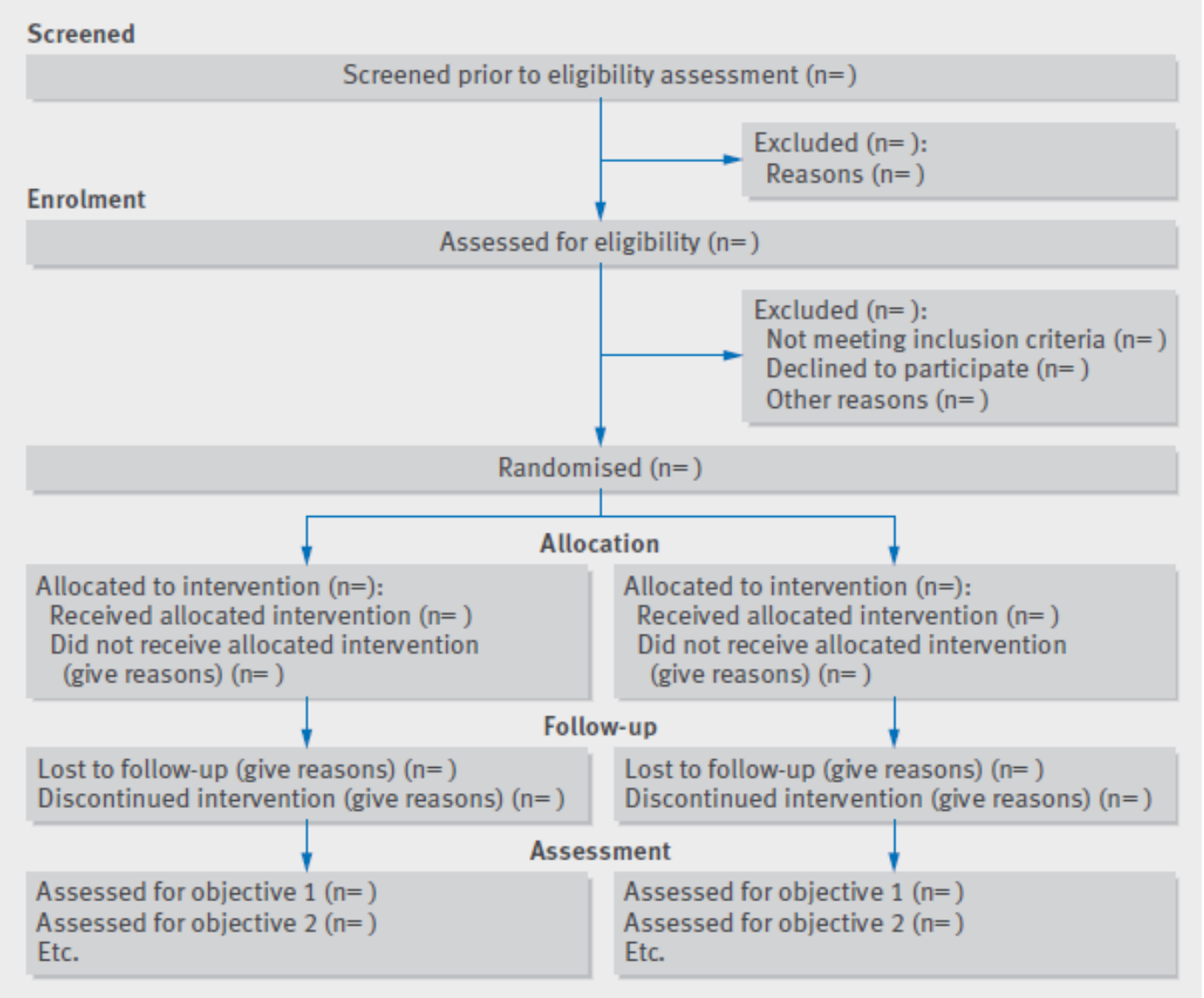
- *Explanation*

This is a new item. The purpose of a pilot trial is to assess the feasibility of proceeding to the next stage in the research process. To do this investigators need some criteria on which to base the decision about whether or not to proceed. The next stage in the research process will normally, although not always, be the future definitive RCT.

The UK National Institute for Health Research requires that pilot or feasibility studies have clear criteria for deciding whether or not to progress to the next stage: "We expect that when pilot or feasibility studies are proposed by applicants, or specified in commissioning briefs, a clear route of progression criteria to the

Blinding:		
11a	If done, who was blinded after assignment to interventions (for example, participants, care providers, those assessing outcomes) and how	
11b	If relevant, description of the similarity of interventions	
Analytical methods:		
12a	Statistical methods used to compare group for primary and secondary outcomes	Methods used to address each pilot trial objective whether qualitative or quantitative
12b	Methods for additional analyses, such as subgroup analyses and adjusted analyses	Not applicable
Results		
Participant flow (a diagram is strongly recommended):		
13a	For each group, the numbers of participants who were randomly assigned, received intended treatment, and were analysed for the primary outcome	For each group, the numbers of participants who were approached and/or assessed for eligibility, randomly assigned, received intended treatment, and were assessed for each objective
13b	For each group, losses and exclusions after randomisation, together with reasons	
Recruitment:		
14a	Dates defining the periods of recruitment and follow up	
14b	Why the trial ended or was stopped	Why the pilot trial ended or was stopped
Baseline data:		
15	A table showing baseline demographic and clinical characteristics for each group	
Numbers analysed:		
16	For each group, number of participants (denominator) included in each analysis and whether the analysis was by original assigned groups	For each objective, number of participants (denominator) included in each analysis. If relevant, these analyses should be by randomised group

13a: Participant flow diagram



Outcomes and estimation:		
17a	For each primary and secondary outcome, results for each group, and the estimated effect size and its precision (such as 95% confidence interval)	For each objective, results including expressions of uncertainty (such as 95% confidence interval) for any estimates. If relevant, these results should be by randomised group
17b	For binary outcomes, presentation of both absolute and relative effect sizes is recommended	Not applicable
Ancillary analyses:		
18	Results of any other analyses performed, including subgroup analyses and adjusted analyses, distinguishing prespecified from exploratory	Results of any other analyses performed that could be used to inform the future definitive trial
Harms:		
19	All important harms or unintended effects in each group (for specific guidance see CONSORT for harms)	
19a		If relevant, other important unintended consequences
Discussion		
Limitations:		
20	Trial limitations, addressing sources of potential bias, imprecision, and, if relevant, multiplicity of analyses	Pilot trial limitations, addressing sources of potential bias and remaining uncertainty about feasibility
Generalisability:		
21	Generalisability (external validity, applicability) of the trial findings	Generalisability (applicability) of pilot trial methods and findings to future definitive trial and other pilot studies
Interpretation:		
22	Interpretation consistent with results, balancing benefits and harms, and considering other relevant evidence	Interpretation consistent with pilot trial objectives and findings, balancing potential benefits and harms, and considering other relevant evidence
22a		Implications for progression from pilot to future definitive trial including any proposed amendments

17a: Outcomes and estimation

- Item 17a

- *Standard CONSORT item*: for each primary and secondary outcome, results for each group, and the estimated effect size and its precision (such as 95% confidence interval)

- *Extension for pilot trials*: for each objective, results including expressions of uncertainty (such as 95% confidence interval) for any estimates. If relevant, these results should be by randomised group

- *Example 1 (feasibility outcome)*

"The ABSORB [A bioabsorbable everolimus-eluting coronary stent system for patients with single de-novo coronary artery lesions] study aimed to assess the feasibility and safety of the BVS [bioasorbable everolimus-eluting stent] stent in patients with single de-novo coronary artery lesions . . .Procedural success was 100% (30/30 patients), and device success 94% (29/31 attempts at implantation of the stent)."[78]

- *Explanation*

It is important that the reported results of a pilot trial reflect the objectives. Results might include, for example, recruitment, retention or response rates, or other sorts of rates, as in example 1. Because the sample size in a pilot trial is likely to be small, estimates of these rates will be imprecise and this imprecision should be recognised, for example, by calculating a confidence interval around the estimate. Commonly, authors do not give such a confidence interval, but if the numerator and denominator are given the confidence interval can be calculated. In example 1 the Wilson 95% confidence

Other information		
Registration:		
23	Registration number and name of trial registry	Registration number for pilot trial and name of trial registry
Protocol:		
24	Where the full trial protocol can be accessed, if available	Where the pilot trial protocol can be accessed, if available
Funding:		
25	Sources of funding and other support (such as supply of drugs), role of funders	
26		Ethical approval/research review committee approval confirmed with reference number

CONSORT for Abstracts: for reporting pilot and feasibility trials

Table 3 | Extension of CONSORT for abstracts for reporting pilot trials

Item	Standard checklist item	Extension for pilot trials
Title	Identification of study as randomised	Identification of study as randomised pilot or feasibility trial
Trial design	Description of the trial design (eg, parallel, cluster, non-inferiority)	Description of pilot trial design (eg, parallel, cluster)
Methods:		
Participants	Eligibility criteria for participants and the settings where the data were collected	Eligibility criteria for participants and the settings where the pilot trial was conducted
Interventions	Interventions intended for each group	
Objective	Specific objective or hypothesis	Specific objectives of the pilot trial
Outcome	Clearly defined primary outcome for this report	Prespecified assessment or measurement to address the pilot trial objectives*
Randomisation	How participants were allocated to interventions	
Blinding (masking)	Whether or not participants, caregivers, and those assessing the outcomes were blinded to group assignment	
Results:		
Numbers randomised	Number of participants randomised to each group	Number of participants screened and randomised to each group for the pilot trial objectives*
Recruitment	Trial status†	
Numbers analysed	Number of participants analysed in each group	Number of participants analysed in each group for the pilot objectives*
Outcome	For the primary outcome, a result for each group and the estimated effect size and its precision	Results for the pilot objectives, including any expressions of uncertainty*
Harms	Important adverse events or side effects	
Conclusions	General interpretation of the results	General interpretation of the results of pilot trial and their implications for the future definitive trial
Trial registration	Registration number and name of trial register	Registration number for pilot trial and name of trial register
Funding	Source of funding	Source of funding for pilot trial

*Space permitting, list all pilot trial objectives and give the results for each. Otherwise, report those that are a priori agreed as the most important to the decision to proceed with the future definitive RCT.
†For conference abstracts.

Summary of changes to original CONSORT statement (2010)

- 26 items (instead of 25)
- New item:
  - ethical approval/research review committee approval confirmed with reference number
- 40 sub-items: 2 removed, 5 new, 21 adapted, 14 unchanged,
- 2 sub-items removed:
  - Subgroup analyses (in methods section)
  - Absolute and relative effect sizes (in results section)
- New sub-items:
  - how participants were identified and consented
  - if applicable, pre-specified criteria used to judge whether, or how, to proceed with future definitive trial
  - if relevant, other important unintended consequences
  - implications for progression from pilot to future definitive trial including any proposed amendments

Summary of changes (continued)

Adapted sub-items:

- Minor, mostly added word “pilot” (8)
- Introduction: need scientific background for main trial ***and*** rationale for pilot (1)
- Removed the word “hypotheses” (1)
- Changed sample size sub-item to **“Rationale for numbers in the pilot trial”** (1)
- Changed item on subgroup analyses in results to **“Results of any other analyses performed that could be used to inform the future definitive trial”**(1)
- Emphasised reporting applying to each *objective* (and using *assessments and measurements*) rather than each *outcome* (6)
- Discussion: report remaining uncertainty, make clear what results can be generalised to, interpretation consistent with objectives (3)

Small group: focusing on participants examples and different parts of the CONSORT extension

Christine Bond
Gill Lancaster

Focus on CONSORT items:

2a: rationale
2b: objectives
6a: how measure objectives

COFFEE BREAK

Progression criteria, sample size and analysis

Sandra Eldridge

Progression criteria: a pilot trial is about….

Making a decision about whether to proceed with the next stage

- Which may be a main trial
- Or may be another feasibility study

NIHR guidelines

"We expect that when pilot or feasibility studies are proposed by applicants, or specified in commissioning briefs, a clear route of progression criteria to the substantive study will be described. Listing ***clear progression criteria*** *will apply whether the brief or proposal describes just the preliminary study or both together. "*

Pre-specified criteria to aid decision making about next stage

Example: DECISION+ pilot trial (Leblanc et al 2011)

Aim of main study: Optimal use of antibiotics for treating acute respiratory infections in primary care

Intervention: Education in shared decision-making among family physicians and patients

Objective of pilot trial: To assess feasibility and acceptability of study design, procedures, and intervention

Pre-specified criteria for judging whether to proceed to main trial

Family medicine groups participating >=50%

Recruited family physicians participating in all three workshops >=70%

Mean level of satisfaction from family physicians regarding the workshops >=65%

Missing data in each completed questionnaire <10%

Example result : Only 24% of family medicine groups agreed to participate

"Not reaching the pre-established criteria does not necessarily indicate unfeasibility of the trial but rather underlines changes to be made to the protocol"

Advice from the literature on size of a pilot

- **Browne (1995)** gave as a general rule to take a minimum of 30 patients to estimate a parameter
- **Julious (2005)** recommends a minimum sample size of 12 per group as a rule of thumb and justifies this based on rationale about feasibility and precision about the mean and variance;
- **Stallard (2012)** proposed that the sample size should be approximately 0.03 times that the sample size planned for the definitive study
- **Sim and Lewis (2012)** suggest a sample size of at least 50 per group based on upper CI of variance estimate
- **Cocks and Torgerson (2013)** suggest 9% of the sample size of the main planned study
- **Teare et al (2014)** suggest 35 per group to estimate SD or 60-100 per group for event rate

However……

- Most methods in literature assume that main objective is to estimate inputs for sample size calculation for main trial
- Many investigators justify 30 using Gill Lancaster's 2004 paper
- If **main objective** measured via proportion can try choosing sample size that will give certain precision
- If **main objective** is e.g. assessing acceptability of intervention may want to use ideas of purposive sampling
- There may be logistic, resource restrictions…

Analysis: Recommendations from Lancaster et al (2004)

The analysis of a pilot study should be mainly descriptive and should focus on confidence intervals

Reason: pilot is too small and underpowered to test reasonable alternative hypotheses

If it were large enough to do so it would not be a pilot!

Example (Boogerd et al 2014)

Feasibility of an online treatment environment for adolescents with type 1 diabetes

62 adolescents aged 11–21 assigned to usual-care (n=31) or usual-care+intervention (n=31)

The authors started out well by defining feasibility objectives and matching data collection with those objectives..

Objectives

(i) Acceptability (do recipients use the intervention?)

(ii) Demand (do recipients continue to use the intervention?)

(iii) Practicability (can recipients access the intervention?)

(iv) Integration (does the intervention fit with guidelines for pediatric diabetes care?)

(v) Efficacy (what is the effect on adolescents' self efficacy?)

Data collection to match objectives

For example:

*"**Acceptability** **and** **demand*** were assessed in terms of the usage and repeated usage of the intervention by the patients in the trial indicated by logged user statistics."

Alas good intentions were not evident in the results...

They started well “65% (20/31) used the system”

But sadly went on to Hypothesis tests

1)Assessment of efficacy revealed improvement in the intervention group in evaluation of care (Patients’ Evaluation of Quality of Diabetes), $F(1,30)=5.35$, $p < 0.05$, and quality of life, communication (PedsQL), $F(1,30)=11.65$, $p <0.05$.

2) No significant differences in change over time between the intervention and the control group concerning HbA1c ($F(1,61)=0.16$, $p=0.693$)

Can we use a pilot study to assess surrogate endpoints?

Sometimes trials described as ‘pilot’ because they use surrogate endpoints (eg endpoints of more interest to clinicians than patients)

Usually because surrogate endpoints result in smaller/shorter trials

Trial with ‘hard endpoints may be planned if pilot successful

However, in most respects the ‘pilot’ trial resembles a conventional trial (eg sample size and hypothesis testing)

Not a pilot in the sense we intend

“Trials which use surrogate endpoints should only be described as ‘pilot’ when a definitive trial is a distinct possibility and the authors consider conditions which would indicate whether the definitive main trial was worthwhile and feasible. Simply because a trial uses a surrogate endpoint is not justification for calling it a pilot trial” (Campbell et al. 2018)

Can we use a pilot study to estimate an effect size?

- Effect sizes are not “what we expect” but rather what is clinically important
- Problem is that on occasion clinicians don’t know what is “clinically important”
- Usually a pilot is not our only source of information – should combine information from pilot with prior data
- Evidence from Kraemer et al (2006) if you use the effect size found in a pilot as the value of the effect in a main trial you will usually end up with an underpowered trial

Small group: focusing on participants examples and different parts of the CONSORT extension

Sandra Eldridge

Focus on CONSORT items:

6c: progression criteria
7a: sample size
12: method of analysis

Guidance on abstracts and flow diagrams

Sally Hopewell

Importance of abstracts

- Well-written journal and conference abstracts reporting randomized trials are important:
  - readers often base their initial assessment of a trial based on information reported in an abstract.
- They may then use this information to decide whether or not to seek more information about a trial.
- In some parts of the world, health practitioners often have access to the abstracts only,
  - so healthcare decisions are made on the basis of the abstract.

International Committee of Medical Journal Editors

Uniform Requirements for Manuscripts Submitted to Biomedical Journals: Writing and Editing for Biomedical Publication

Updated October 2008

"Articles on clinical trials should contain abstracts that include the items that the CONSORT group has identified as essential."

CONSORT for Abstracts

Item 1b

Standard CONSORT item: structured summary of

- trial design, methods, results, and conclusions (for specific guidance see CONSORT for abstracts)

Extension for pilot trials: structured summary of

- pilot trial design, methods, results, and conclusions (for specific guidance see CONSORT abstract extension for pilot trials)

Table 3 | Extension of CONSORT for abstracts for reporting pilot trials

Item	Standard checklist item	Extension for pilot trials
Title	Identification of study as randomised	Identification of study as randomised pilot or feasibility trial
Trial design	Description of the trial design (eg, parallel, cluster, non-inferiority)	Description of pilot trial design (eg, parallel, cluster)
Methods:		
Participants	Eligibility criteria for participants and the settings where the data were collected	Eligibility criteria for participants and the settings where the pilot trial was conducted
Interventions	Interventions intended for each group	
Objective	Specific objective or hypothesis	Specific objectives of the pilot trial
Outcome	Clearly defined primary outcome for this report	Prespecified assessment or measurement to address the pilot trial objectives*
Randomisation	How participants were allocated to interventions	
Blinding (masking)	Whether or not participants, caregivers, and those assessing the outcomes were blinded to group assignment	
Results:		
Numbers randomised	Number of participants randomised to each group	Number of participants screened and randomised to each group for the pilot trial objectives*
Recruitment	Trial status†	
Numbers analysed	Number of participants analysed in each group	Number of participants analysed in each group for the pilot objectives*
Outcome	For the primary outcome, a result for each group and the estimated effect size and its precision	Results for the pilot objectives, including any expressions of uncertainty*
Harms	Important adverse events or side effects	
Conclusions	General interpretation of the results	General interpretation of the results of pilot trial and their implications for the future definitive trial
Trial registration	Registration number and name of trial register	Registration number for pilot trial and name of trial register
Funding	Source of funding	Source of funding for pilot trial

*Space permitting, list all pilot trial objectives and give the results for each. Otherwise, report those that are a priori agreed as the most important to the decision to proceed with the future definitive RCT.
†For conference abstracts.

Original abstract – checklist items reported by the authors are in green

Title: "Not just another walking program": Everyday Activity Supports You (EASY) model – a randomized pilot study for a parallel randomized controlled trial

Background: Maintaining physical activity is an important goal with positive health benefits, yet many people spend most of their day sitting. Our Everyday Activity Supports You (EASY) model aims to encourage movement through daily activities and utilitarian walking. The primary objective of this phase was to test study feasibility (recruitment and retention rates) for the EASY model.

Methods: This 6-month study took place in Vancouver, Canada, from May to December 2013, with data analyses in February 2014. Participants were healthy, inactive, community-dwelling women aged 55–70 years. We recruited through advertisements in local community newspapers and randomized participants using a remote web service. The model included the following: group-based education and social support, individualized physical activity prescription (called Activity 4-1-1), and use of a Fitbit activity monitor. The control group received health-related information only. The main outcome measures were descriptions of study feasibility (recruitment and retention rates). We also collected information on activity patterns (ActiGraph GT3X+ accelerometers) and health-related outcomes such as body composition (height and weight using standard techniques), blood pressure (automatic blood pressure monitor), and psychosocial variables (questionnaires).

Results: We advertised in local community newspapers to recruit participants. Over 3 weeks, 82 participants telephoned; following screening, 68% (56/82) met the inclusion criteria and 45% (25/56) were randomized by remote web-based allocation. This included 13 participants in the intervention group and 12 participants in the control group (education). At 6 months, 12/13 (92%) intervention and 8/12 (67%) control participants completed the final assessment. Controlling for baseline values, the intervention group had an average of 2,080 [95% confidence intervals (CIs) 704, 4,918] more steps/day at 6 months compared with the control group. There was an average between group difference in weight loss of −4.3 [95% CI −6.22, −2.40] kg and reduction in diastolic blood pressure of −8.54 [95% CI −16.89, −0.198] mmHg, in favor of EASY.

Conclusions: The EASY pilot study was feasible to deliver; there was an increase in physical activity and reduction in weight and blood pressure for intervention participants at 6 months.

Trial registration: ClinicalTrials.gov identifier: NCT01842061

Pilot and Feasibility Studies. 2015, 1:4. doi:10.1186/2055-5784-1-4

Word count: 335

Item	Extension for pilot trials	Reported
Title	Identification of study as randomised pilot trial	✓
Trial design	Description of pilot trial design (e.g. parallel, cluster)	x
METHODS		
Participants	Eligibility criteria for participants and the settings where the pilot trial was conducted	✓
Interventions	*Interventions intended for each group*	✓
Objective	Specific objectives of the pilot trial	✓
Outcome	Pre-specified assessment or measurement to address the pilot trial objective(s)[1]	✓
Randomisation	*How participants were allocated to interventions*	✓
Blinding (masking)	*Whether or not participants, care givers, and those assessing the objectives were blinded to group assignment*	x
RESULTS		
Numbers randomised	Number of participants screened and randomised to each group for the pilot trial objective(s)[1]	✓
Recruitment	*Trial status*[2]	*N/A*
Numbers analysed	Number of participants analysed in each group for the pilot objective(s)[1]	✓
Outcome	Results for the pilot objective(s); including any expressions of uncertainty[1]	partly
Harms	*Important adverse events or side-effects*	x
Conclusions	General interpretation of the results of pilot trial and their implications for the future definitive trial	partly
Trial registration	Registration number for pilot trial and name of trial register	✓
Funding	Source of funding for pilot trial	x

Items above in italics are pilot trial checklist items unchanged from the standard RCT checklist.
[1] Space permitting, list all pilot trial objectives and give the results for each. Otherwise, report those which are a priori agreed as the most important (main) to the decision to proceed with the future definitive trial. [2] For conference abstracts.

BEFORE

Revised abstract – items in red are added to meet the checklist requirements

Title: "Not just another walking program": Everyday Activity Supports You (EASY) model – a randomized pilot study for a parallel randomized controlled trial

Background: Maintaining physical activity is an important goal with positive health benefits, yet many people spend most of their day sitting. Our Everyday Activity Supports You (EASY) model aims to encourage movement through daily activities and utilitarian walking. The primary objective of this pilot trial was to test study feasibility (recruitment and retention rates) for the EASY model.

Methods: This 6-month parallel two-arm pilot trial took place in Vancouver, Canada (May to December 2013). Participants were healthy, inactive, community-dwelling women aged 55–70 years. We recruited through advertisements in local community newspapers and randomized participants using a remote web service. The model included: group-based education and social support, individualized physical activity prescription, and use of a Fitbit activity monitor. The control group received health-related information only. The main outcome measures were descriptions of study feasibility (recruitment and retention rates). We also collected information (blinded outcome assessment) on activity patterns, height and weight, blood pressure, and psychosocial variables.

Results: We advertised in local community newspapers to recruit participants. Over 3 weeks, 82 participants telephoned; following screening, 68% (56/82) met the inclusion criteria and 45% (25/56) were randomized by remote web-based allocation: 13 participants in the intervention group and 12 in the control group (education). At 6 months, 12/13 (92%; 95% CI 65% to 100%) intervention and 8/12 (67%; 95% CI 35% to 90%) control participants completed the final assessment. This met our a priori recruitment and retention criteria for success. Of those who declined 21/30 gave reasons of timing of sessions within working hours. There were no adverse events related to study participation.

Conclusions: The EASY pilot study was feasible to deliver; there was an increase in physical activity and reduction in weight and blood pressure for intervention participants at 6 months. In a future definitive trial greater drop-out in the control arm may be reduced by using a different design and alternative sources of recruitment might be considered.

Trial registration: ClinicalTrials.gov identifier: NCT01842061

Trial funding: Canadian Institute of Health Research, Michael Smith Foundation, Australian National Health and Medical Research Council.

Word count: 340

Item	Extension for pilot trials	Reported
Title	Identification of study as randomised pilot trial	✓
Trial design	Description of pilot trial design (e.g. parallel, cluster)	✓
METHODS		
Participants	Eligibility criteria for participants and the settings where the pilot trial was conducted	✓
Interventions	*Interventions intended for each group*	✓
Objective	Specific objectives of the pilot trial	✓
Outcome	Pre-specified assessment or measurement to address the pilot trial objective(s)[1]	✓
Randomisation	*How participants were allocated to interventions*	✓
Blinding (masking)	*Whether or not participants, care givers, and those assessing the objectives were blinded to group assignment*	✓
RESULTS		
Numbers randomised	Number of participants screened and randomised to each group for the pilot trial objective(s)[1]	✓
Recruitment	*Trial status*[2]	*N/A*
Numbers analysed	Number of participants analysed in each group for the pilot objective(s)[1]	✓
Outcome	Results for the pilot objective(s); including any expressions of uncertainty[1]	✓
Harms	*Important adverse events or side-effects*	✓
Conclusions	General interpretation of the results of pilot trial and their implications for the future definitive trial	✓
Trial registration	Registration number for pilot trial and name of trial register	✓
Funding	Source of funding for pilot trial	✓

Items above in italics are pilot trial checklist items unchanged from the standard RCT checklist.
[1] Space permitting, list all pilot trial objectives and give the results for each. Otherwise, report those which are a priori agreed as the most important (main) to the decision to proceed with the future definitive trial. [2] For conference abstracts.

AFTER

Importance of flow diagrams

- A flow diagram is a key element of CONSORT and has been widely adopted.
- CONSORT flow diagrams include the number:
  - assessed for eligibility
  - randomly assigned to each group
  - received treatment as allocated
  - completed treatment as allocated
  - were analysed for the primary outcome, with numbers and reasons for exclusions

CONSORT flow diagram

Table 2 | CONSORT checklist of information to include when reporting a pilot trial

Section/topic and item No	Standard checklist item	Extension for pilot trials	Page No where item is reported
Results			
Participant flow (a diagram is strongly recommended):			
13a	For each group, the numbers of participants who were randomly assigned, received intended treatment, and were analysed for the primary outcome	For each group, the numbers of participants who were approached and/or assessed for eligibility, randomly assigned, received intended treatment, and were assessed for each objective	
13b	For each group, losses and exclusions after randomisation, together with reasons		
Recruitment:			
14a	Dates defining the periods of recruitment and follow-up		
14b	Why the trial ended or was stopped	Why the pilot trial ended or was stopped	

- In addition, for pilot trials include the number of participants who were:
  - approached and/or assessed for eligibility
    - in order to assess external validity, and how easy it is to recruit participants
  - assessed for each objective
    - if multiple then decide a priori which are most important to decide whether to proceed to a future definitive trial

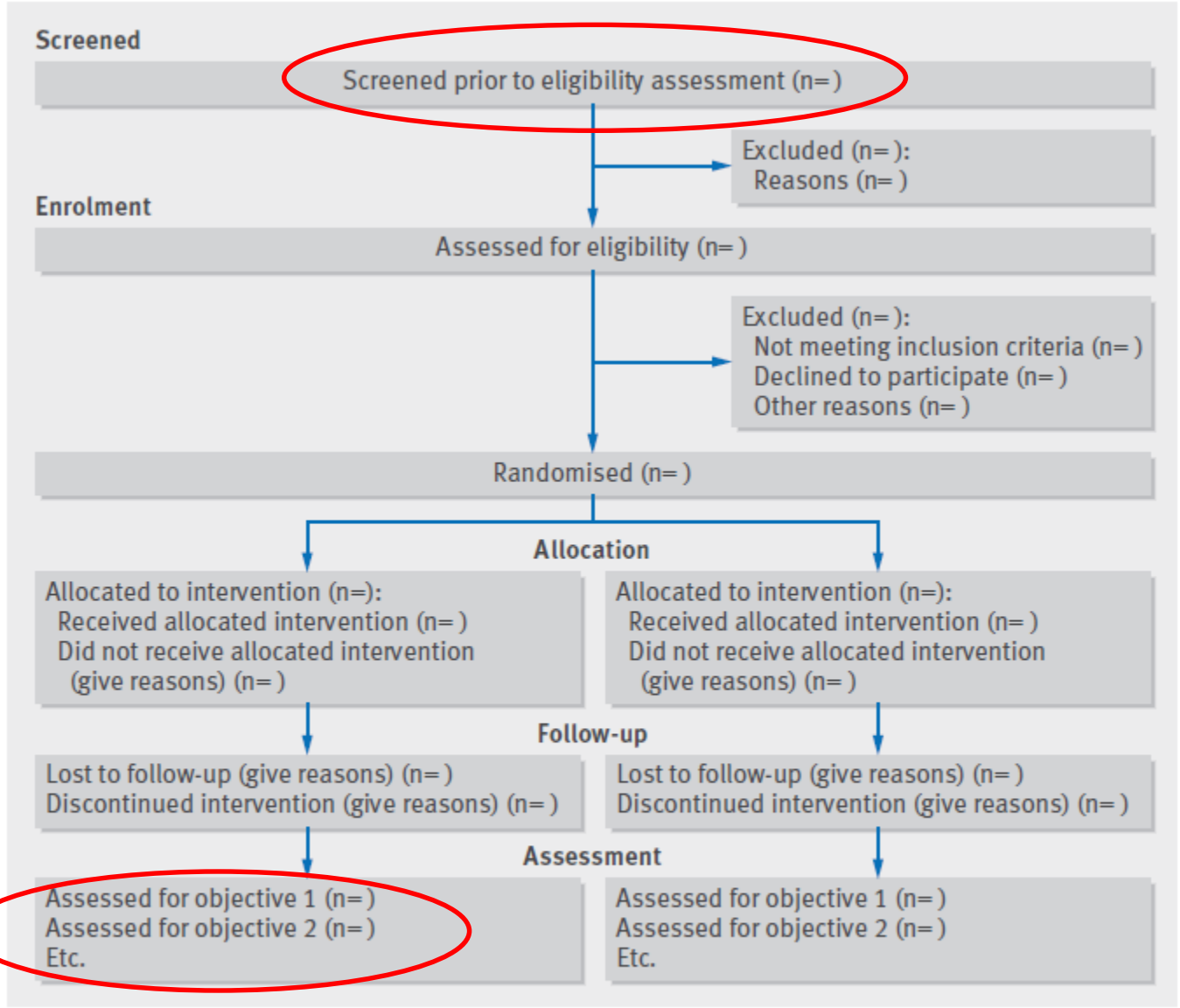


Fig 5 | Recommended flow diagram of progress through phases of a parallel randomised pilot trial of two groups—that is, screening, enrolment, intervention allocation, follow-up, and assessment for each pilot trial objective. Adapted from Moher et al[2]

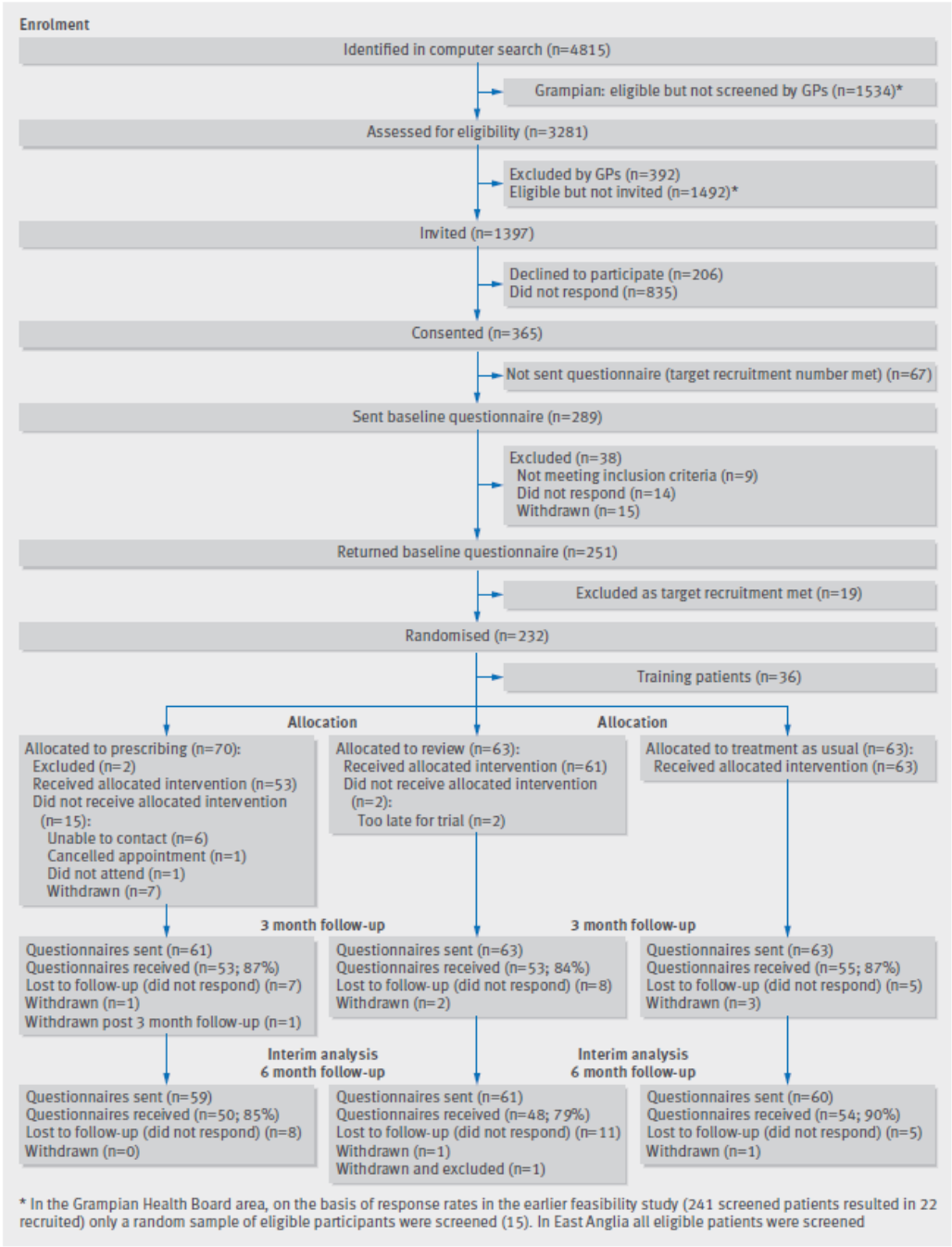
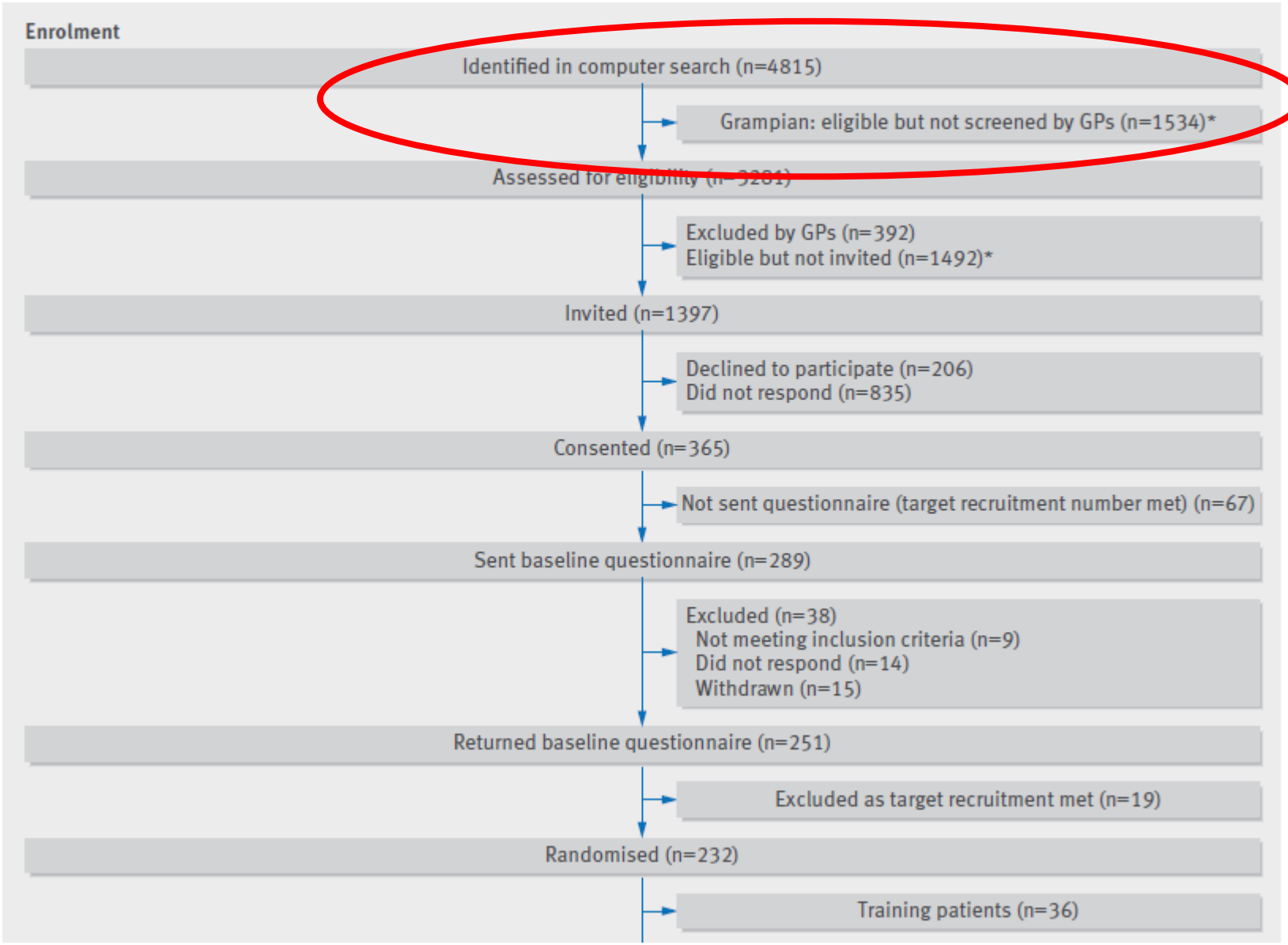


Fig 4 | Flow diagram of a randomised pilot trial of pharmacist led management of chronic pain in primary care (reproduced from Bruhn et al[69])


Enrolment
Identified in computer search (n=4815)
Grampian: eligible but not screened by GPs (n=1534)*
Assessed for eligibility (n=3281)
Excluded by GPs (n=392)
Eligible but not invited (n=1492)*
Invited (n=1397)
Declined to participate (n=206)
Did not respond (n=835)
Consented (n=365)
Not sent questionnaire (target recruitment number met) (n=67)
Sent baseline questionnaire (n=289)
Excluded (n=38)
Not meeting inclusion criteria (n=9)
Did not respond (n=14)
Withdrawn (n=15)
Returned baseline questionnaire (n=251)
Excluded as target recruitment met (n=19)
Randomised (n=232)
Training patients (n=36)

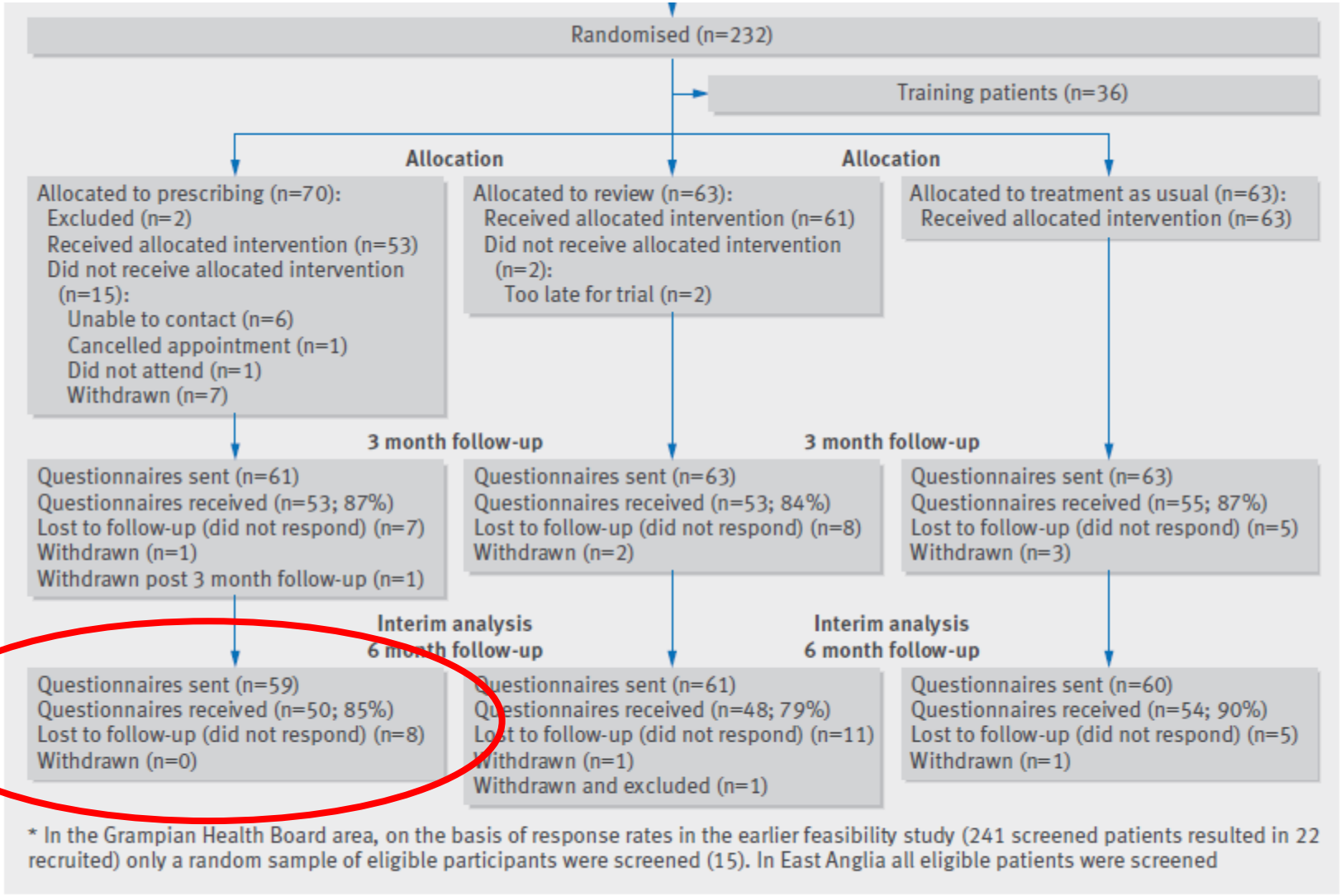


Fig 4 | Flow diagram of a randomised pilot trial of pharmacist led management of chronic pain in primary care (reproduced from Bruhn et al[69])

Group exercise: using the CONSORT extension to assess completeness of pilot trial reporting

Sally Hopewell
Claire Chan

Focus on CONSORT items:

2a: rationale
2b: objectives
6a: how measure objectives
6c: progression criteria
7a: sample size
12: method of analysis

Hind et al. Pilot and Feasibility Studies (2017) 3:16
DOI 10.1186/s40814-017-0132-0

Pilot and Feasibility Studies

RESEARCH Open Access

Aquatic therapy for boys with Duchenne muscular dystrophy (DMD): an external pilot randomised controlled trial

Daniel Hind[1*], James Parkin[1], Victoria Whitworth[1], Saleema Rex[1], Tracey Young[2], Lisa Hampson[3], Jennie Sheehan[4], Chin Maguire[1], Hannah Cantrill[1], Elaine Scott[2], Heather Epps[5], Marion Main[6], Michelle Geary[7], Heather McMurchie[8], Lindsey Pallant[9], Daniel Woods[10], Jennifer Freeman[11], Ellen Lee[1], Michelle Eagle[12], Tracey Willis[13], Francesco Muntoni[6] and Peter Baxter[10]

Abstract

Background: Standard treatment of Duchenne muscular dystrophy (DMD) includes regular physiotherapy. There are no data to show whether adding aquatic therapy (AT) to land-based exercises helps maintain motor function. We assessed the feasibility of recruiting and collecting data from boys with DMD in a parallel-group pilot randomised trial (primary objective), also assessing how intervention and trial procedures work.

Methods: Ambulant boys with DMD aged 7–16 years established on steroids, with North Star Ambulatory Assessment (NSAA) score ≥8, who were able to complete a 10-m walk test without aids or assistance, were randomly allocated (1:1) to 6 months of either optimised land-based exercises 4 to 6 days/week, defined by local community physiotherapists, or the same 4 days/week plus AT 2 days/week. Those unable to commit to a programme, with >20% variation between NSAA scores 4 weeks apart, or contraindications to AT were excluded. The main outcome measures included feasibility of recruiting 40 participants in 6 months from six UK centres, clinical outcomes including NSAA, independent assessment of treatment optimisation, participant/therapist views on acceptability of intervention and research protocols, value of information (VoI) analysis and cost-impact analysis.

Results: Over 6 months, 348 boys were screened: most lived too far from centres or were enrolled in other trials; 12 (30% of the targets) were randomised to AT ($n = 8$) or control ($n = 4$). The mean change in NSAA at 6 months was −5.5 (SD 7.8) in the control arm and −2.8 (SD 4.1) in the AT arm. Harms included fatigue in two boys, pain in one. Physiotherapists and parents valued AT but believed it should be delivered in community settings. Randomisation was unattractive to families, who had already decided that AT was useful and who often preferred to enrol in drug studies. The AT prescription was considered to be optimised for three boys, with other boys given programmes that were too extensive and insufficiently focused. Recruitment was insufficient for VoI analysis.

Conclusions: Neither a UK-based RCT of AT nor a twice weekly AT therapy delivered at tertiary centres is feasible. Our study will help in the optimisation of AT service provision and the design of future research.

Trial registration: ISRCTN41002956

CONSORT item 2a

- “Scientific background and explanation of rationale for future definitive trial, and reasons for randomised pilot trial”

CONSORT item 2a

"International guidelines for the multidisciplinary management of people with Duchenne muscular dystrophy (DMD) recommend…evidence base for these recommendations is weak and do not detail specific therapy interventions or dosage, nor do they discuss aquatic therapy…There are limited data on the effectiveness of AT in general, and none in people with DMD."

"Our study addressed a 2012 commission from the UK NIHR HTA programme for a feasibility study. The specific objective was to collect data that would tell us whether it was feasible to run a full-scale trial, assessing the clinical effectiveness of AT in maintaining physical function in people with Duchenne muscular dystrophy. The principal focus of this paper is the feasibility of a full-scale research study."

CONSORT item 2b

- “Specific objectives or research questions for pilot trial”

CONSORT item 2b

(Abstract)“We assessed the feasibility of recruiting and collecting data from boys with DMD in a parallel-group pilot randomised trial (primary objective), also assessing how intervention and trial procedures work.”

“The specific objective was to collect data that would tell us whether it was feasible to run a full-scale trial, assessing the clinical effectiveness of AT in maintaining physical function in people with Duchenne muscular dystrophy.”

CONSORT item 6a

- “Completely defined prespecified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed”

CONSORT item 6a

"The primary outcome was the feasibility of recruitment of 40 participants within 6 months from six centres. Additional feasibility outcomes were a decision on the primary endpoint for a subsequent larger trial; the number and characteristics of eligible participants who were approached for the study; the number of participants randomised, withdrawn, and lost to follow-up; the number of participants who discontinued AT and were included and excluded from analysis with reasons; the recruitment rate; reasons for refused consent; participant attrition rates and reasons; data completeness; feasibility of recruiting participating sites and estimation of the costs; participant views on acceptability of research procedures and intervention; physiotherapist views on the intervention/research protocol and perceived contamination of the control group; and intervention optimisation. The following clinical data were collected for all participants: 6-min walk distance (6MWD); North Star Ambulatory Assessment (NSAA) ; forced vital capacity (FVC); … Children's OMNI Scale of Perceived Exertion were assessed before and after each AT session. The therapists also recorded attendance as well as the AT stretches and exercises performed."

CONSORT item 6c

- “If applicable, prespecified criteria used to judge whether, or how, to proceed with future definitive trial”

CONSORT item 6c

“This pilot aimed to recruit 40 children in 6 months and deliver AT to 20 of them. If this objective success criterion was met, then we could deem a full-scale study potentially feasible. Other feasibility outcomes did not involve objective stop-go (success) criteria but provided a basis for improving the research procedures.”

CONSORT item 7a

- “Rationale for numbers in the pilot trial”

CONSORT item 7a

"The sample size for this external pilot trial was based on a recommended minimum of 30 participants (15 per group) for feasibility objectives involving parameter estimation *. Assuming a drop-out rate at 6 months of 20%, we set a target of randomising at least 40 participants (20 per group). While this decision was principally informed by the need to calculate a sample size for a full-scale study, we believed the recruitment of 40 boys in 6 months might indicate the feasibility of a trial of n = 100 to 150 in UK centres alone, given a longer recruitment window that would still be acceptable to funding bodies (1 to 2 years)."

**Browne RH. On the use of a pilot sample for sample size determination. Stat Med. 1995;14:1933–40*

CONSORT item 12

- “Methods used to address each pilot trial objective whether qualitative or quantitative”

CONSORT item 12

- “The ITT population included all patients who were consented and randomised. This was the primary analysis set, and unless stated otherwise, all endpoints are summarised for the ITT population. Depending on the distribution of the data, continuous variables (e.g., age) were summarised by either the mean and standard deviation or the median and interquartile range (IQR). AT adherence was assessed by the number and percentage of AT sessions attended, with mean (SD), median (IQR) and minimum–maximum numbers. LBT adherence was measured by the number of days on which the prescribed exercises were performed and the percentage of the prescribed exercises that were performed on across the total number of days on which exercise adherence was recorded. Descriptive statistics (mean differences between groups and 95% CIs) were derived for clinical outcomes. Categorical outcomes are presented as the difference between groups in the percentages in each category, together with 95% CIs. Available clinical outcomes at 6 months are presented for the ITT set, by group and overall. For continuous outcomes, we present change from baseline by group and overall... All interviews were audio recorded and transcribed. Transcripts were coded in NVivo with analysis completed using a framework analysis.”

Guidance on planning pilot and feasibility studies and writing study protocols

Lehana Thabane

Using the checklist in planning

- Many study protocols are published in the Pilot and Feasibility Studies journal
- Authors follow SPIRIT guideline for main RCT protocol – not ideal as often they do not specify explicit feasibility objectives & outcomes until asked to do so
- How can the CONSORT extension items supplement SPIRIT for planning a pilot trial?

Interim guidance on reporting of protocols of pilot RCTs—based adaptation of both the SPIRIT guideline plus the CONSORT extension to pilot RCTs

Thabane and Lancaster *Pilot and Feasibility Studies* (2019) 5:37
https://doi.org/10.1186/s40814-019-0423-8

Pilot and Feasibility Studies

EDITORIAL Open Access

A guide to the reporting of protocols of pilot and feasibility trials

Lehana Thabane[1*] and Gillian Lancaster[2]

Item supplementation

SPIRIT	CONSORT Extension
Item 6a Justification for undertaking the trial	Item 2a Reasons for randomized pilot trial
Item 7 Specific objectives or hypotheses	Item 2b Specific (feasibility) objectives or research questions
Item 12 Methods - Primary and secondary outcome measures	Item 6a Assessments to address each pilot trial objective (primary and secondary)
Item 14 Sample size	Item 7a Rationale for numbers
Item 20a Statistical methods	Item 12a Methods (of analysis) used to address each pilot trial objective

Interim guidance on reporting of non randomized pilot studies—based on adaptation of the CONSORT extension to pilot trials and guidelines for reporting of the non-RCTs (eg. STROBE)

Lancaster and Thabane *Pilot and Feasibility Studies* (2019) 5:114
https://doi.org/10.1186/s40814-019-0499-1

Pilot and Feasibility Studies

EDITORIAL Open Access

Guidelines for reporting non-randomised pilot and feasibility studies

Gillian A. Lancaster* and Lehana Thabane

First:

One can use the CONSORT extension to pilot trials checklist, and declare the parts that deal with randomization as not applicable

Table 1 Main types of non-randomised feasibility studies submitted to the journal, where to find guidance and published examples

Type of study	Equator website checklists and other helpful guidance	Published examples
Intervention development	TIDieR http://www.equator-network.org/reporting-guidelines/tidier/ Maximising the impact of qualitative research in feasibility studies for randomised controlled trials: guidance for researchers (O'Cathain et al): https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-015-0026-y	Thematic series on intervention development available at: https://www.biomedcentral.com/collections/interventiondevelopment
Patient-Reported Outcome Measures (PROMS) development	CONSORT PRO (adapt alongside CONSORT extension to pilot trials) http://www.equator-network.org/reporting-guidelines/consort-pro/ COSMIN User Manual (comprehensive reference, useful risk of bias tool) https://cosmin.nl/wp-content/uploads/COSMIN-syst-review-for-PROMs-manual_version-1_feb-2018.pdf	Thematic series on pilot and feasibility testing of patient-reported outcome measures available at: https://www.biomedcentral.com/collections/pilotfeasibilityPROMs
Piloting several components of the trial	CONSORT extension to pilot trials (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/consort-2010-statement-extension-to-randomised-pilot-and-feasibility-trials/	Aging, Community and Health—Community Partnership Program before-after study [25]: https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-016-0063-1 POWeR-RN non-randomised study with wait-list control [26] https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-017-0122-2#Sec16

Implementation of research findings	CONSORT extension to pilot trials (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/consort-2010-statement-extension-to-randomised-pilot-and-feasibility-trials/ RE-AIM (Reach, Effectiveness, Adoption, Implementation, Maintenance) framework for evaluating interventions http://www.re-aim.org/ Please note that when applying RE-AIM to pilot and feasibility studies, 'potential effectiveness' only should be addressed.	Thematic series on implementation science and practice forthcoming at: https://www.biomedcentral.com/collections/implementationscience-pilotstudies GLA:D® Back before-after study [28]: https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-019-0448-z GenerationPMTO before-after study [29] https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-019-0476-8
Feasibility studies in preparation for a cohort or other large scale study	STROBE (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/strobe/ CONSORT extension to pilot trials (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/consort-2010-statement-extension-to-randomised-pilot-and-feasibility-trials/ • Ensure there is adequate explanation as to why the study is a feasibility study, and state clear feasibility objectives • Ensure a formal sample size calculation is reported if hypothesis testing is carried out	Community-based paediatric respiratory infection surveillance cohort study [31]: https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-018-0371-8 Prognosis of patients with apparent treatment-resistant hypertension [32]: https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-018-0232-5
Feasibility studies that test preliminary hypotheses of association	STROBE (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/strobe/ CONSORT extension to pilot trials (ignoring items not applicable) http://www.equator-network.org/reporting-guidelines/consort-2010-statement-extension-to-randomised-pilot-and-feasibility-trials/ • Ensure there is adequate explanation as to why the study is a feasibility study, and state clear feasibility objectives • Ensure a formal sample size calculation is reported if hypothesis testing is carried out	Is cognitive function in delirium associated with EEG frequency band connectivity (case-control study) [33]? https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-018-0388-z Are foetus mouth movements associated with sound stimulation in the womb [34]? https://pilotfeasibilitystudies.biomedcentral.com/articles/10.1186/s40814-016-0053-3

Future plans and close

Lehana Thabane

Challenges with Pilot Studies

- ✓ **Most are not well designed**
  - ❑ No clear feasibility objectives
  - ❑ No clear rationale for piloting
  - ❑ No clear analytic plans
  - ❑ No clear criteria for success of feasibility
- ✓ **Most are not reported/published**
- ✓ **They should be registered**

African Proverb (Ashanti, Ghana)

You never test the depth of a river with both feet

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