Core Guide: Pilot and Feasibility Studies

Part of a series addressing common issues in statistical and epidemiological design and analysis

Definition of a Feasibility Study. A feasibility study is performed to assess whether some aspect of a proposed project or study will work [1]. They may also be used to estimate important parameters that are needed to design a larger study. The following are examples of values an investigator may be interested in measuring [2]:

- Time commitment needed by interventionists and study participants
- Willingness of participants to be randomized
- Willingness of clinicians to recruit participants
- Number of eligible patients, caretakers or other appropriate participants
- Characteristics of the proposed outcome measure (in some cases feasibility studies might involve designing a suitable outcome measure)
- Follow-up rates, response rates to questionnaires, adherence/compliance rates
- Availability of data needed or the usefulness and limitations of a particular database
- Ability to validly and precisely measure variables of interest
- Time needed to collect and analyze data

Definition of a Feasibility Study that is a Pilot: A pilot study is a type of feasibility study in which an investigator is testing a potential future study protocol as a whole to see if it will work. [1] Pilot studies are used to test whether all components of a study can work together, with the intent that findings from the pilot will lead to a larger full-scale study in the future.

In some cases, this will be the first phase of the larger substantive study and data from the pilot phase may contribute to the final analysis; this can be referred to as an internal pilot. Internal pilot studies are often incorporated into the main study design of a larger randomized controlled trial. In contrast, an external pilot is a stand-alone piece of work planned and carried out independently of and prior to the main study [2, 3].

What is the difference between a pilot study and a feasibility study?
In the literature, it is not uncommon to see “pilot” and “feasibility” used interchangeably. In fact, all pilot studies are a type of feasibility study, though the contrary may not be true. The distinguishing feature of a pilot study is that it tests the feasibility of all aspects of the protocol as whole and how the components of the protocol work together [4]. On the other hand, a feasibility study that is NOT a pilot study might test various subsets of future full-scale study protocol but not the protocol as a whole.

Some simple definitions have been proposed to distinguish between three main types of feasibility studies [5]:

---

Core Guide: Pilot & Feasibility Studies
1. **Randomized pilot studies**: Studies in which the future RCT, including the randomization of participants, is conducted on a smaller scale to see if it can be done.

2. **Non-randomized pilot studies**: Similar to randomized pilot studies, these are studies in which all or part of the intervention to be evaluated and other processes to be undertaken in a future trial is/are carried out but without randomization of participants.

3. **Feasibility studies that are not pilot studies**: Studies in which investigators attempt to answer a question about whether some element of the future trial can be done but do not implement the intervention to be evaluated or other processes to be undertaken in a future trial, though they may be addressing intervention development in some way.

It is important to note that exact definitions and distinctions between randomized pilot studies, non-randomized pilot studies, and feasibility studies that are not pilot studies are not necessarily consistent in the literature. See Eldridge et al [5], for examples of different classifications and proposed definitions for the three types of studies.

**Objectives of Internal Pilot Study**

Internal pilot studies are usually planned simultaneously with a larger study and conducted using a pre-specified number of the initial participants in the full trial. The main purpose of an internal pilot study is to provide a check on the adequacy of the sample size calculation [6]. One drawback to such a design is that analyzing the pilot participants along with participants in the rest of the study may lead to increased Type I error due to non-independence of samples (i.e. sample size in the second stage is allowed to depend on observed responses in the pilot phase). However, the inflation is likely to be small in all but very small pilot sample sizes and can be remedied by incorporating the non-independence into the sample size calculation [7].

Though both internal pilot studies and interim analyses allow for sample size reviews for clinical trials, there is an important distinction between them. An interim analysis involves a formal calculation of the treatment effect and a corresponding hypothesis test for the purposes of determining futility or success of the treatment in order to determine whether early stopping is appropriate. In contrast, internal pilot studies involve calculation or re-calculation of nuisance parameters\(^1\) (e.g. sample variance) for the purpose of determining whether the original sample size calculation was appropriate[7].

Use of an internal pilot study ultimately depends on the goals of investigators. For instance, internal pilot studies do NOT allow for the pre-testing of the feasibility, acceptability, or many other components of the larger study since the internal pilot is already a part of the larger study [7], thus establishment of the feasibility of the study must occur before the internal pilot.

---

\(^1\) A nuisance parameter is any parameter that is NOT of primary interest in an analysis but must be accounted for in order to make inference about parameters that ARE of primary interest (e.g. variance is often considered a nuisance parameter when the mean of a distribution is the parameter of interest).
Objective of an External Pilot Study

The main objective of an external pilot study is to test all aspects of the integrity of a study protocol and feasibility of the intervention AND of the trial design. The following are a list of objectives of a pilot study, which include objectives meant to establish feasibility of the intervention itself, as well as objectives meant to test the feasibility of the future trial designed to test the efficacy or effectiveness of the intervention.

In order to test the feasibility of the intervention itself, the pilot study may establish, for example:

1. Resources needs, such as:
   - Intervention and administrative staffing needs
   - Training needed for intervention and administrative staff
   - Mobilization of equipment/materials and other logistics involved in the roll-out of the intervention
   - Establishing and/or testing ongoing regulatory and reporting procedures
   - Refining or establishing monitoring/oversight procedures (especially in cases of multiple sites)

2. Acceptability of the intervention:
   - Is the intervention appealing to participants given any known side effects?
   - Are there any difficulties with administration of intervention to participants?
   - How long does the intervention take to administer, and is the time commitment and length of time the intervention takes acceptable to participants?
   - What are the rates of compliance to the intervention?
   - What is the retention rate of participants in the intervention?
   - Are the inclusion/exclusion criteria for the intervention acceptable/appropriate?

In order to test the feasibility of the full-scale trial, the pilot study may be used to, for example:

1. Test appropriateness of collection forms, questionnaires:
   - Ensure the form is comprehensible and appropriate, and that questions are well defined, clearly understood and presented in a consistent manner.

2. Test the randomization procedure:
   - Testing the logistics of the implementation of randomization procedure
   - Testing the success of the randomization procedure
   - Testing the acceptability of the randomization procedure for participants (i.e. does knowing that they may be randomized to control affect recruitment?)

3. Test recruitment and consent:
   - Testing the informed consent procedures
   - Estimating recruitment, consent, and retention rates
   - Identifying barriers to recruitment
   - Identify issues with treatment cross-over and/or potential contamination (i.e. are control participants inadvertently exposed to the treatment?)
4. Assist in the selection of most appropriate primary outcome measure:
   - Is it feasible to measure the primary outcome?
   - Can the chosen outcome be reliably measured?

Because the goal of the pilot is to identify issues with implementation of BOTH the intervention and the trial, investigators should strongly consider collecting qualitative data to supplement quantitative data collection [2].

**Using pilot studies to inform sample size calculations**

A common goal of pilot studies is to inform the sample size calculation for the future full-scale trial. There are conflicting opinions about whether and how pilot results should be used for this purpose.

Some feel that pilot study results should NOT be used for sample size determination due to the inherent imprecision in between group effect size estimates and elevated levels of Type I and II error [8]. For example, a pilot trial that finds an overly large effect may lead to underpowering of the full scale trial. A pilot trial that finds an overly small effect could lead to termination of an intervention that may in fact be effective OR the overpoweirng of the larger trial. Instead, it is recommended that sample size for larger trials be based on the smallest difference that is clinically meaningful.

Others feel that providing data for the sample size calculation of a larger trial is a major objective of conducting a pilot study [1]. In such cases, one might use the pilot sample to estimate central tendency (e.g. mean or proportion) and/or variability (e.g. standard deviation) in the population of interest. When a major objective of the pilot/feasibility study is to provide data for a sample size calculation, investigators must factor this into the proposed sample size for the pilot study itself, as discussed in the next section.

There is some consensus in the fact that the results of pilot/feasibility studies can be used for sample size determination if investigators proceed with extreme caution, preferably basing such calculation on a range of possible parameter values (i.e. not just those obtained from the pilot/feasibility study)[1, 2].

**Determining Samples Size for a Pilot/Feasibility Study**

The size of the sample needed for a pilot/feasibility study is determined by the precision by which investigators wish to estimate various end targets, which may range from participant adherence to study protocol (which may require few participants) to having estimates for the length of time needed to fill out a questionnaire, determine response rates, estimate adherence and attrition [9], or to estimate parameters needed for calculation of sample size of a larger study (which may require more participants).

For example, if an investigator wants to estimate potential dropout, expected to be around 20%, within 10 percentage points, with a confidence level of 90%, then s/he will need N=52 participants total to estimate that quantity². If the investigator wishes to be conservative in planning for dropout in the larger trial, he/she might choose to use the upper end of the confidence interval of that estimate (i.e. if

---

² Example uses Exact (Clopper Pearson) formula for two-sided confidence interval estimation.
the realized dropout rate was 20% with a 90% CI of [10%, 30%] then 30% may be assumed for the purposes of sample size calculation for the larger trial).

If one of the goals of the pilot study is to determine whether an instrument is appropriate for the population that will be studied in the larger trial, then he/she may wish to use the pilot to calculate statistics such as Cronbach’s alpha or test-retest reliability, then calculate the sample needed for a desired level of precision at an acceptable confidence level.

If a major goal of the pilot study is to estimate parameters related to treatment effect size for the full study (e.g. means, proportions, standard deviations), then the investigator will need to factor this into planned sample size for the pilot (note: pilots are not meant to be powered to find the true effect size as this is the goal of the larger study). Some authors have advocated simple rules of thumb, such as having N=30 per parameter estimated (e.g. mean or proportion) for the pilot study, then to use at least an 80% upper one-sided confidence limit of the estimated treatment effect to estimate power for the larger trial [10].

Overall, precision needed to estimate feasibility targets will need to be balanced between what is realistic financially and logistically and what the consequences of imprecision of that estimate will be as it applies to the larger trial.

Recommendations for Analysis of Pilot Studies

Analysis of Feasibility. Criteria for success of a pilot study should be stated clearly and should be based primarily on feasibility objectives [2]. Feasibility objectives are determined by the investigator and study team. For example, the study team may decide that feasibility is defined as having at least 70% sample retention, having a recruitment rate of at least 10 participants per month, and average satisfaction rating by participants of “very good” or “excellent,” and no more than 10% missing data for outcome measures.

The end result of a pilot study should be one of the following:

1. **Stop**: The main study is not feasible
2. **Continue, but modify the protocol**: The study would be feasible with modifications
3. **Continue without modifications, but monitor closely**: The study would be feasible with modification and close monitoring
4. **Continue without modifications**: The study is feasible as is.

Analysis of Estimated Treatment Effects. A pilot study should NOT be considered a preliminary test of the intervention hypothesis. Analysis of pilot/feasibility studies should be mainly descriptive. Any estimation or description of treatment outcomes should focus on confidence interval estimation. Inferential statistical tests should NOT be proposed as part of the pilot proposal/protocol. Any hypothesis testing undertaken should be done with extreme caution as covariates are likely to be imbalanced due to low sample size and confidence intervals are likely to be imprecise even when significant differences do exist. All results should be treated as preliminary and interpreted with caution.
Some may wonder whether a control arm is needed at all if inferential comparisons are discouraged. For the purposes of a study pilot, having a control arm provides a more realistic examination of recruitment, randomization, implementation, blinding procedures, and differences in loss to follow-up by treatment arm. It is important to understand how feasibility, consistency and acceptability will occur in a control arm.

It is also important to determine the possible differences in recruitment, retention, and acceptability that occur when there is a control arm, e.g. if a participant knows that they may receive placebo OR they know that they will be part of the intervention arm, it may change their willingness to participate, acceptability of the intervention or reported outcomes [8].

*Investigators should be compelled to publish the results of a pilot study.* There is a publication bias against pilot/feasibility studies, particularly those with negative or null results. It is important to the research community to have access to the results of pilot/feasibility studies to save resources from being unnecessarily spent on studies that are NOT feasible. Publishing the results of a pilot/feasibility study also helps avoid duplication of effort when assessing feasibility [2]. Examples of types of pilot/feasibility study findings potentially suitable for publication can be found in Table 1.

Authors should state in the conclusion of a manuscript whether the aims and objectives of pilot/feasibility work have been met and whether the results obtained from the pilot/feasibility study will lead to a future large-scale study[11].

Reporting guidelines from CONSORT’s extension to Pilot/Feasibility Studies [12] can be found in Appendix 1 (note: guidelines are ONLY for pilot/feasibility studies for randomized controlled trials).

**Words of Caution on Pilot and Feasibility Studies**

A *pilot should never be conducted simply because of small available sample size.*

A *pilot should NOT be considered a preliminary test of the intervention hypothesis.* Despite the view that pilot studies should NOT be used to assess treatment effectiveness, Arain et al (2010)[13] found that 81% of studies (in a meta-analysis of pilot/feasibility studies) incorporated hypothesis testing and some included tests of treatment effectiveness [11].

*Pilot studies are NOT, in general, appropriate for determining safety due to small sample size,* except in extreme, unfortunate cases where a death occurs or repeated serious adverse events occur [8]. A pilot/feasibility study COULD, however, be used to examine feasibility of adverse event reporting system.

*Prepared by: Alyssa Platt, MA*

*Reviewed by: Duke Global Health Institute | Research Design & Analysis Core*

*Version: 1.4, last updated September 28th, 2017*
Table 1. Pilot Study Findings Potentially Suitable for Publication

**Sampling information**
- Number of eligible subjects in research sites
- Proportion of eligible persons who consent
- Reasons for non-consent of eligible potential subjects
- Flow of eligible subjects over time
- Relative efficacy of different recruitment approaches or locations
- Differences in subjects recruited in different sites
- Setting practice and organizational features that affect recruitment
- Sample characteristics compared to intended population
- Sample attributes that might be potential confounding variables
- Attrition rates and patterns over time
- Differential attrition by subject attributes or arm assignment
- Causes of attrition

**Intervention delivery**
- Intervention content integrity
- Intervention purity
- Intervention dose integrity
- Interventionist training adequacy and requirements
- Interventionist competence
- Intervention reliability between interventionists
- Intervention effectiveness between interventionists
- Participant responses to interventions beyond outcome measures

**Measures in pilot studies**
- Respondent burden
- Participant difficulties with particular measures or parts of measures
- Appropriateness of order of measures
- Instrument reliability and validity estimates
- Missing data rates and patterns

**Study implementation**
- Protocol integrity: Interventions and measures delivered consistent with protocol
- Adequacy of randomization procedures
- Success of masking arm assignment from subjects and data collectors
- Identification of potential site or sample extraneous variables
- Anticipated and unanticipated human subjects protection issues
- Management of sensitive or legal issues
- Personnel time for recruitment, retention, intervention delivery, and measurement of study variables

**Pilot study outcomes**
- Measures of central tendency and variability
- Effect size estimates
Estimated sample size for parent study to detect clinically and statistically meaningful findings
Inferential tests when appropriate
Characteristics of data
Patterns of findings over time
Comparisons of different measures of the same construct
Results for subsamples with particular characteristics or individual subjects
Potential mediator variable findings
Safety and unanticipated outcomes
Lessons learned when predicted outcomes are not achieved

*Source: Conn et al., 2010 [14]
## Appendix 1. CONSORT 2010 checklist of information to include when reporting a pilot or feasibility trial [12]

<table>
<thead>
<tr>
<th>Section/Topic</th>
<th>Item No</th>
<th>Checklist item</th>
<th>Reported on page No</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Title and abstract</strong></td>
<td>1a</td>
<td>Identification as a pilot or feasibility randomised trial in the title</td>
<td></td>
</tr>
<tr>
<td></td>
<td>1b</td>
<td>Structured summary of pilot trial design, methods, results, and conclusions (for specific guidance see CONSORT abstract extension for pilot trials)</td>
<td></td>
</tr>
<tr>
<td><strong>Introduction</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Background and objectives</strong></td>
<td>2a</td>
<td>Scientific background and explanation of rationale for future definitive trial, and reasons for randomised pilot trial</td>
<td></td>
</tr>
<tr>
<td></td>
<td>2b</td>
<td>Specific objectives or research questions for pilot trial</td>
<td></td>
</tr>
<tr>
<td><strong>Methods</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Trial design</strong></td>
<td>3a</td>
<td>Description of pilot trial design (such as parallel, factorial) including allocation ratio</td>
<td></td>
</tr>
<tr>
<td></td>
<td>3b</td>
<td>Important changes to methods after pilot trial commencement (such as eligibility criteria), with reasons</td>
<td></td>
</tr>
<tr>
<td><strong>Participants</strong></td>
<td>4a</td>
<td>Eligibility criteria for participants</td>
<td></td>
</tr>
<tr>
<td></td>
<td>4b</td>
<td>Settings and locations where the data were collected</td>
<td></td>
</tr>
<tr>
<td></td>
<td>4c</td>
<td>How participants were identified and consented</td>
<td></td>
</tr>
<tr>
<td><strong>Interventions</strong></td>
<td>5</td>
<td>The interventions for each group with sufficient details to allow replication, including how and when they were actually administered</td>
<td></td>
</tr>
<tr>
<td><strong>Outcomes</strong></td>
<td>6a</td>
<td>Completely defined pre-specified assessments or measurements to address each pilot trial objective specified in 2b, including how and when they were assessed</td>
<td></td>
</tr>
<tr>
<td></td>
<td>6b</td>
<td>Any changes to pilot trial assessments or measurements after the pilot trial commenced, with reasons</td>
<td></td>
</tr>
<tr>
<td></td>
<td>6c</td>
<td>If applicable, prespecified criteria used to judge whether, or how, to proceed with future definitive trial</td>
<td></td>
</tr>
<tr>
<td><strong>Sample size</strong></td>
<td>7a</td>
<td>Rationale for numbers in the pilot trial</td>
<td></td>
</tr>
<tr>
<td></td>
<td>7b</td>
<td>When applicable, explanation of any interim analyses and stopping guidelines</td>
<td></td>
</tr>
<tr>
<td><strong>Randomization:</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Sequence generation</strong></td>
<td>8a</td>
<td>Method used to generate the random allocation sequence</td>
<td></td>
</tr>
<tr>
<td></td>
<td>8b</td>
<td>Type of randomisation(s); details of any restriction (such as blocking and block size)</td>
<td></td>
</tr>
<tr>
<td><strong>Allocation concealment mechanism</strong></td>
<td>9</td>
<td>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</td>
<td></td>
</tr>
<tr>
<td><strong>Implementation</strong></td>
<td>10</td>
<td>Who generated the random allocation sequence, who enrolled participants, and who assigned participants to interventions</td>
<td></td>
</tr>
<tr>
<td><strong>Blinding</strong></td>
<td>11a</td>
<td>If done, who was blinded after assignment to interventions (for example, participants, care providers, those assessing outcomes) and how</td>
<td></td>
</tr>
<tr>
<td></td>
<td>11b</td>
<td>If relevant, description of the similarity of interventions</td>
<td></td>
</tr>
<tr>
<td><strong>Statistical methods</strong></td>
<td>12</td>
<td>Methods used to address each pilot trial objective whether qualitative or quantitative</td>
<td></td>
</tr>
<tr>
<td><strong>Results</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
## Participant flow (a diagram is strongly recommended)

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>13a</td>
<td>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.</td>
</tr>
<tr>
<td>13b</td>
<td>For each group, losses and exclusions after randomisation, together with reasons.</td>
</tr>
</tbody>
</table>

## Recruitment

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>14a</td>
<td>Dates defining the periods of recruitment and follow-up.</td>
</tr>
<tr>
<td>14b</td>
<td>Why the pilot trial ended or was stopped.</td>
</tr>
</tbody>
</table>

## Baseline data

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>15</td>
<td>A table showing baseline demographic and clinical characteristics for each group.</td>
</tr>
</tbody>
</table>

## Numbers analyzed

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>16</td>
<td>For each objective, number of participants (denominator) included in each analysis. If relevant, these numbers should be by randomized group.</td>
</tr>
</tbody>
</table>

## Outcomes and estimation

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>17</td>
<td>For each objective, results including expressions of uncertainty (such as 95% confidence interval) for any estimates. If relevant, these results should be by randomized group.</td>
</tr>
</tbody>
</table>

## Ancillary analyses

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>18</td>
<td>Results of any other analyses performed that could be used to inform the future definitive trial.</td>
</tr>
</tbody>
</table>

## Harms

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>19</td>
<td>All important harms or unintended effects in each group (for specific guidance see CONSORT for harms).</td>
</tr>
<tr>
<td>19a</td>
<td>If relevant, other important unintended consequences.</td>
</tr>
</tbody>
</table>

## Discussion

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>20</td>
<td>Pilot trial limitations, addressing sources of potential bias and remaining uncertainty about feasibility.</td>
</tr>
</tbody>
</table>

## Generalizability

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>21</td>
<td>Generalizability (applicability) of pilot trial methods and findings to future definitive trial and other studies.</td>
</tr>
</tbody>
</table>

## Interpretation

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>22</td>
<td>Interpretation consistent with pilot trial objectives and findings, balancing potential benefits and harms, and considering other relevant evidence.</td>
</tr>
<tr>
<td>22a</td>
<td>Implications for progression from pilot to future definitive trial, including any proposed amendments.</td>
</tr>
</tbody>
</table>

## Other information

<table>
<thead>
<tr>
<th>Section</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>23</td>
<td>Registration number for pilot trial and name of trial registry.</td>
</tr>
<tr>
<td>24</td>
<td>Where the pilot trial protocol can be accessed, if available.</td>
</tr>
<tr>
<td>25</td>
<td>Sources of funding and other support (such as supply of drugs), role of funders.</td>
</tr>
<tr>
<td>26</td>
<td>Ethical approval or approval by research review committee, confirmed with reference number.</td>
</tr>
</tbody>
</table>
Appendix 2. Example Abstracts from *Pilot and Feasibility Studies Journal*

**Assessing the feasibility of evaluating and delivering a physical activity intervention for pre-school children: a pilot randomized controlled trial** [15]

*Published: 18 February 2016*

Sally E. Barber, Cath Jackson, Catherine Hewitt, Hannah R. Ainsworth, Hannah Buckley, Shaheen Akhtar, Daniel D. Bingham, Ash C. Routen, Carolyn Summerbell, Gerry Richardson, Helen J. Moore, Kate E. Pickett, Claire O’Malley, Shirley Brierley and John Wright

**ABSTRACT**

**Background**

Few evidence-based physical activity interventions for pre-school children are available. This two-armed pilot cluster randomized controlled trial aimed to evaluate the feasibility of conducting a full-scale trial and of delivering an outdoor physical activity intervention for pre-school children.

**Methods**

School was the unit of randomization, and follow-up occurred at 10 and 52 weeks. Trial feasibility was assessed by recruitment, retention and completion rates of primary (daily moderate-to-vigorous physical activity (MVPA)) and secondary (anthropometric, quality of life, self-efficacy) outcomes. Potential effectiveness was assessed for the primary outcome using a linear regression model comparing MVPA between trial arms adjusting for clustering by school. Feasibility of delivering the intervention was assessed by intervention fidelity and attendance. Semi-structured interviews with parents, intervention facilitators, and head teachers explored acceptability and capability to deliver the intervention as well as acceptability of the study design.

**Results**

Recruitment rates were 37% of schools (n = 10 schools) and 48% of pre-school children (n = 164 children). Retention of children to the trial at 52 weeks was 83.5%. Thirty-nine percent of children had valid primary outcome accelerometer data at baseline and 52 weeks. Response rates for secondary outcome measures ranged from 52 to 88% at 10 weeks and 59 to 80% at 52 weeks. The mean difference in daily MVPA between trial arms at 52 weeks was 0.4, 95% CI 16.3 to 17.0; p = 0.96. Fidelity of intervention implementation was 81%. Intervention attendance was higher (82%) during the summer initiation phase compared to autumn/spring initiation (50%). Parents, facilitators and head teachers found the intervention acceptable and beneficial.

**Conclusions**

Recruitment and retention rates suggest a trial in this outdoor setting with this population was feasible but is weather sensitive. However, strategies to increase accelerometer wear-time would need to be implemented for reliable primary outcome data to be obtained. There was high implementation fidelity by facilitators, and the intervention was seen as acceptable and deliverable. However, attendance was low and preliminary data showed no evidence of intervention effectiveness. A revised intervention, building on the successful elements of this pilot alongside adapting implementation strategies to improve attendance, should therefore be considered.

**Trial registration**

Trial registry name and number: Current Controlled Trials, ISRCTN54165860. Date of registration: 4 September 2012.

**Keywords**: Physical activity intervention Pre-school children Pilot randomized controlled trial Process evaluation Deprivation Ethnicity
Western medical acupuncture in a group setting for knee osteoarthritis: results of a pilot randomized controlled trial [16]

Published: 16 February 2016

Adrian White, Liz Tough, Vicky Eyre, Jane Vickery, Anthea Asprey, Cath Quinn, Fiona Warren, Colin Pritchard, Nadine E. Foster, Rod S. Taylor, Martin Underwood and Paul Dieppe

ABSTRACT

Background

Evidence suggests acupuncture may be effective for treating the symptoms of knee osteoarthritis. Offering this in a group setting may offer cost savings. The aim of this study was to establish the feasibility of a definitive trial to assess the clinical and cost-effectiveness of Western medical acupuncture given in groups, or given individually, for adults with severe knee pain attributable to osteoarthritis.

Methods

A pilot randomized controlled trial (RCT) was conducted. Participants were recruited from seven general practices in Plymouth, Devon. Acupuncture was provided, at a dosage that increased up to and including electroacupuncture if no pain relief was reported, by one experienced acupuncturist in a community clinic. Potentially eligible adults aged at least 45 years with knee osteoarthritis were identified from practice registers, screened and randomized to either: (1) standardised advice and exercise booklet alone (‘standard’); (2) booklet plus group acupuncture (‘group’); and (3) booklet plus individual acupuncture (‘individual’). Both acupuncture arms received up to ten treatments over 12 weeks.

Recruitment, retention and data completion rates were recorded, and participants completed questionnaires on acceptability. We collected pain, stiffness and function data (using the Western Ontario McMaster Universities Osteoarthritis Index; WOMAC) and general health (EQ-5D) and economic measures at baseline and 14 weeks post-randomization.

Results

We screened 149 people and randomized 60 (40 %), 20 per arm. The overall 14 week follow-up rate was 77 %, but only 70 % in the ‘standard’ group; 4.1 % of data points were missing. The study was acceptable to participants. Changes in WOMAC pain score (intention to treat complete case analysis) from baseline to 14 week follow-up were: ‘standard’, 0.4 (95 % confidence interval (CI) −1.4, 2.2, n = 14); ‘group’ −3.2 (95 % CI −5.1, −1.4, n = 17); ‘individual’ −2.4 (95 % CI −4.1, −0.7, n = 15).

Conclusions

A definitive three-arm trial is feasible. Further follow-up reminders, minimum data collection and incentives should be considered to improve participant retention in the follow-up processes in the standardised advice and exercise booklet arm.

Trial registration

ISRCTN05305406

Keywords: Primary health care Knee osteoarthritis Randomised controlled trial Acupuncture Healthcare delivery Pilot project
Prescribed computer games in addition to occlusion versus standard occlusion treatment for childhood amblyopia: a pilot randomized controlled trial [17]

Published: 11 June 2015

Vijay K. Tailor, Selina Glaze, Payal Khandelwal, Alison Davis, Gillian G. W. Adams, Wen Xing, Catey Bunce and Annegret Dahlmann-Noor

ABSTRACT

Background

Amblyopia ("lazy eye") is the commonest vision deficit in children. If not fully corrected by glasses, amblyopia is treated by patching or blurring the better-seeing eye. Compliance with patching is often poor. Computer-based activities are increasingly topical, both as an adjunct to standard treatment and as a platform for novel treatments. Acceptability by families has not been explored, and feasibility of a randomized controlled trial (RCT) using computer games in terms of recruitment and treatment acceptability is uncertain.

Methods

We carried out a pilot RCT to test whether computer-based activities are acceptable and accessible to families and to test trial methods such as recruitment and retention rates, randomization, trial-specific data collection tools and analysis. The trial had three arms: standard near activity advice, Eye Five, a package developed for children with amblyopia, and an off-the-shelf handheld games console with pre-installed games. We enrolled 60 children age 3–8 years with moderate or severe amblyopia after completion of optical treatment.

Results

This trial was registered as UKCRN-ID 11074. Pre-screening of 3600 medical notes identified 189 potentially eligible children, of whom 60 remained eligible after optical treatment, and were enrolled between April 2012 and March 2013. One participant was randomized twice and withdrawn from the study. Of the 58 remaining, 37 were boys. The mean (SD) age was 4.6 (1.7) years. Thirty-seven had moderate and 21 severe amblyopia. Three participants were withdrawn at week 6, and in total, four were lost to follow-up at week 12. Most children and parents/carers found the study procedures, i.e. occlusion treatment, usage of the allocated near activity and completion of a study diary, easy. The prescribed cumulative dose of near activity was 84 h at 12 weeks. Reported near activity usage numbers were close to prescribed numbers in moderate amlyopes (94 % of prescribed) but markedly less in severe amlyopes (64 %). Reported occlusion usage at 12 weeks was 90 % of prescribed dose for moderate and 33 % for severe amlyopes.

Conclusions

Computer-based games and activities appear acceptable to families as part of their child's amblyopia treatment. Trial methods were appropriate and accepted by families.

Keywords: Amblyopia Child Clinical trial
References


