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Pilot and feasibility studies: Is there a difference from each other and from a randomised controlled trial?

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ABSTRACT

Background: A crucial part in the development of any intervention is the preliminary work carried out prior to a large-scale definitive trial. However, the definitions of these terms are not clear cut and many authors redefine them. Because of this, the terms *feasibility* and *pilot* are often misused.
Aim: To provide an introduction to the topic area of pilot and feasibility trials and draw together the work of others in the area on defining what is a pilot or feasibility study.
Methods: This study used a review of definitions and advice from the published literature and from funders' websites. Examples are used to show evidence of good practice and poor practice.
Results: We found that researchers use different terms to describe the various stages of the research process. Some define the terms feasibility and pilot as being different whereas others argue that these terms are synonymous. All reflective papers agree that feasibility/pilot studies should not test treatment comparisons nor estimate feasible effect sizes. However, this is not universally observed in practice.
Summary: We believe that the term 'feasibility' should be used as an overarching term for preliminary studies and the term 'pilot' refers to a specific type of study which resembles the intended trial in aspects such as, having a control group and randomisation. However, studies labelled 'pilot' should have different aims and objectives to main trials and also should include an intention for future work. Researchers should not use the title 'pilot' for a trial which evaluates a treatment effect.

1. Introduction

During recent years, there has also been an increasing emphasis on the importance of preliminary work prior to the organisation of large-scale, publicly funded randomised controlled trials. Many large public funding bodies now expect substantial work to have been done prior to the main bid. Some funding streams, such as UK National Institute for Health Research (NIHR) Research for Patient Benefit (RfPB) [1] and the US NIH R34 funding mechanism [2], recognise this through the provision of substantial sums of money to support such work. The value of preliminary work is now recognised and researchers are encouraged to publish their pilot work in advance of their main trial, and some publishers are willing to publish such results. However, there remains much confusion about the purpose of preliminary work and also of terminology used. The NIHR use the terms 'feasibility' and 'pilot' to distinguish between different stages in the research process [3]. Although these terms are frequently used in the literature, they are used inconsistently and interchangeably [4], while other authors choose to use different terms completely to define the stages of development [5].

There is also the temptation to label a trial 'pilot' to excuse a small sample size, or one conducted in one locality, but still with the intention of running a study with treatment comparison as the main objective.

The aim of this paper is to provide an introduction to the topic area of pilot and feasibility trials. We will draw together the work of others that has been done in this area, describing

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current definitions, their overlaps and points of divergence. We use examples to illustrate good and poor practice and conclude with some recommendations on the use of the terms. This paper adds to our earlier work [4] by critiquing earlier definitions, and providing examples to support our criticism.

1.1. Current definitions

Within the pharmaceutical sector testing, drug efficacy has long had a tradition of clearly defined stages, from the initial phase 1 first-into-man studies through the phase 4 post-marketing studies. However, for large publicly funded trials, particularly of complex interventions and modes of care, the definitions and stages of trials have been less well defined/clear-cut. There have been several attempts to provide guidance on the definitions of a pilot and feasibility study. A review of papers published in 2001 in seven major journals looked at the objectives of pilot studies in the literature [6] to clarify the definition of pilot study. This was repeated in 2010, and the work extended to distinguish between pilot and feasibility studies in the article search and looking at the components of the studies [4]. The authors of these studies found that studies labelled 'pilot' generally used stricter methodology than studies labelled 'feasibility' and that pilot studies mostly reported their results as inconclusive and suggested further work, whereas feasibility studies did not state the same intention. They argue that the distinction between the two terms is not clear cut. However, they suggest the adoption of the NETSCC (NIHR Evaluation, Trials and Studies Coordinating Centre) definition which does distinguish between the two types of study [3].

The NETSCC [3] define feasibility studies as studies used to estimate important parameters that are needed to design the main study, e.g., standard deviation of the outcome measure, willingness of patients to be randomised, willingness of clinicians to recruit participants, number of people eligible, follow-up rates, response rates and adherence/compliance rates. Feasibility studies may have no plan for further work and their aim is to assess whether it is possible to perform a full-scale study.

The NETSCC [3] define a pilot study as a version of the main study run in miniature to determine whether the components of the main study can all work together. They suggest that a pilot should focus on the processes of running the main study, i.e., to ensure the mechanisms of recruitment, randomisation, treatment and follow-up assessments. The aim of the pilot is to provide training and experience in the running of the trial and to highlight any problems so they may be corrected before the main study begins. There must also be a plan for further work. A pilot study can be either external or internal to the main study.

This latter definition is comparable to the UK NICE definition of a pilot study as 'a small-scale "test" of a particular approach … The aim would be to highlight any problems or areas of concern and amend it before the full-scale study begins [7].'

However, in contrast, Arnold et al. [5] provided three separate definitions for different types of pre-clinical work: pilot work, pilot studies and pilot trials. They defined pilot *work* as 'any background research that informs a future study'; pilot *studies* as 'studies with a specific hypothesis, objective and methodology'; and a pilot *trial* as 'a stand-alone pilot study with a randomisation procedure'. Indeed the authors advocated against using the term *feasibility study*, arguing that it 'does not reflect the scope of many pilot studies'. These definitions differ from most others in that they distinguish between the different possible objectives of pilot studies, but do not include the term feasibility whatsoever. The movement through development stages is defined by using the words; work, study and trial instead of the terms feasibility and pilot.

Thabane et al. [8], in their tutorial on pilot studies, do not distinguish between feasibility and pilot studies and simply note that the terms are used synonymously. They do however note that the main focus of a pilot study should be to test the feasibility of conducting a full study rather than statistical significance, and that many pilot studies fail to recognise this.

Leon et al. [9] state that a pilot study can be used to evaluate the feasibility of recruitment, randomization, retention, assessment procedures, new methods and implementation of the novel intervention. A pilot study is not a hypothesis testing study. Safety, efficacy and effectiveness are not evaluated in a pilot. Contrary to tradition, a pilot study does not provide a meaningful effect size estimate for planning subsequent studies due to the imprecision inherent in data from small samples. Thus, effect sizes provided by pilot studies should not be used to power a subsequent full trial. Instead clinical experience should be used to define a *clinically meaningful* effect. A pilot study is a requisite initial step in exploring a novel intervention or an innovative application of an intervention. Pilot results can inform feasibility and identify modifications needed in the design of a larger, ensuing hypothesis testing study.

This is similar to the British Medical Research Council's (MRC's) complex interventions guidelines, which urge the reader to exercise caution when using the results of a pilot study to make assumptions about the required sample size, likely response rates, etc., when the evaluation is scaled up [10]. These guidelines do not give an exact definition of a pilot or feasibility study; instead, they focus on the outcomes of the feasibility and piloting stage. Investigators should be confident that the intervention can be delivered as intended and be able to make safe assumptions about the effect sizes, variability, recruitment rates and retention to aid in the designing of the main study. They do note that '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'.

1.2. Examples

Krarup et al. [11] describe a trial, the ExSTroke Pilot trial, to examine the benefits of exercise in patients who have had a stroke. They intended to recruit 300 subjects, but this was powered on a postulated difference in treatment groups from a surrogate outcome, the Physical Activity Scale for the Elderly (PACE). The reason for the term 'pilot' in the title could be inferred because the study was not powered for recurrent stroke, MI, or mortality. The results were published [12] as a randomised controlled trial. The trial was criticised because it did not follow guidelines for the developing of complex interventions such as those of the MRC [10], and 'we