

**Research for Patient Benefit Programme
Director's Message 6 -- March 2009**

Thinking about applying for funding for a pilot study?

Effective pilot work is often the key to a sound and successful research design. Lack of preliminary feasibility work can mean wasted resources, especially when a trial of a complex intervention is proposed, and applications are often turned down on the grounds that that pilot work has not been done. NIHR is well aware that it can be difficult to find funding for pilot work. For this reason, and notwithstanding its strong emphasis on patient benefit outcomes in the short to medium term, the RfPB Programme welcomes requests for funding pilot work. Both applicants and committee members, however, have asked for more guidance. What do we mean by pilot studies? What are the criteria by which they should be judged? How do pilot studies fit with the strong emphasis in the Programme on demonstrating patient benefit?

Many kinds of pilots

Clinicians, health services researchers and other social scientists share a similar basic understanding of pilot work. Pilot work is research conducted in advance of a main study. It aims to reduce some of the uncertainties associated with that study and to increase confidence that a research design will be appropriate and generate a successful result. For clinical researchers, the logic of the drug trial is often the starting point. Box 1 sets out a widely used adaptation of this, designed for complex clinical and non-clinical interventions. Using this framework, a pilot study is a Phase II trial/exploratory trial. It tests all the components of a larger study on a smaller scale. In essence, it is a main study in miniature. Anything short of this will tend to be regarded as 'pre-protocol' or 'preliminary' work.

Box 1: Phases for Development of a RCT

Preclinical	explores theory to select intervention, develop hypotheses and explore potential confounders
Phase I	identifies components of intervention and models impact on each other and on outcomes
Phase II	carries out an exploratory trial with a feasible protocol for comparing intervention with an alternative or with treatment as usual
Phase III	aims to conduct a definitive trial using a theoretically defensible, fully defined intervention that is reproducible with appropriate statistical power
Phase IV	determination of whether results can be replicated in uncontrolled settings over the long term

Source: Adapted from M. Campbell et al *Framework for the Design of and Evaluation of Complex Interventions to Improve Health*, BMJ 2000;321, 694-6

While these terms are likely to remain in widespread use, new and updated guidance www.mrc.ac.uk/complexinterventionsguidance is now in print. This extends coverage to non-experimental methods and broadens its concept of feasibility/piloting to encompass wider ranges and mixes of uncertainties. This helps to encompass differential understandings across the health research community of what pilot work might entail. Some, for example, will use the term 'pilot study' (or feasibility study) to refer to an examination of a particular subset of components of a planned research design that have been identified as unknowns or as particular challenges given the context of the study. The aims of a pilot in such a case may be focused very specifically on whether an intervention is acceptable to the target group, for example, or whether the proposed outcome measures need testing and further refinement for use with a specific study population. It is important and quite possible also to regard a fairly extended period of qualitative research as a pilot – where, for example, the research is intended as a test of whether there is a fit between the perspectives of patients and those of practitioners and researchers and hence whether the research question needs to be refined or reformulated. A pilot study therefore, does not need always to be a Phase II trial to be accepted into the RfPB Programme. Projects already accepted into the Programme portfolio bear witness to this.

Spelling it out

The key message to applicants, here as elsewhere, is 'never assume'. Never assume that your terminology will be understood in the same way by all who read your proposal. Be very clear what you mean by the terms you use. Whatever the terminology, committee members will want to be sure that a proposal provides satisfactory answers to some basic questions:

- What exactly is being piloted, and why?
- How will the pilot work be carried out?
- What are the outcomes that are envisaged as the end points of the pilot and what are the next steps likely to be?

The primary aim of a pilot is always to strengthen the design of a main study. Some pilots will be large, using the full amount of time and the full funding available. Others will be of more modest size. The key is to provide a clear rationale and an appropriate combination of goals. Box 2 provides an illustration of some of these.

Box 2: Why carry out a pilot study?

- to assess the feasibility of a protocol that has been developed for a full-scale trial
- to clarify the components of a complex intervention
- to refine a research question/set of objectives/hypothesis
- to refine one or more research instruments or measures
- to gauge the acceptability of a research plan to proposed participants and estimate likely attrition rates
- to establish the likely success of alternative recruitment strategies
- to provide data on which to begin to estimate required sample size
- to establish the likely staffing requirements and/or timetable needed for a main study
- to try out data analysis techniques
- to establish training requirements for those required to implement an intervention
- to explore variation in usual practice and consider its implications as the control arm of a trial
- to create interest in and support for the project in a local setting, building commitment and local ownership for the ultimate results

and ultimately...

- to convince a later funding body of the soundness of the design and the competence of the team.

Much standard guidance on preparing research proposals applies equally to pilot studies. It is always wise, for example, to include as much detail as possible on how the work will be done, what the sequence of events will be, and how the composition of the team is appropriate for the work. Pilot studies are a little more complex, however, when it comes to the crucial business for the Programme specifying outcomes and addressing the patient benefit case.

Assessing pilots in RfPB

A pilot should amount to a self-contained study, the intended outcome of which is a convincing protocol for a definitive trial or other well-designed study. While it will be rare that the pilot meets the criterion of 'showing tangible benefit for patients in the short to medium term' or 'feeding into daily practice', the committee will need to have some demonstration that the subsequent study will have a clear potential for such outcomes. Thus committee members need to be persuaded:

- that the pilot is necessary
- that the main study has potential patient benefit
- that there will be a good chance of securing funding for the main study.

In short, the overall trajectory for the research is of relevance alongside the design for the pilot and also needs to be given attention. In the long term, RfPB itself will be judged among other things on whether it has helped to secure good quality definitive studies through the pilots it has funded. If you know which funder you plan to approach to fund the full study then it is useful to include this information in your application. Success in gaining the resources for a pilot study, however, does not imply that this Programme or any other NIHR programme would fund the full study. The key message, however, is that a case for a pilot must also, in outline, be a case for the main study.

Box 3: Seven top tips for applying to RfPB for a pilot

Do distinguish clearly between the likely results of the pilot and the likely results of the main study. Both are part of the case you need to make.

Do ensure that there is evidence of some kind that provides support for the intervention you are proposing to pilot and that you cite this evidence.

Don't 'over claim' for the results of the pilot. In some cases the pilot will have a strong chance of producing findings that could result in service change and patient benefit. Often, however, it is appropriate simply that it strengthens the design of a trial.

Do consider involving patients and giving attention to process issues. These are often at the very heart of making good choices about what will work for a definitive study.

Do pay attention to value for money. Is it necessary to include all the elements you have identified or to have a sample size as large as you first anticipate? Is a full trial in miniature needed for this particular research question?

Do tick the box on the application form indicating that the application is for a pilot and make clear in your title that this is to be judged as a pilot.

Do consult the new complex interventions guidance at www.mrc.ac.uk/complexinterventionsguidance. It sets pilot work in the wider development-evaluation-implementation process.

Box 4: Some common questions

Do pilots stand less of a chance of being funded than definitive studies?

No. Proposals are treated individually and on their merits. If this changes, applicants will be informed.

Do some kinds of pilots stand a better chance than others?

No. While some committee members will take the view that a 'proper pilot' is the phase II exploratory trial, a sound and well-described case will persuade them that action short of this is appropriate.

Can I ever come back to the Programme for the main study?

Yes. It is likely, however, that a definitive study will need to involve a large and probably multi-centre trial with sums that way exceed the Programme's budget. In this case, you will stand a better chance at the pilot stage if you have already identified a likely funder for the main study as well as showing that the main study has potential for patient benefit. Bear in mind also that RfPB should not be seen as an opportunity to build a staged programme of work through repeated applications but more as an opportunity to 'kick-start' a project that has patient benefit but has little chance of development without some preliminary work.

Where do you stand on stages 0 (preclinical) and I in the initial complex interventions framework?

RfPB will fund pilot work where a case can be made that a clear and fairly rapid trajectory to a main study is likely. As noted above, it is not a source of funds for initiating a long-term programme of work from basic to applied. It is likely therefore that such work will be ruled out of scope. Preliminary scrutiny committees will always, however, look at cases on their merits, paying particular attention to the route to patient benefit.

Is RfPB the only place to go in NIHR for pilot studies?

RfPB is a significant funding stream for pilot work; however, the NIHR funds a wide range of programmes many of which have a track record in the funding pilot studies. Other sources of NIHR funding for pilots will include the HTA Programme and Programme Grants for Applied Research, but there may be other options as well and applicants should seek advice from either NIHR CCF or NETSCC, as appropriate, in order to check that the aims and remit of a particular programme are appropriate for the proposal being developed. Some programmes will have an ongoing funding stream for pilot work whereas others may fund pilots from time to time. There is no definitive answer on the best route to fund pilot work and applicants should check the NIHR website frequently for the most up to date advice and guidance.

Professor Celia Davies