

The case for more pilot studies

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The purpose of conducting a pilot study is to examine the feasibility of the study intervention, as it will be employed in a subsequent larger scale investigation.^{1,2} “Piloting” is focused on assessing the processes of the main study, for example, to ensure that recruitment, randomisation, treatment and follow-up assessments all run smoothly, especially when multiple sites and investigators are involved. Pilot studies can be especially useful and are recommended when evaluating interventions that contain several interacting components.^{3,4} Fine-tuning or even significant modification of the protocol may then take place, before committing to a larger study.

It is important to appreciate that pilot studies have different objectives compared with randomised controlled trials, and given their specific design and sample size, they should not simply be considered a preliminary test of the research hypothesis. Indeed, the null hypothesis for a pilot study is that it is not feasible to undertake the main investigation, with a type 1 error falsely committing the investigator to a larger body of work.⁵ As such, a pilot study’s sample size should be adequate to estimate key feasibility parameters (eg, eligibility criteria, recruitment rate, and separation of study interventions), so as to ensure an accurate assessment of study viability.⁶

Under certain circumstances, data from the pilot phase may contribute to the final analysis — which is usually referred to as an internal pilot study.⁷ However, pilot study data should not be combined with the main study if the methods have been modified or significant changes to the protocol have been made after the pilot phase.

In this issue of *Critical Care and Resuscitation*, Young and colleagues⁸ report the outcomes of the pilot phase of the ICU-ROX trial, a randomised controlled trial comparing conservative versus standard oxygen therapy in patients in the intensive care unit who are mechanically ventilated. The primary focus of this internal pilot study was to establish the feasibility of the study design in the first 100 randomised patients. Predefined and quantifiable outcomes were employed, and it was pre-specified that if some or all the aims of the pilot study were not achieved, then either the protocol would be modified or, if necessary, the study would be abandoned altogether in its current form.

Over a 10-month period, six medical-surgical intensive care units in Australia and New Zealand enrolled eligible patients. Forty-nine participants received conservative oxygen therapy and 51 standard care, yielding a median recruitment rate of 3.6 patients per site per month, higher

than that required for the main study. Ninety-five per cent of the participants were confirmed by independent monitors to fulfil all study eligibility criteria. Moreover, a conservative approach to oxygen therapy significantly reduced oxygen exposure compared with standard care, without increasing the incidence of hypoxaemia. As such, the authors concluded that it is highly feasible to complete the ICU-ROX trial in the remaining 900 participants. In addition, minor changes to the protocol were implemented in order to improve the quality and safety of the ongoing study. In this manner, the article neatly illustrates how piloting can help to develop consistent practices to enhance data integrity, and most importantly, it reassures us that the study design will actually result in a quantifiable difference in the treatment received by each group.

Despite their essential role, pilot studies are often poorly reported, with inappropriate emphasis on hypothesis testing.⁶ Moreover, authors often fail to identify the reason for undertaking a pilot study.⁵ Of note, a review of pilot studies in critical care medicine² suggests that few of them progress to published major trials. Likewise, Lancaster and colleagues⁹ found that while 50% of pilot studies reported the intention of further work, less than 10% were followed by a major study.⁶

The value of a precise, well conducted pilot study can, therefore, not be overstated, in that the results of such work invariably have an impact on the next phase of investigation. Indeed, given that poorly planned or executed clinical trials are essentially unethical¹⁰ — if not simply wasteful — pilot studies should really be viewed as a mandatory component of the larger research program. In many cases, it may be more appropriate to refocus scarce research resources elsewhere, rather than try to progress a study question with limited viability. Dissemination of these data is crucial, as they may affect several research groups working in the same area.

This effect on research groups reinforces that pilot studies are specifically designed to assess feasibility, and the term “pilot” should be used accordingly.¹¹ Clearly labelling an underpowered clinical trial as a pilot study (particularly post hoc), in order to justify the potential risk to patients and the associated expense, is manifestly inappropriate. In this respect, readers should be aware of the specific features of pilot studies when reviewing them,¹² because publishing the results of well conducted pilot or feasibility studies is a vital part of successful critical care research, irrespective of their outcome.

Competing interests

None declared.

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