TENOVUS SCOTLAND
SUPPORTING MEDICAL RESEARCH

Why Are Pilot Studies Necessary In Medical Research?

Medical research studies generally require hundreds, sometimes thousands, of patients to participate because researchers aim to identify relatively small effects of a new treatment amongst all the other influences on disease outcomes**.** The cost of including a single patient in a study is rarely less than £1000 and may be well over £10,000, and thus the overall cost of a research trial may reach many millions of pounds. To avoid influencing the analysis and interpretation of these large trials, no-one is allowed access to the results until the trial is finished**;** it is also not possible to adjust, change or improve procedures once the trial is underway. Researchers therefore will not embark on a large trial until some basic issues are understood. It would be both wasteful of resources and unethical to involve large numbers of patients in a study that was not properly informed by this basic groundwork.

Pilot studies undertake this basic groundwork. Major funding bodies rarely support a large trial without evidence of relevant pilot work.

Issues tested in **pilot studies** would include, for example -

- **The feasibility of patient recruitment procedures**
  Are there enough patients willing and able to participate? There is no point trying to undertake a large study if it will not be able to recruit enough participants.

- **The appropriate dose of a new medicine to use**
  Too low and it will not be effective**:** too high and it may cause an unacceptable level of side effects – it is important to get the dose right **before** starting a larger study

- **The efficacy of the treatment**
  Does the new medicine actually do what is expected of it? For example, if a new preventative treatment for stroke is supposed to work by 'thinning' the blood, it would be important to see convincing evidence of this blood-thinning effect in a pilot study before testing the stroke prevention effect in a large study.

- **The potential adverse effects of a new treatment**
  An initial assessment of side effects has to be made. If side effects are shown to be common or poorly tolerated, this could negate any benefits of treatment and would suggest a re-think before starting a large study.

- **The logistics of the study**
  Evidence is required that a) the various tests and treatments involved can be co-ordinated without interfering with each other or with the normal care and social activities of the patient, and that b) the study procedures are workable in the study centres.

- **The written materials in the trial**
  Information for study participants (and researchers), patient consent forms etc must all be checked to ensure that they are easily understood, informative and unambiguous.

It is important to note that success in a **pilot study** is not assessed in the same way as success in a larger trial – the outcome will not be a new medicine (or other treatment) immediately but it will underpin the processes and further studies that may eventually see a new treatment become available. A **pilot study** showing that something is impractical, unsuccessful or even dangerous is also valuable as it will prevent resources from being wasted (and patients from perhaps being placed at risk) in a major trial; it may also lead to a second **pilot study** with the treatment altered and refined. Even a **pilot study** with largely negative outcomes may help in building a research team whose members will work well together on future, hopefully more positive, projects.

Pilot studies are the foundation of clinical research: to start with a shaky foundation risks disaster while laying a solid foundation maximises the likelihood of success.

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