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Pilot Studies in clinical research

Steven A Julious[1]*

University of Sheffield, UK

Steven A Julious, Medical Statistics Group, ScHARR, University of Sheffield, Regent Court, 30 Regent Street, Sheffield, S1 4DA, UK. Email: S.A.Julious@Sheffield.ac.uk

A well designed pilot study is important in the evaluation of therapies [1,2]. In this special issue, different aspects of pilot trials are reviewed in different research settings to practically illustrate the methodological issues with these studies.

One of the objectives of a pilot trial is to assess the feasibility of undertaking a large definitive assessment of efficacy. This is true for all pilot trials and Wilson and colleagues describes the challenges of undertaking pilot trials of complex interventions [3]. They highlight how the ideas developed for Phase II drug development could be extended to the assessment of complex interventions [4]. In particular they considered how the efficacy of complex interventions could be assessed in the context of current early phase feasibility or pilot studies.

In oncology the assessment of a new treatment in a Phase II trial includes an assessment of whether there are sufficient signs of effectiveness to justify being tested in a phase III trial [5]. Wason and Jaki highlight how there are a large number of recent methodological developments that have aimed to improve phase early phase oncology trials and they describenovel approaches that they believe should be considered as alternatives to traditional designs.

Hee and colleagues highlight how pilot studies are often done to serve a variety of purposes with little consensus on their design [7,8]. The reviewed the literature on methods for pilot studies that are based on the use of Bayesian decision theory and under took a systematic reviewing methodology to identify relevant published work in the area. Bayesian decision approaches are appealing as can help to inform decisions at the end of the pilot trial [9,10]. Decisions such as whether there has been sufficient has been discharged to enable to the start of the definitive trial [11]. Hee and colleagues contend that Bayesian decision-theoretic approaches to be appropriate for the design of pilot due to their role in informing decisions regarding further future clinical research

All trials need a sample size justification. Not all trials need a sample size calculation [12]. This is particularly true for pilot trials. Cluster randomised trials are a common study design in health services research [13-14]. Eldridge and colleagues highlight the issues in estimating the sample size for a cluster pilot trial [14]. They undertake simulations to provide the distribution of the expected number of clusters for the main trial under different assumptions and conclude that pilot studies will usually be too small to estimate parameters required for estimating a sample size for a main cluster randomised trial with sufficient precision.

For individually randomised trials a pilot sample size is usually recommended in terms of a flat rule of thumb [16]. Whitehead and colleagues discuss how when the outcome is continuous, the sample size estimation requires an accurate estimate of the standard deviation of the outcome measure and

how a pilot trial can be used to get an estimate of this [17]. They describe how an external pilot trial sample size can be chosen in order to minimise the sample size of the overall clinical trial programme - the pilot and the main trial added together - and propose stepped rules of thumb for pilot sample sizes.

In summary, this special issue covers many concerns with pilot trials from assessing health technologies to drug trials and from cluster trials to individually randomised trials with many practical recommendations.

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