

Why do a pilot study?

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Pilot studies, or feasibility studies provide important information for design and justification of randomised controlled trials (RCTs). As large-scale RCTs are time-and resource-consuming, and most of funding bodies require preliminary evidence before supporting main RCTs, pilot studies should be as carefully designed and rigorously conducted as main RCTs should be.

In this sense, I was puzzled to read the article "a pilot clinical study on the Traditional Korean Medicine treatment of amyotrophic lateral sclerosis" by Kim et al.¹⁾

Pilot studies are usually conducted with the following objectives: 1) sample size calculation, 2) checking the integrity and feasibility of study protocol, 3) checking the feasibility of outcome measures, 4) checking the randomisation procedures, 5) checking the feasibility of recruitment and consent, 6) checking the acceptability of intervention, and 7) selection of most appropriate primary outcome measure.²⁾

Although Kim et al. stated their study as pilot, it is not a randomised study and sample size calculation or checking the randomisation procedures may not be possible. Nevertheless, the study protocol including intervention, outcome measures and analysis, and adverse events should have been

under careful scrutiny. In the discussion, the authors provide only general information on the intervention and outcome measures used but it is questionable whether future large-scale trials can be designed and performed based on the information presented in this article. It would have been more helpful if the authors reported whether amyotrophic lateral sclerosis functional rating scale revised (ALS-FRS-R) and modified Medical Research Council (MRC) scale used in the study worked well and which of the two would fit for the primary outcome measure, how and what unexpected problems were sorted out, or how patient informed consent were obtained.

Another important characteristic of pilot studies is that they cannot provide the definite conclusion, thus analysis and presentation of results should be descriptive and/or focus on confidence interval estimation. In Kim et al.'s study, however, statistical test results are presented, which should be avoided without formal hypothesis testing and proper power calculation.

Pilot studies should not be conducted simply because only a small number of patients are available or because of lack of funding. "Case series" would better fit for labelling Kim et al.'s study, rather than "a pilot study".

1. Kim S, Na W, Lim N, Lee D, Jang E, Song B.
A pilot clinical study on the Traditional Korean Medicine treatment of amyotrophic lateral sclerosis. Journal of Korean Pharmacopuncture Institute. 2009;12(1):53-65.
2. Lancaster GA, Dodd S, Williamson PR.
Design and analysis of pilot studies: recommendations for good practice. J Eval Clin Pract 2004;10(2):307-12.