Title of Your Poster Here: Panacea Cures All Ills in Weeks

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Introduction

The choice of control group is always a critical decision in designing a clinical trial. That choice affects the inferences that can be drawn from the trial, the ethical acceptability of the trial, the degree to which bias in conducting and analyzing the study can be minimized, the types of subjects that can be recruited and the pace of recruitment, the kind of endpoints that can be studied, the public and scientific credibility of the results, the acceptability of the results by regulatory authorities, and many other features of the study, its conduct, and its interpretation

Methods

The purpose of this guideance is to describe the general principles involved in choosing a control group for clinical trials intended to demonstrate the efficacy of a treatment and to discuss related trial design and conduct issues. This guidance does not address the regulatory requirements in any region, but describes what trials using each design can demonstrate. The general principles described in this guidance are relevant to any controlled trial but the choice of control group is of particularly critical importance to clinical trials carried out during drug development to demonstrate efficacy. The choice of the control group should be considered in the context of available standard therapies, the adequacy of the evidence to support the chosen design, and ethical considerations.

This guidance first describes the purpose of the control group and the types of control groups commonly employed to demonstrate efficacy. It then discusses the critical design and interpretation issues associated with the use of an active control trial to demonstrate efficacy by showing non-inferiority or equivalence to the control (Section 1.5). There are circumstances in which a finding of non-inferiority cannot be interpreted as evidence of efficacy. Specifically, for a finding of non-inferiority to be interpreted as showing efficacy, the trial needs to have had the ability to distinguish effective from less effective or ineffective treatments.

RESULTS

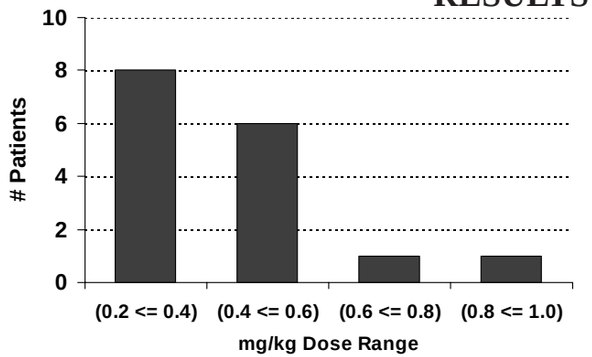
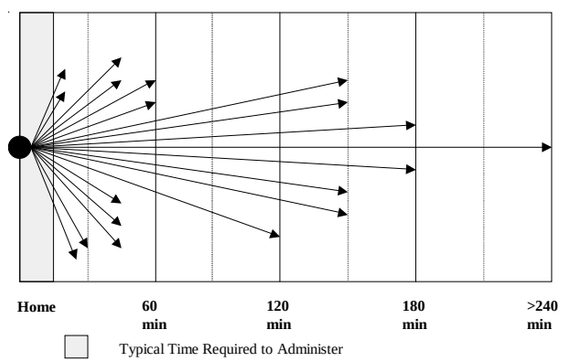


Figure 1. A concurrent control group is one chosen from the same population as the test group and treated in a defined way as part of the same trial that studies the test treatment, and over the same period of time. The test and control groups should be similar with regard to all baseline and on-treatment variables that could influence outcome.

Figure 2. Failure to achieve this similarity can introduce a bias into the study. Bias here (and as used in ICH E9) means the systematic tendency of any aspects of the design, conduct, analysis, and interpretation of the results of clinical trials to make the estimate of a treatment effect deviate from its true value.



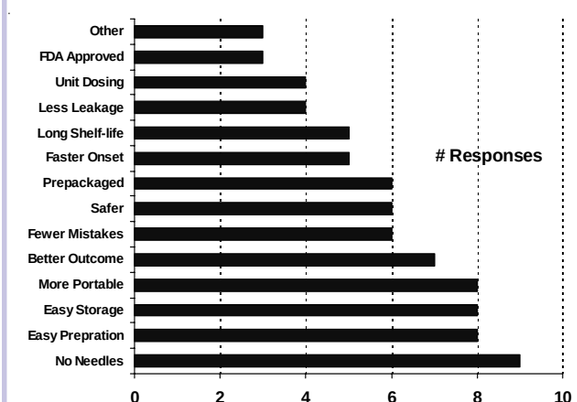


Figure 3. Randomization and blinding are the two techniques usually used to minimize the chance of such bias and to ensure that the test treatment and control groups are similar at the start of the study and are treated similarly in the course of the study (see ICH E9).

Control groups have one major purpose: to allow discrimination of patient outcomes (for example, changes in symptoms, signs, or other morbidity) caused by the test treatment from outcomes caused by other factors, such as the natural progression of the disease, observer or patient expectations, or other treatment.

The control group experience tells us what would have happened to patients if they had not received the test treatment or if they had received a different treatment known to be effective.

If the course of a disease were uniform in a given patient population, or predictable from patient characteristics such that outcome could be predicted reliably for any given subject or group of subjects, results of treatment could simply be compared with the known outcome without treatment.

For example, one could assume that pain would have persisted for a defined time, blood pressure would not have changed, depression would have lasted for a defined time, tumors would have progressed, or the mortality after an acute infarction would have been the same as previously seen. (see section 1.3.5).

Conclusions

- Panacea was effective and well-tolerated with minimal adverse effects in a rural pediatric population with acute prolonged seizures and seizure clusters.
- Numerous advantages were identified by the families and caregivers for Panacea use compared to other treatments
- Most issues of concern can be handled by adequate caregiver training in recognition of prolonged or cluster seizures and administration techniques for Panacea.