

ICU Clinical Trials: Are we expecting too much?

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Background

In reviewing the literature on ICU clinical trials, there is a paucity of "positive" results. This may be due to the outcomes or the statistical parameters chosen. It is also possible that post-randomization decision making during the hospital stay can play a significant role in the results obtained. Recent published clinical trials from a high tier journal were identified and analyzed to compare sample size, expected outcomes and statistical parameters utilized with an aim to identify trends within studies with positive and negative results. The primary aim of the study was to identify trends in clinical trial results and methodology which can support opinion that there should be a change in the way research in the ICU setting is conducted.

Methods

Literature search of the New England Journal of Medicine identified 39 randomized control trials (RCTs) between the years 2009 and 2019. Supporting publications regarding planned statistical analysis and research methodology were also identified for each study. The trials were compared with regards to sample size, expected outcomes, error (α , β), effect size (δ) and the statistical significance of results obtained.

Results

Of the 39 RCTs identified, only 8 had achieved statistically significant results based on their identified primary outcomes. The study was unable to identify significant trends to provide insight into why these trials had produced positive results as opposed to their counterparts. Amongst all trials, primary outcomes of mortality were the most common, with no notable difference between positive and negative trials. With regards to statistical parameters, studies were planned most frequently with a power of 0.8. Expected effect size varied significantly between studies and a trend was not able to be identified in general given the widely varying expected outcomes.

Discussion

While it was not possible to delineate a clear explanation as to why so few RCTs produce positive results, this small study supports the opinion that further work is needed to improve clinical trial design in the ICU to obtain more significant results to guide further practice.