Effect of intravenous ferric carboxymaltose vs placebo among patients with acute isovolemic anemia following gastrectomy: the FAIRY randomized clinical trial

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14. Sample Size and Statistics

14.1. Sample size consideration

The sample size is based on a superiority design assuming an FCM response (per primary endpoint definition) of 75% by week 12 and a response of 60% in the control group.

For patients with intervention, the improvement is expected to be at least 15% higher (ie., 75% responders). This change would also be considered medically significant and warrant early intervention.

Using these estimates, 400 patients are required to have a 90% chance of detecting, as significant at the 5% level, an increase in the primary outcome measure from 60% in the control group to 75% in the experimental group.

Calculation based on the formula (Pocock): \( n = f(\alpha, \beta) \times \left[ p_1 \times (100 - p_1) + p_2 \times (100 - p_2) \right] / (p_2 - p_1)^2 \) where \( p_1 \) and \( p_2 \) are the percent ‘success’ in the control and experimental group respectively and \( f(\alpha, \beta) = \left( \Phi^{-1}(\alpha/2) + \Phi^{-1}(\beta) \right)^2 \).

To account for potential patient drop-outs over the 12 week study period, the sample size is estimated at 450 patients (225 per group).

The parameters will be analyzed by a Pearson chi-square test or Fisher’s exact test (patient age and gender, clinicopathologic data, and morbidity), and Student’s t-test (Hb level before treatment and hospital days after treatment). The Z test will be used to determine whether or not a significant difference existed between two groups with respect to the slopes for changes in the Hb level during follow-up. (Pocock SJ. Clinical Trials: A Practical Approach. Wiley; 1983)

14.2. Analysis set

A. Efficacy Analysis Set

1) Intention to Treatment: That participants in the trials should be analysed in the groups to which they were randomized

2) Full analysis set (FAS): That participants who have results of at least one post baseline Hb value among the safety set

3) Per-Protocol set: The participants who fulfil the protocol in the terms of the eligibility, interventions, and outcome assessment.

B. Safety Analysis Set:
That participants in the trials should be analyzed in the groups to which they were randomized and who took study medication.