Official title:

The use of intravenous ferric carboxymaltose (FCM) without erythropoiesis-stimulating agents (ESA) in the treatment of anemia in cancer patients undergoing chemotherapy with or without radiotherapy

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Statistical Considerations

Sample Size Calculation

Patients will be recruited over a whole year tentatively between February and December, 2016. It is estimated that 2-5 % of encountered patients are anemic and eligible for enrollment.

Assuming at 90 % power, 5 % level of significance, based on previous pilot study: a proportion of responders of 0.54 and a standard deviation of 1.21, a response rate of 50 % or more being clinically meaningful, and testing for superiority; a minim of 25 subjects are required. To account for a 10 % chance for drop-outs the required sample is adjusted to 47.5 and approximated to the nearest tens. A total of fifty subjects will be required for the assessment. Since, each strata will be analyzed separately, and assuming both follow the same assumptions 50 subjects per strata will be recruited to make a total study sample of one hundred.[i,ii].

Recruitment of 100 patients in 10 months is doable.

East 6.3 software was used in the estimation of sample size.

Statistical Analysis for data presentation

Descriptive statistics will be used to present the two groups baseline characteristics and disease information. in addition to the patient's responses after the whole follow up period.

- Analysis will be done for the two groups separately:
    - **Group I**: Patient with iron-deficiency anemia based on iron studies at base line
    - **Group II**: Patient with non-iron deficiency anemia
- Hb test results will be presented as mean, median and range through all twelve weeks.
- Average percent change from baseline will be used to show the changes of Hb levels. Comparison between means of Hb level will be made between the baseline Hb and Hb levels in the following weeks using Paired T-test.
- Response to IV iron therapy will be assessed in relation to Hepcidin and CRP levels.
- Univariate analysis will be used to assess the effect of patient characteristics and disease information on the response using Chi square test or fisher's exact test.
- Adjusting for all significant factors that may affect the response will be done using Logistic regression.
- Odds ratio out of the Logistic regression will be reported with their corresponding 95% CI.
- A significance criterion of $p< 0.05$ will be used in the analysis.

All analyses will be performed using SAS version 9.4 (SAS Institute Inc, Cary, NC).

Safety Analysis

Rate and incidence of adverse events will be analyzed and reported. Number of patients requiring ESA or transfusions will be reported using percentages and counts.

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[i] Shein-Chung Chow, Jun Shao and Hansheng Wand, Sample Size Calculations in clinical research

[ii] Abhays Indrayan, Medical Biostatistics, CRC Press Taylor and Francis 2013.