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The Comparative Efficacy of the Masquelet versus Titanium Mesh Cage Reconstruction Techniques for the Treatment of Large Long Bone Deficiencies

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STATISTICAL PLAN AND DATA ANALYSIS:

Statistical Methodology

Descriptive statistics will be used to analyze the collected data specifically to:

- characterize the patient with segmental bone deficiency;
- estimate the proportion of healing with/without complications;
- cross-compare the recorded variables.

Paired Student t-test will be used to test the effects of the Masquelet versus the CTMC defect reconstruction on the outcome measures.

Analysis of covariance (ANCOVA) will be used for pair-wise comparison within the two treatment arms (graft Option A vs. Option B).

A mixed model for repeated measures will be conducted to evaluate:

- the effect of the Masquelet versus CTMC defect reconstruction on the outcome measures, adjusting for patient demographics and clinical characteristics;
- the effect of the specific defect characteristics (etiology, size, location, adjacent soft tissue involvement, etc) on the treatment outcome;
- the possible interaction effects between treatment and defect characteristics.

Multiple models will be used in case the proposed sample size would not have enough power to produce an accurate predictive model which accounts for all possible morbidities and interactions. Simple models using modern penalty function techniques (lasso, elastic net, boosted trees, etc.) to control model complexity should provide a useful initial predictive tool for each clinical and quality of life outcome.

Both receiver operating curve (ROC) analysis and clinical judgment will be used in determining the optimal clinical indications and timing for defect reconstitution with the definitive defect reconstruction using the Masquelet versus the CTMC technique.

Sample Size Justification

Based on the data from the PIs' preliminary clinical series of 20 patients, it can be assumed that 75% of patients with segmental bone defects reconstructed with the CTMC technique will heal without complications. In the present study we propose to enroll and follow-up 30 patients throughout 4 years of the clinical trial duration.

This sample size of 30 is largely determined by clinical feasibility. For the sample size 30, a two-sided 95% confidence interval for a single proportion using the large sample normal approximation will extend 0.134 from the observed proportion for an expected proportion of 0.75.

Our past experience with a prospectively investigating 20 patients with traumatic segmental defects presented to our institution (single site) within 3.5-year period suggests that the 30 patients to be enrolled in the proposed study within the anticipated time frame is very feasible considering broader inclusion criteria and multi-site study location.

Data Analysis

Prospectively collecting pertinent pre-, intra-, and postoperative study variables will establish a database of patients with segmental bone defects reconstructed with CTMC in combination with bone graft of bone graft substitute. A series of multivariate models will be constructed to model sizeable complex interactions between the patient, defect etiology and characteristics, process of care variables and surgical outcomes. Specific analyses will be performed to address the following questions:

- What are the characteristics of the patients who present with segmental bone deficiency warranting surgical reconstruction that achieve superior functional and quality of life outcomes when treated with the Masquelet versus the CTMC technique?
- What is the nature of a segmental bone defect and its related variables that determine superior outcomes after the Masquelet versus the CTMC reconstruction?
- What are the aspects of pre-, intra-, and postoperative care that differ for patients who:
  A) achieve poor outcomes;
  B) sustain treatment related complications;
  C) incur resource usage well in access of the average?