

Effect of Elemental Diet on Adult Patients with Eosinophilic Gastroenteritis (ELEMENT)

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13.3 Analysis Plan

Distribution appropriate statistics on age, sex, race will be presented to describe the cohort.

Since we anticipate a small (N=20) sample size for analyses, we will explore each one of the potential predictors one-at-a-time for association with outcome (as opposed to developing a full predictive model for outcome). All analyses will be performed using SAS 9.4 or higher version. A p value of 0.05 or less will be used to indicate the statistical significance.

13.3.2 Primary Analysis of Primary Endpoint(s)/Outcome(s)

Analysis of the complete histologic remission endpoint will give the frequency and proportion of those meeting criteria for complete histologic remission (mucosal eosinophilia <30 eos/HPF). Exact 95% confidence limits of the proportion will be presented. An exact confidence interval with a lower bound of no less than 0.75 will be used to demonstrate the efficacy of ED in EG/EGE patients seen in our initial pilot study.

We will also compare the proportion of patients who achieve complete histologic remission to a reference value of 0.56, the proportion who achieved complete histologic remission in the SFED group in our pilot trial, to compare efficacy of ED in EG/EGE patients to other dietary therapy. A one sample proportion test will be utilized to assess the difference in proportion of complete histologic remission compared to a value of 0.56.

13.3.4 Analyses of Secondary and Other Endpoint(s)/Outcome(s)

Secondary outcomes will include eosinophil density, symptoms, endoscopic features, peripheral eosinophilia, and quality of life scores. Outcomes will be measured pre and post the elemental diet, and paired t tests will be used to test whether the paired changes in outcomes significantly differ from 0. Relevant statistical assumptions will be assessed, and in cases of violation of these assumptions (i.e., extreme skewness or non-normally distributed data), the nonparametric Wilcoxon signed-rank test will be employed.

For eosinophil density, we will also calculate the reduction% as (baseline absolute peak eosinophil density - final absolute peak eosinophil density)/baseline absolute peak eosinophil density. A 50% reduction will be considered as partial histologic remission. The frequency and proportion (exact 95% confidence limits) of patients who have had partial histologic remission will be presented.