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A Phase 2 Study Evaluating a Proprietary Amino Acid Based Medical Food (Enterade) in Patients with Quality of Life Limiting Diarrhea Due to Carcinoid Syndrome and Other Neuroendocrine Tumors

Aman Chauhan[1]; Mark Evers[2]; Heidi Weiss[1]; Val Adams[1]; Jill Kolesar[1]; Donald Cohen[1]; Susanne Arnold[1]; Lowell Anthony[2]

[1]University of Kentucky; [2]Markey Cancer Center University of Kentucky

BACKGROUND: Diarrhea in neuroendocrine tumor (NET) patients is a major symptom that severely affects the quality of life. Enterade is an amino acid based oral rehydration solution which has been shown to restore intestinal villi and decrease secretory diarrhea and has also shown to improved survival and body weight in preclinical mice model. Retrospective data from NET patients presented at GI ASCO (2018) suggests antidiarrheal clinical activity in NET patients.

METHODS: This is an investigator-initiated, single center, open label, phase II study involving well differentiated neuroendocrine tumors with quality of life limiting diarrhea (4 or more stools/day). Two distinct subject cohorts (carcinoid syndrome diarrhea and non-carcinoid syndrome diarrhea) will be enrolled. The primary endpoint is a reduction in the frequency of diarrhea for individual subjects before and after enterade®. Subjects will maintain a daily stool diary. The mean of daily stool frequency between Day 1 and 28 will be considered baseline. The diarrheal frequency of each patient will be compared to their own baseline during the observation period. On day 29 (+/- 3 days), subjects will start enterade ® BID for 28 days (D 29-D56). On Day 57 +/-3 days) subject will return to clinic for assessment of response. Based on a prior published study (Kulke et.al), we will assume that the mean daily reduction in Bowel Movements

from baseline is equal to 1.5 (SD of change = 1.5) representing a large effect size = 1.0. A sample of 12 subjects in each cohort will provide over 90% power in detecting this effect size based on a two-sided paired t-test with % significance level. Additional 3 subjects will be added to each cohort to account for potential dropouts. Final sample size will be 15 subjects for each cohort.

Results: Trial is currently accruing.

CONCLUSION: ClinicalTrials.gov Identifier: NCT03722511