Trial of Eflornithine plus Sulindac in Patients with Familial Adenomatous Polyposis (FAP)

Due to disease progression, the current standard of care for patients diagnosed with FAP involves having a prophylactic colectomy or proctocolectomy with a lifetime of follow-up procedures involving endoscopies and surgical polypectomies.

Purpose: The purpose of this randomized, double-blind, Phase III trial is to determine if the combination of two study drugs (eflornithine plus sulindac) is superior to taking one of the study drugs alone (sulindac or eflornithine) in delaying time to the first occurrence of any FAP-related event. This includes: 1) FAP-related disease progression indicating the need for excisional intervention involving the colon, rectum, pouch, duodenum and/or 2) clinically important events which includes progression to more advanced duodenal polyposis, cancer or death.

Eligibility Criteria

To be eligible for this study, participants must be:

- Adults at least 18 years old
- Clinical diagnosis of classical FAP with positive APC gene mutation
- Ability to tolerate endoscopies and colonoscopies every 6 months
- Must meet criteria regarding extensiveness of prior surgeries (i.e. intact colon, rectum, etc.); more details can be found here: [http://www.clinicaltrials.gov/ct2/show/NCT01483144?term=FAP&recr=Open&rank=5](http://www.clinicaltrials.gov/ct2/show/NCT01483144?term=FAP&recr=Open&rank=5)
- Must meet criteria for hematopoietic, hepatic, and renal status taken within 30 days of randomization
- No clinically significant hearing loss
- Not pregnant or lactating; if necessary, must be willing to undergo pregnancy tests and use of contraception
- No significant blood in stool
- Must not have had cancer within past 5 years, excluding non-melanoma skin cancer, papillary thyroid cancer, or precancerous cervical dysplasia
- Must not be taking warfarin, fluconazole, lithium, Pradaxa® or other direct thrombin inhibitors, Plavix®, cyclosporine, other NSAIDs (such as ibuprofen, aspirin, diflunisal), diuretics (furosemide and thiazides), DMSO, methotrexate, probenecid, propoxyphene hydrochloride, Tylenol® (acetaminophen) preparations containing aspirin or cytotoxic chemotherapy drugs, Omega-3 fatty acids, corticosteroids, or other FAP directed drug therapy during study
- Minimal use of aspirin, 81 mg daily or 650 mg not more than once a week - eligible
- Must be 3 years out from colectomy with IRA/proto-colectomy or intact colon
- No intra-abdominal desmoid disease, stage III or IV
- Able to provide informed consent and follow protocol requirements

Length of Study Commitment

Participation lasts for approximately 24 months.
What is involved?

- Approximately 7 research appointments over 24 months which may include all or some of the following: physical exam, upper and lower endoscopy, blood and urine collection, hearing test, EKG, pregnancy test, and completion of questionnaires
- Phone call interviews and questionnaires between appointments
- Review of medical records to collect data about upper and lower endoscopies, treatments, and/or procedures until the study ends

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