Confidential

CC-CHOC Pediatric Point Person Project (P4)
Protocol Information Form

Follow this link to indicate your interest in Protocol **#22 Crohn's Disease**
https://redcap.ctsacentral.org/surveys/?s=7Gifps

Submitted November 8, 2012

Participant ID #22 Crohn's Disease

Sponsor CRO

Study Title A Pediatric Sequential Dose-Escalation Double-blinded, Placebo-controlled, Phase 1 Study to Evaluate the Safety and Tolerability of Oral Suspension XXXX in Pediatric Patients with Crohn's Disease

Short Study Synopsis This US Phase 1 study is intended to assess the safety and efficacy of a single dose level of study drug in pediatric patients with moderately to severely active Crohn's disease.

Target Population 24 pediatric patients: 2 groups, 5-11 and 12-17 year olds with Crohn's Disease for a minimum of 3 months

Study Objectives Safety and Efficacy. Unmet medical need.

Key Inclusion/Exclusion Criteria

Inclusion:

1. Patient is willing and able to stay on current treatments for Crohn's disease during the pre-treatment diary collection period and the two weeks following dosing with Investigational Product (IP) or placebo.
2. Hemoglobin, white blood cell and platelet counts within normal limits at screening.

Exclusion:

1. Patient with ulcerative colitis, indeterminate colitis, or ulcerative proctitis.
2. Bowel surgery in past 6 months prior to Screening.
3. Resection of more than 50 cm of the ileum.
4. Current ileostomy or colostomy.
5. Ongoing or active septic complications, is hospitalized or exhibiting signs of toxicity (sepsis), has symptomatic strictures, or impending obstruction or anticipating a need for blood transfusion for gastrointestinal bleeding or in whom surgical intervention may be imminent.
6. Patient with gastrointestinal abscess, perforation, active draining fistulae or fistulae that are considered clinically significant or perianal lesions.
7. Patient with history of colorectal cancer or colorectal dysplasia.
8. Parenteral or tube feeding.
9. Patient with evidence of infectious colitis, e.g., Clostridium difficile, Amoebiasis, Giardia lamblia or stools positive for other enteric pathogens, ova or parasites at Screening.
10. Patient with active hepatitis B virus, hepatitis C virus, liver cirrhosis or portal hypertension, cytomegalovirus, Epstein Barr Virus or herpes simplex virus or is known to be human immunodeficiency virus (HIV) positive.

11. Patient with primary sclerosing cholangitis.
12. Patient with active malignancy or treatment with anticancer drugs in the past 5 years.
13. Patient received methotrexate 4 weeks prior to Screening.
14. Patient received metronidazole 2 weeks prior to Screening.
15. Patient received antibiotic, antifungal or antiparasitic medication in the last 2 weeks prior to Screening and/or would potentially require this during the study treatment period. However, topical antibiotics and antifungals are allowed.

Total Number of Patients to be Enrolled 24

Enrollment Period 1 year

Miscellaneous Details/Requirements Subjects will be broken into two (2) age groups, 5-11 year olds and 12-17 year olds. For each age group there will be up to three cohorts of patients enrolled in a sequential manner. The first cohort within each age group will be treated with a low dose of IP or placebo (3:1). If well-tolerated, the next cohort within each age group will be treated with the next higher dose of IP (or placebo). If well-tolerated, the next cohort within each age group will be treated with the highest dose of IP (or placebo). Subjects will be closely followed for 2 weeks after the dose to assess safety and tolerability outcomes; a daily gastrointestinal symptom diary will be provided to the subject to complete each day during these two weeks. Subjects will return for a Week 2 visit and again at Month 6.

Has This Study Been Approved by the FDA? No

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Any question? Email ctsa_childhealth@ctsaC4.org