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

Follow this link to indicate your interest in Protocol **#23 T2 DM** https://redcap.ctsacentral.org/surveys/?s=7Gifps

Submitted 11-27-2012

Participant ID 23

Sponsor Industry

Study Title Single Dose PK and PD Study of an investigational glucose lowering drug in children aged 10-17 with Type 2 Diabetes

Short Study Synopsis Study Design: This is an open-label, randomized, parallel group designed study. Subjects will undergo screening evaluations to determine eligibility within 21 days prior to administration of study medication. Twenty-four (24) children or adolescents with T2DM will be randomly assigned to receive a single oral dose of 2.5, 5, or 10 mg of study drug (8 per dose group). Subjects will be confined to the clinical facility for at least 24 hours after study drug administration. Subjects will return to the clinical facility on the morning of Day 3 for the 48 hour PK sample collection and complete study discharge procedures. Physical examinations, vital sign measurements, 12-lead electrocardiograms (ECGs), and clinical laboratory evaluations will be performed at selected times during the study. Blood samples will be collected for up to 48 hours after study drug administration for pharmacokinetic (PK) analysis.

Target Population Children or adolescents with T2DM, male or female, between the ages of 10 to 17 years, inclusive.

Study Objectives **Primary Objective:** To assess the pharmacokinetics of a single dose of Study Drug in the range of 2.5 to 10 mg in pediatric subjects aged 10 to 17 years with T2DM.

Secondary Objectives:

• To assess the pharmacodynamics of a single dose of study drug in the range of 2.5 to 10 mg in pediatric subjects aged 10 to 17 years with T2DM.

• To assess the safety and tolerability of 2.5 to 10 mg single oral doses of study drug in pediatric subjects aged 10 to 17 years with T2DM.

• To characterize the pharmacokinetics of study drug following 2.5 to 10 mg single oral doses of study drug to pediatric subjects aged 10 to 17 years with T2DM.

Exploratory Objectives:

• To assess the swallowability of 2.5, 5, and 10 mg study drug tablets in pediatric subjects aged 10 to 17 years with T2DM.

• To assess the utility of dried blood spot (DBS) analysis relative to plasma quantification of concentrations of Study Drug and metabolites.

Key Inclusion/Exclusion Criteria Subjects must be treated with metformin or diet and exercise alone (drug naive) during the study period. Subjects must have a fasting plasma or serum glucose (FG) concentration ≤ 240 mg/dL, a plasma or serum C-peptide ≥0.8 ng/mL and a hemoglobin A1c >6.5 and <10.0% inclusive while on metformin and/or diet and exercise.

Total Number of Patients to be Enrolled 24

Enrollment Period Study anticipated to be complete Aug 30 2013

Miscellaneous Details/Requirements Site should have capabilities to house the subject for overnight stay and PK collection capabilities.

Has This Study Been Approved by the FDA? No

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Any question? Email ctsa_childhealth@ctsaC4.org