

A Pilot Study to Assess the Safety, Tolerability and Pharmacokinetics (PK) and Pharmacodynamics (PD) of Cysteamine Bitartrate Delayed-release Capsules (RP103), Compared to Cysteamine Bitartrate Capsules, (Cystagon®) in Patients with Nephropathic Cystinosis.

The University of California, San Diego (UCSD) will be studying an investigational cysteamine drug (RP103) for the potential treatment of cystinosis. RP103 will be compared to the existing treatment, Cystagon®. Dr. Bruce Barshop is the principal investigator for this study.

The study requires a 5 day/4 night in-patient stay in the Clinical Research Center and involves blood draws and other evaluations. Travel arrangements will be made at no expense to the participant and compensation for participating in the study will be provided. Daily living expenses for a family member or guardian accompanying a minor will also be provided.

Eligible patients must be on a stable dose of Cystagon® for 21 days prior to starting the study, be able to swallow the 150 mg capsules whole, and not have received a transplant. More information is available at clinicaltrials.gov [link to: <http://clinicaltrials.gov/ct2/show/NCT00872729?term=cystinosis&rank=3>].

Raptor Pharmaceuticals is sponsoring this study. It is the first clinical study planned with Cysteamine Bitartrate Delayed-release Capsules.

Participation in research is always entirely voluntary. If you are interested in participating and would like more information, please contact Betty Cabrera B.S., M.P.H. at University of California San Diego Medical Center, San Diego, California, United States, 92103. Phone: (619) 471-9554 or email: blcabrera@ucsd.edu.