

Phase 3 Study of Cysteamine Bitartrate Delayed-release Capsules (RP103)

This multi-center research study will investigate a new cysteamine drug (RP103) for the potential twice a day treatment of cystinosis. RP103 will be compared to the existing four times a day treatment (Cystagon[®]),

The study will require the time commitment of 20 clinic visits over 10-11 weeks. Most of these clinic visits occur in clusters of 3-4 days in a row:

- Screening Visit
- Run-in: Weeks 1, 2, 3
 - take Cystagon[®]
 - 3 clinic visits during week 1; 1 clinic visit during week 3
- Period 1: Weeks 4, 5, 6
 - take RP103 or Cystagon[®]
 - 3 clinic visits during week 4; 3 clinic visits during week 6
- Period 2: Weeks 7, 8, 9
 - take the opposite of what was taken during Period 1
 - 3 clinic visits during week 7; 3 clinic visits during week 9
- Follow-up Visit, 1 week later
 - or the option to enter a longer study with RP103

There will be blood draws, overnight fasts and other evaluations. Travel and accommodations for participating in the study will be provided at no cost to the patient. Daily living expenses for a family member or guardian accompanying a minor will also be covered.

Eligible patients must be on a stable therapeutic dose of Cystagon[®], be able to swallow Cystagon[®] capsules whole, not have received a kidney transplant and take all medications orally, not through a gastric tube. More information can be found at www.clinicaltrials.gov.

An extension study to determine the safety of long-term administration of Cysteamine Bitartrate Delayed-release Capsules is planned. Patients who complete this Phase 3 study will be offered the opportunity to be treated with Cysteamine Bitartrate Delayed-release Capsules until they are approved by the FDA or until Raptor Therapeutics withdraws its application with the FDA (for whatever reason).

If you are interested in participating and would like more information, please contact:

Margo Kamel, working with Dr. Larry Greenbaum at Emory University in Atlanta, GA
Email: cystinosistrial@oz.ped.Emory.edu
Phone: 404-712-9923

Heather Price, working with Dr. Craig Langman at Children's Memorial Hospital in Chicago, IL
Email: hprice@childrensmemorial.org
Phone: 773-755-6368