

Delayed-Release Cysteamine Bitartrate Capsules (DR Cysteamine) Clinical Trial Plan Update

Raptor is currently planning a pivotal Phase III clinical trial for DR Cysteamine (delayed-release cysteamine bitartrate) as a drug candidate for the potential improvement in the management of nephropathic cystinosis (cystinosis), a serious lysosomal storage disease.

We expect that this clinical trial will enroll approximately 15 patients. The documentation for this trial has not yet been submitted to the FDA, but it is Raptor's hope to begin this study in the summer or fall of 2009. In order to be eligible for this clinical trial, patients must be on a stable dose of the currently marketed Cystagon® therapy, be able to swallow capsules whole and not yet have had a kidney transplant.

Raptor is currently recruiting clinical centers to participate in this study. Clinical trial centers will be selected primarily for their experience with the care of cystinosis patients and expertise with white blood cell cystine assay sample preparation. Clinical trial participants will have to travel weekly for 14 weeks to the clinical center and a laboratory that has experience preparing white blood cells for the cystine assay. During this 14-week study period, patients also will be hospitalized for two long weekends in a clinical research unit for more detailed evaluations.

Reimbursement will be available to assist with the costs associated with clinical trial participation, such as travel.

The FDA granted an orphan drug designation for DR Cysteamine for the treatment of cystinosis in 2006. According to the FDA, "[h]istorically, the approval time for orphan products as a group has been considerably shorter than the approval time for other drugs ... often due to the fact that many orphan products receive expedited review or accelerated approval because they are for serious or life-threatening disease." Although there are no guarantees that this drug will be approved, Raptor believes that their study plan, together with an orphan drug designation, will help in bringing this new product to the market as soon as possible.

Raptor remains committed to improving drug targeting to expand the reach and benefit of existing therapeutics into underserved patient populations, especially the cystinosis community.

For more information, please talk with your doctor.