

Raptor Pharmaceuticals Presents

at International Cystinosis Congress

By Valerie Hotz

Ted Daley, President of the clinical division of Raptor Pharmaceuticals Corp., was one of over 20 featured speakers at the 5th International Cystinosis Congress, held in Dublin Ireland this summer. Daley presented information in the poster session about his company's collaborative work with researchers at the University of California, San Diego, to develop DR Cysteamine, a delayed-release form of cysteamine, the only drug treatment for cystinosis approved for sale by the Food and Drug Administration and the European Medicines Agency. Daley's presentation was of special interest to all conference attendees.

After returning from the conference I had an opportunity to meet with Ted Daley. "Current Cystagon therapy is the benchmark for efficacy and this is what we want to match with our final form of DR Cysteamine. We are looking into dosage forms that can be applicable to infants and toddlers, as well as older patients. We believe it will improve patient compliance for two reasons. First, we expect that we'll be able to reduce dosing frequency and second, because it is absorbed in the lower intestine rather than the stomach, there may be reduction in gastrointestinal side effects compared with current therapy," says Daley.

The idea for a delayed-release form of cysteamine was conceived by Dr. Jerry A. Schneider and Dr. Ranjan Dohil of the University of California, San Diego. Dr. Jess Thoene's pioneering research conducted at Dr. Schneider's lab led to the initial development of cysteamine therapy in 1978. Cysteamine removes Cystine from cells and thus slows progression of the disease. Mylan Laboratories manufactured cysteamine in capsule form and has been marketing it under the trademark Cystagon since 1994.

With the support of a research grant from the Cystinosis Research Foundation, Dr. Schnieder and Dr. Dohil initiated a series of studies at UCSD to test the effectiveness of bypassing the stomach and delivering cysteamine further down in the gastrointestinal tract. Cystagon TM capsules were generously donated by Mylan Laboratories for the studies. Based on their findings, they filed patents for delayed-release forms of cysteamine, which they believe will be better tolerated by patients. Raptor obtained worldwide exclusive license to UCSD patents in 2007.

"Without Dr. Schneider and Dr. Dohil, we would have no program. The concept of delayed-release cysteamine was proven by them in their initial study and they have been ingenious in how they have gone about it. Their dedication to their patients is wonderful.

Improving the quality of life for Cystinosis patients is our goal. Thanks to them, we have the opportunity to work together collaboratively to bring the product to market,” explains Daley.

Raptor Pharmaceuticals’ plan is to design its clinical studies in such a way that results are achieved that support a New Drug Application (NDA) at the earliest possible date. “We are consulting with Drs. Schneider and Dohil on the study design. For the patient study we anticipate an outpatient study with visits to the clinic for initial screening and blood draws. We plan to gauge efficacy by measuring Cystine levels in white blood cells.”

Raptor’s planned clinical trial of DR Cysteamine will be separate from the ongoing UCSD study, and will likely take place at multiple locations which include UCSD and other academic medical research centers. “We want to make it an easy trip to the clinic for the individuals involved in trials.”

Daley anticipates beginning clinical studies of DR Cysteamine for Cystinosis in the first half of 2009 and hopes to submit an NDA to the Food and Drug Administration by the end of 2009. “Many factors affect the timeline, some of which are out of our control. For instance, there may be complications in the formulation stage that is taking place at this time. Our general goal is to have an improved form of the drug available by early 2010, but there are still many issues to be resolved that can affect this timeline,” says Daley.

DR Cysteamine potentially has applications to other metabolic and neurodegenerative diseases including Huntington’s disease and Batten disease. Raptor is in discussions with clinical researchers about collaborating on clinical studies for other diseases. “These are much earlier stage clinical development programs than Cystinosis. Our primary lead focus is Cystinosis. We have great respect for Drs. Schneider and Dohil and feel our experience and capabilities will complement their efforts well,” says Daley. For more information about Raptor Pharmaceuticals, please visit the web site at www.raptorpharma.com