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RE: Cystinosis Research Foundation

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Leading Cystinosis Researcher Says Advancements In Treatments, Cure Of Rare Disease May Be Hastened Following First International Symposium

The world's leading expert on the rare disease cystinosis said the first International Cystinosis Research Symposium held recently in Irvine may speed up progress for new treatments and a cure for the fatal ailment that strikes mostly children.

Dr. Jerry Schneider, a pediatrics research professor at the University of California at San Diego who's regarded as the most experienced and knowledgeable cystinosis researcher, said the event was the first meeting for most of the more than 60 researchers from the United States and Europe who have been working on saving cystinosis sufferers.

"The symposium was very important for cystinosis because many of the participants began sharing information on their research efforts – information that generally isn't shared until it's published, which often takes months or years. This can only help accelerate new medical and scientific advancements we've been working to achieve for so many years," Dr. Schneider said.

Cystinosis is a metabolic disease that slowly destroys every organ in the body, including the liver, kidneys, eyes, muscles, thyroid and brain. In patients with cystinosis, the amino acid cystine accumulates in the tissue due to the inability of the body to transport cystine out of the cell. This causes development of crystals, resulting in early cell death. There is a drug, Cysteamine, that can prolong the patient's life, but there is no cure. Almost all sufferers succumb before 40 years old. The disease afflicts an estimated 500 people, mostly children, in the United States and 2,000 worldwide.

Investigators from the United States, France, Germany, England, Italy and The Netherlands attended the two-day April symposium at the Arnold and Mabel Beckman Center of the National Academies of Sciences and Engineering, adjacent to the University of California at Irvine.

Twenty-four researchers presented their current bench and clinical studies on cystinosis. Four of the presenters are researchers from the University of California at Irvine. All the

research presented was funded by grants from the Cystinosis Research Foundation of Irvine.

“The symposium also was unusual in that it included not only major researchers in this field but researchers new to the field. Several investigators who had just received funding to study cystinosis told me how much they learned about cystinosis at the symposium and how helpful it would be to their research plans,” Dr. Schneider said.

“I think many of the participants made contacts that will lead to important cooperative efforts. Additionally, several participants were able to meet a cystinotic patient for the first time in their careers,” he said.

The scientific exchange was sponsored by the CRF, Sigma-Tau Pharmaceuticals Inc. and Bennu Pharmaceuticals Inc., which recently obtained an exclusive, worldwide license for EC Cysteamine. EC Cysteamine was developed with CRF grants by Drs. Schneider and Ranjan Dohil at the University of California at San Diego.

Development of EC Cysteamine represents the first breakthrough in treatment in 20 years. It cuts the patient’s dosing frequency of the medication from 12 hours to six hours. As a result of taking the medication less frequently, compliance improves, side effects are reduced and most importantly, children are able to sleep through the night.

The active ingredient in EC Cysteamine also has demonstrated potential in clinical studies as a treatment for other metabolic and neurodegenerative diseases, including Huntington’s Disease and Batten Disease.

Drs. Dohil and Schneider, pediatric research professors at the UCSD’s School of Medicine, co-chaired the event. Dr. Schneider, chairman of the CRF’s Scientific Review Board, has been involved in cystinosis research for more than 40 years.

The CRF was founded by Jeff and Nancy Stack. Jeff is a managing director of the SARES●REGIS Group, a diversified real estate company in Irvine. The Stack’s daughter Natalie, 17, was diagnosed with cystinosis as an infant.

The Cystinosis Research Foundation is dedicated to finding better treatments to improve the quality of life for those with cystinosis and to finding a cure for this devastating disease. The CRF raises funds to support bench and clinical research toward improved treatments and a cure for cystinosis. The CRF also seeks to educate the medical and public communities about cystinosis to ensure early diagnosis and proper treatment.

Since its formation in 2003, the CRF has raised more than $6.5 million, $5.1 million of which has been funded or committed for cystinosis research, making the CRF the largest non-profit fund provider of cystinosis research in the world. Currently the CRF funds studies with researchers in The Netherlands, France, Germany, Italy and the United States.

For more information, call the Cystinosis Research Foundation at 949-223-7610 or visit www.natalieswish.org.

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