CURE CYSTINOSIS INTERNATIONAL PATIENT REGISTRY LAUNCHED

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A partnership between the Cystinosis Research Foundation and the Cystinosis Foundation along with a collaboration of 12 advocate foundations from around the world has launched the first global cystinosis patient registry. The registry's goal is to connect patients with researchers and others developing potential new treatments and a cure for the metabolic and fatal disorder that afflicts about 2,000 persons, mostly children, worldwide.

The purpose of the Cure Cystinosis International Registry is to identify people with cystinosis worldwide and collect their medical histories and information. This information will allow clinicians, researchers and pharmaceutical companies to accelerate novel treatments and a cure for cystinosis.

"The cystinosis community has experienced exciting scientific advancements to treat and cure cystinosis. Currently there is a proliferation of research activity, breakthroughs and hope. The CCIR is a central hub of information and will be used as a resource for the research community and could prove vital to advances in the care and treatment for those with cystinosis," said Nancy Stack, President of the Cystinosis Research Foundation.

The CCIR is the only registry created specifically for individuals with cystinosis and will contain current information regarding cystinosis clinical trials and studies. All patient information is de-identified (anonymous) and held in a secure data base accessible only by the CCIR curator. Information that could identify participants and their family members will not be shared without their expressed written approval. Participants will also be able to view aggregate data allowing them to view how they fit within the larger cystinosis community.

The registry was formed following discussions which began in 2009 among leaders in the cystinosis community who saw the

need to establish a new and comprehensive resource to connect and serve the needs of the entire cystinosis community. The CCIR's organizers come from family foundations around the world and the cystinosis academic and scientific communities. These groups are focused on the need to accelerate the research process in the quest to find the cure for cystinosis.

In patients with cystinosis, the amino acid cystine accumulates in the tissue due to the inability of the body to transport cystine out of one of the compartments of the cell. Cystinosis is a metabolic disease that slowly destroys every organ in the body, including the liver, kidneys, eyes, muscles, thyroid and brain. There is a medicine that prolongs the children's lives, but there is no cure. Most cystinosis sufferers succumb to the disease or its complications by age 40.

CCIR officials say recruitment for clinical trials can be a lengthy process, especially for a rare disease like cystinosis. Participation in this registry will help speed up the recruitment process and facilitate and expedite clinical trials, officials said.

Plans are to offer the website in French, Italian and Spanish following the launch.

One of the major features of the registry is a professional/researcher portal that will allow the scientific and pharmaceutical communities to request access to de-identified patient information. Those seeking patient information have to meet stringent requirements governing patient medical data, including patient approval. Each request will be reviewed by the CCIR curator and operations board.

Twelve other cystinosis foundations have joined as advocates of the CCIR. They are: the Cystinosis Awareness & Research Effort in Canada; the Australian Cystinosis Support Group; Cystinosis France, Cystinosis Foundation UK, Cystinosis Ireland, The Cystinosis Foundation, New Jersey Chapter; Cystinosis Support Group South Africa, 24 Hours for Hank; Hope For Holt; Jenna & Patrick's Foundation Of Hope, Joshua's Journey of Hope and Tina's Hope for a Cure.