The North American Pediatric Renal Trials and Collaborative Studies and Horizon Pharma plc Announce New Long-Term Cystinosis Registry

September 27, 2018

CHICAGO--(BUSINESS WIRE)--Sep. 27, 2018-- A prospective longitudinal natural history registry designed to provide long-term data evaluating people living with cystinosis was launched today at a meeting of The North American Pediatric Renal Trials and Collaborative Studies (NAPRTCS) in Chicago, Ill. The registry will collect and organize physician-reported data that will help healthcare professionals better understand the natural history of cystinosis. Data collected will also be available to researchers exploring new potential treatments for cystinosis. Utilizing the established NAPRTCS infrastructure which collects data on patients with chronic kidney disease, on dialysis and following kidney transplant, the cystinosis registry was made possible through a partnership between NAPRTCS, leading physicians in the cystinosis community, and the medical leadership at Horizon Pharma plc (Nasdaq: HZNP).

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20180927005242/en/

“Initial cystinosis natural history studies were conducted decades ago by the National Institutes of Health and there have been few studies since that time that track the progression of cystinosis,” said Bradley Warady, M.D., professor of pediatrics at the University of Missouri-Kansas City School of Medicine, director of pediatric nephrology at Children’s Mercy Kansas City and member of the NAPRTCS board of directors. “With recent advancements, people with cystinosis are living longer lives; however, our understanding of the disease in the context of these recent advancements and new treatments is limited. By gathering data from people living with cystinosis over an extended period of time, the NAPRTCS registry will provide key information for physicians and researchers.”

People living with cystinosis under the age of 25, with consent from their guardian, can be enrolled into the registry by their physician. All of the patient data will be blinded, which means that individual names or any other information identifying a specific person will not be made available to those exploring registry data. Any person under the age of 25 can be enrolled in the study, regardless of what therapies or medications they are receiving.

“This registry will help us better understand cystinosis, identify management improvements, and explore new treatment options that arise,” said Paul Grimm, M.D., professor of pediatrics (nephrology) at the Lucile Salter Packard Children’s Hospital at Stanford. “The registry will also help us answer a number of questions related to the long-term outcomes for patients and the long-term effects of cystinosis on the kidneys and all other organ systems.”

While Horizon provided NAPRTCS financial support to develop the registry, the company does not own any of the data – it belongs entirely to the medical community and is accessible through a securely protected online portal managed by NAPRTCS. Interested healthcare professionals should visit www.naprtcs.org.

“This registry is an example of a prioritized initiative based on input we’ve heard from physicians and researchers,” said Jeffrey Kent, M.D., senior vice president, medical affairs, Horizon Pharma plc. “A key component of understanding the opportunity for new medicines for rare diseases is understanding the natural history of the disease. In order to do that, there needs to be a system where this information can be collected. This registry will allow the medical community to gather and synthesize data that can be evaluated to better understand cystinosis and explore new approaches that can advance the treatment of the disease.”

About Cystinosis

Nephropathic cystinosis is a rare, life-threatening metabolic lysosomal storage disorder that causes toxic accumulation of cystine in all cells, tissues, and organs in the body. If untreated, elevated cystine accumulation leads to progressive, irreversible tissue damage and multi-organ failure, including kidney failure, blindness, muscle wasting and premature death. It is estimated that only about 2,000 people worldwide are currently diagnosed with nephropathic cystinosis. Nephropathic or “classic infantile” cystinosis – the most common and most severe form of the disease – is typically diagnosed in infancy and requires lifelong cystine depleting therapy.[1]

About NAPRTCS

The North American Pediatric Renal Trials and Collaborative Studies (NAPRTCS) is a research effort organized in 1987. The NAPRTCS patient registry follows the clinical course and natural history of children with renal dysfunction from participating sites across North America. It follows these patients as they move across the treatment continuum from chronic kidney disease to dialysis and transplantation. For more information, visit www.naprtcs.org.

About Horizon Pharma plc

Horizon Pharma plc is focused on researching, developing and commercializing innovative medicines that address unmet treatment needs for rare and rheumatic diseases. By fostering a growing pipeline of medicines in development and exploring all potential uses for currently marketed medicines, we strive to make a powerful difference for patients, their caregivers and physicians. For us, it’s personal: by living up to our own potential, we are helping others live up to theirs. For more information, please visit www.horizonpharma.com, follow us @HZNPplc on Twitter, like us on Facebook or explore career opportunities on LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential benefits of the cystinosis registry. Forward-looking statements speak only as of the date of this press release and Horizon Pharma does not undertake any obligation to update or revise these

statements, except as may be required by law. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include, but are not limited to, whether physicians and patients provide information to the registry and whether the registry is ultimately used in the medical community. For a further description of these and other risks, please see the risk factors described in Horizon Pharma's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption “Risk Factors” in those filings. Forward-looking statements speak only as of the date of this press release and Horizon Pharma undertakes no obligation to update or revise these statements, except as may be required by law.

1. https://ghr.nlm.nih.gov/condition/cystinosis. Accessed Sept. 17, 2018

C-HZN-00107

View source version on businesswire.com: https://www.businesswire.com/news/home/20180927005242/en/

Source: Horizon Pharma plc

Horizon Pharma plc
U.S. Media Contact:
Matt Flesch
Executive Director, Product Communications
media@horizonpharma.com