



NephCure Kidney International® Launches the NephCure Accelerating Cures Institute® Aims to accelerate treatments and a cure for glomerular kidney diseases

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CONTACT: Jessica Martin NephCure Kidney International (610) 540-0186
jmartin@nephcure.org NephCure Kidney International® Launches the NephCure Accelerating Cures Institute® Aims to accelerate treatments and a cure for glomerular kidney diseases

King of Prussia, PA: With a goal of bringing more effective treatments for rare glomerular kidney diseases (including Focal Segmental Glomerulosclerosis (FSGS), Minimal Change Disease (MCD), IgA Nephropathy, and Membranous Nephropathy) to the market faster, NephCure Kidney International (NKI), in partnership with the University of Michigan (UM), has launched a global clinical trials network and supporting infrastructure: ***The NephCure Accelerating Cures Institute (NACI)***. The network has the potential to allow both pharmaceutical companies and academic investigators to expedite their trials with a unified institutional review board and improved access to this rare disease patient, clinician, and research community.

During the building phase of this 30-plus-site network, NKI has received financial support from Pfizer's Centers for Therapeutic Innovation (PFE) and Retrophin (RTRX) and is planning to add additional sponsors to help overcome obstacles that impede new therapy development.

Reactions to the establishment of NACI from the nephrology community – patients, researchers, clinicians, and industry – have been extremely favorable. NCI, in partnership with UM, raised over \$1.6mm in 2 years to launch this groundbreaking initiative.

Dr. Deb Gipson, Professor of Pediatrics at the University of Michigan School of Medicine, Pediatric Nephrologist at University of Michigan’s C.S. Mott Children’s Hospital and NACI Medical Director notes “NACI is launching a clinical trials network as part of its strategic approach. Its success will be demonstrated by the generation of data to support new and effective therapies and improved health outcomes for patients with glomerular kidney disease.”

NACI’s goals for nephrotic syndrome diseases include:

- Creating strength in numbers by engaging a motivated patient and provider community in the drug discovery process
- Finding more effective treatments in a timely and efficient manner
- Implementing a globally accepted and effective standard of care
- Moving towards finding a cure

NACI’s strategic approach to meet these goals is to:

1. Develop and implement well-designed, data driven, patient-centered clinical trials
2. Create a network of trial ready patients, practices, and research teams
3. Improve and expand clinical trial endpoints

Questions about supporting NACI should be directed to Mark Stone at mstone@nephcure.org.

To express interest in joining the NACI trial network, please see the Request for Applications.

Additional information can be found at www.NephCureACI.org

About NephCure Kidney International: NephCure Kidney International® is committed to eradicating glomerular diseases by advancing research, educating patients, their families and healthcare professionals and providing emotional support for everyone affected by these diseases.

About Retrophin: Retrophin is a fully integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company’s approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease, and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening

neurological disorder that typically begins in early childhood. Research exploring the potential of early-stage assets in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola®, Cholbam® and Chenodal®. Additional information can be found at www.Retrophin.com.

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