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**ON BEHALF OF THE  
NEPHCURE FOUNDATION  
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**INNOVATION HEARING – OUTSIDE WITNESS TESTINOMY**

**SUBMITTED TO THE  
SENATE COMMITTEE ON APPROPRIATIONS**

**APRIL 24, 2014**

Thank you for the opportunity to present the views of the NephCure Foundation regarding federal investments in research. NephCure strongly supports a funding level of \$32 billion for the National Institutes of Health in fiscal year 2015 and encourages continued support for research on idiopathic focal segmental glomerulosclerosis (FSGS) and primary nephrotic syndrome (NS). FSGS is a leading cause of end-stage renal disease (ESRD) and treatment advancements could achieve tremendous savings in federal health care costs and reduce health status disparities.

NephCure is the only non-profit organization exclusively devoted to fighting FSGS and the NS disease group. Driven by a panel of respected medical experts and a dedicated band of patients and families, NephCure works tirelessly to support kidney disease research and awareness.

NS is a collection of signs and symptoms caused by diseases that attack the kidney's filtering system. These diseases include FSGS, Minimal Change Disease and Membranous Nephropathy. When affected, the kidney filters leak protein from the blood into the urine and often cause kidney failure, which requires dialysis or kidney transplantation. According to a Harvard University report, 73,000 people in the United States have lost their kidneys as a result of FSGS. Unfortunately, the causes of FSGS and other filter diseases are poorly understood.

FSGS is the second leading cause of NS and is especially difficult to treat. There is no known cure for FSGS and current treatments are difficult for patients to endure. These treatments include the use of steroids and other dangerous substances which lower the immune system and contribute to severe bacterial infections, high blood pressure and other problems in patients, particularly child patients. In addition, children with NS often experience growth retardation and heart disease. Finally, NS caused by FSGS, MCD or MN is idiopathic and can often reoccur, even after a kidney transplant.

FSGS disproportionately affects minority populations and is five times more prevalent in the African American community. In a groundbreaking study funded by NIH, researchers found that FSGS is associated with two APOL1 gene variants. These variants developed as an

evolutionary response to African sleeping sickness and are common in the African American patient population with FSGS/NS.

FSGS has a large social impact in the United States. FSGS leads to end-stage renal disease (ESRD) which is one of the most costly chronic diseases to manage. In 2008, the Medicare program alone spent \$26.8 billion, 7.9% of its entire budget, on ESRD. In 2005, FSGS accounted for 12% of ESRD cases in the U.S., at an annual cost of \$3 billion. It is estimated that there are currently approximately 20,000 Americans living with ESRD due to FSGS.

On behalf of the thousands of families that are significantly affected by this disease, we encourage support for expanding the research portfolio on FSGS/NS at the NIH.

### **Encourage FSGS/NS Research at NIH**

There is no known cause or cure for FSGS and scientists tell us that much more research needs to be done on the basic science behind FSGS/NS. More research could lead to fewer patients undergoing ESRD and tremendous savings in health care costs in the United States.

With collaboration from other Institutes and Centers, ORDR established the *Rare Disease Clinical Research Network*. This network provided an opportunity for the NephCure Foundation, the University of Michigan, and other university research health centers to come together to form the Nephrotic Syndrome Study Network (NEPTUNE). NEPTUNE is developing a database of NS patients who are interested in participating in clinical trials which would alleviate the problem faced by many rare disease groups of not having access to enough patients for research.

The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) leads the NIH research portfolio on FSGS/NS. NIDDK is currently launching an exciting new project called CURE Glomerulonephropathy that will build on past research.

Furthermore, the National Institute of Minority Health and Health Disparities (NIMHD) has recently supported research on the disproportionate burden of FSGS on minority populations. NephCure encourages NIMHD to expand this important work.

Thank you for the opportunity to present the views of the FSGS/NS community. Please contact the NephCure Foundation for additional information.