C-Path's PKD Outcomes Consortium Receives BAA Award for Project to Advance Drug Development Tools for Autosomal Dominant Tubulointerstitial Kidney Disease

TUCSON, Ariz., Sept. 19, 2024 — Critical Path Institute (C-Path) is thrilled to announce its Polycystic Kidney Disease Outcomes Consortium (PKDOC) has been awarded an Autosomal Dominant Tubulointerstitial Kidney Disease (ADTKD) focused Broad Agency Announcement (BAA) contract from the U.S. Food and Drug Administration (FDA). The overarching objective of the work supported by the BAA award is to leverage collaboration with the Wake Forest Rare Inherited Kidney Disease team and its ADTKD registry, to analyze clinical and laboratory data that will help evaluate prognosis in ADTKD and help set the stage for future clinical trials.

ADTKD only affects the kidney, resulting in slowly worsening kidney function and the need for dialysis or kidney transplant at an average age of 45 years. It is caused predominantly by genetic changes (mutations) in the UMOD and MUC1 genes. Because of the inheritance pattern (autosomal dominant), a child of an affected parent has a 50% chance of also being affected. Thus, many family members have kidney disease and will eventually need a kidney transplant or dialysis. Unlike many other kidney diseases, there is no blood or protein in the urine. While this condition was infrequently recognized in the past, identification of the genetic causes of this condition has resulted in increased detection. It is now estimated that ADTKD is the third most common form of inherited kidney disease and affects more than 25,000 individuals in the U.S.

Given its recent identification as a cause of kidney disease and its rarity, little is known about factors that affect progression of kidney disease in ADTKD. Understanding rates of progression is important for patients (to learn about prognosis) and important for the development of future clinical trials. C-Path will now play a major role in analyzing available clinical data about ADTKD and helping to better understand the factors associated with disease progression. The goal of this proposal is to identify endpoints for future clinical trials and benefit ADTKD drug development efforts and regulatory review of these medications by providing tools to increase the ability to analyze the effectiveness of medications as treatments for ADTKD.

"We are very grateful for the support we have received from FDA to pursue this important project," said Sorin Fedeles, Ph.D., MBA, Executive Director of PKDOC. "This proposal represents our commitment to advancing the quantitative understanding of ADTKD progression through incorporation of relevant biomarkers into disease progression models, thus allowing a path forward for more efficient clinical trials and eventual drug approvals for this devastating disease. Our close collaboration with Wake Forest will allow us to accelerate progress towards future ADTKD treatments. In addition, this support will enhance our outreach efforts to leverage expertise across ADTKD stakeholders in order to collaborate and accelerate the development of life-changing therapies for the ADTKD community."

"We are really delighted to work with C-Path on this important project. C-Path has made great strides in its work in autosomal dominant polycystic kidney disease, and we are happy to be working with them on this new endeavor. It is really a great day for patients with ADTKD, and we are happy that the FDA has provided

our patients with hope and funding as we move towards finding a treatment for ADTKD. We invite all patients who have or think they have ADTKD to contact us at kidney@wakehealth.edu and become part of this work," said Anthony Bleyer, Ph.D., professor of kidney disease at Wake Forest School of Medicine and leader of the Rare Inherited Kidney Disease team.

About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the FDA's Critical Path Initiative. **C-Path's mission is to lead collaborations that advance better treatments for people worldwide**. Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path's global headquarters is in Tucson, Arizona and C-Path's Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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About Wake Forest Rare Inherited Kidney Disease Team

The Wake Forest Rare Inherited Kidney Disease team helps families identify the genetic cause of inherited kidney disease. Its mission statement is "To help one patient, one family at a time." The team has identified mutations in the UMOD, REN, MUC1, and APOA4 gene as causes of inherited kidney disease and has provided help to over 1,000 families. The team is interested in working with individuals with UMOD mutations or unknown causes of inherited kidney disease and can be contacted at kidney@wakehealth.edu.

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