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TUCSON, Ariz., November 13, 2015 – The Critical Path Institute (C-Path) announced today that the European Medicines Agency (EMA) rendered a positive qualification opinion to C-Path's Polycystic Kidney Disease Outcomes Consortium (PKDOC) for total kidney volume (TKV) as a prognostic biomarker to select patients for clinical trials of new therapies for Autosomal Dominant Polycystic Kidney Disease (ADPKD).

ADPKD is a debilitating genetic disease affecting approximately 600,000 Americans and 12 million people worldwide. There is only one medication developed to treat ADPKD, called tolvaptan, which has been approved in Europe, Japan, and Canada, but has not yet been approved in the United States.

TKV is a measurement of the impact of ADPKD on the size of the kidneys and is considered to be predictive of a future decline in kidney function. The EMA opinion states, "CHMP [Committee for Medicinal Products for Human Use] supports baseline total kidney volume, in combination with patient age and eGFR [estimated glomerular filtration rate] as a prognostic biomarker to identify patients likely to experience a progressive decline in renal function, as characterized by a decline in eGFR or progression to end-stage renal disease.

"From the data provided it is reasonable to expect that baseline TKV can predict disease progression and is a biomarker valuable for risk stratification."

"This qualification, along with a similar one from FDA, confirms the relationship between TKV and ADPKD disease progression, and will help in the design of clinical trials for new therapies for ADPKD" says C-Path Chief Operating Officer and PKDOC Co-Director Steve Broadbent.

The PKDOC created a Clinical Data Interchange Standards Consortium (CDISC) data standard for ADPKD and used it to remap the data from several patient registries and observational studies. The database was then used to develop a joint model linking baseline TKV with clinical outcomes.

About PKDOC:
The PKDOC is a successful collaboration between C-Path, the PKD Foundation, CDISC, four leading academic medical centers (Tufts University, University of Colorado Denver, Emory University, and Mayo Clinic), and three pharmaceutical companies. Its mission is to develop tools and promote research that will lead to the discovery of treatments for PKD and improve the lives of all it affects. The consortium is led by C-Path and funded through a grant from the PKD Foundation

and philanthropic donations.

About the organizations:

C-Path (Critical Path Institute) is an independent, nonprofit organization established in 2005 with public and private philanthropic support from the Arizona community, Science Foundation Arizona, and the US Food and Drug Administration (FDA). C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established 12 global, public-private partnerships that currently include over 1,300 scientists from government and regulatory agencies, academia, patient advocacy organizations, and dozens of major pharmaceutical companies. C-Path is headquartered in Tucson, Arizona. For more information, visit www.c-path.org.

The **PKD Foundation** is the only organization in the United States solely dedicated to finding treatments and a cure for polycystic kidney disease (PKD) to improve the lives of those it affects. This is done through promoting programs of research, education, advocacy, support, and awareness on a national level, along with direct services in local communities across the country. Their vision is that one day no one will suffer the full effects of PKD. Visit pkdcure.org to learn more about PKD and the Foundation.

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