

Regulatory Support For Autosomal Dominant Polycystic Kidney Disease Biomarker

June 1, 2015

 Critical Path Institute Secures Regulatory Support For Autosomal Dominant Polycystic Kidney Disease (ADPKD) Biomarker

TUCSON, Ariz., June 1, 2015 — [The Critical Path Institute \(C-Path\)](#) announced today that the [U.S. Food and Drug Administration \(FDA\)](#) has issued a Letter of Support to C-Path’s Polycystic Kidney Disease Outcomes Consortium ([PKDOC](#)) for the use of 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 more than 600,000 Americans and 12 million people worldwide. It is characterized by progressive enlargement of the kidneys due to the formation and growth of cysts. TKV is a measurement of the impact of ADPKD on the size of the kidneys and is believed to be predictive of a future decline in kidney function.

This Letter of Support is intended to encourage the use of TKV as an exploratory prognostic biomarker in clinical studies to identify patients likely to experience a progressive decline in renal function. “This represents a very significant milestone in the PKDOC’s continuing effort to qualify TKV as a prognostic biomarker,” said Martha Brumfield, PhD, President and Chief Executive Officer of C-Path. “It also constitutes a significant endorsement of the innovative consensus science model that C-Path has helped foster for the past 10 years.”

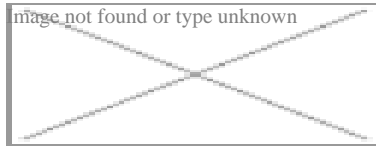
PKDOC created a Clinical Data Interchange Standards Consortium (CDISC) data standard for ADPKD and used it to remap the data from several patient registries and observational studies. The database was then used to develop a joint model linking the trajectory of TKV with clinical outcomes.

David Baron, PhD, Chief Scientific Officer of the PKD Foundation, said, “As a patient advocate, I believe this is important because it shows that the FDA acknowledges variances in the disease progression of ADPKD patients. This also encourages drug companies to investigate potential treatments for ADPKD that can be used earlier in the progression of the disease.”

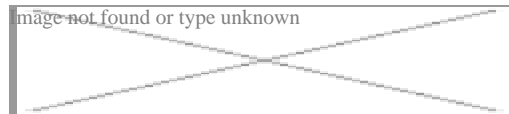
“This Letter of Support is the result of a tremendous collaborative effort,” said Ronald Perrone, M.D., Medical Director, Kidney Transplantation, Professor of Medicine at Tufts University School of Medicine, and Co-Director of the PKDOC. “Data and expertise were provided by the C-Path, the PKD Foundation, CDISC, four leading academic medical centers, and a number of pharmaceutical companies.”

The Letter of Support is posted on the [FDA website](#) and can also be accessed via the [C-Path PKDOC website](#)

About the organizations:



C-Path (Critical Path Institute) is an independent, non-profit organization established in 2005 with public and private philanthropic support from the Arizona community, Science Foundation Arizona, and the U.S. 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 eight global, public-private partnerships that currently include over 1,000 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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