# Polycystic Kidney Disease Outcomes Consortium

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## Research Areas

**Biomarker Research**
Diagnostic

## At a Glance

- Status: **Active Consortium**
- Year Launched: **2010**
- Initiating Organization: **C-Path**
- Initiator Type: **Nonprofit foundation**
- **Rare** disease
- Location: **North America**

## Abstract

The Polycystic Kidney Disease Outcomes Consortium (PKDOC) is a consortium initiated by the Polycystic Kidney Disease (PKD) Foundation and managed by the Critical Path Institute (C-Path) to develop evidence supporting the use of imaging total kidney volume (TKV) as a prognostic biomarker that measures the rate of disease progression. The goal is to develop a tool that can be used to select patients who are more likely to respond to a therapy in a clinical trial setting.

PKDOC was initiated by the PKD Foundation as a public-private partnership between the U.S. Food and Drug Administration (FDA), Clinical Data Interchange Standards Consortium (CDISC), and C-Path, the latter of which manages the effort and helps coordinate the efforts of several academic and industry partners. The overall goal is to establish TKV as a measure that predicts the progression of autosomal dominant polycystic kidney disease (ADPKD). It aims to replace the currently used measurement of glomerular filtration rate (GFR).

PKDOC has the following goals:

- Develop standard clinical data elements and definitions that are specific to ADPKD

- Create a database of aggregated data from existing multiple, longitudinal, and well-characterized research registries maintained over decades by the leading institutions in ADPKD clinical investigation

- Advance and harmonize the missions of regulatory agencies by creating tools that help with the evaluation of new pharmaceutical compounds

- Develop a quantitative disease progression model to examine the linkage between TKV and disease outcomes

- Quality Total Kidney Volume as a prognostic biomarker for use in clinical trials of therapies for patients with Autosomal Dominant Polycystic Kidney Disease.

## Consortium History

2007/2008: Held several meetings with FDA resulting in recommendations to construct a disease model to ascertain the linkage between total kidney volume, rate of size increase, and the most commonly encountered secondary features of ADPKD

2011: Developed standard common data elements, which were reviewed by the public

2012: Developed a database and mapped the data

2013: Released version 1.0 of the PKD therapeutic user guide; submitted PKDOC TKV biomarker qualification to FDA and the European Medicines Agency (EMA)

2015: Received FDA Qualification, in the form of a draft guidance, for Total Kidney Volume as a prognostic biomarker. Received a Draft Qualification Opinion from the EMA for the use of TKV as a prognostic biomarker.

## Structure & Governance

As the umbrella organization for PKDOC, C-Path's revenue is generated mostly from membership dues and grants. The PKD Foundation provides the primary financial support for PKDOC, which is supplemented by additional donations. C-Path also receives financial support through a grant from FDA.

## Intellectual Property

The output of the collaboration is within the precompetitive space, and intellectual property created is owned by the consortium as a whole. The content and information from the consortium is deemed confidential to consortium members, and all external disclosures are approved by members.

## Patent Engagement

Patients are involved in PKDOC projects via the PKD Foundation.

PKDOC's goal is to evaluate and demonstrate the fitness of TKV as a biomarker that predicts the progression of ADPKD.

## Data Sharing

The partnership between C-Path, CDISC, and the PKD Foundation aims to enable greater sharing by the development of tools, methods, and standards that can be used to report clinical research data to regulatory authorities. It brings together CDISC, the clinical data standard-setting organization, the PKD Foundation, the organization with research expertise, and C-Path, a third-party consortium manager.

The PKDOC used patient registries from three academic research institutions that agreed to share their data, and two observational studies sponsored by the NIH.

## Links/Social Media Feed

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## Sponsors & Partners

Clinical Data Interchange Standards Consortium
Critical Path Institute
Emory University
European Medicines Agency
Mayo Clinic College of Medicine
Otsuka Pharmaceutical
Pfizer, Inc.
Polycystic Kidney Disease Foundation
Sanofi/Genzyme Corp
Tufts Medical Center
U.S. Food and Drug Administration
University of Colorado, Denver

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