Traditional endpoints of renal function only show changes very late in the course of the disease, making it difficult to assess the effectiveness of new medications. There is critical need for a biomarker that will assess disease progression at an earlier stage, when patients may be more likely to respond to new therapies.

A major stride toward achieving this goal of qualifying a new endpoint occurred when in August 2015 the FDA issued a qualification decision, in the form of a draft guidance to C-Path’s Polycystic Kidney Disease Outcomes Consortium (PKDOC) for total kidney volume (TKV) as a prognostic biomarker to select patients for clinical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).

TKV is a measurement of the size of the kidneys and is considered to be predictive of a future decline in kidney function. This draft guidance provides qualification recommendations for the use of TKV, measured at baseline, as a prognostic enrichment biomarker to select patients with ADPKD at high risk for a progressive decline in renal function (defined as a confirmed 30% decline in the patient’s estimated glomerular filtration rate [eGFR]). The use of TKV as a biomarker—along with the patient’s age and baseline eGFR—can help those conducting clinical trials in finding appropriate candidates, potentially improving the accuracy and efficiency of those trials.

Fig. 1: Increase in kidney size and change in kidney function with age