Biomarker Evaluation in Patients with Autosomal Dominant Polycystic Kidney Disease

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Autosomal dominant polycystic kidney disease (ADPKD) is the most common hereditary kidney disease, which is characterized by progressive multiple cyst formation, proliferation, and apoptosis, finally causing interstitial fibrosis and end-stage renal disease (ESRD). It is caused by genetic mutation on either *PKD1* or *PKD2* gene, but phenotypic presentation only begins in young adulthood. Furthermore, renal function remains stable at the early stage because glomerular hyperfiltration compensates for the progressive loss of healthy glomeruli, which result in renal failure only after several decades from diagnosis.

Recently, several novel therapies have been introduced to slow the rate of disease progression in ADPKD. Since ADPKD progresses slowly after cyst formation, typical hard outcomes such as time to end-stage renal disease or patient survival are often not useful in studying ADPKD treatment effect. Therefore, there has been a great interest to develop alternative endpoints or surrogate biomarkers for patient and renal outcomes. Surrogate biomarkers should fulfil following conditions. Firstly, they should reflect disease severity. Second, they should predict rapid disease progression, which will distinguish high-risk patients who will benefit from the treatment. Lastly, they should be easy and convenient to evaluate disease progression in a short-term interval. I will discuss diagnostic evaluation of currently available surrogate biomarkers in the patients with ADPKD.