【APSN-TSN CME Symposium 2-1】

ADPKD: Basic Research

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Autosomal dominant polycystic kidney disease (ADPKD) is one of the most common “rare” diseases that clinicians deal with in daily practice. A unique feature of ADPKD is the initiation and continuous expansion of fluid-filled cysts protruding from renal tubules, eventually occupying the whole kidneys and causing the loss of renal function. PKD1 and PKD2 mutations are the major causative genes for ADPKD but other novel cystic genes including DNAJB11, GANAB, HNF1B, and IFT140 may also lead to similar cystic phenotypes. Basic research in ADPKD has discovered the “cystogenesis” mechanisms such as cell proliferation, fluid secretion, metabolic reprogramming, immunomodulation, cilia dysfunction, matrix alteration, and epigenetic control. Identification of the most important signaling pathways involved in cystogenesis including cAMP, mTOR, CFTR, STAT3, and glycolysis has led to the current treatment armamentarium for ADPKD. Recent progress of potential treatment developed from basic research including PKA inhibitors, metformin, caloric restriction, ketone ester, tolvaptan and octreotide-long-acting combination, CFTR correctors, and miR-17 inhibitors, The availability of orthologous animal models of ADPKD paves the way for finding new targeted treatments and is of paramount importance to examine the efficacy and safety of any new treatment preclinically.