Polycystic Kidney Disease: Translating Mechanisms into Therapy

Program Description and Objectives
Polycystic kidney disease (PKD) is the most common hereditary kidney disease in humans. Autosomal dominant PKD causes ESRD in 50% of affected patients, can also cause liver cysts, and is associated with pathology in other organs including the gastrointestinal tract and the vascular system. The genes responsible for PKD have been identified and sequenced, and the sequences of encoded proteins have been deduced. Functions for these proteins are being elucidated. Significant progress has been made in identifying intracellular pathways that are altered as part of the pathophysiologic mechanisms responsible for the cystic phenotype. These pathways are potential targets for specific therapy, which has been sorely lacking. Numerous clinical trials of specific therapy for PKD are underway. Results of several clinic trials, including some long-awaited large clinical trials, have been published recently. This program provides a comprehensive review of PKD mutations, pathophysiologic mechanisms of PKD, renal and extrarenal manifestations of PKD, clinical trials and emerging therapies in PKD, and appropriate management of patients with PKD.

Upon completion of this program, the participants will be able to: 1) describe the genetics of PKD; 2) discuss pathophysiologic mechanisms in PKD; 3) explain the role of imaging in management of PKD; 4) assess renal and extrarenal complications of PKD; 5) describe emerging therapies and large-scale clinical trials of treatments in PKD; and 6) adopt current concepts regarding management of PKD.

Target Audience
- Physicians
- PhDs and Other Researchers
- Medical and Other Trainees—Including medical students, residents, graduate students, post-docs, and fellows
- Nurses and Nurse Practitioners
- Pharmacists
- Physician Assistants
- Other Health Care Professionals
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