New approaches in treating complicated childhood polycystic kidney disease

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A collaborative team of physician-scientists at the Medical College of Wisconsin (MCW) and Children's Hospital of Wisconsin Research Institute has developed a new evidence-based, clinical algorithm to help physicians treat complex patients with autosomal recessive polycystic kidney disease (ARPKD).

Their invited manuscript, written by Grzegorz Telega, M.D., associate professor of pediatrics (gastroenterology and hepatology) at MCW and program director of hepatology at Children's Hospital of Wisconsin; David Cronin, II, M.D., Ph.D., professor of surgery and member of the new Transplantation Institute; and Ellis D. Avner, M.D. professor of pediatrics (nephrology) and physiology at MCW, and director of the Multidisciplinary Childhood PKD Program (MCPP) at Children's Hospital of Wisconsin Research Institute, appears in the April 17 edition of *Pediatric Transplantation*.

ARPKD is a rare genetic disorder that causes progressive disease of the kidneys and liver. Of the patients with ARPKD who survive the first year of life, more than 85 percent will reach their tenth birthday. However, despite dramatic improvements in overall survival and quality of life, nearly 50 percent of those survivors develop end stage kidney disease during that time.

Based on a comprehensive analysis of published medical literature, unique insights generated from the MCPP (established in 2005 and the only such program in the U.S.) and more than 50 years of clinical

experience by the authors in treating complex problems in ARPKD patients, an algorithm was developed to guide patient therapy. Of particular note, the authors recommend an innovative approach for a subgroup of ARPKD patients with severe kidney and liver disease: simultaneous kidney and liver transplantation.

"We believe combined liver-kidney transplantation can potentially decrease overall mortality and morbidity in carefully selected ARPKD patients with end stage renal disease and clinically significant congenital hepatic fibrosis," said Dr. Avner. "This is a particularly attractive option at our center, given the rapid growth of the joint solid organ transplantation program between Children's Hospital of Wisconsin, Froedtert Hospital, the Medical College of Wisconsin, and the BloodCenter of Wisconsin, directed by Dr. Johnny Hong."

The authors also emphasize that this therapy only be considered in major pediatric organ transplant centers with experienced pediatric transplant surgeons, subspecialty physicians and nurses, and complete pediatric ancillary services. The commentary also includes a "tree" to help guide clinicians making decisions about the appropriate course of treatment for complications of ARPKD.

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