Autosomal dominant polycystic kidney disease (ADPKD) is the most common inherited renal disease and accounts for 6–10% of patients on renal replacement therapy. Management of ADPKD has seen a major paradigm shift in recent years from mere supportive treatment to the establishment of targeted therapies. While research in the 1990s primarily focused on the search for the causative genes, the understanding of the molecular mechanisms underlying cystogenesis and cyst expansion has been rapidly growing in the last 10–20 years. This knowledge was the crucial basis in the development of pharmacological strategies aiming for disease-modifying effects to prevent both kidney growth and, more importantly, loss of renal function. It is remarkable that these joint efforts have now led to the approval of tolvaptan as the first drug specifically for ADPKD patients, with an assortment of other interesting approaches showing high efficacy in animal models but yet to be tested in clinical trials. Furthermore, the fact that a disease-modifying strategy is now at hand has led to another important paradigm shift. An early initiation of treatment appears to be the logical choice for a genetic disease – potentially already starting in childhood. Consequently, despite ADPKD having been considered a classic adult-onset disease in the past, this view paves the way for a much more intense interaction between adult and pediatric nephrologists caring for these patients and their children. This supplement reviews the recent advances in the management of ADPKD developed to date and anticipates future advances from three different angles. The authors discuss the current state of the art in the management of adult patients, highlighting the evidence for both targeted and supportive interventions. This review is accompanied by a detailed description of the view of pediatric nephrologists on the changing care of ADPKD in childhood. Another article adds the latest knowledge on emerging targeted (non-)pharmacological strategies based on both cellular biology and animal models. Importantly, with the targeting of drugs to the cyst epithelium being one of the key problems impairing the translation of new compounds, the authors place a special focus on this important aspect.

Taken together, this supplement provides the reader with a concise overview of the most important questions and answers at the crossroads of clinical care and basic research on ADPKD and points toward the key challenges in the future.

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