

Tolvaptan Use in Young Adults with Rapidly Progressing Autosomal Dominant Polycystic Kidney Disease

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Background: Autosomal polycystic kidney disease (ADPKD) is a common and inherited kidney disease characterized by the formation and progression of fluid-filled cysts. Regulatory approval of tolvaptan as a treatment for ADPKD in adults with evidence of rapidly progressing disease has changed the management of this condition. The phase 3 Tolvaptan Efficacy and Safety in Management of Autosomal Dominant Polycystic Kidney Disease and Its Outcomes (TEMPO 3:4; NCT00428948) clinical trial evaluated tolvaptan in a large population (N=1,445) of subjects aged 18–50 years over a 3-year period. However, it did not specifically assess the use of tolvaptan in adolescents and young adults (AYAs) with ADPKD.

Methods: A post hoc analysis of the TEMPO 3:4 trials was performed for patients 18-24 years old. The inclusion criteria were a diagnosis of ADPKD by Ravine criteria, and estimated creatinine clearance ≥ 60 mL/min (by Cockcroft-Gault) or rapidly progressive kidney growth (total volume ≥ 750 mL) by MRI at randomization. The primary outcome was the annual rate of change in total kidney volume (TKV).

Results: A total of 51 patients in the 18-24 group were analyzed. Out of the 51 patients, 29 were subjected to tolvaptan treatment while 22 were given placebo. The tolvaptan group had a lower mean % TKV growth per year compared to the placebo group (3.9% vs. 6.5%, $p=0.0491$).

Conclusion: Tolvaptan, with appropriate patient selection and management, can provide effective and acceptably safe treatment in AYAs with ADPKD.