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P366 -Tolvaptan, An innovative treatment or a disappointment for ADKPD patients? A single centre's experience.

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Introduction and Aims

In 2015 the National Institute for Health and Care Excellence (NICE) licensed Tolvaptan as a treatment option for adults with autosomal polycystic kidney disease (APKD) and evidence of rapidly declining renal function.1
NICE hailed Tolvaptan 'an innovative treatment', the first treatment shown to specifically impact on the progression of ADPKD'.

Data from the TEMPO 3:4 Study 2012 showed Tolvaptan was associated with 'a statistically significant 31.6% relative reduction in the annual rate of renal function decline compared to placebo'2 with results supported by the follow on TEMPO 4:4 study 2018 and REPRISE Study 2017. However dropout rates of 23% in TEMPO 3:4, side effects such as thirst, additional cost of monthly liver function tests have called into question the clinical and cost effectiveness of Tolvaptan in the management of APKD.

In light of the above we therefore reviewed our centre's APKD population, first to assess if they would qualify for Tolvaptan and second our patient's experience of Tolvaptan, and in particular dropout rates and associated side effects.

Method

All individuals with a diagnosis of ADPKD in our database were included. Current GFR-EPI was calculated as were the historical rate of decline in GFR to determine if the patient would qualify for Tolvaptan under currently licensing, GFR trends, reported side effects and drop out data were recorded.

Results

85 patients were identified has having ADPKD. 68 patients were excluded (15 transplant patients, 5 hemodialysis patients, 19 patients chronic kidney disease (CKD) stages 1,4 or 5 and 29 patients shown no evidence of rapidly declining renal function.)
6 patients had been prescribed Tolvaptan with the demographics of this cohort: mean age 46 years 4:2 male to female ratio, mean GRP-EPI 41.6ml/min.
In our center we had a significant drop out rate of 66% 16% LFT derangement, 16% pollakiuria, 16% electrolytes derangement and 16% patient choice .
The longest duration for Tolvaptan administration was 15 months and the shortest one month. All patients taking Tolvaptan showed progressive decline in GFR whilst on Tolvaptan, see figure 1. Only two patients currently remain taking Tolvaptan.
11 patients had been approached about commencing Tolvaptan, of which two patient have declined taking Tolvaptan due to the reported side effects.

Conclusion

Whilst our centre's experience of Tolvaptan is limited, the observed dropout rate was much higher than that in TEMPO3:4, 66% vs 23% Only one patient in our center has been able to tolerate Tolvaptan to 15 months. As further experience is gained, the real life cost effectiveness of this new therapy for patients with ADPKD may be more fully evaluated.

References

1. NICE. Tolvaptan for treatment autosomal dominant polycystic kidney disease (TA358)
2. Tolvaptan in Patients with Autosomal Dominant Polycystic Kidney Disease. Vicente.E.Torres et al. N Engl J Med 2012; 367:2407-2418