**Introduction**

mTOR inhibitors have been shown to be highly effective in treating many serious complications in TSC; renal angiomyolipomas (AML) [1], brain Sub Ependymal Giant Cell Astrocytomas (SEGA) [2], pulmonary lymphangioleiomyomatosis (LAM) [3], refractory epilepsy [4] and facial angiofibromas (FA) [5]. However their use in cancer and transplantation is associated with frequent and serious side effects. Here we present real world data from a survey of their use in a regional TSC clinic patient population.

**Methods**

We included all patients for whom mTOR inhibitors have been prescribed at the St Georges/Brighton regional TSC-clinic. Doses were titrated to manage side effects while maintaining efficacy according to previous recommendation (6). Their demographics, indication for use and outcome are presented below.

**Results**

68 patients (47 Female) were treated – 17 with Sirolimus and 38 Everolimus, 13 with both. Starting dose was 2mg/day for Sirolimus and 5mg/day for Everolimus. Median age was 34 (Range 3-68), and median duration of therapy was 29 months (Range 2-129). Indications for treatment were primarily AML in 58, SEGA in 2 and AML and LAM in 2, liver AML in 1. 4 patients discontinued therapy; 1 due to side effects, 2 were non-compliant and 1 died of an unrelated cause. The rest (94%) have continued on treatment. Most patients had one or more adverse events including; mouth ulcers (35), infection (6), acne / rash (5), dyspepsia (2), and one each of anorexia, menorrhagia, epistaxis and hyophosphataemia. Serious AEs were rare and included pyelonephritis and cellulitis. Hyperlipidaemia and intermittent proteinuria were also common. In most patients side effects were successfully managed by temporary reduction or withdrawal of drug. Recurrent or serious side effects were managed with permanent dose reduction.

The primary aim of halting disease progress and preventing new complications was achieved in all patients who continued on therapy for > 6 months.

**Conclusion**

mTOR inhibitors have a much more benign side effect profile when used as monotherapy for TSC if they are actively managed with dose alterations.

Everolimus is now licensed for treatment of AML, SEGA and refractory epilepsy in TSC and Sirolimus for LAM. Nephrologists will receive increasing referral for advice about these patients.

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