**Tuberous Sclerosis Complex: From Molecular**

**Biology to Novel Therapeutic Approaches**

Summary

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Tuberous sclerosis complex (TSC) is a rare autosomal dominant genetic disorder, which causes tumors to form in many different organs, primarily in the brain, eyes, heart, kidney, skin and lungs (multi-system). TSC patients suffer from epilepsy, mental retardation, and autism spectrum disorder (ASD). This explains why, TSC, it has gained substantial attention as a model of epilepsy, autism spectrum disorder (ASD) and tumorigenesis, with an evident genetic trigger. Bourneville-Pringle disease or TSC is very rare, being inherited during 1 out of 6000 births. *TSC1* and *TSC2* genes form the TSC and encode for two proteins hamartin and tuberin. TSC is caused by the lack of functional Tsc1-Tsc2 complex, which functions as a major cellular inhibitor of mammalian Target of Rapamycin Complex 1 (mTORC1). MTORC1 is a kinase that regulates most of anabolic processes in cells. Inhibitors of mTORC1, for example rapamycin, serve as experimental or already approved drugs for several TSC symptoms. Rapamycin and its derivatives, called rapalogs, are used in clinical practice to target several TSC symptoms such as epilepsy. However, rapalogs have to be administered chronically and for a lifetime. Furthermore, mTORC1 is not the sole target of Tsc1-Tsc2-Rheb signaling cascade. Based on recently innovation researchers have begun a search for novel, alternative strategies to combat the neurological manifestations and tumorigenesis in TSC.