

Mr Simon Stevens  
Chief Executive  
NHS England  
4W12 Quarry House  
Leeds  
LS2 7UE

26 October 2016

Dear Mr Stevens,

**Prescription of Everolimus to Treat Tuberous Sclerosis Complex-related Brain Tumours**

We the undersigned write with regard to NHS England's lack of a prescribing policy for Everolimus to treat Tuberous Sclerosis Complex (TSC)-related brain tumours.

TSC is a potentially life-threatening genetic disease that is present from birth and causes non-cancerous tumours to develop in different parts of the body. The tumours most commonly affect the skin, kidneys, heart, eyes, lungs and brain which results in various health problems.

In 2011 Everolimus was approved for use in the EU for the treatment of subependymal giant cell astrocytoma (SEGA), more commonly known as brain tumours. If left untreated young patients with inoperable SEGA suffer progressive neurological deterioration, with most dying within two years. With this treatment up to 95% go on to live long and healthy lives. Sadly 20 young people are dying every year as a result of inoperable SEGA, the majority of whom could have been saved by this treatment.

In July 2016 NHS England decided not to approve a prescribing policy for Everolimus as they deemed it to be 'unaffordable'. This is despite treatment costing less than dialysis for a patient suffering from kidney failure. Whilst TSC sufferers can apply for Everolimus treatment through individual funding requests, the majority have historically been turned down. This is because even the small number of individuals with inoperable tumours presenting (an estimated 20 per year), it proves impossible to demonstrate "exceptionality". All too often victims of rare diseases do not receive the support and treatment they need simply because it only affects a small proportion of the population.

We appreciate that the NHS must be run on a sustainable basis and cannot sign blank cheques to pharmaceutical companies for their medicines. But we are concerned about the prioritisation process itself and the disadvantage at which rare disease medicines are placed. We vehemently disagree with the decision taken by NHS England to abandon young sufferers of inoperable TSC-related brain tumours and their families to their fate when a proven and effective course of treatment exists.

We understand that NHS England are looking again at the prescribing policies originally placed into the June prioritisation round as a result of the outcome on the judicial review on prophylaxis for HIV.

We await the outcome of Clinical Priorities Advisory Group's deliberations; but if the decision is negative, we the undersigned call for an immediate and thorough reassessment of the prescribing policy for Everolimus through a fair and transparent process, and urge NHS England to make this treatment available to the young people who desperately need it.

Yours sincerely,

**Julian Sturdy MP**

**Greg Mulholland MP**