

## ***Help make medical treatment accessible for people with SEGAs***



*Send your comments about Afinitor – a medicine for Tuberous Sclerosis tumours – to the Pharmaceutical Benefits Advisory Committee by 10 October 2012*

### **Afinitor approved for use, but expensive...**

Early in 2012, Afinitor was approved by the Therapeutic Goods Administration (TGA) for: “the treatment of patients with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis (TS) who require therapeutic intervention but are not candidates for curative surgical resection.”

Unfortunately, the medicine can cost more than a thousand dollars per month for just one patient. Because the treatment is ongoing, Afinitor is very expensive for the patient or their hospital.

### **The ATSS is advocating for Afinitor to be listed on the PBS**

Pharmaceutical Benefits Scheme listing would mean that the Australian Government would pay for most of the cost of the medicine, making the treatment much more accessible for those who need it.

At its forthcoming November meeting the Pharmaceutical Benefits Advisory Committee (PBAC) will consider listing Afinitor for PBS subsidy. As is routine, the medicine’s manufacturer (Novartis) has supplied the PBAC with the necessary detailed clinical and economic data.

The Australasian Tuberous Sclerosis Society (ATSS) is making a submission, and we think you should too. We’re concerned that unless the medicine is subsidised by the PBS, it is not going to be affordable for many families.

### **Your comments about SEGAs in TS are needed**

This will be a first. The PBAC has never considered a medicine specifically for TS. What’s needed – to give the PBAC the complete picture – are the direct views of TS patients and their families.

So we’d like to make sure the PBAC hears why you want Afinitor listed by the PBS for the treatment of SEGAs in TS, and what life is like as a TS affected family. Your family doesn’t need to have experienced a SEGA to make a useful comment.

### **How to comment**

**Submissions close 10 October 2012 and must be made via the PBAC’s online consumer comments form at [http://www.health.gov.au/internet/main/publishing.nsf/Content/PBAC\\_online\\_submission\\_form](http://www.health.gov.au/internet/main/publishing.nsf/Content/PBAC_online_submission_form)**

The Medicine to which this submission relates: **Afinitor**. The date of the PBAC meeting: **November 2012**. Use your own name and contact details. Then there are five questions to complete (with space for about 200 words each). Some inspiration is over the page.

To discuss your submission you can email ([clare@atss.org.au](mailto:clare@atss.org.au)) or call (0410 685 181) Clare Stuart, ATSS Project Manager.

**Need some inspiration?** (written from the perspective of a parent of a child with TS)

**1: What treatment (if any) are you using now?**

Describe your child's situation – do they have/ have they had a SEGA(s)? Are they currently taking Afinitor or Everolimus or Rapamycin?

Mention that there aren't other medicines available approved for SEGAs in TS, the only other treatment option is surgery, possibly more than once, if the SEGA grows back.

You could also describe the many and varied treatments that your child needs because of TS (medicines, early interventions, special education etc.), and other challenges of living with TS.

**2: What do you see as the benefits of this new medicine for you?**

If your child is taking Afinitor for SEGAs, you could write about the benefits e.g. shrinking the tumour, avoiding hydrocephalus and more serious complications, including death. You may also like to write about the benefits of avoiding surgery.

If your child has not had a SEGA, write about the benefits you could imagine were your child to develop a SEGA.

You could also express your hopes about potential co-benefits from Afinitor for TS, like improvements in seizures, kidney AMLs, facial angiofibromas and cognition. Remember that none of these benefits have enough data from clinical trials to make a decision on yet, but it may provide extra information for the PBAC members.

**3: How will your life and that of your family and carers be improved by this new medicine?**

For example, less time out of school for your child; less time out of the workforce for yourself or other carers; fewer or shorter stays in hospital, fewer doctors appointments; less travel and associated costs like fuel/transport/accommodation; less time away from home and your other children; affect on family relationships; ability to have leisure time...

**4: What other benefits can you see from having this medicine on the PBS?**

If your child is/has used Afinitor - what difficulties have you experienced in meeting its specific cost? What have you had to change when it comes to the family budget – have you gone without things, deferred other purchases etc?

You might also wish to describe the health costs that your family experiences from having a child with TS, and some of range of uncertainties that surround having a child with a diagnosis of TS (for example in terms of your child's future health and consequent future demands on your finances as well as your family's emotional reserves).

### **Why is the ATSS facilitating submissions?**

Advocacy is one of the roles of ATSS. The TGA approval means that there is sufficient evidence of the usefulness and safety of Afinitor to treat SEGAs in TS. Given this ATSS believes that listing the medicine on the PBS will ensure that patients with a SEGA caused by TS requiring Afinitor can access it.

The ATSS Committee makes its own decisions about what to advocate on, in the best interests of families with TS. The ATSS has received funding from Novartis, the manufacturers of Afinitor, for certain ATSS projects including the 2011 and 2012 conferences and the new ATSS website. ATSS committee and staff have full control over ATSS strategy and the delivery of all projects. No external assistance was given in preparing this information sheet.

To read ATSS policy about when and how ATSS works with Pharmaceutical companies, please visit:

<https://www.atss.org.au/about-atss/atss-policies/>

### **Further information about some of the terms in this document**

**Afinitor** is the brand name given to Everolimus, an mTOR inhibiting medicine. Afinitor has only been approved for the treatment of one particular type of TS tumour: SEGAs. Other uses of Afinitor that are currently being researched include for the treatment of kidney angiomyolipomas (AMLs), for the treatment of epilepsy and for improvements in cognition. The manufacturer of Afinitor, Novartis, may seek approval from the TGA for other uses of Afinitor in the future.

**SEGAs** are a brain tumour that affects approximately 15% of people with Tuberous Sclerosis. For more information on SEGAs, visit <https://www.atss.org.au/i-need-to-know-more/signs/brain/>

The **Pharmaceutical Benefits Scheme (PBS)** is an Australian Government program that subsidises medicines to make them more affordable for Australians. For more information about the PBS, visit:

<http://www.pbs.gov.au/info/general/faq>

The **Pharmaceutical Benefits Advisory Committee (PBAC)** is the group of people that make a recommendation on whether a medicine should be included on the PBS. Most are medical doctors or researchers.