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# FDA approves kidney cancer drug to treat brain tumors

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The United States has approved a drug normally used for advanced kidney cancer to treat brain tumors caused by a rare genetic disease, US officials and the Swiss manufacturer said Monday.

Everolimus, which is marketed under the name Afinitor by Swiss pharmaceutical giant Novartis, was approved to treat tumors caused by the genetic condition tuberous sclerosis that cannot be resolved with surgery, the US Food and Drug Administration (FDA) and Novartis said.

Tuberous sclerosis causes slow-growing non-cancerous tumors called subependymal giant cell astrocytoma (SEGA) in the brain, and can cause growths in other parts of the body.

The genetic disorder, which affects mainly children and teens, can be fatal for patients who develop complications from tumor growth on the brain.

"Patients with this disease currently have limited treatment options beyond surgical intervention," said Richard Pazdur, director of the Office of Oncology Drug Products in the FDA's Center for Drug Evaluation and Research.

Afinitor was approved to treat SEGA tumors under the FDA's accelerated approval program after a study found the drug visibly shrank the largest SEGA tumor in a third of the 28 patients who took part in a trial.

Patients participating in the study at Cincinnati Children's Hospital did not develop any new tumors, but, on the other hand, none of their tumors healed completely, the FDA and Novartis said.

Four patients on the study had previous surgery but their tumor grew back. After receiving Afinitor, three of those patients saw a reduction in tumor volume of more than 50 percent, the FDA said.

The most commonly reported side effects of Afinitor seen in the study included upper respiratory tract infections, sinus and ear infections, mouth sores and fever.

The fast-track process used by the FDA to approve Afinitor to treat SEGA tumors is intended to give patients who suffer from serious diseases with few or no treatment options access to promising new or existing drugs, even as trials of the drug continue.

Between 25,000 and 40,000 people in the United States are affected by tuberous sclerosis, and SEGA tumors occur in up to 20 percent of them, Novartis said.

The company is conducting a Phase III trial with patients in Australia, Belgium, Britain, Canada, Germany, Italy, the Netherlands, Poland, Russia and the United States to study the efficacy and clinical benefits of Afinitor for patients with SEGA tumors.

Afinitor, which brought in 70 million dollars in sales last year and has already reaped 67 million dollars in sales in the first three quarters of 2010, was first approved in the United States in March last year to treat kidney cancer in patients in whom other treatments had failed.

It is also approved in Europe for the treatment of kidney cancer.

The generic drug everolimus is also approved in the European Union and the United States in different dosages and under different names to Afinitor for the prevention of organ rejection in heart and kidney transplant recipients.

Everolimus has not been approved outside the United States for the treatment of SEGA tumors caused by tuberous sclerosis, but applications to market it for this indication have been submitted to regulatory bodies in the European Union, Switzerland and elsewhere around the world, Novartis said.

"Because of the uncertainty of clinical trials, there is no guarantee that everolimus will become commercially available for SEGAs anywhere else in the world," the company added.