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Questions and answers on the use of Votubia in children from 6 months of age

The European Medicines Agency has finalised its assessment of an application to extend the use of Votubia to the treatment of seizures (fits) associated with tuberous sclerosis in children from the age of six months.

Although EMA's human medicines committee (CHMP) did not recommend this extension, it agreed that relevant data from the study submitted with the application be included in the medicine's product information.

What is Votubia and what is it used for?

Votubia is a medicine used to treat the following benign (non-cancerous) tumours caused by the genetic disease tuberous sclerosis:

- subependymal giant cell astrocytoma (SEGA), a benign tumour of the brain, where it is used in adults and children whose brain tumour cannot be surgically removed;
- renal angiomyolipoma, a benign tumour of the kidneys, where it is used in adults who are at risk of complications but who do not require immediate surgery.

The medicine is also used as an add-on treatment in patients from 2 years of age with seizures related to tuberous sclerosis that have not responded to other treatments. Votubia is used for partial-onset seizures (seizures that start in one part of the brain), which may or may not spread to affect the whole brain (secondary generalisation).

Votubia contains the active substance everolimus and is available as tablets to be taken by mouth.

Further information on Votubia's current uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/votubia.

What change had the company applied for?

The company applied to extend the use of Votubia in the treatment of seizures associated with tuberous sclerosis to children between six months and two years of age.

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How does Votubia work?

The active substance in Votubia, everolimus, acts by blocking an enzyme called 'mammalian target of rapamycin' (mTOR), which is thought to play a role in the seizures that occur in patients with tuberous sclerosis but it is not fully understood how the medicine acts to prevent them.

What did the company present to support its application?

To support its application, the company did not conduct a study in children between 6 months and 2 years of age, but instead estimated what the blood levels of Votubia in these children would be with a mathematical model using data from studies conducted in older children and adults.

What were EMA's conclusions?

The Agency noted that the error margin of this model was too wide, meaning that the model's predictions for the appropriate dose could not be considered accurate enough to support extending the use of Votubia to younger children (aged 6 months to 2 years).

Although Votubia will therefore not be authorised in this age group, the prescribing information for Votubia will be updated to include relevant data.

Does this outcome affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Votubia.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your doctor.