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Hyftor (*sirolimus*)

An overview of Hyftor and why it is authorised in the EU

What is Hyftor and what is it used for?

Hyftor is a medicine used to treat benign (non-cancerous) skin tumours on the face (facial angiofibroma) that are caused by a genetic disease called tuberous sclerosis complex. It is used in adults and children aged 6 years and older.

Tuberous sclerosis complex is rare, and Hyftor was designated an 'orphan medicine' (a medicine used in rare diseases) on 23 August 2017. Further information on the orphan designation can be found on the EMA website.

Hyftor is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but Hyftor is given in a different way. While the reference medicine, Rapamune, is given by mouth (tablets or a liquid to be drunk), Hyftor is available as a gel to be applied to the skin.

Hyftor contains the active substance sirolimus.

How is Hyftor used?

Hyftor can only be obtained with a prescription and is available as a gel that is applied twice a day to the areas of the face affected by angiofibroma. Treatment should be stopped after 12 weeks if there is no effect.

For more information about using Hyftor, see the package leaflet or contact your doctor or pharmacist.

How does Hyftor work?

The way in which Hyftor works in facial angiofibroma due to tuberous sclerosis complex is not fully understood. The active substance of Hyftor, sirolimus, works by blocking an enzyme called 'mammalian target of rapamycin' (mTOR). This enzyme is involved in the control of cell division and has increased activity in tumour cells in patients with tuberous sclerosis complex. By blocking mTOR, sirolimus stops tumour cells dividing, which is expected to reduce the growth of skin tumours associated with the disease.

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What benefits of Hyftor have been shown in studies?

A main study involving adults and children aged 6 years and above with facial angiofibroma due to tuberous sclerosis complex has shown Hyftor to be effective at improving facial angiofibroma. After 12 weeks, facial angiofibroma size and redness improved or greatly improved in 18 out of 30 patients (60%) who used Hyftor compared with none of the 32 patients (0%) who used a placebo gel (dummy treatment).

What are the risks associated with Hyftor?

For the full list of side effects and restrictions with Hyftor, see the package leaflet.

The most common side effects with Hyftor (which may affect more than 1 in 10 people) include irritation at the application site, dry skin, acne and pruritus (itching).

Why is Hyftor authorised in the EU?

Hyftor has been shown to improve the size and redness of facial angiofibroma due to tuberous sclerosis complex in adults and children aged 6 years and older, which are considered relevant benefits. The medicine's safety profile is considered acceptable. The European Medicines Agency therefore decided that Hyftor's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Hyftor?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Hyftor have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Hyftor are continuously monitored. Suspected side effects reported with Hyftor are carefully evaluated and any necessary action taken to protect patients.

Other information about Hyftor

Hyftor received a marketing authorisation valid throughout the EU on 15 May 2023.

Further information on Hyftor can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/hyftor.

This overview was last updated in 07-2023.