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Duvyzat (*givinostat*)

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

What is Duvyzat and what is it used for?

Duvyzat is a medicine used to treat people aged 6 years and older with Duchenne muscular dystrophy who are able to walk and are already being treated with corticosteroids. Duchenne muscular dystrophy is a genetic disease that gradually causes weakness and loss of muscle function.

Duchenne muscular distrophy is rare, and Duvyzat was designated an 'orphan medicine' (a medicine used in rare diseases) on 4 July 2012. Further information on the orphan designation can be found on the EMA website.

Duvyzat contains the active substance givinostat.

How is Duvyzat used?

Duvyzat can only be obtained with a prescription and should be prescribed by a doctor experienced in treating patients with Duchenne muscular dystrophy.

Duvyzat is available as a liquid to be taken by mouth with food twice a day. Before starting treatment with Duvyzat, doctors will ensure that patients have normal platelet levels (platelets are components that help the blood to clot). During treatment, platelet and triglyceride levels should be monitored regularly and the dose of Duvyzat may need to be adjusted based on the results. In patients who get diarrhoea (more than 4 stools a day) during treatment, the dose of Duvyzat may also need to be adjusted.

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

How does Duvyzat work?

The active substance in Duvyzat, givinostat, blocks the activity of certain enzymes called histone deacetylases (HDAC), which control how genes are read and used to make proteins, including those involved in muscle repair.

It is thought that dystrophic muscle cells have increased HDAC activity, which reduces the production of proteins that repair muscles, leading to inflammation, scarring and thickening, and fat build-up in muscles.

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By blocking HDAC activity, givinostat helps restore the production of proteins involved in muscle repair, reducing muscle damage and helping to slow the progression of the disease.

What benefits of Duvyzat have been shown in studies?

In a main study in boys aged 6 years and older with Duchenne muscular dystrophy who were able to walk and were being treated with corticosteroids, Duvyzat was shown to slow the decline in patients' ability to walk.

In the study, 81 boys received Duvyzat and 39 received placebo (a dummy treatment), both in addition to corticosteroids. The main measure of effectiveness was the change in patients' ability to walk, measured by the time needed to climb 4 steps.

After 18 months of treatment, patients taking Duvyzat took an average of 1.25 seconds longer to climb four steps. Those given placebo had a greater increase, taking 3.03 seconds longer on average.

What are the risks associated with Duvyzat?

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

The most common side effects with Duvyzat (which may affect more than 1 in 10 people) include diarrhoea, abdominal (belly) pain, thrombocytopenia (low levels of blood platelets), vomiting and hypertriglyceridaemia (high blood levels of triglycerides, a type of fat).

Why is Duvyzat authorised in the EU?

At the time of authorisation, corticosteroids were the only medicines used for the treatment of Duchenne muscular dystrophy. Based on the study results, Duvyzat is expected to slow the progression of the disease, especially when started early. However, there were uncertainties regarding the magnitude of Duvyzat's effect and the European Medicines Agency therefore requested that further studies be conducted to confirm the medicine's effectiveness. The side effects with Duvyzat were mostly mild to moderate and considered manageable. The European Medicines Agency therefore decided that Duvyzat's benefits are greater than its risks and it can be authorised for use in the EU.

Duvyzat has been given conditional authorisation. This means that it has been authorised on the basis of less comprehensive data than are normally required because it fulfils an unmet medical need. The Agency considers that the benefit of having the medicine available earlier outweighs any risks associated with using it while awaiting further evidence.

The company must provide further data on Duvyzat. It must submit results from two studies to confirm the safety and effectiveness of the medicine, including in the long-term. Every year, the Agency will review any new information that becomes available.

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

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

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

Other information about Duvyzat

Duvyzat received a conditional marketing authorisation valid throughout the EU on 6 June 2025

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

This overview was last updated in 06-2025.