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Nexviadyme (*avalglucosidase alfa*)

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

What is Nexviadyme and what is it used for?

Nexviadyme is an enzyme replacement therapy used to treat patients with Pompe disease, a rare inherited disorder caused by the lack of an enzyme called alpha-glucosidase. Patients with Pompe disease have a build-up of glycogen (complex sugars) in body tissues, including the heart, lung and skeletal muscles, causing enlarged heart, breathing difficulties and muscle weakness.

Nexviadyme contains the active substance avalglucosidase alfa.

Pompe disease is rare, and Nexviadyme was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 March 2014. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3141251.

How is Nexviadyme used?

Nexviadyme treatment should be supervised by a doctor who has experience managing patients with Pompe disease or other inherited diseases of the same type.

Nexviadyme is given as an infusion (drip) into a vein once every two weeks, and the dose depends on the patient's body weight. A doctor may decide to increase the dose for patients with infantile-onset Pompe disease (Pompe disease that appears at an early age) who do not improve with the regular dose. Patients who have no major side effects with the first few infusions may be able to have their infusions given at home.

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

How does Nexviadyme work?

The active substance in Nexviadyme, avalglucosidase alfa, is a version of the enzyme alpha-glucosidase, which is lacking in people with Pompe disease. Alpha-glucosidase breaks down glycogen into glucose that can be used for energy by the body's cells. If the enzyme is not present, glycogen builds up in certain tissues, including the heart and diaphragm (the main breathing muscle under the lungs), causing damage to them. By replacing the missing enzyme, avalglucosidase alfa helps break down glycogen and stop it from building up and causing the symptoms of the condition.

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What benefits of Nexviadyme have been shown in studies?

One main study involving 100 patients between 16 and 78 years of age with Pompe disease showed that Nexviadyme was at least as effective in improving lung function as another replacement therapy for Pompe disease (alglucosidase alfa). The patients' lung function was measured as the percentage change in their forced vital capacity (FVC, the maximum amount of air exhaled forcefully in one breath).

In the study, patients receiving Nexviadyme for 49 weeks increased their lung function by 2.9% compared with patients receiving alglucosidase alfa, who increased their lung function by 0.5%.

What are the risks associated with Nexviadyme?

The most common side effects with Nexviadyme are hypersensitivity (allergic) reactions and infusion-associated reactions, which may affect more than 1 in 4 people; severe allergic reactions (anaphylaxis) have been reported in less than 2 in 100 people. Other frequently reported side effects (in up to 1 in 10 people) are itching, rash, headache, urticaria (itchy rash), tiredness, nausea (feeling sick), and chills.

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

Why is Nexviadyme authorised in the EU?

A main study showed that Nexviadyme improved the lung function of patients with Pompe disease. The most common side effects are allergic and infusion reactions and are comparable to those reported in patients treated with similar medicines.

The European Medicines Agency, therefore, decided that Nexviadyme'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 Nexviadyme?

The company that markets Nexviadyme will provide educational material for healthcare professionals including guidance on how to monitor patients for risk of infection and how to arrange infusions at home.

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

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

Other information about Nexviadyme

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

This overview was last updated in 05-2022.