EMA/CHMP/631749/2021 Rev.2
Committee for Medicinal Products for Human Use (CHMP)
11 November 2021

Summary of opinion[1] (initial authorisation)

Nexviadyme

avalglucosidase alfa

On 23 July 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Nexviadyme[2], intended for the treatment of glycogen storage disease type II (Pompe disease). The Committee also concluded at that time that the active substance, avalglucosidase alfa, which is a modified version of the enzyme alglucosidase alfa, could not be considered a new active substance.

The applicant subsequently requested a re-examination with respect to the new active substance status of avalglucosidase alfa. After considering the grounds for the request, the CHMP re-examined the initial opinion and confirmed, in its conclusion on 11 November 2021, that avalglucosidase alfa could not be considered to be a new active substance because there was not enough difference between avalglucosidase alfa and alglucosidase alfa as regards efficacy and safety.

The applicant for this medicinal product is Genzyme Europe BV.

Nexviadyme will be available as a 100 mg powder for concentrate for solution for infusion. The active substance of Nexviadyme is avalglucosidase alfa, a recombinant human acid α-glucosidase (ATC code: not yet assigned), which is an enzyme replacement therapy that provides an exogenous source of acid α-glucosidase.

The benefit of Nexviadyme is its ability to improve the respiratory function (also called force vital capacity) of Pompe disease patients. The most common side effects are hypersensitivity (including anaphylaxis), infusion associated reactions (pruritus, rash, headache, urticaria, fatigue, nausea and chills).

The full indication is:

> Nexviadyme (avalglucosidase alfa) is indicated for long-term enzyme replacement therapy for the treatment of patients with Pompe disease (acid α-glucosidase deficiency).

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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Nexviadyme should be prescribed by physicians experienced in the management of patients with Pompe disease or other inherited metabolic or neuromuscular diseases.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.