



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

21 July 2011  
EMA/CHMP/546201/2011  
Committee for medicinal products for human use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

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### Vyndaqel tafamidis

On 21 July 2011 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Vyndaqel, 20mg, capsule intended for the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay neurologic impairment. Vyndaqel was designated as an orphan medicinal product on 28 August 2006. The applicant for this medicinal product is Pfizer Specialty UK Ltd. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Vyndaqel is tafamidis, a novel specific stabilizer of transthyretin, ATC code: N07XX08.

The benefits with Vyndaqel are its ability to stabilise transthyretin and thereby inhibit amyloid formation and progression of the transthyretin amyloidosis. Following 18 months of treatment in a pivotal clinical study, more patients treated with Vyndaqel met the definition of response with respect to the Neuropathy Impairment Score of the Lower Limb than patients on placebo. Less deterioration of neurologic function as measured by other endpoints and improved nutritional status were also observed in patients treated with Vyndaqel.

The most common side effects are urinary tract infection, diarrhoea, vaginal infection and upper abdominal pain.

A pharmacovigilance plan for Vyndaqel will be implemented as part of the marketing authorisation.

The approved indication is: "Vyndaqel is indicated for the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment."

Treatment should be initiated by and remain under the supervision of a physician knowledgeable in the management of patients with transthyretin amyloid polyneuropathy.

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<sup>1</sup> Summaries of positive opinion are published without prejudice to the commission decision, which will normally be issued 67 days from adoption of the opinion.



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 will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit to risk balance for Vyndaqel and therefore recommends the granting of the marketing authorisation under exceptional circumstances<sup>2</sup>.

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<sup>2</sup> In exceptional circumstances an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.