12 December 2024
EMA/CHMP/546392/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

BEYONTTRA

acoramidis

On 12 December 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product BEYONTTRA[2], intended for the treatment of transthyretin amyloidosis in adult patients with cardiomyopathy.

The applicant for this medicinal product is BridgeBio Europe B.V.

BEYONTTRA will be available as 356 mg film-coated tablet. The active substance of BEYONTTRA is acoramidis hydrochloride, belonging to the pharmacotherapeutic group: cardiac therapy, other cardiac preparations (ATC code: C01EB25). Acoramidis is a specific stabilizer of transthyretin, inhibiting its dissociation from a tetramer into monomers. By doing so, it slows the formation of amyloid fibrils in the heart, a process which leads to cardiomyopathy in patients with transthyretin amyloidosis.

The benefits of BEYONTTRA were showed in a pivotal study involving 632 participants with transthyretin amyloid cardiomyopathy (ATTR-CM) and heart failure NYHA Class I-III, and current or prior symptoms of heart failure. Compared with placebo, treatment with acoramidis resulted in a significant reduction in the hierarchical combination of all-cause mortality and cumulative frequency of cardiovascular hospitalisation over the 30-month treatment period. The most common side effects with BEYONTTRA are diarrhoea and gout.

The full indication is:

> BEYONTTRA is indicated for the treatment of wild-type or variant transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM).

Treatment with BEYONTTRA should be initiated by physicians experienced in the management of patients with transthyretin amyloid cardiomyopathy.

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

[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. The applicant requested the removal from the Community Register of Orphan Medicinal Products on 5 December 2024.

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