EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 May 2022
EMA/CHMP/264223/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Zokinvy

lonafarnib

On 19 May 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Zokinvy[3], intended for the treatment of patients with progeroid syndromes.

The applicant for this medicinal product is EigerBio Europe Limited.

Zokinvy will be available as 50 mg and 75 mg hard capsules. The active substance of Zokinvy is lonafarnib, an alimentary tract and metabolism product (ATC code: A16AX20), which prevents the formation of aberrant progerin and progerin-like proteins in cells, thereby promoting maintenance of cell integrity and function.

The benefit of Zokinvy is that it increases patient lifespan in a historical comparison with untreated patients, as observed in a pooled analysis of 62 patients from two single-arm cohorts. The most common side effects are gastrointestinal disturbances such as vomiting and diarrhoea, especially during the first 4 months of treatment, and increased liver enzymes.

The full indication is:

> Zokinvy is indicated for the treatment of patients 12 months of age and older with a genetically confirmed diagnosis of Hutchinson-Gilford progeria syndrome or a processing-deficient progeroid laminopathy associated with either a heterozygous *LMNA* mutation with progerin-like protein accumulation or a homozygous or compound heterozygous *ZMPSTE24* mutation.

Treatment should be initiated by a physician experienced in the treatment of patients with progeroid syndromes or patients with rare genetic metabolic syndromes.

Detailed recommendations for the use of this product will be described in the summary of product

[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] 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.
[3] 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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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.