20 May 2021
EMA/CHMP/273940/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Skysona

elivaldogene autotemcel

On 20 May 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 Skysona,[2] intended for the treatment of early cerebral adrenoleukodystrophy (CALD).

As Skysona is a gene therapy, the CHMP's positive opinion is based on an assessment by the Committee for Advanced Therapies. The applicant for this medicinal product is bluebird bio (Netherlands) B.V.

Skysona will be available as a 2-30 x 10^6 cells/ml dispersion for infusion. The active substance of Skysona is elivaldogene autotemcel, which is made specifically for each patient, using the patient's haematopoietic stem cells. The stem cells are modified in a laboratory to insert a working gene for making human adrenoleukodystrophy protein (ALDP). When the patient is given Skysona, which is made up of these modified cells, the cells start making ALDP, which will then break down the very long chain fatty acids that build-up in patients with CALD.

Skysona provides clinically meaningful benefits by preserving motor function and communication ability, and it improves survival in the early stage of cerebral disease.

The most serious adverse reaction attributed to Skysona was pancytopenia. There are some adverse reactions related to the mobilisation medicine and blood stem cell collection and to the conditioning medicine used to prepare the bone marrow for treatment with Skysona.

The full indication is:

> Skysona is indicated for the treatment of early cerebral adrenoleukodystrophy in patients less than 18 years of age, with an *ABCD1* genetic mutation, and for whom a human leukocyte antigen (HLA)-matched sibling haematopoietic stem cell (HSC) donor is not available.

Skysona must be administered in a qualified treatment centre by physicians with experience in haematopoietic stem cell transplantation and in the treatment of patients with neurological disorders.

[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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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.