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Review of sickle cell disease medicine Adakveo started

EMA has started a review of Adakveo, a medicine for preventing painful crises (called vaso-occlusive pain crises) in patients with sickle cell disease.

The review was prompted by preliminary results from an ongoing study in patients with sickle cell disease which indicate that, after one year of treatment, Adakveo did not reduce the number of painful crises leading to a healthcare visit compared with placebo (a dummy treatment). The study, called the STAND study, looks at the effectiveness and safety of Adakveo compared with placebo in adolescents and adults who had previously had painful crises leading to a healthcare visit. Data from this study were requested by EMA as part of the conditions to the marketing authorisation.

EMA will review these findings in the context of all available data and assess their impact on the benefit-risk balance of Adakveo in its approved indication. The Agency will then recommend whether the medicine's marketing authorisation should be amended.

A letter will be sent in due course to relevant healthcare professionals to inform them of these preliminary results and the ongoing review. The letter will also be published on the EMA website.

More about the medicine

Adakveo is a medicine for preventing painful crises in patients aged 16 years and older with sickle cell disease, a genetic condition in which the red blood cells become rigid and sticky and change from being disc-shaped to being crescent-shaped (like a sickle).

Adakveo was granted a conditional marketing authorisation in October 2020. At the time of approval, the main study showed that Adakveo was effective at reducing the number of painful crises in patients with sickle cell disease. Although there was some uncertainty about the size of Adakveo's effect in the main study, the available evidence showed consistent improvements with Adakveo, including a reduction in hospitalisations. The medicine was therefore granted a marketing authorisation on condition that the company provided further data on the effectiveness and safety from two additional studies, including the STAND study.

More information about the medicine can be found on the EMA website.

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More about the procedure

The review of Adakveo has been initiated at the request of the European Commission, under Article 20 of Regulation (EC) No 726/2004.

The review is being carried out by the Committee for Medicinal Products for Human Use (CHMP), responsible for questions concerning medicines for human use, which will adopt the Agency's opinion. The CHMP opinion will then be forwarded to the European Commission, which will issue a final legally binding decision applicable in all EU Member States.