Review of sickle cell disease medicine Adakveo started

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Information on Adakveo

- 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.

The following product is authorised via centralised procedure.

Active Ingredients	Product Name	Pharmaceutical Form	Classif-cation	Authorisation Number	MAH/license holder
Crizanlizumab	Adakveo	Concentrate for solution for infusion	POM	EMEA/H/C/004874	Novartis Europharm Limited

Information from the EMA about the safety concern

- 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.

Information about the pricedure

- 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.

For more information, please see the European Medicines Agency's press release.

Reporting Adverse Drug Reactions

Healthcare professionals and patients are encouraged to maintain vigilance with Adakveo. Suspected Adverse Drug Reactions (side effects) may be reported using the Medicines Authority Form and sending it to: Sir Temi Żammit Buildings, Malta Life Sciences Park, San Ġwann SĠN 3000 **or** online to http://www.medicinesauthority.gov.mt/adrportal or to the marketing authorisation holder or their local representatives.

Post-Licensing Directorate

Medicines Authority

Healthcare professionals and patients are encouraged to regularly check the Medicines Authority website for product safety updates as these are issued on an ongoing basis.

Feedback Form

The Medicines Authority thanks you for the time taken to read this safety circular. The dissemination of safety circulars is an important process whereby Regulatory Authorities can communicate important issues with respect to the safety of medicines, in order to protect and enhance public health.

The Medicines Authority kindly invites your anonymous feedback about the regulatory action being communicated. This may be returned by folding this form (address side up), stapling the ends and then posting (no stamp required).

Feedback:

We thank you for your interest and look forward to hearing your opinion.

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Pharmacovigilance Section

Post-Licensing Directorate

Medicines Authority

Sir Temi Żammit Buildings

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