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Withdrawal of application to change the marketing authorisation for Scenesse (afamelanotide)

Clinuvel Europe Limited withdrew its application for a change to the marketing authorisation for Scenesse to extend its use to adolescents with erythropoietic protoporphyria (EPP), a rare disease that causes intolerance to light.

The company withdrew the application on 24 April 2024.

What is Scenesse and what is it used for?

Scenesse is a medicine used to treat adults with EPP. In patients with EPP, exposure to light can lead to symptoms such as pain and swelling of the skin, which prevent them from being able to spend time outdoors or in places with bright light. Scenesse is used to help prevent or reduce these symptoms so that these patients can lead more normal lives.

Scenesse has been authorised in the EU since December 2014.

Scenesse contains the active substance afamelanotide. The medicine is available as implants that are injected under the patient's skin once every 2 months, before and during periods of high sunlight exposure, e.g. from spring to autumn. The number of implants per year depends on how much protection from the sun is needed. Three implants per year are recommended; the maximum number is 4.

Scenesse was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 May 2008 for EPP. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-08-541.

Further information on Scenesse's uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/scenesse

What change had the company applied for?

The company applied to extend the use of Scenesse to adolescents aged 12 to 17 years with EPP. During the assessment, the company modified the request to adolescents from 15 to 17 years and weighing more than 60 kg.

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How does Scenesse work?

Patients with EPP have high levels of a substance called protoporphyrin IX. When exposed to light, protoporphyrin IX causes the painful skin reactions seen in people with this condition.

The active substance in Scenesse, afamelanotide, stimulates the production of a brown-black pigment in the skin, known as eumelanin, which is produced during exposure to sunlight to protect the skin cells from harmful rays of light. By stimulating the production of eumelanin in the skin, Scenesse reduces the amount of light absorbed by the skin, thus helping to prevent or reduce these painful reactions.

What did the company present to support its application?

The company presented data from a registry study which included 7 adolescents aged 15 to 17 years with EPP, who were treated with the adult formulation of Scenesse (implants that are injected under the skin at regular intervals). The data are based on reports from patients on which sun protection measures they used and on patients' responses to a quality-of-life questionnaire.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and had prepared questions. After the Agency had assessed the company's responses to the questions, there were still some unresolved issues.

What did the Agency recommend at that time?

Based on the review of the information and the company's response to the Agency's questions, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that Scenesse could not have been authorised for the treatment of adolescents with EPP.

The Agency considered that the company had not provided sufficient and robust evidence to determine the effectiveness and safety of Scenesse in adolescents, or to define the most appropriate dose to be used in this age group. The adolescents included in the study received the adult formulation of Scenesse. There were no data on how the medicine is absorbed, modified and removed from the body in adolescents and on its effects in the body in this group of patients. Data on the safety of Scenesse in adolescents were also very limited.

Therefore, the Agency's opinion was that the balance of benefits and risks of Scenesse in the treatment of adolescents with EPP could not be established.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of application, the company stated that it withdrew its application as it was not possible to provide the comprehensive safety and efficacy data expected by the Agency.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Scenesse.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your clinical trial doctor.

What is happening with Scenesse for the treatment of adults with EPP?

Scenesse continues to be authorised in adults with EPP.