EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 October 2014
EMA/638997/2014
Press Office

Press release

Scenesse recommended for rare disease that causes intolerance to sunlight

Patients involved in discussions on benefits and risks of a medicine at CHMP for the first time

The European Medicines Agency has recommended granting a marketing authorisation under exceptional circumstances for Scenesse (afamelanotide) for the prevention of phototoxicity in adults with erythropoietic protoporphyria (EPP), a rare genetic disease which causes intolerance to light. Scenesse is the first medicine for patients with this condition.

EPP affects fewer than 10,000 people in the European Union (EU). After exposure to sunlight, patients with EPP feel a stinging pain in sun-exposed skin. Prolonged exposure to sunlight can lead to an incapacitating pain, often followed by redness and swelling of the skin. Patients with EPP are often not able to lead normal lives, because they cannot spend time outdoors.

Scenesse acts by stimulating the production of a pigment called eumelanin, which naturally protects the skin against phototoxic reactions caused by sunlight.

During the evaluation of Scenesse, the EMA's Committee for Medicinal Products for Human Use (CHMP) invited patients to share their experience of living with this condition. This was the first time that patients were involved in CHMP discussions on the benefits and risks of a medicine.

The CHMP decided that Scenesse should be recommended for marketing authorisation under exceptional circumstances. This type of authorisation can be granted for medicines that offer new or improved treatment options for patients with no or only limited alternatives, in cases where the applicant is not able to provide comprehensive data. In the case of Scenesse, there is a lack of robust efficacy data due to the difficulties in conducting placebo-controlled trials in this indication. Patients recruited in these studies are not willing to expose themselves to sunlight for fear of developing painful symptoms; therefore, the beneficial effect of the medicine on phototoxicity is difficult to assess.

The assessment of Scenesse was supported by data from the use of the medicine in more than 110 patients who were recruited in compassionate use programmes globally. These programmes allow the use of a medicine before it has been authorised when no satisfactory authorised therapies are available or when patients cannot enter a clinical trial. The CHMP heard from patients and healthcare

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

professionals involved in an expert group that patients treated with Scenesse consistently reported improvements to their quality of life.

The CHMP has recommended approval for Scenesse on the condition that the applicant puts in place a robust risk management plan that ensures close surveillance of the safety and efficacy of the medicine. As part of this plan, the company will establish a registry of patients to collect safety and efficacy data.

Scenesse has benefitted from a range of EMA tools to support innovation. The applicant for Scenesse is registered as a micro-, small- or medium-sized-enterprise (SME), and as such benefited from support and incentives offered by EMA. The Agency also provided free scientific advice to the applicant during the development of the medicine because Scenesse has an orphan designation.

The opinion adopted by the CHMP at its October 2014 meeting is an intermediary step on Scenesse's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The marketing-authorisation applicant for Scenesse is Clinuvel.
3. A marketing authorisation under exceptional circumstances foresees an annual reassessment based on data collected from the real-life use of the medicine.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu