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Questions and answers

Withdrawal of the marketing authorisation application for OraNera (autologous oral mucosal epithelial cells)

On 14 March 2013, CellSeed Europe Ltd. officially notified the European Medicines Agency that it wishes to withdraw its application for a marketing authorisation for OraNera (previously known as Caomecs), intended for the treatment of limbal stem cell deficiency in adults.

What is OraNera?

OraNera is a medicinal product consisting of cells obtained from the lining of the patient's mouth (autologous oral mucosal epithelial cells). It was to be available as multilayer cell sheets.

OraNera is an advanced therapy medicine called 'tissue engineered product'. This is a type of medicine that contains cells or tissues that have been 'engineered' (modified) so they can be used to repair, regenerate or replace tissue.

What was OraNera expected to be used for?

OraNera was expected to be used to treat limbal stem cell deficiency (LSCD) in adults. LSCD is an eye condition in which the patient lacks cells called limbal stem cells, which are found at the edge of the cornea (the transparent layer in front of the eye) and which continuously renew and repair the cornea. The deficiency of limbal stem cells leads to clouding of the cornea and may result in visual impairment or blindness.

How is OraNera expected to work?

One cell sheet of OraNera is expected to be grafted onto the patient's eye. As patients with LSCD are unable to replace the cells of the cornea, the cells in the OraNera sheet were expected to be used to replace damaged corneal cells, thereby relieving any symptoms the patient may have.

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What did the company present to support its application?

The main data submitted by the company came from a main study in 26 patients with LSCD who received OraNera.

How far into the evaluation was the application when it was withdrawn?

The evaluation of advanced therapy products involves an assessment by the Committee for Advanced Therapies (CAT) before an opinion is adopted by the Committee for Medicinal Products for Human Use (CHMP).

This application was withdrawn after the CAT had evaluated the documentation provided by the company and formulated a list of questions. The company had not yet responded to the last round of questions at the time of the withdrawal.

What was the recommendation of the CAT at that time?

At the time of the withdrawal, the CAT had not issued its final recommendation but had concerns about the data submitted in the application.

The CAT was concerned that the data from the main study did not allow any conclusions to be drawn on the beneficial effects of OraNera in patients with LSCD. There were concerns about the quality of the product and the way the study was conducted, such as how treatment success was defined. In addition, the CAT noted that the company would not be able to generate new data necessary to support the current application for marketing authorisation.

Therefore, at the time of the withdrawal, the CAT was of the opinion that the benefits of OraNera did not outweigh its risks.

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 state that it was withdrawing its application because, on the basis of the CAT's preliminary assessment, the Committee could not conclude on a positive benefit-risk balance and the company was unable to generate additional data required for a favourable opinion within the regulatory timeframe.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are currently no clinical trials or compassionate use programmes using OraNera.