EUROPEAN MEDICINES AGENCY
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Press Office

Press release

First stem-cell therapy recommended for approval in EU

New treatment for rare condition caused by burns to the eye

The European Medicines Agency (EMA) has recommended Holoclar, the first advanced therapy medicinal product (ATMP) containing stem cells, for approval in the European Union (EU). Holoclar is a treatment for moderate to severe limbal stem cell deficiency (LSCD) due to physical or chemical burns to the eye(s) in adults. It is the first medicine recommended for LSCD, a rare eye condition that can result in blindness.

The recommendation was made by the Committee for Medicinal Products for Human Use (CHMP) based on a robust assessment carried out by the Committee for Advanced Therapies (CAT), the Agency's expert committee for ATMPs.

"This recommendation represents a major step forward in delivering new and innovative medicines to patients," says Enrica Alteri, Head of EMA's Human Medicines Evaluation Division. "EMA has used all available support tools to facilitate the development and assessment of Holoclar. It is an advanced therapy medicinal product that has been designated as an orphan medicine. This allowed the Agency to provide support including several rounds of free scientific advice to the applicant during Holoclar's development."

Stem cells can act as a repair system for the body. Limbal stem cells are located in the eye at the border between the cornea (clear front part of the eye) and the sclera (white of the eye). These cells are important for regenerating and healing damage to the outer layer of the cornea (corneal epithelium). Physical or chemical burns can cause loss of these stem cells, resulting in LSCD, a condition that is estimated to affect about 3.3 out of 100,000 people in the European Union. Symptoms include pain, photophobia (painful sensitivity to light), inflammation, corneal neovascularisation (excessive ingrowth of blood vessels into the cornea), loss of corneal transparency, and eventually blindness.

Holoclar is a living tissue equivalent intended to be transplanted in the affected eye(s) after removal of the altered corneal epithelium. It is made from a biopsy taken from a small undamaged area (minimum of 1-2 mm^2) of the patient's cornea and grown in the laboratory using cell culture.

Holoclar can offer an alternative to transplantation for replacing altered corneal epithelium in some cases, and it has been shown to increase the chances of a successful corneal transplant where the injury has caused extensive eye damage. It reduces the risk of rejection compared with transplanting

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tissue from a donor and does not require surgery on the patient's other eye as only a small biopsy is performed to collect the cells, thus reducing the risk of damage to the healthy eye. Therefore, Holoclar may also be suitable where both eyes are affected by moderate to severe LSCD.

The CAT and the CHMP considered that Holoclar provided a first treatment option for this rare eye condition and recommended a conditional marketing authorisation because, although the data supplied by the applicant show that the medicine's benefits outweigh its risks, the data are based on retrospective studies and are not yet comprehensive. Therefore, an additional study on the use of Holoclar should be conducted.

The opinion adopted by the CHMP at its December 2014 meeting is an intermediary step on Holoclar'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 applicant for Holoclar is Chiesi Farmaceutici S.p.A.
3. The active substance of Holoclar is *ex-vivo* expanded autologous human corneal epithelial cells containing stem cells.
4. Holoclar was designated as an orphan medicine and an advanced therapy medicinal product (ATMP) and EMA provided protocol assistance to the applicant during the development of the medicine on multiple occasions. Both orphan and ATMP designations and the associated incentives, such as free scientific advice or protocol assistance, are among the Agency's most important instruments to encourage the development of medicines for patients suffering from rare diseases, and the development of innovative medicinal products.
5. ATMPs are innovative medicines that are intended for gene therapy, cell therapy or tissue engineering. The CHMP recommendation follows the draft opinion of the Committee for Advanced Therapies (CAT), the Agency's expert committee for ATMPs.
6. Conditional approval allows the marketing authorisation of medicines that target areas of unmet medical need before comprehensive data sets are available, to speed up patient access to much needed new medicines.
7. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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