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Press release

New cell-based therapy to support stem cell transplantation in patients with high-risk blood cancer

Orphan medicine Zalmoxis recommended by CAT and CHMP for marketing authorisation

The European Medicines Agency (EMA) has recommended granting a conditional marketing authorisation in the European Union (EU) for a new advanced therapy medicinal product (ATMP).

Zalmoxis is recommended as an adjunctive, or add on, treatment for adult patients receiving a haploidentical haematopoietic stem cell transplant (HSCT) for various types of blood cancer to aid immune reconstitution and reduce the risk of graft-versus-host disease.

Haploidentical HSCT involves a patient receiving haematopoietic stem cells (non-specialised haematopoietic cells that can develop into different specialised types of blood cell) from a partially matched donor to help the bone marrow produce healthy blood cells. This type of transplant is used to treat serious blood diseases, such as haematological malignancies, that include leukaemia and lymphoma. Transplants from partially-matched donors are more readily available than perfect matches, but carry a higher risk of graft-versus-host disease, where transplanted cells attack the recipient's organs, leading to organ damage.

Zalmoxis consists of T cells (a type of immune cell) from the stem cell donor, which have been separated from the rest of the cells in the transplant and have been genetically modified to include a 'suicide gene' called HSV-TK. T cells are given to transplant patients to help the body fight off infection, enhance the success of the transplant and support long-lasting anti-cancer effects; however T cells can also cause graft-versus-host disease. The suicide gene in Zalmoxis makes the T cells susceptible to a medicine called ganciclovir. If the patient develops graft-versus-host disease, ganciclovir is given, which kills the T cells that have the suicide gene, so preventing further development of the disease.

EMA's Committee for Medicinal Products for Human Use (CHMP) recommended conditional approval for Zalmoxis. Conditional approval is one of the Agency's main mechanisms to facilitate earlier access by patients to medicines that fulfil unmet medical needs. Conditional approval allows EMA to recommend a medicine for marketing authorisation before the availability of confirmatory clinical trial data, if the benefits of making this medicine available to patients immediately outweigh the risks inherent in the lack of comprehensive data.

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The effects of Zalmoxis were studied in a trial in which 30 patients with blood cancers who had haploidentical HSCT were given Zalmoxis. Of these patients, 23 had their immune systems restored. Graft-versus-host disease occurred in ten patients. Nine of those were given ganciclovir and none of them died or suffered long-term serious effects from graft-versus-host disease.

When overall survival rates from a total of 45 patients (30 from this trial and 15 from a second ongoing trial) treated with Zalmoxis were compared to rates from databases of patients who have undergone haploidentical HSCT, survival rates were higher for patients who received Zalmoxis (49% survival after one year, compared to 37% for patients who did not receive Zalmoxis).

Zalmoxis was assessed by the Committee on Advanced Therapies (CAT), EMA's specialised scientific committee for ATMPs, such as gene or cell therapies. At its June 2016 meeting, the CAT recommended a conditional marketing authorisation for Zalmoxis. The CAT's recommendation was considered by the CHMP which agreed with the CAT's recommendation.

As part of the conditional marketing authorisation, the applicant for Zalmoxis has to provide results from an ongoing, comparative phase III trial which looks at disease-free survival rates. Until availability of full data, the CAT and the CHMP will review the benefits and risks of Zalmoxis annually to determine whether the conditional marketing authorisation can be maintained.

Zalmoxis was designated as an orphan medicinal product in 2003. Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice, or the possibility to obtain ten years' market exclusivity for an authorised orphan-designated medicine. It is a key instrument in the EU to encourage the development of medicines for patients with rare diseases.

The applicant received scientific advice from the Agency on various aspects of the application dossier throughout the medicine's development.

The opinion adopted by the CHMP at its June 2016 meeting is an intermediary step on Zalmoxis' 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 a 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 Zalmoxis 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 common name of Zalmoxis is allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2).
3. The applicant for Zalmoxis is MolMed SpA.
4. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
5. ATMPs are innovative medicines that are derived from gene therapy, cell therapy or tissue engineering. Read more on our website here.
6. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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