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Media and Public Relations

Press release

New medicine to treat perianal fistulas in patients with Crohn's disease

Alofisel is the tenth advanced therapy recommended for marketing authorisation

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for a new advanced therapy medicinal product (ATMP) for the treatment of complex perianal fistulas in patients with Crohn's disease. Alofisel is the tenth ATMP that has received a positive opinion from the Agency's Committee for Medicinal Products for Human Use (CHMP).

Crohn's disease is a long-term condition that causes inflammation of the digestive system or gut. Apart from affecting the lining of the bowel, inflammation may also go deeper into the bowel wall. Perianal fistulas are common complications of Crohn's disease and occur when an abnormal passageway develops between the rectum and the outside of the body. These can lead to incontinence (a lack of control over the opening of the bowels) and sepsis (blood infection). Complex fistulas are known to be more treatment resistant than simple fistulas. There is currently no cure for Crohn's disease, so the aim of treatment is to stop the inflammatory process, relieve symptoms and avoid surgery wherever possible. Crohn's disease can affect people of all ages, with a higher incidence in the younger population.

The active substance of Alofisel is darvadstrocel. Darvadstrocel contains expanded adipose stem cells which, once activated, impair proliferation of lymphocytes and reduce the release of pro-inflammatory cytokines at inflammation sites. This immunoregulatory activity reduces inflammation and may allow the tissues around the fistula tract to heal.

The benefits of Alofisel were studied in a main phase III clinical trial involving 212 patients. After 24 weeks of treatment, half of the patients treated with Alofisel (49.5%) were in remission, compared to a third of the patients under placebo. An extended ongoing follow-up study, which will cover a period of up to 104 weeks of treatment, has supported this result to date.

Although there is a moderate difference between the treatment groups, the effect is considered to be clinically meaningful when other treatment options for fistulas have failed. The most common side effects observed include anal abscess and fistula, as well as procedural pain and proctalgia.

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Alofisel was assessed by the Committee for Advanced Therapies (CAT), EMA's specialised scientific committee for ATMPs, such as gene or cell therapies. At its December 2017 meeting, the CAT recommended a positive opinion for Alofisel to the CHMP. The CHMP agreed with the CAT's recommendation and adopted a positive opinion for the authorisation of Alofisel across the EU at its 11-14 December 2017 meeting.

Because complex perianal fistulas are rare, Alofisel was granted an orphan designation. As always at time of approval, this orphan designation will now be reviewed by EMA's Committee for Orphan Medicinal Products (COMP) to determine whether the information available to date allows maintaining Alofisel's orphan status and granting this medicine ten years of market exclusivity.

The opinion adopted by the CHMP is an intermediary step on Alofisel'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 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 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 Alofisel is Tigenix, S.A.U.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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