EUROPEAN MEDICINES AGENCY
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Media and Public Relations

Press release

New medicine for hereditary angioedema, a rare disease causing swelling beneath the skin

The European Medicines Agency has recommended granting a marketing authorisation for Takhzyro (lanadelumab), the first monoclonal antibody therapy for the prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged 12 years and older.

HAE is a long-term debilitating disease characterised by attacks of swelling beneath the skin that can occur anywhere in the body, such as in the face, limbs, gut and larynx. It is caused by abnormalities in the gene responsible for the production of C1 esterase inhibitor. This results in low C protein levels in the blood which induce increased kallikrein protein activity and ultimately cause the swelling. When the swelling occurs in the larynx it may be life-threatening as it can obstruct the airways and impede breathing.

Takhzyro is the first monoclonal antibody for the prevention of this disease. It has been designed to recognise and attach to kallikrein proteins, and thereby block the activity of the kallikrein-kinin system and reduce the number of angioedema attacks. Takhzyro is administered subcutaneously every 2-4 weeks, offering an improvement in patient care compared to current alternative therapies which are administered either intravenously or more frequently via subcutaneous route. Therefore, the Committee for Medicinal Products for Human Use (CHMP) considered that Takhzyro was of major interest for public health and agreed to the applicant's request for an accelerated assessment of this medicine.

The benefits and safety of Takhzyro were studied in a phase 3 clinical study involving 125 patients. During 26 weeks of treatment, patients who received Takhzyro showed a significant reduction in the total HAE attack rate and severity. The most common adverse reactions were injection site reactions of mild intensity.

Because HAE is a very rare disease which is estimated to affect less than 0.5 in 10,000 people in the EU, Takhzyro was granted an orphan designation in October 2015. 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 Takhzyro's orphan status and granting this medicine ten years of market exclusivity.

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The opinion adopted by the CHMP is an intermediary step on Takhzyro's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once the 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 Takhzyro is Shire Pharmaceuticals Ireland Limited.
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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