



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

1 April 2016  
EMA/CHMP/224720/2016  
Press office

## Press release

---

# First oral treatment for Fabry disease recommended for approval in the EU

## Galafold to provide additional treatment option for this rare genetic disease

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for Galafold (migalastat) for the treatment of Fabry disease, a rare genetic disorder.

Patients with Fabry disease do not have enough of an enzyme called alpha-galactosidase A. This enzyme normally breaks down a fatty substance called globotriaosylceramide (GL-3). If the enzyme is missing or is defective, GL-3 is not broken down and it builds up in the body's cells, such as heart and kidney cells. Patients with Fabry disease may have a wide range of signs and symptoms, including severe conditions such as kidney failure, heart problems and increased risk of strokes.

Currently, the standard treatment for this disease is enzyme replacement therapy (ERT), which consists of an intravenous infusion (drip) of a copy of the missing enzyme. Galafold is the first oral treatment for Fabry disease and may provide a more convenient treatment option for patients. It works in a different way to ERT, acting as a 'pharmacological chaperone' which binds to the defective alpha-galactosidase A enzyme, allowing it to be transported to where its action is needed and restore its activity. Galafold is to be used only in patients with specific mutations of the disease which are known to be responsive to the active substance in the medicine, migalastat.

The evaluation of EMA's Committee for Medicinal Products for Human Use (CHMP) was based on the results of two phase III clinical trials in about 110 patients with Fabry disease who had a genetic mutation which responds to migalastat. Galafold demonstrated its efficacy compared to placebo (a dummy treatment) and to ERT in a long-term comparative study.

In these studies, patients taking Galafold did not generally have troublesome side effects; the most common side effect was headache.

Because Fabry disease is rare, Galafold was designated as an orphan medicine by the Committee for Orphan Medicinal Products (COMP). Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice, or the possibility to obtain 10 years' market exclusivity for an authorised orphan-designated medicine. It is a key instrument available in the EU to encourage the



development of medicines for patients with rare diseases. The applicant also received scientific advice on quality, non-clinical and clinical aspects of the application.

The opinion adopted by the CHMP at its March 2016 meeting is an intermediary step on Galafold'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 Galafold is Amicus Therapeutics UK Ltd.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: [www.ema.europa.eu](http://www.ema.europa.eu)

## Contact our press officers

---

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: [press@ema.europa.eu](mailto:press@ema.europa.eu)

Follow us on Twitter [@EMA\\_News](https://twitter.com/EMA_News)