



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 April 2010
EMA/257628/2010
Press Office

Press release

European Medicines Agency gives new temporary treatment recommendations for Fabrazyme

Supply shortages for Genzyme's Cerezyme and Fabrazyme to last longer than expected

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has been informed by Genzyme, the manufacturer of Cerezyme (imiglucerase) and Fabrazyme (agalsidase beta), that the supply shortages for these medicines are expected to continue at least until the end of September 2010, because of a new manufacturing problem.

Cerezyme and Fabrazyme are both used to treat rare, inherited enzyme-deficiency disorders. Cerezyme is used in patients with Gaucher disease, and Fabrazyme is used in patients with Fabry disease. Temporary treatment recommendations to manage patients relying on these medicines during the ongoing supply shortages have been in place since June 2009 and have been regularly updated.

For Cerezyme, the temporary treatment recommendations given on 22 October 2009 are still valid. However, for Fabrazyme, the Committee decided to revise the recommendations made on 25 September 2009, since, based on information supplied by the manufacturer, at least 12% of patients on the reduced Fabrazyme dose regimen have already experienced a worsening of their disease. For such patients, the CHMP recommended that physicians should either consider restarting the original treatment with the full dose of Fabrazyme or switching to an alternative treatment, such as Replagal.

Given the new information, the temporary treatment recommendations for Fabrazyme are as follows:

- Children and adolescents (<18 years) should receive Fabrazyme according to the standard dose and frequency of one infusion every two weeks.
- Adult patients already treated/stabilised may receive Fabrazyme with an adjusted dose of 0.3 mg/kg as maintenance dose every two weeks.
- All patients, especially those with adjusted dose regimens, should be under close clinical surveillance. A full medical examination, including all relevant clinical parameters, should be



performed every two months. It is of the utmost importance to monitor the plasma GL-3 or urinary GL-3 levels, as for the moment the GL-3 level is the most sensitive parameter.

- For patients on the reduced dose who demonstrate a deterioration of the disease, physicians should consider restarting the original treatment with the full dose of Fabrazyme or switching to an alternative treatment, such as Replagal.

These recommendations are temporary and do not change the currently approved product information for Fabrazyme.

The new manufacturing problem has occurred at the production site in Allston Landing, in the United States of America, where Cerezyme and Fabrazyme are produced. It relates to a problem with the plant's water system. The defect has now been corrected, but supplies for both medicines will not return to normal before the end of September 2010, according to Genzyme.

Because of a series of manufacturing difficulties since June 2009, the CHMP remains concerned about the continued supply shortages of Genzyme's medicines, and is, together with the European network, closely monitoring the situation. Genzyme has been invited to present its manufacturing quality assurance system to the Committee during the May plenary meeting. The Agency will make further updates as appropriate.

Notes

1. Initially the supply shortage for Cerezyme and Fabrazyme was caused by the shutting down of Genzyme's production site in Allston Landing, in the United States of America, for the sanitisation of the bioreactors because of a viral contamination. See here: <http://www.ema.europa.eu/pdfs/human/press/pr/38999509en.pdf>
2. For Cerezyme, the temporary treatment recommendations given on 22 October 2009 are still valid and can be found here: http://www.ema.europa.eu/humandocs/PDFs/EPAR/Cerezyme/Cerezyme_66511209en.pdf
3. More information on Cerezyme, including the currently approved product information, is available in the European public assessment report: <http://www.ema.europa.eu/humandocs/Humans/EPAR/cerezyme/cerezyme.htm>
4. More information on Fabrazyme, including the currently approved product information, is available in the European public assessment report: <http://www.ema.europa.eu/humandocs/Humans/EPAR/fabrazyme/fabrazyme.htm>
5. This press release, together with other information on the work of the European Medicines Agency, can be found on the Agency's website: www.ema.europa.eu

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