European Medicines Agency
Press office

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PRESS RELEASE
Priority access for children during Myozyme supply shortage

The European Medicines Agency's (EMEA) Committee for Medicinal Products (CHMP) has recommended that infants, children and adolescents be given priority access to Myozyme (alglucosidase alfa), from Genzyme, during the expected supply shortage of the medicine over the next few months. This is intended to ensure that these patients continue to receive Myozyme while the company solves its supply problems.

The supply shortage has been caused by the demand for Myozyme outgrowing current manufacturing capacity, as well as problems with the manufacture of the medicine at some sites. While these problems are under investigation and the company takes steps to extend its manufacturing facilities, the temporary treatment recommendations will help doctors to manage potential interruptions in supply.

Myozyme is used to treat patients with Pompe disease, a rare, inherited enzyme-deficiency disorder. Pompe disease leads to a progressive build-up of glycogen in certain tissues, particularly the heart and other muscles. This causes a wide range of signs and symptoms, including an enlarged heart, breathing difficulties and muscle weakness. When the disease occurs early in life (early-onset), it progresses rapidly and is usually fatal without treatment. Pompe disease can also occur in adults (late-onset), when it progresses at a slower rate and is less life-threatening.

The approved treatment schedule for Myozyme is one infusion every two weeks. Because early-onset patients have the most rapid disease progression and are at risk of serious long-term developmental problems, the CHMP is recommending that:

- Infants, children and adolescents should continue Myozyme treatment without any interruption. New treatments should be initiated when necessary without restriction in this age group.
- In adults, no new treatments with Myozyme should be initiated. Until the supply problems are resolved prescribers should consider temporary treatment interruption in adults already being treated except in patients in whom interruption may have life-threatening consequences.

These are temporary recommendations and do not change the currently approved Product Information for Myozyme. The recommendations will apply until the company has resolved its supply problems.

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Notes:

1. More information on Myozyme, including the currently approved Product Information, is available in the European Public Assessment Report: http://www.emea.europa.eu/humandocs/Humans/EPAR/myozyme/myozyme.htm
2. This press release, together with other information on the work of the EMEA, can be found on the EMEA website: www.emea.europa.eu

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