

Package leaflet: Information for the user

Myozyme 50 mg powder for concentrate for solution for infusion

Alglucosidase alfa

Read all of this leaflet carefully before you start using this medicine because it contains important information for you.

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- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor, pharmacist or nurse.
- If you get any side effects, talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in this leaflet. See section 4

What is in this leaflet

1. What Myozyme is and what it is used for
2. What you need to know before you are given Myozyme
3. How Myozyme is given
4. Possible side effects
5. How to store Myozyme
6. Contents of the pack and other information

1. What Myozyme is and what it is used for

Myozyme is used to treat adults, children and adolescents of all ages who have a confirmed diagnosis of Pompe disease.

People with Pompe disease have low levels of an enzyme called alpha-glucosidase. This enzyme helps the body control levels of glycogen (a type of carbohydrate). Glycogen provides the body with energy, but in Pompe disease the levels of glycogen can get too high.

Myozyme contains an artificial enzyme called alglucosidase alfa – this can replace the natural enzyme which is lacking in Pompe disease.

2. What you need to know before you are given Myozyme

Do not use Myozyme:

If you have experienced life-threatening allergic (hypersensitive) reactions to alglucosidase alfa or any of the other ingredients of this medicine (listed in section 6) and re-administration of the medicine was not successful. Symptoms of life-threatening allergic reactions include, but are not limited to, low blood pressure, very fast heart rate, difficulty breathing, vomiting, facial swelling, hives or rash.

Warnings and Precautions

If you are treated with Myozyme, you may experience an infusion-associated reaction while you are being given the medicine or during the hours following the infusion. Such a reaction comprises different symptoms like low blood pressure, chest discomfort, throat tightness, face, lips or tongue swelling (angioedema), hives (urticaria), dizziness, rash, itchy skin, nausea, vomiting, cough and bronchospasm (see section 4 for an overview of all infusion-associated reactions). An infusion-associated reaction can sometimes be very severe. If you experience a reaction like this, you should **tell your doctor immediately**. You may need to be given pre-treatment medicines to prevent an allergic reaction (e.g. antihistamines and/or corticosteroids) or to reduce fever (antipyretics). In studies doctors have used medicines to suppress the immune system to reduce the production of antibodies. Because you have Pompe disease, there is a risk that you get a severe infection of your airways or lungs. Using these medicines to suppress the immune system may further increase this risk.

If you experience severe ulcerative lesions of your skin, please inform your doctor. If you experience swelling of your lower limbs or generalized swelling, please inform your doctor. Your doctor should consider discontinuation of the administration of Myozyme and initiate appropriate medical treatment. Your doctor should consider the risks and benefits of re-administering Myozyme.

Other medicines and Myozyme

Tell your doctor or pharmacist if you are using, have recently used or might use any other medicines.

Pregnancy and breast-feeding and fertility

There is no experience of the use of Myozyme in pregnant women. You should not be given Myozyme during pregnancy unless clearly necessary. You are recommended to stop breast-feeding when you are given Myozyme. If you are pregnant or breast-feeding, think you may be pregnant or are planning to have a baby, ask your doctor or pharmacist for advice before taking this medicine.

Driving and using machines

Take care when driving or using any tools or machines shortly after infusion of Myozyme, since you may experience dizziness.

Myozyme contains sodium

This medicine contains less than 1 mmol sodium (23 mg) per vial, i.e. essentially 'sodium free'.

3. How Myozyme is given

Myozyme will be given to you under the supervision of a doctor who is experienced in the treatment of Pompe disease.

The dose you receive is based on your body weight. The recommended dosage of Myozyme is 20 mg per kg of body weight. It will be given to you once every 2 weeks.

Use in children and adolescents

The recommended dosage of Myozyme in children and adolescents is the same as in adults.

Instructions for proper use

Myozyme is given through a drip into a vein (by intravenous infusion). It is supplied as a powder which will be mixed with sterile water before it is given.

If you are given more Myozyme than you should

There is no experience with overdose of Myozyme.

If you forget to use Myozyme

If you have missed an infusion, please contact your doctor.

If you have any further questions on the use of this medicine, ask your doctor, pharmacist or nurse.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

Side effects were mainly seen while patients were being given the medicine or shortly after ("infusion related effects"). Some of these infusion related side effects were serious or life-threatening. Life threatening reactions, including very severe generalised allergic reactions and anaphylactic shock, have been reported in some patients. Symptoms of such reactions include low blood pressure, very fast heart rate, difficulty breathing, vomiting, facial, lip or tongue swelling, hives or rash. Some patients

have experienced infusion related side effects in the form of flu-like symptoms, which lasted for a few days after completion of the infusion.

Should you experience any reaction like this, please **tell your doctor immediately**. You may need to be given pre-treatment medicines to prevent an allergic reaction (e.g. antihistamines and/or corticosteroids) or to reduce fever (antipyretics).

Very common: may affect more than 1 in 10 people

- Hives
- Rash
- Increased heart rate
- (Facial) flushing
- Fever or increased body temperature
- Cough
- Increased breathing rate
- Vomiting
- Low level of oxygen in the blood

Common: may affect up to 1 in 10 people

- Paleness
- Increased or high blood pressure
- Bluish discolouration of the skin
- Chills
- Agitation
- Tremor
- Headache
- Tingling
- Pain or local reaction at the site of the drip
- Dizziness
- Irritability
- Itchy skin
- Retching
- Swelling of the face, swelling of the throat or severe combined swelling of the face, throat and tongue due to a severe allergic reaction
- Swelling of the arms and legs
- Nausea
- Chest discomfort
- Throat tightness
- Diarrhoea
- Tiredness
- Muscle pain
- Muscle spasms
- Severe ulcerative lesions of the skin
- Redness of the skin

Not known: frequency cannot be estimated from the available data

- Swelling around the eyes
- Abnormal breathing sounds, including a whistling sound
- Difficulty in breathing (including shortness of breath)
- Cold extremities (e.g. hands, feet)
- Low blood pressure
- Narrowing of the blood vessels causing blood flow to be decreased
- Sudden constriction of bronchi restricting air going in and out the lungs (bronchospasm)
- Feeling hot
- Increased sweating

- Eyes tearing
- Mottled skin
- Restlessness
- Wheezing
- Decreased heart rate
- Heart stopping
- Chest pain (not in the heart)
- Inflammation of membrane that covers eyeball and eyelid
- Abdominal pain
- Joint pain
- Temporary suspension or sudden cessation of breathing
- Protein loss in urine
- Nephrotic Syndrome: swelling of lower limbs, generalized swelling and protein loss in urine

Reporting of side effects

If you get any side effects, talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in this leaflet.

You can also report side effects directly via [the national reporting system listed in Appendix V](#). By reporting side effects you can help provide more information on the safety of this medicine

5. How to store Myozyme

Keep this medicine out of the sight and reach of children

Do not use this medicine after the expiry date which is stated on the label after 'EXP'. The expiry date refers to the last day of that month.

Store in a refrigerator (2°C – 8°C).

After dilution, an immediate use is recommended. However, chemical and physical in-use stability has been demonstrated for 24 hours at 2 to 8°C when stored under protection from light.

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What Myozyme contains

-The active substance is alglucosidase alfa. One vial contains 50 mg of alglucosidase alfa. After reconstitution, the solution contains 5 mg of alglucosidase alfa per ml and after dilution the concentration varies from 0.5 mg to 4 mg/ml.

-The other ingredients are

- mannitol (E421),
- sodium dihydrogen phosphate monohydrate (E339)
- disodium phosphate heptahydrate (E339)
- polysorbate 80 (E433).

What Myozyme looks like and contents of the pack

Myozyme is a powder for concentrate for solution for infusion in a vial (50 mg/vial). Each pack contains 1, 10 or 25 vials. Not all pack sizes may be marketed.

The powder is white to off-white. After reconstitution it is a clear, colourless to pale yellow solution, which may contain particles. The reconstituted solution must be further diluted.

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Detailed information on this medicine is available on the European Medicines Agency web site:
<http://www.ema.europa.eu>. There are also links to other websites about rare diseases and treatments.
