For the use only of a Registered Medical Practitioners (Specialist in Medicine) or a Hospital or a Laboratory

Abridged Prescribing Information

MYOZYME®

Alglucosidase alfa for injection (r-DNA origin) 50mg in single-use vial.

Lyophilized Powder for concentrate for solution for infusion

COMPOSITION

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.

Alglucosidase alfa is a recombinant form of human acid α-glucosidase and is produced in Chinese hamster ovary cells (CHO) by recombinant DNA technology.

THERAPEUTIC INDICATION: Myozyme is indicated for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid α-glucosidase deficiency).

DOSAGE & ADMINISTRATION: Myozyme treatment should be supervised by a physician experienced in the management of patients with Pompe disease or other inherited metabolic or neuromuscular diseases. The recommended dose regimen of alglucosidase alfa is 20 mg/kg of body weight administered as intravenous infusion once every 2 weeks. Myozyme should be administered as an intravenous infusion. Myozyme has to be reconstituted with water for injections, then diluted with sodium chloride 9 mg/ml (0.9%) solution for injection and then administered by intravenous infusion using aseptic techniques. A 0.2 micron low protein binding in-line filter should be used for administration. Infusions should be administered incrementally. It is recommended that the infusion begin at an initial rate of 1 mg/kg/h and be gradually increased by 2 mg/kg/h every 30 minutes if there are no signs of infusion associated reactions (IARs) until a maximum rate of 7 mg/kg/h is reached.

Home infusion: Infusion of Myozyme at home may be considered for patients who are tolerating their infusions well and have no history of moderate or severe IARs for a few months. The decision to have a patient move to home infusion should be made after evaluation and upon recommendation by the treating physician. Home infusion infrastructure, resources, and procedures, including training, must be established and available to the healthcare professional. Home infusion should be supervised by a healthcare professional who should be always available during the home infusion and for a specified time after infusion. Dose and infusion rate should remain constant while at home and not be changed without supervision of a healthcare professional. Appropriate information should be given by the treating physician and/or nurse to the patient and/or caregiver prior to initiation of home infusion.If the patient experiences adverse reactions during the home infusion, the infusion process should be stopped immediately, and appropriate medical treatment should be initiated. Subsequent infusions may need to occur in a hospital or in an appropriate setting of outpatient care until no such adverse reaction is present.

SAFETY RELATED INFORMATION

Contraindications: Life threatening hypersensitivity (anaphylactic reaction) to the active substance or to any of the excipients, when rechallenge was unsuccessful.

Special warnings and precautions for use :

Traceability:In order to improve the traceability of biological medicinal products, the name and the batch number of the administered product should be clearly recorded. Hypersensitivity/Anaphylactic reactions: Serious and life-threatening anaphylactic reactions, including anaphylactic shock, have been reported in infantile- and late-onset patients during Myozyme infusions. If severe hypersensitivity or anaphylactic reactions occur, immediate discontinuation of Myozyme infusion should be considered and appropriate medical treatment should be initiated The current medical standards for emergency treatment of anaphylactic reactions are to be observed. Infusion Associated Reactions: Approximately half of the patients treated with Myozyme in infantile-onset clinical studies and 28% of the patients treated with Myozyme in a late-onset clinical study developed infusion associated reactions (IARs. IARs are defined as any related adverse event occurring during the infusion or during the hours following infusion. Infantile onset patients who develop high IgG antibody titres appear to be at higher risk for developing more frequent IARs. However, IARs occurred regardless of antibody titres. Patients with an acute illness (e.g. pneumonia, sepsis) at the time of Myozyme infusion appear to be at greater risk for IARs. Patients with advanced Pompe disease may have compromised cardiac and respiratory function, which may predispose them to a higher risk of severe complications from infusion associated reactions. Therefore, these patients should be monitored more closely during administration of Myozyme. Immunogenicity: IgG antibody titres should be monitored based on clinical phenotype. Patients who experience hypersensitivity reactions may also be tested for IgE antibodies to alglucosidase alfa and other mediators of anaphylaxis. Patients who develop IgE antibodies to alglucosidase alfa appear to be at a higher risk for the occurrence of IARs when Myozyme is re-administered. Therefore, these patients should be monitored more closely during administration of Myozyme. Some IgE positive patients were successfully rechallenged with Myozyme using a slower infusion rate at lower initial doses and have continued to receive Myozyme under close clinical supervision.

Immune-mediated reactions: Severe cutaneous reactions, possibly immune mediated, have been reported with alglucosidase alfa, including ulcerative and necrotizing skin lesions. Nephrotic syndrome was observed in a few patients with Pompe disease treated with alglucosidase alfa and who had high IgG antibody titres (≥ 102,400) Patients should be monitored for signs and symptoms of systemic immune-mediated reactions involving skin and other organs while receiving alglucosidase alfa.

Immunomodulation: Immunogenicity data from clinical trials and published literature in CRIM-negative infantile-onset patients (IOPD) suggests that the administration of immune tolerance induction (ITI) regimen given to alglucosidase alfa naive patients (prophylactic ITI) may be effective in preventing or reducing the development of High Sustained Antibody Titer (HSAT) against alglucosidase alfa. Patients with Pompe disease are at risk of respiratory infections due to the progressive effects of the disease

on the respiratory muscles. Fatal and life-threatening respiratory infections have been observed in some of these patients
Pregnancy: There is limited data from the use of alglucosidase alfa in pregnant women. Myozyme should not be used during pregnancy unless the clinical condition of the woman requires treatment with alglucosidase alfa.
Lactation: Limited data suggest that alglucosidase alfa is excreted in breast milk in very low concentrations. No clinical effect is expected in a breastfed infant due to low breast milk transfer and poor bioavailability. Breastfeeding during treatment with Myozyme may therefore be considered. As a precautionary measure, breastfeeding interruption for the first 24 hours after treatment may be considered

Effects on ability to drive and use machines: Dizziness, somnolence, tremor and hypotension have been reported as an infusion associated reaction, this may affect the ability to drive and use machines on the day of the infusion.
Undesirable effects :Very Common: Flushing,Tachycardia, Tachypnoea, Cough, Vomiting, Urticaria, Rash, Pyrexia, Oxygen saturation decreased. **Common:** Agitation, Hypersensitivity, Tremor, Dizziness, Paraesthesia, Headache, Cyanosis, Hypertension, Pallor, Flushing, Throat tightness, Retching , Nausea, Diarrhoea,, Vomiting, Erythema, Rash maculopapular, Rash macular, Rash popular, Pruritus, Urticaria, Hyperhidrosis, Muscle spasms, Muscle twitching, Myalgia, Pyrexia, Chest discomfort , Peripheral oedema, Local swelling, Fatigue, Feeling hot, Blood pressure increased. **Overdose :** IARs are more likely to occur with higher dose or infusion rates than recommended. In the event of overdose, the infusion rate should be reduced, or the infusion temporarily interrupted. There is no known specific antidote for alglucosidase alfa overdose. The patient should be monitored for any signs or symptoms of adverse reactions and administered appropriate symptomatic treatment immediately. **Special precautions for storage**:Store in a refrigerator (2^{o}C - 8^{o}C)..

For full and latest prescribing information, refer to company website www.sanofi.in.

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