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

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.

**SAFETY RELATED INFORMATION**

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

**Warnings & Precautions:**

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. 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: 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

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:** Myozyme should not be used during pregnancy unless clearly necessary.

**Lactation:** Alglucosidase alfa may be excreted in breast milk. Because there are no data available on effects in neonates exposed to alglucosidase alfa via breast milk, it is recommended to stop breast-feeding when Myozyme is used.

**ADVERSE REACTIONS :Very Common:** 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, Nausea, Erythema, Rash maculopapular, Rash macular, Rash popular, Pruritus, Urticaria, Rash popular, Hyperhidrosis, Muscle spasms, Muscle twitching, Myalgia, Pyrexia, Chest discomfort , Peripheral oedema, Local swelling, Fatigue, Feeling hot, Blood pressure

increased. **Not Known:** Agitation, Restlessness, Tremor, Headache, Conjunctivitis, Cardiac arrest, Bradycardia, Tachycardia, Cyanosis, Hypertension, Hypotension, Vasoconstriction, Pallor, Respiratory arrest, Apnea, Respiratory distress, Bronchospasm, Wheezing, Pharyngeal oedema, Dyspnoea, Tachypnoea, Throat tightness, Stridor, Cough, Abdominal pain, Retching, Periorbital edema, Livedo reticularis, Lacrimation increased, Rash, Erythema, Hyperhidrosis, Arthralgia, Nephrotic syndrome, Proteinuria, Chest pain, Face edema, Feeling hot, Pyrexia, Chills, Chest discomfort, Irritability, Peripheral coldness, Infusion site pain , Infusion site reaction, Oxygen saturation decreased, Heart rate increased

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*For full prescribing information please contact: Sanofi Healthcare India Pvt Ltd, Sanofi House, CT Survey No 117-B, L& T Business Park, Saki Vihar Road, Powai, Mumbai-400072*

Source: EU summary of Product Characteristics (SmPC) for CCDS V9 dated 25-April-2019
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