HAUTE AUTORITÉ DE SANTÉ

TRANSPARENCY COMMITTEE
SUMMARY
17 NOVEMBER 2021

The legally binding text is the original French opinion version

Alpha alglucosidase
MYOZYME 50 mg powder for concentrate for solution for infusion

Re-evaluation

Key points

Favourable opinion for reimbursement in the late-onset form of Pompe disease.

Long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid α-glucosidase deficiency).
Myozyme is indicated in adults and paediatric patients of all ages.

The re-evaluation only concerns late-onset forms of Pompe disease.

Role in the care pathway?

The multidisciplinary management of the disease is coordinated by a hospital physician in liaison with a reference or expert centre for metabolic or neuromuscular diseases and in liaison with the patient's primary care physician.

The specific treatment of Pompe disease is based on enzyme replacement therapy.

The objectives of treatment are as follows: to improve or stabilise hypertrophy and cardiac function in the event of initial damage (infantile-onset form); to stabilise or slow worsening of muscle weakness and avoid or delay loss of walking ability; to stabilise or slow deterioration of respiratory function and avoid or delay the need for ventilatory support.

Role of the medicinal product in the care pathway

MYOZYME (alpha alglucosidase) is the only enzyme replacement therapy with an MA in Pompe disease.

COMMITTEE'S CONCLUSIONS

Considering all of this information and further to debate and voting, the Committee considers:

Clinical benefit

◗ Pompe disease or glycogen storage disease type II is a chronic, progressive hereditary disease that gradually impairs functional capacities and reduces the patient's life expectancy. The disease causes motor disability, with an impact on all aspects of life: occupational, familial, social. Treatment of the disease generates its own constraints (repeated hospitalisations, potential side effects). The clinical symptoms in patients with the late-onset form of the disease (the most common form) are very variable and difficult to predict.
◗ The proprietary medicinal product MYOZYME is an enzyme replacement therapy.
◗ The efficacy/adverse effects ratio of MYOZYME is high in the short term but remains to be specified in the longer term in view of the level of evidence of efficacy data on disease progression and the overall survival of patients.
◗ There are no therapeutic alternatives with an MA in this disease.
◗ This is a first-line treatment.

Public health impact

Considering:
- the rarity of Pompe disease
- the medical need to have access to new treatments
- the lack of response to the identified medical need in view of the absence of a demonstrated impact on the long-term progression of the disease and the overall survival of patients,
- the potential negative impact on the care and/or life pathway of the patient (intravenous infusion every two weeks),

Despite the severity of certain late-onset forms of the disease, the absence of a therapeutic alternative with an MA in the indication and the potential positive impact on the management of patients (limitation of respiratory and/or motor complications and hospitalisations)
MYOZYME is unlikely to have an additional impact on public health in the late-onset form of Pompe disease.

Considering all these elements, the Committee deems that the clinical benefit of MYOZYME (alpha alglucosidase) remains low in the late-onset form of Pompe disease.

The Committee issues a favourable opinion for maintenance of inclusion in the hospital formulary list of reimbursed proprietary medicinal products approved for use in the late-onset form of Pompe disease.