# Sebelipase alfa in the treatment of lysosomal acid lipase deficiency

**Approved at the meeting of COHERE on 12 June 2018**

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| Recommendation by COHERE | | Sebelipase alfa treatment would be included in the national range of services for treating lysosomal acid lipase deficiency in infantile-onset patients if its price were significantly lower. However, the current wholesale price of the medicine per patient is unreasonably high in relation to the expected effectiveness for the drug to be included in the range of services even for infantile-onset patients.<br>For infantile-onset patients whose vital functions have been significantly reduced due to changes caused by the disease, initiation or prolongation of sebelipase alfa therapy is not medically justified. Instead, these patients should be offered symptomatic treatment.<br>In patients with the later-onset form of the disease, sebelipase alfa treatment is not included in the range of services owing to insufficient evidence of its effectiveness and the availability of other medicines. |
| Grounds | Severity and prevalence of the health issue | Lysosomal acid lipase deficiency is a lipid metabolism disorder caused by mutations in the LIPA gene, where lipids accumulate in the body. It is a rare disease, which can be divided roughly into two different forms: the rapidly progressive infantile-onset form (Wolman disease) that typically presents itself in the first weeks of life and leads to death before the age of one year; and the less severe later-onset form (Cholesteryl ester storage disease, CESD), where the life expectancy at its best may be normal but the symptoms, severity and prognosis of the disease vary between individuals.<br>One new infantile-onset patient might be born in Finland every three years. The prevalence of later-onset patients is estimated to be 2–56. |
| | Effectiveness | A higher percentage of infantile-onset patients who had received sebelipase alfa treatment reached the age of two years when compared to patients in the control group, but there is no information about the effects of the medicine on the quality of life. For later-onset patients, the drug has had a favourable effect on surrogate endpoints, but there is no information on how the treatment affects the length of life or morbidity.<br>Sebelipase alfa does not cure the gene defect underlying the disease. |
| | Safety | No specific, serious safety risks have been associated with sebelipase alfa treatment, but the studies are based on small numbers of patients. Dosage is accompanied by the normal risks associated with intravenous drug therapy. |
| | Costs and impact on the budget | Drug therapy for one infantile-onset patient would cost EUR 0.5–1.4 million per year, while the cost for one later-onset patient would be EUR 0.2–0.9 million.<br>Estimation of the total impact on the budget is not feasible because both the cost per patient and the number of patients vary over a wide range. |
| | Ethical and financial aspects as a whole | Sebelipase alfa is an exceptionally expensive drug, and it is ethically justified to limit its use to a patient group for which there is evidence of the effectiveness of the drug therapy. |
| | Diagnosis codes | E75.5 Other lipid storage disorders (ICD-10)<br>275761 (ORPHA number) |
| | Background information and references | Memorandum by COHERE (in Finnish), Assessment report by Fimea (in Finnish with English Abstract) |