NICE National Institute for Health and Care Excellence

Highly Specialised Technologies (HST) criteria checklist

Cipaglucosidase alfa with miglustat for treating Pompe disease (ID3771)

Introduction

The NICE HST criteria checklist is to highlight where a technology meets/partially meets or does not meet the criteria for routing to the HST programme. Its purpose is to show the details of why a technology may not be appropriate for HST evaluation, but also where it has been identified as suitable. For more information, please see section 7 of NICE health technology evaluation topic selection: the manual

Key – does the technology meet the criteria? Please use the colour key to advise if the technology meets the criteria

Met	There is clear and strong evidence that this criterion is met
Unclear	There is some evidence, or the evidence available is unclear.
Not met	There is no evidence or limited evidence that the criterion is met.

MA wording: Proposed wording: [redacted]

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
1.	The condition is very rare defined by 1:50,000 in England	The prevalence of Pompe disease in the UK is around **200 cases** (data from AGSD UK) (this includes both infantile and late-onset) Incidence: - 5 cases for infantile onset **and 11 cases for late-onset,** based on European orphanet data – reported birth prevalence is 0.8 per 100,000	**Met**

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
		for infantile onset and **1.75 per 100,000 for late onset** and 625,651 live births in 2018 in England - estimated to 15 cases in England based on an UK incidence of 1 in 40,000 (AGSD UK) and 625,651 live births in 2018 in England	
2.	Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications	Prevalence of both infantile and late-onset is estimated at around 200 [redacted] Incidence rate of 11 cases per year predicted for late-onset [redacted] Clinical trials included only people with **late-onset Pompe disease.**	**Met**
3.	The very rare disease for which the technology is indicated significantly shortens life or severely impairs quality of life	Glycogen storage disease type II, also known as Pompe disease or acid maltase deficiency is a rare inherited genetic disorder caused by the mutation of the GAA gene which makes an enzyme called acid alpha-glucosidase, resulting in the deficiency of this enzyme. Clinical trials included only people with late-onset Pompe disease. Late-onset disease can present from 1 year of age and is characterised by a progressive myopathy (with little or no cardiac involvement). Late-onset disease is less severe than infantile-onset, but can lead to severe morbidity, respiratory failure, wheelchair dependence and early mortality. There may be a range of severity with late-onset disease in adults. The company in their HST checklist form referenced a study which estimated life expectancy for late-onset to be 56 years (median) – but this source is based on a population **without current treatment with**	**Unclear**

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
		Alglucosidase alfa, which would be expected to increase life expectancy. It is uncertain what life expectancy would be with alglucosidase alfa. Alglucosidase alfa is a current treatment option for this population and reduces the severity/progression of the condition, but it is not a cure. TSOP noted that Avalglucosidase alfa for treating Pompe disease ID3737, which includes a more severe population (includes infantile onset Pome disease), was routed to TA in May 2021. Avalglucosidase alfa for treating Pompe disease ID3737 was considered against the HST criteria at the time of decision making and did not meet the following criterion - The condition is chronic and severely disabling.	
4.	There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.	There is an active treatment option available to this population (late-onset/adult), which is also available for people with infantile onset - Alglucosidase alfa. This treatment option (Alglucosidase alfa) reduces the severity/progression of the condition, but it is not a cure and there is likely to be some unmet need in this population. There are concerns regarding treatment effect waning in patients receiving alglucosidase alfa. Results from a recent clinical trial (Schoser et al. 2021) including ERT-naive and ERT-experienced participants found a statistically insignificant benefit from cipaglucosidase alfa versus alglucosidase alfa in the primary outcome of 6-minute walk distance (13.7m 95% CI -1.2m to 28.5m). There were statistically significant benefits in some secondary outcomes including sitting FVC (2.7% of predicted 95% CI 0.4 to 5.0) however it is unclear how clinically meaningful these outcomes are.	**Not met**