NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 34 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Provisional Title	**Eliglustat for treating type 1 Gaucher disease**		
Topic Selection ID Number	6805	**Wave / Round**	R72
HST ID Number	709		
Manufacturer	Genzyme		
Anticipated licensing information	**CONFIDENTIAL INFORMATION REMOVED**		
Draft remit	To evaluate the benefits and costs of eliglustat within its licensed indication for the treatment of type 1 Gaucher disease for national commissioning by NHS England.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that a highly specialised technology evaluation of eliglustat for treating type 1 Gaucher disease is appropriate. The proposed remit is appropriate. No changes are required. Attendees at the workshop confirmed that some people with type 1 Gaucher disease are asymptomatic, and therefore do not need to be treated with enzyme replacement therapy or substrate replacement therapy until symptoms present. It was agreed that this population would not receive eliglustat and therefore the population in the scope should be amended to specify that patients need to be symptomatic. Therefore, the population in the scope has been amended to 'People with symptomatic type 1 Gaucher disease'. **CONFIDENTIAL INFORMATION REMOVED** Scoping workshop attendees also discussed the need for determining CYP2D6 metaboliser status, as slow metabolisers would need to be exposed to a higher dose of eliglustat. **CONFIDENTIAL INFORMATION REMOVED** Consultees discussed the likely place of eliglustat in the treatment pathway. It was agreed that, although it is a substrate reduction therapy, it was likely to be used at the same point in the treatment pathway as enzyme replacement therapy (that is, as a first line treatment option). It was acknowledged that in people for whom enzyme replacement therapy is unsuitable, eliglustat could also be used as a second line treatment instead of miglustat. No changes to the comparators in the scope were required. Consultees considered that a highly specialised technology evaluation was the most appropriate process to consider this topic. However they		

	did also express the view that an MTA of all available therapies for type 1 Gaucher disease (that is, all enzyme replacement therapy and substrate reduction therapy) is needed.
Population size	Approximately 250 people in England and Wales
Process (MTA/STA/HST)	HST evaluation
Proposed changes to remit (in bold)	None
Costing implications of remit change	No significant changes required – original costing comments apply with a slight amendment to eligible population for consistency: Gaucher disease is a rare condition and the most prevalent of the Lysosomal Storage Disorders. Over 90% of affected individuals have type 1 Gaucher disease. The prevalence of type 1 Gaucher disease has been estimated to be 1 in 200,000 (non-Ashkenazi Europeans), equating to just over 250 people in England. Eliglustat is intended to be used for the treatment of type 1 Gaucher disease in treatment naïve and treatment experienced patients and so it is assumed that all 250 would be eligible. The cost of eliglustat is not yet known and in order for it to be considered a high cost topic its annual incremental cost per person would have to be £60,000 or over. By comparing it to its closest comparators Miglustat (£51,144), Imiglucerase (£334,242) and velaglucerase alfa (439,982), if eliglustat is priced comparatively, it is possible that eligustat could be a high cost topic.
Timeliness statement	Assuming that the anticipated date of the marketing authorisation is the latest date that we are aware of and the expected referral date of this topic, issuing timely guidance for this technology will be possible.