NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Proposed Highly Specialised Technology Evaluation

Eliglustat for treating type 1 Gaucher's disease

Draft scope (pre-referral)

Draft remit/evaluation objective

To evaluate the benefits and costs of eliglustat within its licensed indication for the treatment of type 1 Gaucher's disease for national commissioning by NHS England.

Background

Gaucher's disease is an inherited lysosomal storage disorder. It is caused by a deficiency of an enzyme (glucocerebrosidase) which enables complex lipids to be stored in some types of blood cells. This creates Gaucher cells which occur throughout the liver, spleen, bone marrow, skeleton and occasionally the lungs. There are 3 subtypes of Gaucher's disease, of which type 1 (non-neuropathic) is the most prevalent. All types of Gaucher's disease are associated with a variety of symptoms, including pain, fatigue, anaemia, thrombocytopenia, jaundice, bone damage, and enlargement of the liver and spleen.

There is limited data available on the epidemiology of Gaucher's disease. The overall frequency of all types of Gaucher's disease is approximately 1 in 40,000 to 1 in 50,000 live births. Over 90% of people affected have type 1 Gaucher's disease. The prevalence of type 1 Gaucher's disease is estimated as 1 in 200,000 in non-Ashkenazi Europeans, which equates to approximately 250 people in England and Wales. It is more common in people of Ashkenazi family origin, with a frequency of approximately 1 in 450 live births.

Treatment of Gaucher's disease requires an individualised approach that begins with a comprehensive multi-systemic assessment of all possible disease manifestations to accurately classify disease burden. Current management options include enzyme replacement therapy (such as imiglucerase and velaglucerase alfa) or substrate reduction therapy (miglustat) for people for whom enzyme replacement therapy is not suitable, alongside supportive therapy (which may include blood products, bisphosphonate therapy and/or analgesia).

The technology

Eliglustat (brand name unknown, Genzyme) is a glucosylceramide analogue that inhibits glucosylceramide synthase, resulting in reduced production of glucosylceramide and Gaucher cells. It is given orally.

Eliglustat does not currently have a UK marketing authorisation for treating type 1 Gaucher's disease. It has been studied in adults and young people

aged 16 and over in comparison with placebo, alone or compared with imiglucerase.

Intervention(s)	Eliglustat
Population(s)	People with type 1 Gaucher's disease
Comparators	• imiglucerase • velaglucerase alfa For people for whom enzyme therapy is unsuitable: • miglustat
Outcomes	The outcome measures to be considered include: • type 1 Gaucher's disease therapeutic goals • mortality • adverse effects of treatment • health-related quality of life (for patients and carers).
Nature of the condition	• disease morbidity and patient clinical disability with current standard of care • impact of the disease on carer's quality of life • extent and nature of current treatment options
Impact of the new technology	• clinical effectiveness of the technology • overall magnitude of health benefits to patients and, when relevant, carers • heterogeneity of health benefits within the population • robustness of the current evidence and the contribution the guidance might make to strengthen it • treatment continuation rules (if relevant)
Cost to the NHS and Personal Social Services (PSS), and Value for Money	• budget impact in the NHS and PSS, including patient access agreements (if applicable) • robustness of costing and budget impact information • technical efficiency (the incremental benefit of the new technology compared to current treatment)

	• productive efficiency (the nature and extent of the other resources needed to enable the new technology to be used) • allocative efficiency (the impact of the new technology on the budget available for specialised commissioning)
Impact of the technology beyond direct health benefits, and on the delivery of the specialised services	• whether there are significant benefits other than health • whether a substantial proportion of the costs (savings) or benefits are incurred outside of the NHS and personal and social services • the potential for long-term benefits to the NHS of research and innovation • staffing and infrastructure requirements, including training and planning for expertise.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation.
Related NICE recommendations and NICE pathways	None
Related NHS England policy	NHS England, Manual for prescribed specialised services. November 2012. National Specialised Commissioning Advisory Group, UK national guideline for adult Gaucher's disease. 2012

Questions for consultation

Have all relevant comparators for eliglustat been included in the scope? Which treatments are considered to be established practice for treating type 1 Gaucher's disease in England?

What are the most appropriate outcomes to be included in the scope? Can type 1 Gaucher's disease therapeutic goals be more specifically defined?

Where in the treatment pathway is eliglustat likely to be used?

- Is it likely to be used for people who have previously received treatment, or for those who have not previously received treatment, or both?

- Is eliglustat expected to be used as monotherapy only, or will use in combination with imiglucerase and/or other enzyme replacement therapies be possible?

Are there any subgroups of people in whom the technology is expected to provide greater clinical benefits or more value for money, or other groups that should be examined separately?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which eliglustat will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the Highly Specialised Technologies Evaluation Committee to identify and consider such impacts.

Do you consider the technology to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?

NICE intends to evaluate this technology through its Highly Specialised Technologies Programme. We welcome comments on the appropriateness of evaluating this topic through this process. (Information on the Institute's interim Highly Specialised Technologies evaluation methods and process is available at:

http://www.nice.org.uk/media/DE4/9A/HSTCombinedInterimProcessMethods.pdf