NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Proposed Highly Specialised Technology Evaluation

Teduglutide for treating short bowel syndrome

Draft scope (pre-referral)

Draft remit/evaluation objective

To evaluate the benefits and costs of teduglutide within its licensed indication for treating short bowel syndrome for national commissioning by NHS England.

Background

Short bowel syndrome is a chronic and potentially life-threatening condition characterised by reduced absorption of nutrients, water and electrolytes in the intestine. The most common cause of short bowel syndrome in adults is surgical removal of at least half of the small intestine in people with Crohn's disease. People may also have sections of their bowel removed after a major injury, or during surgery to remove a malignant tumour. Short bowel syndrome causes malnutrition, and in severe cases leads to intestinal failure.

The average age of people with short bowel syndrome is 49 years. Based on 2013 population data,[1] it is estimated that between 108 and 270 people in England have short bowel syndrome.[2,3] The company that manufactures teduglutide estimate that there are approximately 194 people in England receiving long-term treatment for short bowel syndrome.

The main treatment for short bowel syndrome is intravenous delivery of nutrients and electrolytes (known as 'parenteral nutrition'). The majority of people are able to self-administer their nutrients at home, using a long-term intravenous tube inserted by a healthcare professional. Nutrients may also be delivered through a tube to the stomach (known as 'enteral nutrition'), to help stimulate the intestine to function better. The intestine naturally recovers its absorptive properties in around half of people with short bowel syndrome, allowing them to reduce their reliance on nutritional support; the other half will require long-term parenteral nutrition. Other symptomatic treatments include antisecretory agents, bile acid sequestrants, anti-diarrhoeal agents, and antibiotics. People whose condition does not respond to treatment, or who develop life-threatening complications from long-term parenteral nutrition (such as blood infections, blood clots, and liver failure) may require intestinal surgery or an intestinal transplant but these are considered only as a last resort.

The technology

Teduglutide (Revestive, NPS Pharma) is an analogue of glucagon-like-peptide 2 (GLP-2), a naturally-occurring hormone which

promotes the growth of nutrient-absorbing cells on the surface of the intestine. Teduglutide is administered by a subcutaneous injection in the abdomen.

Teduglutide has a UK marketing authorisation for the treatment of adult patients with short bowel syndrome. The summary of product characteristics stipulates that patients should be stable following a period of intestinal adaptation after surgery and that teduglutide cannot be used in people with an active malignancy or a history of malignancies in the gastrointestinal within the last 5 years.

Intervention(s)	Teduglutide
Population(s)	Adults with short bowel syndrome who are stable following a period of intestinal adaptation after surgery
Comparators	• Best supportive care, including parenteral nutrition • Intestinal surgery • Intestinal transplant
Outcomes	The outcome measures to be considered include: • reduction in parenteral nutrition requirements (volume and frequency) • 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	• budget impact in the NHS and PSS, including

Personal Social Services (PSS), and Value for Money	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	If the evidence allows the following subgroups will be considered: • adults with short bowel syndrome who are dependent on parenteral nutrition • adults with short bowel syndrome after small bowel transplant. Guidance will only be issued in accordance with the marketing authorisation
Related NICE recommendations and NICE Pathways	**Related Clinical Guidelines** Clinical Guidelines No. 32, Feb 2006, Nutrition support in adults: Oral nutrition support, enteral tube feeding and parenteral nutrition (Review Proposal Date TBC) **Related Quality Standards** Quality Standard No. 24, Nov 2012, Nutrition support in adults (Review Proposal Date TBC) **Related NICE Pathways** NICE Pathway: Nutrition support in adults (Pathway

	updated Sep 2014)
Related National Policy	**NHS England:** NHS England (January 2014) Manual for prescribed specialised services 2013/14, chapter 10 (page 39) and chapter 23 (page 67): adult specialist intestinal failure services and autologous intestinal reconstruction service for adults **National Service Frameworks:** Long Term Conditions (including neurological) - archived **Department of Health:** Department of Health (2014) NHS Outcomes Framework 2015-2016. Domains 1, 2, 4, 5. Department of Health (2013) The UK strategy for rare diseases

Questions for consultation

Have all relevant comparators for teduglutide been included in the scope? Which treatments are considered to be established practice for treating short bowel syndrome?

How should best supportive care be defined?

Are there any other outcomes that should be included in the scope?

Are the subgroups suggested in 'other considerations' appropriate? Are there any other 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?Please describe any existing services in England for the diagnosis and management of this condition.

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 teduglutide is 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 Highly Specialised Technologies interim methods and evaluation processes is available at: http://www.nice.org.uk/media/DE4/9A/HSTCombinedInterimProcessMethods.pdf.

References

[1] Office for National Statistics (26 June 2014) Population Estimates for UK, England and Wales, Scotland and Northern Ireland, Mid-2013 [accessed 7 June 2015]

[2] Patient.co.uk Short bowel syndrome [accessed 7 June 2015]

[3] Koffeman GI, van Gemert WG, George EK et al (2003) Classification, epidemiology and aetiology. Best Pract Res Clin Gastroenterol 17: 879–93