NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Teduglutide for treating short bowel syndrome

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of teduglutide within its marketing authorisation for treating short bowel syndrome.

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 main symptom is diarrhoea, which can lead to dehydration, malnutrition and weight loss. 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 developing complications from previous surgery, a major injury, or during surgery to remove a malignant tumour. The remaining part of the bowel adapts and grows over time to increase its ability to absorb nutrients, but the level of natural adaptation is different from person to person and can take up to 2–3 years.

Paediatric short bowl syndrome is also a chronic and potentially life-threatening condition. Symptoms include malnutrition, dehydration, and metabolic and electrolyte disturbances. . Paediatric short bowl syndrome can occur at birth or shortly after. The most common causes are necrotizing enterocolitis, abdominal wall defects, intestinal atresia, and mid-gut volvulus.

The main treatment for short bowel syndrome is nutritional support (delivery of nutrients, electrolytes and fluids), which is given directly into the vein (parenteral support). Enteral tube feeding into the stomach or small bowel is infrequently used. The majority of people self-administer parenteral support at home, using a long term intravenous tube inserted by a healthcare professional. These tubes are associated with life threatening complications such as blood infections, blood clots, and liver failure. Parenteral support places a huge burden on patients, because it requires them to be attached to an infusion pump for many hours each night, for several nights a week. Some people need treatment every night, and are unable to work. People with short bowel syndrome will also receive drugs to promote nutrient absorption, including antimotility agents (such as loperamide and codeine) to increase the time it takes food to travel through the intestines, and antisecretory agents (such as proton pump inhibitors) to reduce the production of gastric acid. Patients will be advised to restrict their oral fluid intake and change their diet to promote absorption. In addition, where possible, surgery is considered to reconstruct or lengthen the remaining parts of the bowel, to increase the

surface area for absorption. People whose condition does not respond to treatment, or who develop serious complications from long-term parenteral support, may require an intestinal transplant, but this is considered only as a last resort.

Based on the British Artificial Nutrition Survey (BANS) from the British Association for Parenteral and Enteral Nutrition (BAPEN)[1], it is estimated that around 500 adults in England have short bowel syndrome and are dependent on long-term parenteral support (data from 2015).

The technology

Teduglutide (Revestive, Takeda) is an analogue of glucagon like peptide 2, a naturally-occurring hormone which can slow the gastric emptying, reduce gastric secretions, increase intestinal and portal blood flow and promote 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 marketing authorisation in the UK for treating short bowel syndrome in people aged 1 year and over. The summary of product characteristics stipulates that patients should be stable following a period of intestinal adaptation after surgery and that intravenous fluid and nutrition support should be optimised and stabilised before starting treatment. Teduglutide cannot be used in people with a hypersensitivity to the active substance, any excipients or trace residues of tetracycline, an active or suspected malignancy, or a history of malignancies in the gastrointestinal tract within the last 5 years.

Intervention(s)	Teduglutide in addition to established clinical management
Population(s)	People with short bowel syndrome who are stable following a period of intestinal adaptation after surgery
Comparators	Established clinical management without teduglutide (including parenteral support, antimotility and antisecretory agents, fluid restriction and dietary optimisation)
Outcomes	The outcome measures to be considered include: • reduction in parenteral support requirements (volume and frequency) • overall survival • adverse effects of treatment • health-related quality of life • impact on carers.

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations and NICE Pathways	**Related Clinical Guidelines** Clinical Guideline 32, Aug 2017, Nutrition support in adults: Oral nutrition support, enteral tube feeding and parenteral nutrition **Related Quality Standards** Quality Standard No. 24, Nov 2012, Nutrition support in adults **Related NICE Pathways** NICE Pathway: Nutrition support in adults (Pathway updated Aug 2017)
Related National Policy	**NHS England:** Prescribed Specialised Services (PSP) Tool 2020/21 **Department of Health:** Department of Health (2021) NHS Outcomes Framework Indicators - February 2021 Release - NHS Digital Department of Health (2013) The UK strategy for rare diseases

References

1 BANS Report 2016 (2017); Artificial Nutrition Support in the UK 2005-2015. http://www.bapen.org.uk/resources-and-education/publications-and-reports/bans/bans-reports [accessed March 2021]