NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Teplizumab for delaying the onset of type 1 diabetes in people 8 years and over at risk of developing the condition

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of teplizumab within its marketing authorisation for delaying the onset of type 1 diabetes in people aged 8 and over at risk of developing the condition.

Background

Diabetes mellitus is a chronic metabolic disorder characterised by elevated blood glucose levels (hyperglycaemia) resulting from a lack of the hormone insulin or resistance to its action. Type 1 diabetes results from the body's own immune system destroying the cells that make insulin[1]. If not managed effectively, diabetes mellitus can lead to kidney failure, blindness, foot problems, and damage to the nervous system[2]. People with diabetes are also more at risk of cardiovascular disease[3].

In the 2021-2022 financial year, there were 9,760 people diagnosed with type 1 diabetes in England and Wales[4]. Type 1 diabetes can present at any age, with peaks in presentation between ages 5 to 7 and around puberty[5]. Type 1 diabetes is slightly more common in males than females[5].

NICE's clinical guideline on the diagnosis and management of type 1 diabetes in adults (NG17) states that diabetes is typically diagnosed when people present with hyperglycaemia. The type of diabetes can be assessed using diabetes-specific autoantibodies and, if needed, non-fasting serum C-peptide. The management of type 1 diabetes in adults includes structured education, dietary control, physical activity, self-monitoring of blood glucose levels, insulin therapy, hypoglycaemia control, control of cardiovascular risk and treating complications. NG17 recommends flexible basal-bolus insulin regimens. This involves regular daily doses of longer-acting insulin to keep blood glucose levels stable during periods of fasting (basal insulin), and short-acting insulin to prevent increases in blood glucose levels after meals (bolus insulin).

NG17 also recommends consideration of adding metformin, which does not currently have a marketing authorisation in the UK for treating type 1 diabetes, to insulin therapy in some circumstances.

NICE technology appraisal 622 recommends sotagliflozin with insulin in certain circumstances for treating type 1 diabetes in adults with a body mass index of at least 27 kg/m^2 when insulin alone does not provide adequate glycaemic control despite optimal insulin therapy.

The technology

Teplizumab (Tzield, Sanofi) does not currently have a marketing authorisation in the UK for delaying type 1 diabetes. It has been studied in clinical trials alone compared with placebo in people aged 8 and over at risk of developing type 1 diabetes.

Intervention(s)	Teplizumab
Population(s)	People aged 8 and over at risk of developing type 1 diabetes
Comparators	No prophylaxis
Outcomes	The outcome measures to be considered include: • rate of new diabetes per year • time to diabetes diagnosis • mortality • adverse effects of treatment • health-related quality of life.
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. The availability of any managed access arrangement for the intervention will be taken into account The use of teplizumab is conditional on the presence of diabetes antibodies. The economic modelling should include the costs associated with diagnostic testing for diabetes antibodies in people with type 1 diabetes who would not otherwise have been tested. A sensitivity analysis should be provided without the cost of the diagnostic test. See section 4.8 of the guidance development manual (available here: https://www.nice.org.uk/process/pmg36/chapter/introduction-to-health-technology-evaluation).

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	**Related technology appraisals:** Hybrid closed loop systems for managing blood glucose levels in type 1 diabetes. (2023) NICE Technology appraisal guidance TA943 Sotagliflozin with insulin for treating type 1 diabetes. (2020) NICE Technology appraisal guidance TA622 **Related NICE guidelines:** Diabetic foot problems: prevention and management. (2019) NICE guideline NG19 Diabetes (type 1 and type 2) in children and young people: diagnosis and management. (2022) NICE guideline NG18 Type 1 diabetes in adults: diagnosis and management. (2022) NICE guideline NG17 **Related interventional procedures:** Allogeneic pancreatic islet cell transplantation for type 1 diabetes mellitus. (2008) NICE Interventional procedures guidance IPG257 **Related quality standards:** Type 1 diabetes in adults. (2023) NICE quality standard QS208 Diabetes in children and young people. (2022) NICE Quality standard QS125 Diabetes in pregnancy. (2023) NICE Quality standard QS109
Related National Policy	The NHS Long Term Plan (2019) NHS Long Term Plan

Questions for consultation

Are there any interventions used for delaying type 1 diabetes in people at risk of developing the disease?

Are there any subgroups of people in whom teplizumab is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Are the outcomes listed appropriate?

Are there any diagnostic tests required before teplizumab can be administered?

Where do you consider teplizumab will fit into the existing care pathway for type-1 diabetes?

Please select from the following, will teplizumab be:

A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):

For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would teplizumab be a candidate for managed access?

Do you consider that the use of teplizumab can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

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 teplizumab 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 committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. NHS Diabetes. Accessed May 2024
2. Diabetes UK Complications of diabetes. Accessed May 2024
3. Diabetes UK Diabetes and heart disease. Accessed May 2024
4. NHS Digital National Diabetes Audit 2021-22, Report 1: Care Processes and Treatment Targets, Detailed Analysis Report. Accessed May 2024

5. Los, E and Wilt, A (2023). Type 1 Diabetes in Children. Accessed May 2024