NICE National Institute for Health and Care Excellence

NICE guidance

Eltrombopag for treating severe aplastic anaemia refractory to immunosuppressive therapy (terminated appraisal)

Technology appraisal guidance
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www.nice.org.uk/guidance/ta382

Contents

Advice

NICE is unable to make a recommendation about the use in the NHS of eltrombopag for treating severe aplastic anaemia refractory to immunosuppressive therapy because no evidence submission was received from Novartis for the technology.

Background

Novartis was invited to submit evidence for this single technology appraisal for eltrombopag in September 2015.

The company did not make an evidence submission because it was unable to develop robust clinical- or cost-effectiveness analyses. The marketing authorisation for eltrombopag for treating severe aplastic anaemia was based on 1 small (n=43), non-comparative, single-centre study. The primary endpoint (response rate) was measured at 3 months, and the relationship between this and long-term outcomes is unclear. All of these factors severely limited the ability of the company to construct a robust case for clinical or cost effectiveness.

NICE has therefore terminated this single technology appraisal.

Information

NHS organisations should take into account the reasons why the company did not make an evidence submission when considering whether or not to recommend local use of eltrombopag for severe aplastic anaemia refractory to immunosuppressive therapy. If, after doing this, organisations still wish to consider eltrombopag for severe aplastic anaemia refractory to immunosuppressive therapy, they should follow the advice on rational local decision-making in the NHS Constitution for England and the NHS Commissioning Board and Clinical Commissioning Groups (Responsibilities and Standing Rules) Regulations 2012, which outlines the approach that should be adopted in circumstances in which NICE guidance is unavailable.

NICE will review the position at any point if the company indicates that it wishes to make a full submission.

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Accreditation