

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Proposed Health Technology Appraisal

Ruxolitinib for treating polycythaemia vera that is resistant or intolerant to hydroxycarbamide

Draft scope (pre-referral)

Draft remit/appraisal objective

To appraise the clinical and cost effectiveness of ruxolitinib within its licensed indication for treating polycythaemia vera that is resistant or intolerant to hydroxycarbamide.

Background

Polycythaemia vera is a bone marrow disease that leads to an increase in the number of blood cells (primarily red blood cells). As more red blood cells are made, the blood becomes thicker which can lead to complications such as gout, bleeding problems and blood clots. Blood clots can cause strokes, heart attacks, or blockage of an artery in your lungs (pulmonary embolism) or in a vein deep within a muscle (deep vein thrombosis). Polycythaemia vera can also cause an increase in white blood cells which can lead to severe itching. In some cases, the extra cells collect in the spleen which may then become enlarged. Polycythaemia vera can lead to other problems such as scarring of the bone marrow (myelofibrosis) and acute myeloid leukaemia.

Polycythaemia vera can affect people of any age, but is most prevalent in people age 60 years and older. The UK prevalence based on the population enrolled in The Health Improvement Network is approximately 6.8 per 100,000. Approximately 5% to 15% of people with polycythaemia vera go on to have myelofibrosis. No epidemiological data are available about the proportion of people receiving treatment for polycythaemia vera whose disease transforms into acute myeloid leukaemia.

Current treatments for polycythaemia vera aim to prevent symptoms and complications, and to minimise the risk of transformation to acute myeloid leukaemia or myelofibrosis. The British Committee for Standards in Haematology's guidelines for polycythaemia vera recommend a range of treatments including periodic venesection (bloodletting), interferon, hydroxycarbamide, anagrelide, radioactive phosphorus or low dose busulphan. In addition, melphalan has a license for treating polycythaemia vera in the UK.

The technology

Ruxolitinib (Jakavi, Novartis) is an inhibitor of the Janus-associated kinases (JAKs), which are involved blood cells differentiation. Ruxolitinib is administered orally.

Ruxolitinib does not currently have a marketing authorisation in the UK for the treatment of polycythaemia vera. It has been studied in a clinical trial compared with hydroxycarbamide in adults with polycythaemia vera symptoms despite treatment with a stable dose of hydroxycarbamide for at least 12 weeks prior to entering the study. It has also been studied in a clinical trial compared with best available therapy (selected by investigator for each person in the control arm of the study) in adults with polycythaemia vera whose disease is resistant to or intolerant of hydroxycarbamide.

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| Intervention(s) | Ruxolitinib |
| Population(s) | Adults with polycythaemia vera that is resistant or intolerant to hydroxycarbamide |
| Comparators | <ul style="list-style-type: none"> • Hydroxycarbamide • Interferon • Anagrelide • Busulfan • Radioactive phosphorus • Melphalan • Best supportive care |
| Outcomes | <p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • mortality • symptom relief (including a reduction in spleen size, itching, and venesection) • response rate • progression to acute myeloid leukaemia or myelofibrosis • adverse effects of treatment • health-related quality of life |

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| Economic analysis | <p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>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.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p> |
| Other considerations | <p>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.</p> |
| Related NICE recommendations and NICE Pathways | <p>Related Technology Appraisals:</p> <p>Technology Appraisal No. 289, June 2013, 'Ruxolitinib for disease-related splenomegaly or symptoms in adults with myelofibrosis'. Expected review proposal decision date: August 2014.</p> |
| Related National Policy | <p>None.</p> |

Questions for consultation

Have all relevant comparators for ruxolitinib been included in the scope?
Which treatments are considered to be established clinical practice in the NHS for polycythaemia vera? How should 'best supportive care' be defined?

Are there any subgroups of people in whom ruxolitinib is expected to be more clinically effective and cost effective 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 ruxolitinib 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.

Do you consider ruxolitinib 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)?

Do you consider that the use of ruxolitinib can result in any potential significant and 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 Appraisal Committee to take account of these benefits.

NICE intends to appraise this technology through its Single Technology Appraisal (STA) Process. We welcome comments on the appropriateness of appraising this topic through this process. (Information on the Institute's Technology Appraisal processes is available at http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/technology_appraisal_process_guides.jsp)