NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

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

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of ruxolitinib within its marketing authorisation 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 the 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, and between 5 and 10% go on to have 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 phlebotomy (bloodletting), interferon, hydroxycarbamide, anagrelide, radioactive phosphorus or low dose busulphan. In addition, melphalan has a licence for treating polycythaemia vera in the UK, but is rarely used in clinical practice.

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 has a marketing authorisation in the UK for treating polycythaemia vera that is resistant or intolerant to hydroxycarbamide in adults.

Intervention(s)	Ruxolitinib with established clinical practice
Population(s)	Adults with polycythaemia vera that is resistant or intolerant to hydroxycarbamide
Comparators	Established clinical practice without ruxolitinib, comprising treatment with phlebotomy and aspirin, and: • Hydroxycarbamide • Interferon alfa • Anagrelide • Busulfan • Radioactive phosphorus • No additional treatment
Outcomes	The outcome measures to be considered include: • complete haematological remission (including reporting of haematocrit, white blood cell count and platelet count separately) • mortality • symptom relief (including a reduction in spleen size, itching, fatigue and phlebotomy) • thrombosis • progression to acute myeloid leukaemia or myelofibrosis • 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.

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	Appraisals in development 'Ruxolitinib for disease-related splenomegaly or symptoms in adults with myelofibrosis (Review TA289)' NICE technology appraisals guidance ID831. Publication expected February 2016.
Related National Policy	None.