NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Proposed Health Technology Appraisal

Masitinib for treating systemic mastocytosis [ID781]

Draft scope (pre-referral)

Draft remit/appraisal objective

To appraise the clinical and cost effectiveness of masitinib within its marketing authorisation for treating systemic mastocytosis.

Background

Mastocytosis is a rare condition caused by excessive amounts of mast cells gathering in body tissues, such as the skin, organs and bones. The World Health Organization's classification system divides the condition into cutaneous mastocytosis and five main subtypes of systemic mastocytosis. These include indolent systemic mastocytosis (which accounts for over 90% of cases of systemic disease) and smouldering systemic mastocytosis.

The mast cells release large amounts of histamine and other chemicals into the blood, causing symptoms such as skin rash, itchy skin and hot flushes. Other symptoms can include anaphylaxis, vomiting and diarrhoea, muscle and joint pain, mood changes, headaches and tiredness. These wide-ranging symptoms can be disabling or even life-threatening. The systemic condition mainly affects adults and it is estimated that around 1 in 150,000 people in England have systemic mastocytosis.

Various treatments are used to control the symptoms of systemic mastocytosis (many outside their marketing authorisations). They include antihistamines, sodium cromoglicate, corticosteroids, cladribine and tyrosine kinase inhibitors such as imatinib, nilotinib and dasatinib.

The technology

Masitinib (brand name unknown, AB Science) is a tyrosine kinase inhibitor that inhibits c-Kit, platelet-derived growth factor receptor, fibroblast growth factor receptor and kinases, which influence the activation of inflammatory cells. Masitinib is administered orally.

Masitinib does not have a marketing authorisation in the UK for treating systemic mastocytosis. It has been compared with placebo in clinical trials in adults with previously treated smouldering or indolent systemic mastocytosis or cutaneous mastocytosis, who have an overall perception of disability (OPA) score greater than 2 (moderate to intolerable general handicap) as a result of their condition.

Intervention(s)	Masitinib
Population(s)	People with smouldering or indolent systemic mastocytosis with moderate to very severe disability as a result of their condition
Comparators	Established clinical management without masitinib
Outcomes	The outcome measures to be considered include: • symptoms (including itching, flushes, depression and weakness) • 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	None
Related National Policy	Manual for Prescribed Specialised Services (2013/14) 'Highly specialised allergy services (all ages). section B09 – Specialised Immunology and Allergy Services. Department of Health, NHS Outcomes Framework 2014-2015, Nov 2013. Domain 2. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/256456/NHS_outcomes.pdf

Questions for consultation

Is mastinib likely to be used for the treatment of cutaneous mastocytosis?

Have all relevant comparators for masitinib been included in the scope? Which treatments are considered to be established clinical practice in the NHS for systemic mastocytosis? How should established clinical management be defined?

Have the most appropriate mastocytosis-related outcomes been included?

Are there any subgroups of people in whom masitinib 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 masitinib 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 masitinib 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 masitinib 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/article/pmg19/chapter/1-Introduction)