NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Avapritinib for treating moderate to severe indolent systemic mastocytosis ID6578

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of avapritinib within its marketing authorisation for treating moderate to severe indolent systemic mastocytosis.

Background

Mastocytosis is a rare condition caused by an excess number of mast cells gathering in the body's tissues. The cause or causes of mastocytosis are not fully known, but there's thought to be an association with a change in genes, known as the KIT mutation. The KIT mutation makes the mast cells more sensitive to the effects of a signalling protein called stem cell factor (SCF). SCF plays an important role in stimulating the production and survival of certain cells, such as blood cells and mast cells, inside the bone marrow.[1] The mast cells release large amounts of histamine and other mediators into the blood, causing symptoms such as skin rash, itchy skin, hot flushes, blood pressure changes, fainting, tachycardia, headache, vomiting, diarrhoea, brain fog, organ failure and anaphylaxis.

There are 2 main types of mastocytosis, cutaneous mastocytosis, which affects the skin (mainly in children), and systemic mastocytosis, which affects the skin, internal organs and bones (mainly in adults). Systemic mastocytosis has subtypes defined by level of disease: indolent (the most common, about 90% of cases[1]), smouldering, and advanced systemic mastocytosis. For most people, symptoms of indolent systemic mastocytosis are mild to moderate, but vary from person to person. In 1% to 3% of people, indolent systemic mastocytosis can progress to a more aggressive form.[2]

An estimated 1 in 7,700 to 10,400 people in Europe have systemic mastocytosis.[3]

There is no cure for indolent systemic mastocytosis. Treatment aims to relieve symptoms and includes antihistamines, sodium cromoglicate and oral corticosteroids.

The technology

Avapritinib (Ayvakyt, Blueprint Medicines) does not currently have a marketing authorisation in the UK for treating moderate to severe symptoms of indolent systemic mastocytosis.

Avapritinib plus best supportive care is being studied in a clinical trial compared with placebo plus best supportive care in adults with indolent systemic mastocytosis.

Avapritinib has a marketing authorisation to treat aggressive systemic mastocytosis, systemic mastocytosis with an associated haematological neoplasm, or mast cell leukaemia in adults.

Intervention(s)	Avapritinib
Population(s)	Adults with indolent systemic mastocytosis
Comparators	Best supportive care
Outcomes	The outcome measures to be considered include: • mortality • symptom severity • response rate • 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 and cost of biosimilar and generic products should be taken into account.
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:** Avapritinib for treating advanced systemic mastocytosis (2024) NICE technology appraisal guidance 1012. Midostaurin for treating advanced systemic mastocytosis (2021) NICE technology appraisal guidance 728.

Questions for consultation

Where do you consider avapritinib will fit into the existing care pathway for indolent systemic mastocytosis?

What treatments are included in best supportive care for indolent systemic mastocytosis?

Is avapritinib expected to be used instead of or in addition to currently available treatments?

How are symptoms of indolent systemic mastocytosis measured in clinical practice? How would moderate and severe symptoms be defined?

Please select from the following, will avapritinib 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 avapritinib be a candidate for managed access?

Do you consider that the use of avapritinib 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.

Please indicate if any of the treatments in the scope are used in NHS practice differently than advised in their Summary of Product Characteristics. For example, if the dose or dosing schedule for a treatment is different in clinical practice. If so, please indicate the reasons for different usage of the treatment(s) in NHS practice. If stakeholders consider this a relevant issue, please provide references for data on the efficacy of any treatments in the pathway used differently than advised in the Summary of Product Characteristics.

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 avapritinib 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.UK: mastocytosis [accessed July 2025].

2. UK masto.org: mastocytosis [accessed July 2025].

3. Orphanet: systemic mastocytosis [accessed July 2025].