NICE National Institute for Health and Care Excellence

NICE guidance

Avapritinib for treating advanced systemic mastocytosis

Technology appraisal guidance
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Your responsibility

The recommendations in this guidance represent the view of NICE, arrived at after careful consideration of the evidence available. When exercising their judgement, health professionals are expected to take this guidance fully into account, alongside the individual needs, preferences and values of their patients. The application of the recommendations in this guidance is at the discretion of health professionals and their individual patients and do not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian.

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Commissioners and providers have a responsibility to promote an environmentally sustainable health and care system and should assess and reduce the environmental impact of implementing NICE recommendations wherever possible.

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1 Recommendations

1.1 Avapritinib is recommended, within its marketing authorisation, as an option for treating advanced systemic mastocytosis (including aggressive systemic mastocytosis, systemic mastocytosis with an associated haematological neoplasm and mast cell leukaemia) in adults. Avapritinib is only recommended if the company provides it according to the commercial arrangement.

Why the committee made this recommendation

Standard treatments for advanced systemic mastocytosis include midostaurin and cladribine. Midostaurin is used at first line when possible, with cladribine mostly being used at second line or later.

Evidence from clinical trials suggests that avapritinib increases how long people have before their condition gets worse and how long they live. But avapritinib was not compared with any other treatments in these trials, so how it compares with them is uncertain. An indirect comparison suggests that avapritinib increases how long people live compared with midostaurin at first line and with cladribine at second or later lines.

Despite the uncertainty in the clinical-effectiveness evidence, the cost-effectiveness estimate for avapritinib is within the range that NICE considers an acceptable use of NHS resources. So, avapritinib is recommended.

2 Information about avapritinib

Marketing authorisation

2.1 Avapritinib (Ayvakyt) is indicated 'as monotherapy for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated haematological neoplasm (SM-AHN), or mast cell leukaemia (MCL)'.

Dosage in the marketing authorisation

2.2 The dosage schedule is available in the summary of product characteristics for avapritinib.

Price

2.3 The list price for avapritinib is £26,667 for a 30-pack of 25-mg, 50-mg, 100-mg or 200-mg tablets (excluding VAT; company submission).

2.4 The company has a commercial arrangement. This makes avapritinib available to the NHS with a discount. The size of the discount is commercial in confidence.

3 Committee discussion

The evaluation committee considered evidence submitted by Blueprint Medicines, a review of this submission by the external assessment group (EAG) and responses from stakeholders. See the committee papers for full details of the evidence.

The condition

Details of condition

3.1 Mastocytosis is a rare group of heterogenous diseases characterised by excessive mast cells. It includes advanced systemic mastocytosis, which is a severe form of the disease with 3 diverse subtypes:

- aggressive systemic mastocytosis
- systemic mastocytosis with associated haematological neoplasm mast cell leukaemia.

People with advanced systemic mastocytosis have a lot of symptoms because of the systemic nature of the condition. The patient experts explained that the symptoms have a major debilitating effect on their daily activities and quality of life. These include frequent and unexpected anaphylaxis, diarrhoea and vomiting. One patient expert explained how they had previously had 9 episodes of anaphylaxis over a few weeks. They added that avapritinib had been transformative in reducing the frequency of those episodes. One clinical expert described 1 person with the condition who had 20 bowel movements per day before having treatment with avapritinib. The patient experts described how avapritinib reduced the symptomatic burden of advanced systemic mastocytosis, prevented repeated hospital admissions, and allowed people to engage in physical and social activity. The committee concluded that there is an unmet need for people with advanced systemic mastocytosis. It also concluded that people with the condition would welcome a disease-modifying treatment option with less severe side effects.

Clinical management

Treatment options

3.2 The clinical experts explained that the treatment pathway for advanced systemic mastocytosis is complex. Treatment is individualised based on symptoms, and because of the diversity of the disease subtypes. Midostaurin is the only licensed targeted treatment for advanced systemic mastocytosis currently available in the NHS. Other treatments, including cladribine, imatinib, interferon alpha and pegylated interferon, are sometimes used in specific circumstances. The patient and clinical experts agreed that midostaurin is a welcome part of the treatment pathway. But they explained that advanced systemic mastocytosis still has a poor prognosis, particularly for systemic mastocytosis with associated haematological neoplasm and for mast cell leukaemia. The experts explained that most treatments do not treat the condition itself because they are not designed to be disease modifying. They added that people with advanced systemic mastocytosis who have had both midostaurin and avapritinib prefer avapritinib. This was because it causes fewer side effects and has lasting positive effects that improve quality of life dramatically. In particular, midostaurin is associated with gastrointestinal side effects, including nausea and vomiting. The patient experts described this vomiting as intolerable, even when using antinausea medications. This means that people often have to alter their lifestyles or stop treatment. The committee concluded that current treatment options for advanced systemic mastocytosis are limited.

Positioning of avapritinib

3.3 In its submission, the company chose to split its clinical- and cost-effectiveness analyses by lines of treatment. This meant that different comparators were used for the first- and second-line populations. It proposed that midostaurin was the most suitable comparator for avapritinib at first line, and cladribine was the most suitable at second line or later. The EAG agreed that the main comparator for avapritinib was midostaurin. But it said that it would be more appropriate to assess the cost effectiveness of avapritinib compared with midostaurin in the overall population, not just at first line. It noted that the recommendation in

NICE's technology appraisal guidance on midostaurin for treating advanced systemic mastocytosis does not restrict treatment to the first-line population setting only. The EAG did acknowledge that most people are likely to have midostaurin at first line in the NHS, but that some people will have it at second line. The committee asked the clinical experts to clarify the expected treatment pathway for people with advanced systemic mastocytosis in the NHS. They explained that the condition is heterogeneous, resulting in individualised treatment plans. They said that, in the absence of very effective treatments, midostaurin would be used at first line, but response to treatment can often be slow or incomplete. They added that cladribine would usually follow midostaurin, except for in a small group of people who would have cladribine at first line to help a rapid debulking of disease. The NHS England Cancer Drugs Fund clinical lead said that, during the 3 years that midostaurin has been available in the NHS, about 80% of people have had it at first line. The patient experts acknowledged that avapritinib would be used at second line when people have had midostaurin already. But they would expect avapritinib to be used before midostaurin in people who have not had any previous treatment. The committee acknowledged the EAG's concerns around splitting the population into lines of treatment. But it recognised the clinical and patient expert opinion on the anticipated positioning of avapritinib. The committee thought that it was appropriate to consider avapritinib across all lines of treatment, and accepted that the company's evidence was sufficient for decision making.

Clinical effectiveness

Clinical-effectiveness evidence

3.4 The key clinical evidence for avapritinib came from 2 trials:

- PATHFINDER (n=107), a phase 2 open-label single-arm trial
- EXPLORER (n=86), a phase 1 open-label dose-finding single-arm trial.

An external control study (n=141) was used to compare results from people from PATHFINDER and EXPLORER with results from a respective cohort of people having treatment for advanced systemic mastocytosis in clinical

practice. All studies were international multicentre studies and included sites in the UK. The populations of PATHFINDER and EXPLORER included people with advanced systemic mastocytosis. EXPLORER also included people with other myeloid malignancies (n=17). In PATHFINDER, most people had a starting dose of 200 mg once daily (n=105) and around a third of people had avapritinib as a first-line treatment (n=38). People with a confirmed diagnosis of advanced systemic mastocytosis were split into 2 cohorts depending on whether they met the modified Internation Working Group criteria for response-evaluable disease. This provided a response-evaluable population (RAC-RE; n=81) separate to the overall safety population. The primary efficacy endpoint was overall response rate. Secondary endpoints included overall survival (OS), progression-free survival (PFS) and health-related quality-of-life measures. EXPLORER consisted of a dose-finding phase, and a dose-expansion phase to determine the maximum tolerated dose of avapritinib. The characteristics of the RAC-RE population in EXPLORER were similar to those in PATHFINDER, but a larger proportion had had previous treatments.

The EAG noted that both PATHFINDER and EXPLORER were single-arm trials, so could not provide any estimates of avapritinib's clinical effectiveness relative to other treatments such as midostaurin. The company provided an indirect treatment comparison to address this (see section 3.7). The EAG also noted uncertainty with the results of both trials because of the immaturity of the data (see section 3.6). The company decided to pool data from PATHFINDER and EXPLORER to reduce some of this uncertainty, which the EAG agreed was a suitable approach. The pooled evidence provided a median follow up of 36 months. The clinical expert advice to the company and the EAG was that people in the trials were slightly older than the people who would be seen in NHS clinical practice. But the rarity of advanced systemic mastocytosis limited the data available to explore the impact of age on response to avapritinib. The committee noted that the trial evidence was generalisable to the UK population, but it would have preferred a comparative clinical trial. The patient experts emphasised the rarity of advanced systemic mastocytosis, and the potential difficulties of recruiting people to a randomised controlled trial. The clinical experts agreed, stating that the evidence presented in PATHFINDER and EXPLORER was the most robust available. The committee concluded that in the absence of directly

comparative evidence, the trial evidence was suitable for decision making.

Data cuts

3.5 In the company's initial submission, it presented data from the September 2022 data cut for PATHFINDER, and a pooled analysis of PATHFINDER and EXPLORER from April 2021 and June 2020. The EAG requested later data cuts to help reduce some of the uncertainty in the OS and PFS estimates. After technical engagement, the company provided updated effectiveness data from PATHFINDER (September 2023), and pooled PATHFINDER and EXPLORER data (September 2023 and January 2023). Response rates remained mainly consistent with previous data cuts. A marginally lower overall response rate was seen in the updated pooled PATHFINDER and EXPLORER population. But endpoints relating to OS, were still not met in the newer data cuts. The committee concluded that the OS trial data was immature, making its results uncertain.

Treatment effect

3.6 Pooled PATHFINDER and EXPLORER results showed that, across all types of advanced systemic mastocytosis, a large proportion of people were still alive 24 months after starting treatment with avapritinib. The results are confidential and cannot be reported here. Median OS was not reached for people having first-line treatment. But it was reached in the second-line population. Median PFS was reached across all populations. The committee concluded that the results of the trials were promising. But it thought that the immaturity of the OS data introduced uncertainty into the decision-making process.

Indirect treatment comparison

3.7 In its submission, the company provided several indirect treatment comparisons, including an inverse probability of treatment weighting (IPTW) analysis using the pooled PATHFINDER and EXPLORER safety population data and the external control study data. Avapritinib was compared with midostaurin and cladribine

individually according to treatment line. The indirect treatment comparisons suggested that avapritinib increased how long people live compared with midostaurin and cladribine. The results are confidential and cannot be reported here. The IPTW was used to inform the company's base-case analysis. The EAG said that the IPTW was the most appropriate indirect treatment comparison, but it expressed concern with the adjustment for baseline characteristics. It said that the company had not adjusted for some key prognostic variables, including C-findings, bone marrow mast-cell burden and KIT D816V mutation status. But it also may have over-adjusted for non-prognostic factors, such as region. The company explained that data on C-findings and bone marrow mast-cell burden were not available for adjustment. Even though data on KIT D816V mutation status was available, the company did not think that any adjustment was necessary. This was because most people in the external control study and pooled PATHFINDER and EXPLORER analysis were KIT D816V positive. It explained that region was an important factor to adjust for because of potential differences in treatment at study sites. The EAG acknowledged that data may not have been available to allow for adjustment of C-findings and bone marrow mast-cell burden. But it said that they could still affect the direction and size of effect because of their potentially prognostic status. The clinical and patient experts noted that indirect comparisons are less definitive than trials. But they thought that the comparisons were still of interest, particularly because advanced systemic mastocytosis is such a rare disease. The committee acknowledged the uncertainty associated with the indirect treatment comparison. But it also noted that the rarity of the condition would contribute to difficulties in collecting data. It concluded that the indirect treatment comparison was suitable for decision making, but that its results were uncertain.

Economic model

Company's modelling approach

3.8 To compare avapritinib with midostaurin and cladribine in people with advanced systemic mastocytosis, the company used a partitioned survival model with 3 health states (progression free, progressed disease and death). People entered the model in the progression-free health state. Transitions to the progressed-

disease and death health states were determined by the PFS and OS curves. These curves were extrapolated beyond available data to model a lifetime horizon. The effects of subsequent treatment after stopping initial treatment and of prior treatment were also considered. The progression-free and progressed-disease health states included 'on primary treatment' and 'off primary treatment'. This was to reflect switching to different treatments either after stopping treatment (in the progression-free health state) or after progression (in the progressed-disease health state). The EAG noted that the company's base-case model structure was consistent with the final model structure used in NICE's technology appraisal guidance on midostaurin for treating advanced systemic mastocytosis. But it highlighted that the appropriateness of such modelling depended on the maturity of the clinical-effectiveness data. This is because any uncertainty in the long-term survival extrapolations would lead to uncertainty in the cost-effectiveness estimates of avapritinib compared with midostaurin and cladribine. The committee concluded that the company's model was suitable for decision making. But it thought that the company's cost-effectiveness estimates were uncertain because of uncertainty in the survival estimates (see section 3.9 and section 3.11).

OS extrapolation

3.9 When extrapolating the pooled OS data from PATHFINDER and EXPLORER, and the external control study, the company chose the following parametric distributions:

- first line, avapritinib arm: generalised gamma
- first line, midostaurin arm: exponential
- second line, avapritinib arm: exponential
- second line, cladribine arm: log-normal.

The hazard of death for avapritinib was set to equal its respective comparator arm (depending on the line of treatment) after a treatment-benefit duration of 7.5 years (see section 3.10). The EAG agreed that the chosen parametric distributions were the best statistical fit. But it was

concerned with the immaturity of the OS data for avapritinib, and its extrapolation beyond its limited follow up. The EAG also noted that extrapolating avapritinib's OS data using different parametric functions led to very different long-term survival outcomes. This was particularly so for the exponential one, which was the second-best fitting curve. The long-term OS estimates were also dependent on the chosen length of treatment-benefit duration because the difference in OS between the different parametric distributions increased over time. If people moved to the comparator curve's OS extrapolation estimates earlier, there was less time for variability between avapritinib's extrapolations to be captured within the long-term survival estimates. The EAG did not consider that the updated pooled PATHFINDER and EXPLORER data had substantially reduced uncertainty in the OS estimates. This uncertainty affected how easy it was to extrapolate these estimates over the long term. The committee acknowledged this. It asked the clinical experts for their opinions on long-term survival outcomes. They said that it is difficult to ascertain expected survival because of the rarity and heterogeneity of the condition. They explained that avapritinib may provide prolonged periods of remission to some people, but that it is not curative because many people have haematological neoplasms as well as mastocytosis. So, they would predict a slow decline in OS over time. The committee acknowledged that long-term OS estimates depended on the choice of extrapolation curve and of treatment effect duration. It also noted that the results for avapritinib were highly sensitive to both in combination. It concluded that the choices of parametric distribution were the best statistical fits. But it thought that the immaturity of the pooled OS data from PATHFINDER and EXPLORER increased the uncertainty in the long-term OS estimates.

Duration of treatment benefit

3.10 In the company's original model, the duration of treatment benefit was assumed to be 5 years. This was based on the rate of duration of response seen in an earlier data cut of PATHFINDER's RAC-RE population. The EAG thought 5 years was reasonable, but said it could be pessimistic. This was because a reasonable proportion of people were still on treatment after 5 years. After technical engagement, the company updated its assumption from 5 years to 7.5 years. This

was based on the most recent data cut of the pooled PATHFINDER and EXPLORER data, and the external control study IPTW analysis. In this analysis, a greater proportion of people were on treatment at 7.5 years than in the earlier data cut. The EAG noted that this change was reasonable based on this analysis. But it emphasised that the duration of treatment benefit should not be considered in isolation of other survival outcomes. This was because the results were sensitive to combining different parametric extrapolations and different durations of treatment effect. The EAG highlighted that there was still uncertainty about the duration of treatment benefit for avapritinib in relation to its comparators. The clinical experts said that it was difficult to predict what the duration of treatment benefit for avapritinib may be. They suggested that 5 to 7 years seemed reasonable, but this duration may be shorter in the second-line setting than in the first-line setting. When considering other factors such as survival outcomes, the committee concluded that it was appropriate to apply a duration of treatment effect. But it concluded that a duration of 5 years was more suitable for decision making.

PFS extrapolation

3.11 When extrapolating the pooled PFS data from PATHFINDER and EXPLORER, the company chose the following parametric distributions:

- first line, avapritinib arm: exponential
- second line, avapritinib arm: log-normal.

PFS data was not available from the external control study for an IPTW comparison with the pooled PFS data from PATHFINDER and EXPLORER. So, the company used the comparator's time-on-treatment curve as a proxy for its PFS curve. When extrapolating the time-on-treatment data from the external control study, the company chose the following parametric distributions:

- first line, midostaurin arm: log-normal
- second line, cladribine: exponential.

The EAG acknowledged that there was limited PFS data for the comparators, and thought it reasonable to use the company's time-on-treatment curves as a proxy for PFS. It explained that the pooled PATHFINDER and EXPLORER PFS data had reached median PFS, which reduced concerns about the immaturity of the PFS data for avapritinib. But, as with the OS extrapolations (see section 3.9), the EAG expressed concern with the long-term extrapolation of PFS for avapritinib. It noted that extrapolating avapritinib's PFS data using different parametric functions also led to very different long-term PFS outcomes. The long-term PFS estimates were also dependent on the chosen duration of treatment benefit. The committee agreed with the choice of extrapolations but noted the EAG's concerns about the long-term PFS outcomes.

Utility values

Source of utility values

3.12 EQ-5D data could not be used to determine utility values for the progression-free and progressed-disease health states because it was not available from PATHFINDER or EXPLORER. To determine these utility values, the company mapped the European Organisation for Research and Treatment of Cancer Quality of Life Core 30-Item Questionnaire (EORTC QLQ-C30) data collected from the RAC-RE population of PATHFINDER onto the EQ-5D-3L. It did this using an algorithm established by Young et al. (2015). The mapped utility values for each person across all preprogression observations were averaged to derive a single utility value for the progression-free health state. There was only 1 observation noted for the progressed-disease health state, even after pooling the PATHFINDER and EXPLORER data. So, the company used literature to identify relevant health-state utility values for the progressed-disease health state in advanced systemic mastocytosis. It identified 6 studies, 4 of which were used to calculate a ratio between the progression-free and progressed-disease health states. The weighted average of the ratios from each study was calculated. Then, it was applied to the progression-free utility values for the first- and second-line populations to estimate respective utility values for the progressed-disease health state. These utility values were adjusted for aging in the model, and they

were not permitted to exceed gender- and age-adjusted UK general population utilities. The EAG noted that there was a large variation in the ratios derived from the 4 studies identified, and the mean age of people in those studies. All 4 studies also included people with acute myeloid leukaemia, a haematological cancer not associated with advanced systemic mastocytosis. So, the EAG questioned the generalisability of the data. The clinical and patient experts emphasised that quality of life is seriously affected by advanced systemic mastocytosis. They added that the utility values seen for each population aligned with people's experiences of the 2 different health states. The committee understood the EAG's concerns, but thought that the utility values were reasonable for the progression-free and progressed-disease health states. It concluded that the utility values were suitable for decision making.

Costs

Subsequent treatment costs

3.13 In its submission, the company stated that avapritinib could be used before an allogeneic haematopoietic stem cell transplant (allo-HSCT), the only curative option for people with advanced systemic mastocytosis. This is because some people who have avapritinib have complete remission and may then be eligible for an allo-HSCT. But the EAG noted that subsequent treatment costs, and the utility values associated with using subsequent treatments such as an allo-HSCT, were excluded from the company's base case. The clinical experts said that an allo-HSCT may be an option for some people after treatment with avapritinib, but that this cohort is likely to be very small. This is because many people with advanced systemic mastocytosis are older and have comorbidities that mean they are ineligible. Or, they do not have a large enough response to avapritinib to benefit from an allo-HSCT. One patient expert explained that, based on information from several haematologists, they estimated that a maximum of 10% of people with advanced systemic mastocytosis would be eligible for an allo-HSCT. The committee understood that the eligible cohort for an allo-HSCT was low, and that including subsequent treatment costs in the economic model was not needed.

Severity

3.14 NICE's methods on conditions with a high degree of severity did not apply.

Cost-effectiveness results

Committee's preferred assumptions

3.15 After technical engagement, both the company's and EAG's base cases were based on the same modelling assumptions. These were:

- PFS data sourced from the RAC-RE population of pooled PATHFINDER and EXPLORER results (see section 3.6)
- OS data sourced from the IPTW (indirect treatment comparison) between the pooled PATHFINDER and EXPLORER data, and the external control study data (see section 3.7)
- using generalised gamma (first line) and exponential (second line) parametric distributions to extrapolate avapritinib's OS data (see section 3.9)
- using an exponential parametric distribution to extrapolate midostaurin's OS data (see section 3.9)
- using a log-normal parametric distribution to extrapolate cladribine's OS data (see section 3.9)
- a treatment-benefit duration of 7.5 years (see section 3.10)
- progression-free health-related quality-of-life data from pooled PATHFINDER and EXPLORER data (see section 3.12).

  The committee accepted most of the EAG's and company's chosen assumptions but thought that a treatment-benefit duration of 5 years was more appropriate.

Acceptable ICER

3.16 NICE's manual on health technology evaluations notes that, above a most plausible incremental cost-effectiveness ratio (ICER) of £20,000 per quality-adjusted life year (QALY) gained, judgements about the acceptability of a technology as an effective use of NHS resources will consider the degree of certainty around the ICER. The committee will be more cautious about recommending a technology if it is less certain about the ICERs presented. But it will also consider other aspects, including uncaptured health benefits. The committee noted the level of uncertainty, specifically that:

- The clinical and cost-effectiveness analysis was separated by treatment line (see section 3.3).
- The clinical-effectiveness evidence came from single-arm trials (see section 3.4).
- The indirect treatment comparison was uncertain because of the approach taken when adjusting baseline characteristics (see section 3.7).
- The OS data was immature, with the median OS not reached (see section 3.6).
- Both extrapolation of OS and PFS data was highly sensitive to the choice of parametric distribution and duration of treatment benefit (see sections 3.9 to 3.11).

But the committee also acknowledged that the rarity of the condition led to:

- data-collection difficulties underpinning many of the uncertainties
- the additional benefits of avapritinib not being captured in the model (see section 3.18).

So, the committee concluded that an acceptable ICER would be around £30,000 per QALY gained.

Cost–utility analysis

3.17 The committee noted that it was appropriate to recommend avapritinib across all lines of therapy. It took into account ICERs for both first- and second-line positioning, and concluded that the most appropriate ICER for decision making would be within this range. The committee's preferred ICER for decision making was below £30,000 per QALY gained when:

- including all confidential discounts that applied to treatments in the model
- taking account of the committee's preferred assumptions (see section 3.15).

The exact ICERs are confidential and cannot be reported here. The committee specified that the most appropriate ICER for avapritinib across all treatment lines was between the ICERs for first- and second-line treatment. So, the committee concluded that the cost-effectiveness estimate for avapritinib was below what it considered to be a cost-effective use of NHS resources.

Other factors

Uncaptured benefits

3.18 The committee identified additional benefits of avapritinib not captured in the economic modelling. The clinical and patient experts discussed the side effects associated with midostaurin. They explained how these side effects were substantially more unpleasant than those with avapritinib. It was noted that side effects have a further impact on quality of life for people who already have systemic symptoms from their condition. The experts explained that side effects, such as vomiting, are often intolerable and lead to people stopping treatment. The clinical experts said that avapritinib would offer an alternative disease-modifying treatment with less severe side effects than midostaurin. Additionally, the committee noted that avapritinib does not contain gelatine as an excipient, unlike midostaurin. Including gelatine can cause issues for people who follow certain diets. So, having an option without gelatine may benefit them. The committee concluded that the additional benefits of avapritinib had not been

captured and should be taken into account in its decision making.

Equality

3.19 The committee did not identify any other equality issues.

Conclusion

Recommendation

3.20 The committee's preferred ICER for avapritinib across all treatment lines was between the ICERs for first- and second-line treatment. So, it concluded that the most likely cost-effectiveness estimate for avapritinib was below what it considered to be a cost-effective use of NHS resources. This means avapritinib is recommended for treating advanced systemic mastocytosis in adults.

4 Implementation

4.1 Section 7 of the National Institute for Health and Care Excellence (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013 requires integrated care boards, NHS England and, with respect to their public health functions, local authorities to comply with the recommendations in this evaluation within 3 months of its date of publication.

4.2 Chapter 2 of Appraisal and funding of cancer drugs from July 2016 (including the new Cancer Drugs Fund) – A new deal for patients, taxpayers and industry states that for those drugs with a draft recommendation for routine commissioning, interim funding will be available (from the overall Cancer Drugs Fund budget) from the point of marketing authorisation, or from release of positive draft guidance, whichever is later. Interim funding will end 90 days after positive final guidance is published (or 30 days in the case of drugs with an Early Access to Medicines Scheme designation or cost comparison evaluation), at which point funding will switch to routine commissioning budgets. The NHS England Cancer Drugs Fund list provides up-to-date information on all cancer treatments recommended by NICE since 2016. This includes whether they have received a marketing authorisation and been launched in the UK.

4.3 The Welsh ministers have issued directions to the NHS in Wales on implementing NICE technology appraisal guidance. When a NICE technology appraisal guidance recommends the use of a drug or treatment, or other technology, the NHS in Wales must usually provide funding and resources for it within 2 months of the first publication of the final draft guidance.

4.4 When NICE recommends a treatment 'as an option', the NHS must make sure it is available within the period set out in the paragraphs above. This means that, if a patient has advanced systemic mastocytosis and the healthcare professional responsible for their care thinks that avapritinib is the right treatment, it should be available for use, in line with NICE's recommendations.

5 Evaluation committee members and NICE project team

Evaluation committee members

The 4 technology appraisal committees are standing advisory committees of NICE. This topic was considered by committee C.

Committee members are asked to declare any interests in the technology being evaluated. If it is considered there is a conflict of interest, the member is excluded from participating further in that evaluation.

The minutes of each evaluation committee meeting, which include the names of the members who attended and their declarations of interests, are posted on the NICE website.

Chair

Richard Nicholas
Vice chair, technology appraisal committee C

NICE project team

Each evaluation is assigned to a team consisting of 1 or more health technology analysts (who act as technical leads for the evaluation), a technical adviser, a project manager and an associate director.

Emily Leckenby
Technical lead

Caron Jones
Technical adviser

Kate Moore

Project manager

Ian Watson
Associate director

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