

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Asciminib for chronic myeloid leukaemia after two or more tyrosine kinase inhibitors

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of asciminib within its marketing authorisation for treating Philadelphia-chromosome-positive chronic myeloid leukaemia in the chronic phase after two or more tyrosine kinase inhibitors.

Background

Chronic myeloid leukaemia (CML) is a type of cancer that affects the blood-forming cells, called myeloid cells, found in the bone marrow^{1,2}. People with CML have a genetic mutation in their bone marrow cells which causes an abnormal chromosome to form, known as the Philadelphia chromosome¹. The Philadelphia chromosome is made up of 2 genes which join to make a single fusion gene called BCR-ABL¹. The BCR-ABL gene causes the myeloid cells to produce a protein, called tyrosine kinase, that encourages white blood cells to grow and multiply¹. CML usually develops and progresses slowly. It is diagnosed in one of three distinct phases; chronic, accelerated or blast phase³.

The incidence of CML is approximately 1.3 per 100,000 in the UK⁴, it is a rare condition with around 760 people diagnosed in the UK each year⁴. The median age at diagnosis is between 60 and 65 years of age. Around 90% of CML cases are diagnosed at the chronic phase².

NICE technology appraisal [401](#) recommends bosutinib as an option, for chronic, accelerated and blast phase Philadelphia chromosome positive chronic myeloid leukaemia in adults, when they have previously had 1 or more tyrosine kinase inhibitor and imatinib, nilotinib and dasatinib are not appropriate. NICE technology appraisal guidance [425](#) recommends dasatinib and nilotinib as options for treating only chronic- or accelerated-phase Philadelphia-chromosome-positive chronic myeloid leukaemia in adults, if they cannot have imatinib, or their disease is imatinib-resistant. NICE technology appraisal guidance [451](#) recommends ponatinib as an option for treating chronic-, accelerated- or blast-phase chronic myeloid leukaemia in adults when the disease is resistant to dasatinib or nilotinib or they cannot tolerate dasatinib or nilotinib and for whom subsequent treatment with imatinib is not clinically appropriate or the T315I gene mutation is present.

The technology

Asciminib (ABL001, Novartis) is an allosteric inhibitor of the tyrosine kinase BCR-ABL1 fusion protein. It binds to BCR-ABL1, which inhibits BCR-ABL1 mediated cell proliferation. It is administered orally.

Asciminib does not currently have marketing authorisation in the UK for previously treated Philadelphia chromosome-positive CML. It has been studied in clinical trials in comparison with bosutinib in adults with CML in the chronic phase; previously treated with 2 or more tyrosine kinase inhibitors.

Intervention(s)	Asciminib
Population(s)	Adults with chronic-phase Philadelphia chromosome-positive chronic myeloid leukaemia whose disease has previously been treated with 2 or more tyrosine kinase inhibitors
Comparators	<ul style="list-style-type: none"> • Bosutinib • Dasatinib • Nilotinib • Ponatinib
Outcomes	<p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • progression-free survival • overall survival • response rates • time to response • adverse effects of treatment • health related quality of life
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>

<p>Related NICE recommendations and NICE Pathways</p>	<p>Bosutinib for previously treated chronic myeloid leukaemia (2016) NICE technology appraisal guidance 401</p> <p>Dasatinib, nilotinib and high-dose imatinib for treating imatinib-resistant or intolerant chronic myeloid leukaemia (2016) NICE technology appraisal guidance 425</p> <p>Ponatinib for treating chronic myeloid leukaemia and acute lymphoblastic leukaemia (2017) NICE technology appraisal guidance 451</p> <p>Myeloid leukaemia (2021) NICE pathway https://pathways.nice.org.uk/pathways/blood-and-bone-marrow-cancers#path=view%3A/pathways/blood-and-bone-marrow-cancers/myeloid-leukaemia.xml&content=view-node%3Anodes-ponatinib</p>
<p>Related National Policy</p>	<p>The NHS Long Term Plan, 2019. NHS Long Term Plan</p> <p>NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019) : Chapter 29</p> <p>Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 1,2 and 3. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017</p>

References

1. Hochhaus, A., Saussele S., Rosti G., Mahon F. X., Janssen J. J. W. M., Hjorth-Hansen H., et al. *Chronic myeloid leukaemia: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up*. *Annals of oncology : official journal of the European Society for Medical Oncology*. 2017;28(suppl_4):iv41-iv51. Available from: <https://10.1093/annonc/mdx219>
2. Leukaemia Foundation. *Chronic myeloid leukaemia (CML)*. 2021. Available from: <https://www.leukaemia.org.au/disease-information/leukaemias/chronic-myeloid-leukaemia/> [Accessed 16 April 2021].
3. Cancer Research UK. *Chronic myeloid leukaemia (CML): Stages*. 2020. Available from: <https://www.cancerresearchuk.org/about-cancer/chronic-myeloid-leukaemia-cml/stages> [Accessed 27 April 2021].
4. Cancer Research UK. *Chronic myeloid leukaemia (CML) incidence statistics*. Available from: <https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/leukaemia-cml/incidence> [Accessed 16 April 2021].