# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Health Technology Appraisal

## Moxetumomab pasudotox for hairy-cell leukaemia

## Draft scope

### Draft remit/appraisal objective

To appraise the clinical and cost effectiveness of moxetumomab pasudotox within its marketing authorisation for relapsed or refractory hairy-cell leukaemia.

### Background

Hairy cell leukaemia (HCL) is a cancer of the immune system which affects white blood cells known as lymphocytes. It causes anaemia, swollen lymph nodes, spleen enlargement, unexplained weight loss and increased susceptibility to infection. There is an excess number of lymphocytes in the circulating blood. These lymphocytes are abnormal and cannot help the body to defend against infections. They are called hairy cells because the cells have fine projections on the surface which look like hairs under the microscope.

HCL is rare, representing approximately 2% of adult leukaemia.[1] Around 210 people in the UK are diagnosed with HCL each year UK and it is more common in men. [2] The five-year relative survival with HCL is 89%.[2]

Treatment options for HCL depend on whether patients experience symptoms. There is no NICE guidance for the treatment of HCL. The European Society for Medical Oncology recommend purine analogues cladribine and pentostatin as first line treatment of HCL. If a partial response is achieved after the first round of therapy rituximab may be added for the second round of treatment [1]. Patients with relapsed HCL are re-treated with the alternative purine analogue used for initial treatment with or without rituximab (an anti-CD20 monoclonal antibody) or may receive interferon alpha or bendmustine.[1]

### The technology

Moxetumomab pasudotox (brand name unknown, AstraZeneca) is an anti-CD22 mouse monoclonal antibody fused with a toxin. It is administered intravenously.

Moxetumomab pasudotox does not have a marketing authorisation in the UK for HCL. It has been studied in clinical trials with treatment refractory HCL after 2 previous treatments with purine analogues (or at least 1 analogue and 1 either rituximab or BRAF inhibitor) in adults.

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| **Intervention(s)** | Moxetumomab pasudotox |
| **Population(s)** | Adults with relapsed or refractory HCL |
| **Comparators** | • Interferon alpha<br>• Bendamustine+rituximab |
| **Outcomes** | The outcome measures to be considered include:<br>• progression-free survival<br>• overall survival<br>• response rates<br>• adverse effects of treatment<br>• 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.<br>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.<br>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** | Related Technology Appraisals:<br>None.<br>Related Guidelines:<br>Haematological cancers: improving outcomes (2003). NICE guideline 47 Review date May 2016.<br>Related Quality Standards:<br>Haematological cancers (2017).<br>NICE quality standard 150.<br>Related NICE Pathways:<br>Blood and bone marrow cancers overview (2017) NICE pathway |

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| **Related National Policy** | NHS England Manual for prescribed specialised services 2017/18:<br>• Blood and marrow transplantation services (adults and children) [section 29, page 79]<br>• Specialist cancer services (adults) [section 105, page 234].<br><br>Department of Health, NHS Outcomes Framework 2016-2017 (published 2016): Domains 1-5. |

**Questions for consultation**

- At what stage of the treatment pathway would moxetumomab pasudotox be used?
- Is it intended to be used in people with relapsed or refractory HCL? How are relapsed and refractory defined?
- Which treatments are considered to be established clinical practice in the NHS for relapsed or refractory HCL? Is vemurafenib used in the NHS?
- Would moxetumomab pasudotox be used in adults with HCL who are not eligible for chemotherapy?
- Is best supportive case a relevant comparator and if it, how should best supportive care be defined?

Are the outcomes listed appropriate?

Are there any subgroups of people in whom moxetumomab pasudotox is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Where do you consider moxetumomab pasudotox will fit into the existing NICE pathway, Blood and bone marrow cancers overview ?

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 moxetumomab pasudotox 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 moxetumomab pasudotox 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 moxetumomab pasudotox 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.

To help NICE prioritise topics for additional adoption support, do you consider that there will be any barriers to adoption of this technology into practice? If yes, please describe briefly.

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).

**References**

1. Robak et al. 2015. Hairy cell leukaemia: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up. Annals of Oncology, Volume 26, Issue suppl 5.

2. Haematological Malignancy Research Network. Statistics - Survival. https://www.hmrn.org/statistics/survival Accessed November 2017.