Engagement Report

Topic details

Title of policy or policy statement: Vemurafenib plus rituximab for patients with relapsed or refractory classic hairy cell leukaemia (HCL) (Adults)

Programme of Care: Cancer

Clinical Reference Group: Chemotherapy

URN: 2318

1. Summary

This report summarises the feedback NHS England received from engagement during the development of this policy proposition, and how this feedback has been considered.

2. Background

Hairy cell leukaemia (HCL) is a very rare type of leukaemia (blood cancer). In patients with HCL there is an excess number of lymphocytes (a type of white blood cell) in the blood. These lymphocytes are abnormal and cannot help to defend the body against infection. Classic HCL is characterised by a mutation called BRAF V600E which is present in all leukaemic cells. This differentiates classic HCL from hairy cell leukaemia variant (HCL-V), which does not harbour BRAF mutations.

The first-line treatment for patients with classic HCL are cytotoxic drugs called purine nucleoside analogue (PA) therapy. A single PA therapy (either cladribine or pentostatin) is the current standard care. The median time after first-line treatment before relapse (cancer returns) is around 11 years (Cancer Research UK, 2022). However, there is a significant minority of patients who do not respond or become resistant (refractory) to PA therapy. Additionally, some patients will relapse sooner than others following first-line treatment with PA therapy. For patients who are refractory to first-line PA therapy, or who relapse within 2 years following PA therapy, standard second-line treatment is generally with an alternative PA therapy in combination with rituximab. Patients who relapse within 2-5 years following PA therapy, can be retreated with the initial PA therapy plus rituximab. Patients who relapse beyond 5 years from the end of initial treatment with PA therapy can be re-treated with the same, or an alternative, single agent PA therapy, plus or minus rituximab.

The proposed intervention is for vemurafenib plus rituximab in adult patients with classic HCL who are either a) refractory to first-line treatment with a PA therapy; or b) refractory to, or relapse following, treatment with a second-line PA therapy with or without rituximab; or c) for patients who are unsuitable for PA therapy at any stage.

3. Engagement

The Programme of Care has decided that the proposition offers a clear and positive impact on patient treatment, by potentially making a new treatment available which widens the range of treatment options without disrupting current care or limiting patient choice, and therefore further public consultation was not required. This decision has been assured by the Patient Public Voice Advisory Group.

The policy proposition underwent a two-week stakeholder testing between 7th and 22nd February 2024 to registered stakeholders from the following Clinical Reference Groups:

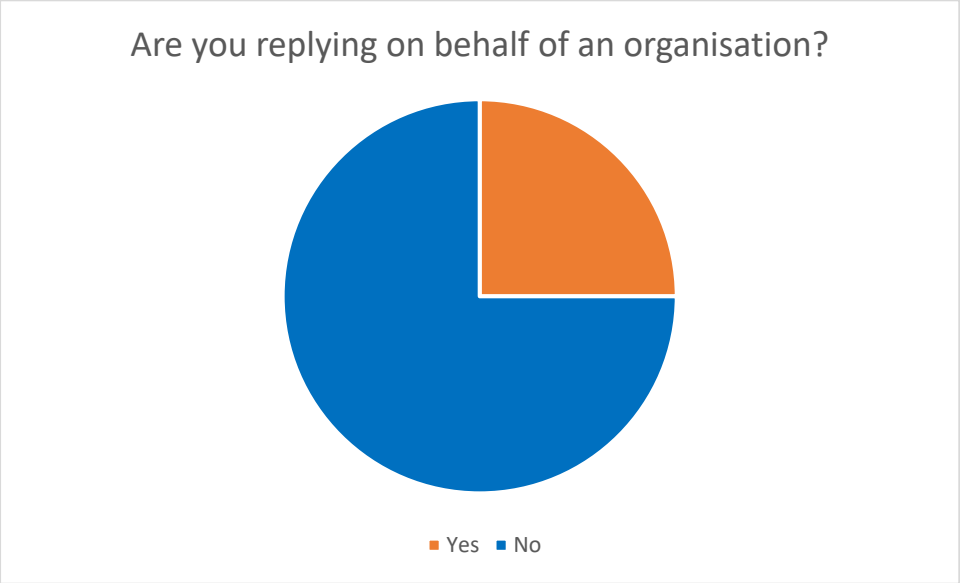
- Chemotherapy
- Radiotherapy
- Specialised blood disorders

Respondents were asked the following consultation questions:

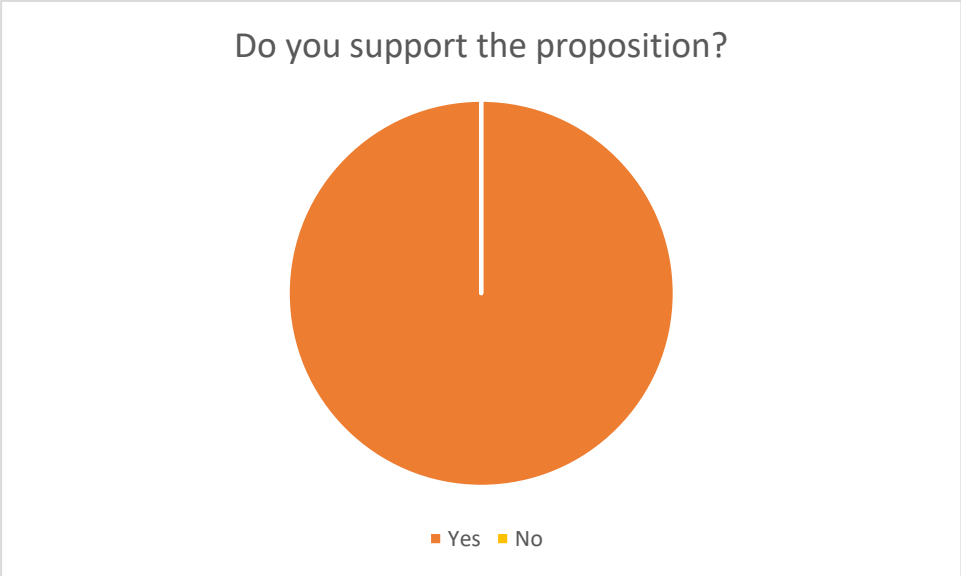
- Do you support the proposal that that vemurafenib plus rituximab will be routinely commissioned for patients with relapsed or refractory classic hairy cell leukaemia (HCL) based on the evidence review and the criteria set out in this document?
- Do you believe that there is any additional information that we should have considered?
- Do you believe that there is any additional information that we should have considered in the evidence review?
- Do you believe that there are any potential positive and/or negative impacts on patient care as a result of making this treatment option available?
- Do you support the Equalities and Health Inequalities Impact Assessment?
- Do you agree with the Patient Impact Assessment?
- Do you have any further comments on the policy proposal? If so, please submit these in under 500 words.

Engagement Results

In total, 4 respondents engaged with stakeholder testing for this proposition. This consisted of 3 individuals and one organisation. This consisted of two clinicians, one patient and one individual who did not categorise themselves.



All respondents were supportive of the policy proposition.



In line with the 13Q assessment it was deemed that further public consultation was not required.

4. How has feedback been considered?

Responses to engagement have been reviewed by the Policy Working Group and the (insert PoC) PoC. The following themes were raised during engagement:

Keys themes in feedback	NHS England Response
Relevant Evidence	
No additional relevant evidence with appropriate references was provided.	No further action required.
Patient Impact Assessment (PIA)	
Two respondents commented that they disagreed with the PIA. One respondent	Comment noted. The Patient Impact Assessment (PIA) is limited to 150

felt that the impact of current treatment should be emphasised with regards to the potential for self-isolation as a result of immunosuppression.	words and is intended to provide a brief summary that is broadly reflective of the experience of the patient cohort. An amendment has been added to the PIA to reflect burden of increased infection risk following immunosuppressive treatment: *Side effects of current treatment (e.g. low white cell count) may mean patients have to adhere to strict self-isolation to reduce infection risk.*
One respondent felt that the impact of relapsed or refractory HCL on mental health should be more strongly emphasised in the PIA.	Comment noted. The PIA is limited to 150 words and is intended to provide a brief summary that is broadly reflective of the experience of the patient cohort. The impact of relapsed or refractory disease on the patient cohort has been captured in the PIA. No further action.
Current Patient Pathway	
No stakeholders commented on the patient pathway.	No further action.
Potential impact on equality and health inequalities (EHIA)	
All respondents agreed with the EHIA and no additional comments were provided.	No further action required.
Changes/addition to policy	
One minor amendment was made to the PIA in line with stakeholder feedback. No changes were made to the policy proposition.	As noted above.

5. Has anything been changed in the policy proposition as a result of the stakeholder testing and consultation?

The following change(s) based on the engagement responses has (have) been made to the policy proposition and/or supporting documents:

Patient Impact Assessment (PIA)	
One respondent felt that the impact of current treatment should be emphasised with regards to the potential for self-isolation as a result of immunosuppression.	An amendment has been added to the PIA to reflect burden of increased infection risk following immunosuppressive treatment: *Side effects of current treatment (e.g. low white cell count) may mean patients have to adhere to strict self-isolation to reduce infection risk.*

6. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposition?

No.