Sent by e-mail onlyXXXXXXXXXXXXXXXXXXXXXXXX

FAO XXXXXXXX

Vitiligo Society

8 August 2024

Dear XXXXXXXXXX

**Re:** **Final Draft Guidance For Ruxolitinib Cream For Treating Non-Segmental Vitiligo In People 12 Years And Over [ID 3998]**

Thank you for your undated letter sent by email on 1 August 2024, lodging an appeal against the above Final Draft Guidance (FDG).

Introduction

The Institute's appeal procedures provide for an initial scrutiny of points that an appellant wishes to raise, to provide an initial view on whether they are within the permitted grounds of appeal ("valid") and are at least arguable. The permitted grounds of appeal are:

* 1(a) NICE has failed to act fairly, or
* 1(b) NICE has exceeded powers;
* (2) the recommendation is unreasonable in the light of the evidence submitted to NICE.

This letter sets out my initial view of the points of appeal you have raised: principally whether they fall within any of the grounds of appeal, or whether further clarification is required of any point. Only if I am satisfied that your points contain the necessary information, are arguable, and fall within any one of the grounds will your appeal be referred to the Appeal Panel.

You have the opportunity to comment on this letter in order to elaborate on or clarify any of the points raised before I will make my final decision as to whether each appeal point should be referred on to the Appeal Panel.

Initial View

I assess each of your points in turn.

***Ground 1(a): In making the assessment that preceded the recommendation, NICE has failed to act fairly***

**Appeal point 1(a).1: NICE failed to act fairly by declining all expert nominations from The Vitiligo Society and therefore excluding representation from the committee meetings**

I am minded to refer this appeal point to the Appeal Panel. While NICE is not bound as a matter of procedural fairness to appoint all nominated experts or to ensure a spread of representation across all stakeholders, I consider there is an arguable point for an Appeal Panel to explore how Vitiligo Society's nomination(s) might have contributed to the decision-making process and whether expert selection was unfair in this case.

**Appeal point 1(a).2: NICE failed to act fairly by giving notice of only 3 days to make a written response to the Phase 1 scoping consultation**

I am minded to refer this appeal point to the Appeal Panel. By "Phase 1 scoping consultation" I understand that you mean the consultation on the scope of the appraisal. I anticipate the Panel will wish to explore the extent and impact of the delay and whether and how this caused any unfairness.

**Appeal point 1(a).3: NICE failed to act fairly by giving notice of only 13 days to make a Phase 2 written submission**

I am minded to refer this appeal point to the Appeal Panel. By "Phase 2 written submission" I understand you mean Vitiligo Society's response to consultation on the appraisal consultation document. I anticipate the Panel will wish to explore NICE's usual notice periods, the impact of any delay and whether and how this caused any unfairness.

***Ground 2: the recommendation is unreasonable in the light of the evidence submitted to NICE***

**Appeal point 2.1: The recommendation is unreasonable in the light of the evidence submitted to NICE about the use of phototherapy in current clinical care for vitiligo**

I am not minded to refer this appeal point to the Appeal Panel. I consider your point makes three main arguments to which I respond below.

1. I understand the first four paragraphs of your appeal point to argue that it was unreasonable, in light of the evidence put to it, for the committee to conclude at para 3.5 that it considered "phototherapy (with or without topical treatments) to be a relevant comparator (see section 3.4)". The Committee's reasoning for that conclusion is detailed in para 3.4 of the FDG, including in particular:

"The committee agreed that the appraisal should consider the clinical effectiveness of phototherapy and if ruxolitinib cream would displace the treatment or move it further down the pathway if it was available in clinical practice. It concluded that, because ruxolitinib cream is proposed to be prescribed in secondary care and likely to be more effective than phototherapy (see section 3.6), ruxolitinib cream would effectively create a new position in the specialist treatment pathway before phototherapy. So, a comparison with no active treatment followed by some people having phototherapy would be most reflective of what ruxolitinib cream would displace in clinical practice."

Your appeal expresses concerns about the limited use of phototherapy, the limited benefits associated with it, insufficient exploration of its use by particularly vulnerable groups (for whom you consider phototherapy is harder to access) and insufficient exploration of links between phototherapy and cancer that may preclude its use. You do not, however, point to evidence that was submitted to the Committee on these issues that arguably renders its conclusion that phototherapy was "a" relevant comparator unreasonable.

I am mindful that the consequence of the Committee's conclusion was the preparation and consideration of an indirect comparative study, and that para 3.7 of the FDG is clear that ultimately the Committee concluded "comparison of no active treatment followed by phototherapy is most representative of the positioning of ruxolitinib cream". It is therefore not the case that the "inadequacy" or limited benefit (or indeed costs) of phototherapy was a main driver for the conclusion that Ruxolitinib is not cost-effective, which I understand to be the essence of your argument (see paragraph 4 of your appeal under this point). I therefore see no arguable unreasonableness point here.

1. The fifth paragraph of your appeal point argues that it was unreasonable that the committee did not do more to consider the extent to which uncaptured benefits of Ruxolitinib might help address some of the perceived uncertainties in Health-Related Quality of Life estimates. I note the FDG refers briefly to uncaptured benefits at para 3.22. Your appeal letter does not provide sufficient information to persuade me that there is an arguable case that the Committee's recommendation or any particular conclusion is unreasonable on the evidence submitted to it. If you wish to pursue this point I invite you to develop your arguments in your response to this letter.
2. I understand the sixth paragraph of your appeal point to argue that it was unreasonable for the Committee to "consider improvement in facial vitiligo as the primary outcome for Ruxolitinib as a flaw", whereas you would argue that this is "an appropriate endpoint to capture the outcome most important to patients.".

I understand this as a challenge to the reasonableness of the statement at para 3.5 (Clinical effectiveness evidence) of the FDG that the Committee:

"considered that improvements in F-VASI did not necessarily correspond directly to the quality of life of people with vitiligo, because changes in F-VASI did not always correlate with changes to T-VASI."

It seems to me that it was reasonable for the Committee to treat this as a limitation to the clinical trial data (and indeed the economic model in the context of a technology appraisal that was considering costs and benefits of the treatment for use on facial and non-facial areas: I refer you to the committee's detailed discussion of this aspect of the model in the papers (see slides 24-27 of the public committee slides[[1]](#footnote-1) and pages 172-175 of the Final Draft Guidance Committee Papers[[2]](#footnote-2)) and paragraphs 3.8-3.10 of the FDG.

Conclusion

The above sets out above my initial views on all of your appeal points.

In respect of your points which I am not minded to refer on you are entitled to submit further clarification and/or evidence to me within the next 10 working days, and I will then give a final decision on the points to put before an appeal panel. Responses must deal only with requested clarifications, or arguments or comments about the lead non-executive director for appeals' initial view that an appeal point is not valid. For the points I am already content to refer on, an oral appeal will be held remotely.

Once I have made my final decision, and where there is more than one appellant, each appellant will receive the valid appeal points of the other appellants and their redacted appeal letter. This is to enable appellants to avoid duplication at the hearing where there are overlapping appeal points. If the appeal letter and/or responses to scrutiny contain confidential information please ensure you have provided a version with this information redacted by 30 August 2024.

Ordinarily appeals are conducted on the basis of the appellants’ written appeal letters, and the material generated during the appraisal process. Use of additional written material is discouraged, and the panel cannot receive any new evidence. If, exceptionally, you feel there is written material that will not be before the panel that you would wish to rely on you must let the NICE Appeal team know by return of letter, indicating what the material is, why it is desirable to submit it, and when it will be available, by no later than 20 September 2024. Please note that the appeal panel cannot accept papers that are tabled late or ad hoc, as this affects the preparation of the panel and other parties for the appeal.

Yours sincerely

XXXXXXXXXXXXX

Dr Mark Chakravarty

Lead Non-Executive Director for Appeals & Vice Chairman

National Institute for Health and Care Excellence

1. <https://www.nice.org.uk/guidance/gid-ta10893/documents/1-2> [↑](#footnote-ref-1)
2. <https://www.nice.org.uk/guidance/gid-ta10893/documents/committee-papers-2> [↑](#footnote-ref-2)