Sent by e-mail only: XXXXXXXXXXXXXXXXXXX

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Sickle Cell Society

54 Station Road

London

NW10 4UA

18 August 2023

Dear XXXXXXXXXXX

**Re: Final Appraisal Document — Voxelotor for treating haemolytic anaemia caused by sickle cell disorder (SCD) (ID1403)**

Thank you for your letter of 8 August 2023, lodging an appeal against the above Final Draft Guidance (FDG). In light of your request that your letter be treated as an appeal should NICE be unable to respond by 5pm on 11 August 2023, your letter has been passed to me as the lead non-executive director for appeals, in accordance with NICE's [*Guide to the technology appraisal and highly specialised technology appeal process*](https://www.nice.org.uk/process/pmg41/resources/guide-to-the-technology-appraisal-and-highly-specialised-technologies-appeal-process-pdf-72286831312837) (the "[Appeal Process Guide](https://www.nice.org.uk/process/pmg41/chapter/making-an-appeal)") [PMG41S].

Introduction

The Appeal Process Guide sets out what an appeal letter must contain. In particular, at 4.6:

*"The appeal letter must clearly and concisely set out the appellant's points of appeal in detail. Appeal points must be made in order of the ground to which they relate, that is, all appeal points made under ground 1(a) must be made first, followed by all points made under ground 1(b) and then all appeal points made under ground 2. Appeal letters must provide enough information for the appeal panel to understand all points being raised.*

*Each point must be headed with the appeal ground to which it relates, and a 1‑sentence description of the appeal point. For example, 'Ground 1(a).1: the change from a positive to negative recommendation following draft guidance without further consultation is unfair'.*

*Each appeal point must be numbered so that the first appeal point under ground 1(a) is numbered 1(a).1 with subsequent ground 1(a) points numbered 1(a).2, 1(a).3, and so on. The same numbering will apply for ground 1(b) and ground 2 points, for example: 1(b).1, 1(b).2, 2.1 and so on.*

*…*

*In summary the appeal letter must include the following information:*

* *the ground(s) of appeal*
* *the aspect(s) of the final draft guidance, or technology appraisal or highly specialised technologies evaluation process, being appealed against*
* *the reasons why the aspect(s) of the final draft guidance, or technology appraisal or highly specialised technologies evaluation process, being appealed against fall within the specified ground(s) of appeal, in enough detail to demonstrate an arguable case*
* *the concluding statement indicating whether the appellant wishes to be heard at an oral or written appeal.*

On receipt of an appeal letter, the Institute's appeal procedures provide for me to carry out 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.

To the extent possible I have indicated what I consider your specific appeal points to be and under what ground they may fall. However, it is imperative that you reply to this letter to confirm the wording of any appeal appoints that I am minded to refer to the Appeal Panel.

Within your response you also 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.

1. **"We believe NICE have not acted fairly during the process of the appraisals."**

I have identified three potential appeal points under your above heading, all of which appear to me most likely to be brought under ground 1(a) ("In making the assessment that preceded the recommendation, NICE has failed to act fairly"). I provide my initial response below with proposed numbering and wording for each point:

**Your first point:** **The Sickle Cell Society had nominated two patient representatives for the first appraisal in December 2022. One of those patients was an individual who had been on the clinical trial. NICE declined this nomination, without any communication to the Society. The first we became aware of this was when the individual concerned told us that she had been stood down by NICE**

I am minded to refer this appeal point to the Appeal Panel as follows: "Appeal point 1(a).1 The Committee has acted unfairly by declining the nomination of a patient representative, without any communication to the Society that their nomination had been declined."

**Your second point:** **NICE decided that the second appraisal meeting in June 2023 would only have patient and clinical representatives as observers**

I am minded to refer this appeal point to the Appeal Panel as follows: "Appeal point 1(a).2 The Committee has acted unfairly by including patient and clinical experts in the second appraisal committee meeting only as observers, which meant they were unable to contribute to the meeting."

**Your third point: You had no audio throughout the entire meeting which was hugely unhelpful and frustrating**

I can see no arguable appeal point here. This might if anything support a ground 1(a) argument of procedural unfairness, but I can see no unfairness arising from a technical issue with the audio of a member of public observing a Committee meeting. I am however minded to refer your argument about the participation of patient and clinical experts (see proposed point 1(a).2 above).

**2. "Health inequalities"**

I am not minded to refer this appeal point to the Appeal Panel

I understand this appeal point disputes the Committee's Equality Impact Assessment but it is unclear what impact you say this had and what part of the FDG or way the evaluation was done you challenge and on what grounds. I note that the Committee did expressly take into account health inequalities: this is evident from the Committee meeting slides, responses to consultation and in various places in the FDG, including in particular the summary on page 2:

*"Voxelotor has the potential to address the health inequalities associated with sickle cell disease and the unmet need for effective treatments, so a higher cost effectiveness estimate could be accepted for decision making. But, the estimates for the company’s proposed second line positioning were extremely uncertain. Any estimate that could be considered sufficiently reliable for decision making would likely be above what NICE considers an acceptable use of NHS resources."*

If you wish to pursue this appeal point I invite you to respond to this letter with clarification as to why you consider the Committee's approach to health inequalities was procedurally unfair (ground 1(a)), exceeded NICE's powers (ground 1(b)) or was unreasonable in light of the evidence submitted to NICE (ground 2).

**3. "Why the Committee made these recommendations"**

I understand your argument to be that the Committee has provided inadequate reasoning for its recommendations in consideration of the position taken by the FDA and EMA, who you refer to as "NICE's peers in the USA and Europe".

I do not consider this to be a valid appeal point.

NICE conducts appraisals with the remit of assessing the evidence for both the clinical and cost-effectiveness of a technology for use within the NHS. The organisations to which you refer do not operate under the same remit but rather (at very high level) are tasked with ensuring medicines meet applicable standards of safety, quality and efficacy (effectiveness) for human use. In the UK this role is provided by the Medicines and Healthcare products Regulatory Agency ("MHRA").

This means that NICE, in considering the cost-effectiveness of a technology, works within different parameters to these other organisations and properly applies different assessment criteria and asks itself different questions. I therefore do not consider there was a "difference between NICE's position and the position of [the FDA and MDA]"; however, I will of course consider any further clarification you wisht to submit in response to this letter.

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. For the points I am already content to refer on, an oral appeal will be held which is likely to 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 1 September 2023.

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 4 September 2023. 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

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Dr Mark Chakravarty

Lead Non-Executive Director for Appeals & Vice Chairman

National Institute for Health and Care Excellence