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30 August 2023

Dear Dr Mark Chakravarty,

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# Re- Final Appraisal Document – Voxelotor for treating hemolytic anaemia caused by sickle cell disorder (SCD) (ID1403)

Thank you for your letter dated 18 August setting out your initial scrutiny of the representations included in my letter dated 08 August.

# 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.

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

I am pleased that you are minded to refer this appeal point to the Appeals Panel.

In my letter of 08 August, I made the point that the patient representative who had been declined was an individual who had taken part in the clinical trial. This is an important level of detail that should be shared with the Appeals Panel. NICE did not hear from any patient who had participated in the clinical trial.

# 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.

Regarding your second point, NICE decided that the second Appraisal meeting in June 2023 would only have patient and clinical experts as observers.

I am pleased that you are minded to refer this appeal point to the Appeal Panel.

# Your third point: You had no audio throughout the entire meeting which was hugely frustrating.

I disagree with your initial view that you see no arguable point here. First, it is factually correct that I had no audio. You should take this point in the round of 1(a)1 and 1(a)2. By this I mean this was part of the Appraisal process. Thus, this point should not be disassociated from the previous two points, which in the round give a picture of the process. In my view, the unfairness relates to the fact that I was disadvantaged in assimilating, analysing and fully understanding the debate, to inform what if any, next steps were necessary from our charity.

# Health Inequalities

I am disappointed and somewhat concerned that you are not minded to refer this appeal point to the Appeal Panel.

I confirm that I am disputing No4 of the NICE Equality Impact Assessment. You have not provided any rationale as to why this dispute on our part is wrong or misplaced. I made clear in my letter of 08 August that the impact is associated with high unmet need in SCD and limited access to new and safe effective disease modifying treatments for SCD. In addition, SCD predominantly affects black, Asian and mixed- race people in the UK.

The contradiction is that you say that the Committee expressly took into account health inequalities, but the Impact Assessment does not, in our view, support that argument. In addition, you have not commented on what weight, if any, was attached to this, in the decision making. Frankly, it is easy to say that it was taken into account, but we found it impossible to discern what weight was attached.

Your statement/sentence is as follows.

“Any estimate that could be considered sufficiently reliable for decision making

would likely be above what NICE considers an acceptable use of NHS resources.”

Why this is a valid point is that this sentence infers that the main reason behind the decision is whether Voxelotor represents value for money for the NHS. If that is the case, then it is important that NICE are transparent about what weight, if any, was given to health inequalities beyond stating that the Committee took it into account.

# Why the Committee made these recommendations.

We are fully aware that it is NICE’s remit and indeed that of the MHRA.

It is really important that when communicating its decision, NICE should do so clearly and without ambiguity, particularly to its patient stakeholder communities.

If the decision of NICE is clearly one about value for money, it should say so clearly, rather than provide a response that is open to interpretation.

I hope these additional points are helpful to answer your questions. Yours sincerely,

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XXXXXXXXX, Sickle Cell Society