National Institute for Health and Care Excellence 2nd Floor

2 Redman Place London

E20 1JQ

08 August 2023

Dear XXXXXXXXXXXXXX and TA Team5,

SICKLE CELL SOCIETY

54 Station Road London NW10 4UA

T 020 8961 7795/4006

F 020 8961 8346

E [info@sicklecellsociety.org](mailto:info@sicklecellsociety.org) W [www.sicklecellsociety.org](http://www.sicklecellsociety.org/) Social Media @SickleCellUK

# Re -Voxelotor for treating haemolytic anaemia caused by sickle cell disorder (SCD) (ID1403) - Final Draft Guidance

I am contacting you to follow up our exchange of emails on 27 and 28 June regarding the final draft guidance for the above disease modifying treatment for SCD.

The Sickle Cell Society is now making representations about the NICE decision as well as the decision- making process. We suspect that it is highly unlikely that you will be able to respond to these representations by 5pm on Friday 11 August 2023. If that is the case, you should therefore treat these representations as an appeal.

Our representations are based on the following points,

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

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.

Furthermore, NICE decided that the second appraisal meeting in June 2023 would only have patient and clinical representatives as observers. Unfortunately, in my case I had no audio throughout the entire meeting which was hugely unhelpful and frustrating.

The two examples above make us question the fairness of the process. In our view not hearing from a patient for whom Voxelotor has made a significant positive difference to her quality of life, is a serious omission in the appraisal process.

If as NICE has stated in its decision-making process there are uncertainties about various aspects of Voxelotor, then surely NICE should be enabling more discussion from patients and clinicians about the matter to help inform a more inclusive assessment rather than restricting the second appraisal discussion to only the NICE committee members. It is also not clear why NICE was not able to work with the company Pfizer to resolve any uncertainties throughout the process.

# P:\Common Area\Dropbox\INFORMATION STD PRODUCTS 1\Branding and logos\IS Logo's 2013\information-standard-member-logo-positive_full.jpgP:\New Common Area\Communications & Media\Matthew Neal\IIP Acreditation\Standard - Accredited to 2020\PNG\IIP_ACCRED_LOGO_2020_CMYK.pngP:\New Common Area\Communications & Media\Matthew Neal\GSK and Kings Fund Award\GSK_IMPACT_2018_winner_RGB.jpgHealth Inequalities

No 4 of the NICE Equality Impact Assessment (Do the preliminary recommendations make it more difficult in practice for a specific group to access the technology compared to other groups? If so, what are the barriers to, or difficulties with, access for a specific group) is marked not applicable. In our view the answer to this question is yes, particularly since the same impact assessment document states clearly that there is high unmet need in the SCD community and limited access to new safe, effective treatments for SCD.

We therefore do not understand this contradiction, nor do we believe that sufficient weight has been given to the health inequalities which affect the SCD community, in this decision- making process.

# Why the Committee made these recommendations.

In my 27 June email, we made the point that any NICE decision should be made clear and unambiguous, particularly having regard to the fact that Voxelotor is licensed by the FDA in the USA and EMA in Europe.

We do not believe, from a patient advocacy perspective, that the reasons why the committee made its decision are clear and unambiguous or addressed the difference between NICE’s decision and the position of your peers in the USA and Europe. The inference is that your methods and processes are superior to those of other countries. Is that correct?

Whilst we accept there is a balance to be struck to meet the communication needs of all stakeholders involved with this process, it is not easy to explain this point to the SCD community, other than NICE does not believe Voxeletor represents value for money for the NHS and the taxpayer. Is that also, correct?

We look forward to hearing from you. Yours sincerely,

XXXXXXXXXXXXXXXX

XXXXXXXXXXXXXXX

XXXXXXXXXXXX