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10 August 2023

National Institute for Health and Care Excellence (NICE) 2nd Floor, 2 Redman Place

London E20 1JQ

Dear Sir/Madam

# Re: 2nd Appraisal Committee Meeting about Voxelotor for treating haemolytic anaemia in people with sickle cell disease

*Re NICE RECOMMENDATION: ''Voxelotor is not recommended, within its marketing authorisation, for treating haemolytic anaemia caused by sickle cell disease, with or without hydroxycarbamide, in people 12 years and older. ‘'*

The **National Haemoglobinopathy Pane**l would like to appeal against the negative NICE appraisal decision for the above-mentioned technology appraisal on the following grounds:

The NICE committee is undoubtedly aware of the limited treatment options for sickle cell patients, resulting of decades of inadequate investment in research and development for this disease, at a clinical trials level. The only non- curative treatments available in the UK which can ameliorate vaso-occlusive crises (VOC) are hydroxyurea (hydroxycarbamide) and blood transfusion. Benefits from hydroxyurea are partial and unpredictable and some patients cannot be transfused. Until very recently, there were no trials on new non-curative therapies for sickle cell disorders (SCD).

So it is especially important that if clinical trials show efficacy and safety for SCD, that this highly disadvantaged patient group benefit in the UK, otherwise the incentive for new investment will be harmed and patients will choose not to participate in future clinical trials in the UK.

We fear that drugs that pass the licensing criteria for treatment of haemoglobinopathies in the UK and have been made available in other countries are not receiving fair appraisal by NICE, to the clinical detriment of this highly disadvantaged patient group. This causes us ethical concerns: if patients with haemoglobin disorders are asked to participate in clinical trials in the UK (at their own risk and inconvenience) and that if treatments are then approved by licensing authorities, there should be a reasonable prospect of treatment becoming available to NHS patients. This is often stated explicitly by ethical committees for clinical trials.

When patients in the UK are asked to participate in international drug development trials, ethics approval in the UK for patients to participate often demands that treatment will be made available to trial participants of the drug passes licensing by MHRA and until such time as it

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becomes available on the NHS. We are concerned that this is repeatedly not happening in the UK. For example several new treatments for haemoglobin disorders have passed the licensing step either internationally and in the UK, but have not been made available in the UK to NHS patients e.g. Gene therapy for thalassaemia, (Bluebird Bio), Crizanlizumab for sickle cell disease (Novartis), Luspatercept for thalassaemia (BMS).

With specific respect to Voxelotor, we and others who treat sickle cell disorders have seen first- hand the benefits to individuals treated with Voxelotor where no alternative treatments are available. This is particularly the case in severely anaemic patients who cannot be transfused (because transfused blood causes severe or fatal transfusion reactions) and where life threatening anaemia cannot therefore be corrected. We urge that NICE and drug sponsors Pfizer work together to recognise patient subgroups that we believe clearly benefit from Voxelotor and, in particular, to analyse already existing data on non-transfused or untransfusable anaemias with this specific question in mind.

# In summary, the NHP strongly recommends that the use of Voxelotor goes before a third NICE panel with the specific aim of identifying more focused indications for its use and with particular emphasis of severe and life threatening anaemia in un-transfusable patients.

Your Sincerely

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| ***XXXXXXXXXXXXXXXXXX***XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX, Guy’s & St Thomas’ HospitalXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX[www.scorecharity.com](http://www.scorecharity.com/)ARISE – African Research and Innovative Initiative for Sickle cell Education: Improving ResearchCapacity for Service Improvement (ARISE) [www.ariseinitiative.org](http://www.ariseinitiative.org/) | ***XXXXXXXXXXXXXXXX******XXXXXXXXXXXX******XXXXXXXXXX***XXXXXXXXXXXXX University College London Hospitals | ***XXXXXXXXXXXXXXX******XXXXXXXXXXXXXXX***University College London Hospitals |

***On Behalf of the UK National Haemoglobinopathy Panel (NHP)***

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