**Crizanlizumab (Adakveo®) Patient Information**

**Why have I been given this information?**

You have been given this information sheet because you have been offered treatment with crizanlizumab. This is a new medicine available for the prevention of recurrent vaso-occlusive crises caused by Sickle Cell Disease (SCD) in patients aged 16 years and older. This information is designed to supplement the patient support pack provided by Novartis (the pharmaceutical company that makes crizanlizumab). If you do not have a crizanlizumab support pack, your Haemoglobinopathy team will be able to provide one.

**Why have I been offered crizanlizumab?**

Clinical trials have shown that people receiving crizanlizumab have fewer vaso-occlusive crises in a year than people who are just receiving best supportive care. This is in patients who take hydroxycarbamide and those who don’t. Based on your current treatment and the number of crises you have within a year, your specialist team think you may benefit from receiving crizanlizumab.

**How did crizanlizumab become available?**

All new medicines in the UK must be assessed for their value for money before they become available to prescribe within the NHS. This is done by an organisation called NICE (National Institute for Health and Care Excellence). NICE have reviewed crizanlizumab and decided that it can only be prescribed under certain conditions. This is known as a “Managed Access Agreement” (MAA). This is because the clinical trial that showed crizanlizumab is safe and effective only included a small number of patients and had quite a short duration so the long-term benefits are uncertain. NICE would like to gather more information from the MAA and another clinical trial called STAND. Information from this trial will be available in 2023. NICE will then re-assess the available data and make a decision on whether they think it is suitable for long-term use in the NHS. If they decide it is not suitable, patients will no longer be able to receive crizanlizumab even if they already started treatment in the NHS (unless they are able to fund it themselves).

In addition to the MAA, NHS England has stated that patients are not allowed to receive crizanlizumab if they are already receiving regular blood transfusions.

**What should I expect from my Haemoglobinopathy team?**

If you think that crizanlizumab may be a good option for you, your specialist team will discuss your case with their colleagues from within the region. This is called a Multi-Disciplinary Team (MDT). The MDT meet on a regular basis to discuss patients and make sure they are being cared for appropriately. Your personal details will be anonymised. MDT approval to prescribe crizanlizumab is mandatory according to the MAA and NHS England. If you are approved, your specialist team will prescribe crizanlizumab and it will be administered in the hospital. They will also monitor how well the medicine is working and if you have any side effects.

**What is expected of me as the patient?**

Since crizanlizumab is an expensive medicine, we expect patients to turn up to every treatment appointment and at the correct time. If you are unable to attend an appointment, please let your team know as soon as you can. Many hospitals will prepare the medicine before your appointment so that it is ready for you when you arrive. Every patient will receive a different dose based on their weight. If you miss an appointment, your dose cannot be used for somebody else and will have to be put in the bin. If you miss your appointments for crizanlizumab without a reasonable explanation, your team may decide to discontinue treatment.

Once your infusion has finished, we expect you to remain in the treatment area for one hour after your first two infusions. This is because some people may have a reaction to the crizanlizumab e.g. a rash or feeling sick. If no reaction occurs, you will have to stay for 30 minutes after future doses.

If you experience any side effects after you have left the hospital or in between appointments, you must contact your team to let them know. This is because crizanlizumab is a new drug and all side effects must be reported to a national body called the Medicines and Healthcare products Regulatory Agency (MHRA).

**NHS England and NICE expectations of the Haemoglobinopathy team**

Both of these organisations expect every hospital offering crizanlizumab to meet the terms of the MAA. This means making sure patients meet all of the eligibility criteria, are discussed and approved for treatment by the MDT and details of treatment entered on to the National Haemoglobinopathy Registry.

**I have some questions about crizanlizumab, who can I ask?**

Please discuss your treatment with your specialist team who will be able to provide further guidance.