THIS INFORMATION IS FOR PATIENTS WITH SICKLE CELL DISEASE IN GREAT BRITAIN.
This information leaflet was developed and funded by Pfizer Ltd.

Information for patients about the medicine Oxbryta ▼(voxelotor), used to treat Sickle Cell Disease

Dear Patient,

We are writing to highlight how deeply saddened we are about the recent need to withdraw Oxbryta (voxelotor) from Great Britain.

This was an extremely difficult decision made in the interest of your safety and that of the wider sickle cell community. Patient safety drives everything we do and is our highest priority.

We have heard directly from the sickle cell community about the challenges of living with sickle cell disease and are very aware of the impact this decision has had.

Please find enclosed further information around the withdrawal.

Warmest regards,

UK Pfizer Team

Reporting of side effects

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See below for how to report side effects.

If you get any side effects, talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. You can help by reporting any side effects you may get. See https://yellowcard.mhra.gov.uk for how to report side effects.

By reporting side effects, you can help provide more information on the safety of this medicine.

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Why am I being contacted about Oxbryta (voxelotor)?

You are being contacted to provide you with information regarding the sudden withdrawal of this medicine, which has been prescribed to you by your healthcare professional as part of the management of your condition.

Oxbryta (voxelotor) has been withdrawn by the makers, Pfizer. This means it will no longer be available treatment for your condition.

Why was I being prescribed this medicine?

Your healthcare professional will have discussed with you the reason why you were prescribed this medicine.

Oxbryta is used to treat haemolytic anaemia due to sickle cell disease in adults and children from 12 years.

Why is Oxbryta (voxelotor) no longer available?

After medicines are approved for use ('licensed') in patients, scientists continue to research the benefits and risks of the new medicine to ensure they work as expected and to monitor any side effects.

New safety information from this ongoing research into Oxbryta (voxelotor) has alerted scientists to potential safety risks. These potential risks were not seen in previous research that supported the approved use ('licence') of the medicine in 2022. This new information suggests that the risks of taking the medicine are higher than originally thought.

Because of this, all supplies of Oxbryta (voxelotor) Tablets are being recalled. This means you can no longer be prescribed this medicine, and it is no longer available in pharmacies. This was a necessary decision due to these safety concerns, but one that the team at Pfizer are deeply saddened to make, recognising it represents a significant setback for the sickle cell community in need of new treatment options. We understand that this news may come as a surprise to the entire community and that safeguarding patients remains the highest priority.

What were the safety risks?

The new safety concerns show an increase in vaso-occlusive crises (acute painful episodes) and fatalities (deaths) following treatment with Oxbryta (voxelotor). Given that patient safety is of utmost importance, we acted quickly to withdraw the medicine whilst we investigate these studies further. Information related to further in-depth analysis of the studies will follow when available.

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What is meant by benefit-risk profile?

The Medicines and Healthcare products Regulatory Agency (MHRA) is responsible for approving the use of medicines in the United Kingdom. This is called 'licensing'. When licensing medicines, the MHRA looks at the benefits patients have by taking the medicine, and risks that might be caused by taking the medicine.

After licensing, pharmaceutical companies and the MHRA continue to monitor the safety of a medicine. The new safety concerns with Oxbryta (voxelotor) suggest that its benefits no longer outweigh the risks.

Before the MHRA issues a licence for a medicine, it considers all the data at that time to decide whether the benefits of the medicine outweigh the risks. Following licensing, the pharmaceutical company (like Pfizer) and the MHRA continue to monitor safety. For some medicines, such as newer medicines, or medicines that contain a new active substance (as is the case for Oxbryta), additional safety monitoring may be required, and these medicines can be identified with an inverted black equilateral triangle (▼). The new safety concerns with Oxbryta (voxelotor) suggest that its benefits no longer outweigh the risks.

What should I do if I am currently taking Oxbryta (voxelotor)?

Your healthcare professional has most likely reached out to discuss discontinuing this medicine; if not, you should contact your healthcare provider without delay. They will advise you about how best to manage your condition.

Will I have withdrawal symptoms if I stop taking Oxbryta (voxelotor)?

It is important to discuss this with your healthcare provider, who can review your individual clinical situation and discuss what this means for you.

Are there any alternative medications available?

Your healthcare provider will be able to advise you. They can advise on how best to manage your condition going forward based on your specific health needs and medical history.

Will I be able to take Oxbryta (voxelotor) in the future?

Pfizer and regulatory authorities like the MHRA are reviewing the data so that it can better understand and decide how to proceed. More information will be shared when possible.

What should I do with any Oxbryta (voxelotor) I have left over?

Please take the Oxbryta bottle with any unused tablets back to the pharmacy you got it from.

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My health was good when I was taking this medicine. Why can't I make the choice to continue?

Pfizer recognises that this is a significant setback for the Sickle Cell Community and we are saddened by the need to take this step. Patient safety is our highest priority.

After looking at new information showing increased risks for patients using this medicine, Pfizer has made the difficult decision to withdraw it to keep patients safe. We understand that this is disappointing for those who have seen benefits from the medicine. Pharmaceutical companies like Pfizer follow strict legal, medical, and ethical regulations to ensure patient safety is not compromised. If there are safety concerns about a medicine, we will work alongside the relevant regulatory authorities to determine what is best in the interest of patient safety. In the case of Oxbryta, Pfizer determined that stopping the supply of the medicine would be the most appropriate way to protect patients from these safety concerns.

If the medicine is not safe, why have I been allowed to take it up until now?

Before a medicine is approved for use ('licensed'), there is a long period of testing and research to gather information to assess if the medicine works and what side effects it may cause. For Oxbryta, this information was reviewed by experts from the UK regulator (MHRA) who determined in 2022 the medicine met quality and safety standards needed to be approved for use in patients in the UK. The benefit-risk evaluation for Oxbryta at that time was positive as assessed by Pfizer, the MHRA and all the regulators in countries where the medicine has been licensed. At that time the original clinical trials that led to the licensing did not show an increase in vaso-occlusive crisis (acute painful episodes) or fatalities (deaths). However, even once a medication is approved for use, the research does not stop. Pharmaceutical companies remain responsible for monitoring the drug's performance in the real world and further clinical trials may also be conducted.

With Oxbryta, Pfizer has continued to conduct clinical trials and collect real world data on the effectiveness and safety during real world use outside clinical trials. It is this more recent data that led to a safety concern which was not there before and given that patient safety is paramount we have had to act quickly.

At this point in time we do not know why the new data is not consistent with the prior data, and why the benefit-risk ratio has changed. The company will be conducting comprehensive analyses alongside the regulatory authorities to understand these findings. However, whilst this is happening, the necessary course of action, in the interest of patient safety, is to withdraw the medicine.

Who should I contact for further information?

You should contact your healthcare provider for any specific questions regarding your care. For queries about Oxbryta (voxelotor) , you can also contact Pfizer Medical Information on www.pfizermedicalinformation.co.uk or telephone 01304 616161.

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