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Pfizer Voluntarily Withdraws All Lots of Sickle Cell Disease Treatment OXBRYTA® (voxelotor) From Worldwide Markets

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NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) announced today that it is voluntarily withdrawing all lots of OXBRYTA ® (voxelotor) for the treatment of sickle cell disease (SCD) at this time, in all markets where it is approved. Pfizer is also discontinuing all active voxelotor clinical trials and expanded access programs worldwide.

Pfizer's decision is based on the totality of clinical data that now indicates the overall benefit of OXBRYTA no longer outweighs the risk in the approved sickle cell patient population. The data suggest an imbalance in vaso-occlusive crises and fatal events which require further assessment. Pfizer has notified regulatory authorities about these findings and its decision to voluntarily withdraw OXBRYTA from the market and discontinue distribution and clinical studies while further reviewing the available data and investigating the findings.

“The safety and well-being of patients is of the utmost importance to Pfizer, and we believe this action is in the best interest of patients,” said Aida Habtezion, Chief Medical Officer and Head of Worldwide Medical and Safety at Pfizer. “Our primary concern is for patients who suffer from SCD, which remains a very serious and difficult-to-treat disease with limited treatment options. We advise patients to contact their physicians to discuss alternative treatment while we continue to investigate the findings from our review of the data.”

Patients, physicians, pharmacists, or other healthcare professionals with additional questions about OXBRYTA should contact Pfizer Medical Information 1-800-438-1985. The company will keep patients, regulatory authorities, investigators and clinicians informed about actions and appropriate next steps for OXBRYTA.

The company does not anticipate that this event will impact its full-year 2024 financial guidance.

About Sickle Cell Disease

SCD is a lifelong, debilitating inherited blood disorder in which hemoglobin S polymerization leads to red blood cell sickling resulting in vascular inflammation and hemolytic anemia. Vascular inflammation, together with sickled RBC’s can lead to acute pain crises, or vaso-occlusive crises, and progressive end organ damage, including stroke. Complications of SCD begin in early childhood and are associated with shortened life expectancy. Early intervention and treatment of SCD have shown potential to modify the course of this disease, reduce symptoms and events, prevent long-term organ damage, and extend life expectancy.

Historically, there has been a high unmet need for therapies that address the root cause of SCD and its acute and chronic complications. While rare in developed markets, there are 4.5 million people living with SCD globally

and more than 45 million people living with the sickle cell trait. SCD occurs particularly among those whose ancestors are from sub-Saharan Africa, though it also occurs in people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.

About OXBRYTA ® (voxelotor)

OXBRYTA (voxelotor) is an oral, once-daily therapy for patients with sickle cell disease (SCD). OXBRYTA works by increasing hemoglobin's affinity for oxygen. OXBRYTA inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells leading to hemolysis and hemolytic anemia, which are primary pathologies faced by every single person living with SCD.

In 2019, the U.S. Food and Drug Administration (FDA) granted accelerated approval for OXBRYTA tablets for the treatment of SCD in adults and children ages 12 years and older. In December 2021, the FDA expanded the approved use of OXBRYTA for the treatment of SCD in patients 4 years of age and older in the U.S.

OXBRYTA was granted Priority Medicines (PRIME) designation by the European Medicines Agency (EMA) and designated an orphan medicinal product for the treatment of patients with SCD by the European Commission (EC). In February 2022, the EC granted Marketing Authorization for OXBRYTA for the treatment of hemolytic anemia due to SCD in adult and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). Since its first approval in 2019, OXBRYTA has been approved in over 35 countries globally.

Important Safety Information

OXBRYTA should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in OXBRYTA. See the end of the patient leaflet for a list of the ingredients in OXBRYTA. OXBRYTA can cause serious side effects, including serious allergic reactions. Patients should tell their healthcare provider or get emergency medical help right away if they get rash, hives, shortness of breath (difficult breathing) or swelling of the face.

The most common side effects of OXBRYTA include headache, diarrhea, stomach-area (abdominal) pain, nausea, rash or hives, and fever. The most common side effects of OXBRYTA in children ages 4 to less than 12 years of age include fever, vomiting, rash, stomach-area (abdominal) pain, diarrhea, and headache. These are not all the possible side effects of OXBRYTA. Before taking OXBRYTA, patients should tell their healthcare provider about all medical conditions, including if they have liver problems; if they are pregnant or plan to become pregnant as it is not known if OXBRYTA can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if OXBRYTA can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with OXBRYTA and for at least 2 weeks after the last dose.

Patients should tell their healthcare provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements. Some medicines may affect how OXBRYTA works. OXBRYTA may also affect how other medicines work and may affect the results of certain blood tests.

Patients are advised to call their doctor for medical advice about side effects. Side effects can be reported to FDA at 1-800-FDA-1088. Side effects can also be reported at 1-833-428-4968.

Full Prescribing Information for OXBRYTA is available at https://labeling.pfizer.com/ShowLabeling.aspx?id=19593 .

About Pfizer: Breakthroughs That Change Patients' Lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For 175 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.Pfizer.com . In addition, to learn more, please visit us on www.Pfizer.com and follow us on X at @Pfizer and @Pfizer News , LinkedIn , YouTube and like us on Facebook at Facebook.com/Pfizer .

DISCLOSURE NOTICE:

The information contained in this release is as of September 25, 2024. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about a voluntary withdrawal of all lots of OXBRYTA (voxelotor) from worldwide markets, including the anticipated impact on the company's full-year 2024 guidance, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, uncertainties regarding the future of OXBRYTA (voxelotor), as well as uncertainties related to other sickle cell disease assets in our portfolio; the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; risks associated with interim data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of OXBRYTA (voxelotor) or other sickle cell disease assets in our portfolio; the uncertainties inherent in business and financial planning, including, without limitation, risks related to Pfizer's business and prospects, legal proceedings, adverse developments in Pfizer's markets, or adverse developments in the U.S. or global capital markets, credit markets, regulatory environment or economies generally; uncertainties regarding the impact of COVID-19 on our business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results," as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com .

Category: Prescription Medicines

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