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FDA and EMA Accept Marstacimab Regulatory Submissions for the Treatment of Hemophilia A and B

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- *Submissions based on positive data from the Phase 3 BASIS trial, which were presented this past weekend at the American Society of Hematology (ASH) Annual Meeting*
- *If approved in the U.S. and EU, marstacimab could become the first once-weekly subcutaneous treatment for people living with hemophilia B and the first treatment administered as a flat dose for people living with hemophilia A or B*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the U.S. Food and Drug Administration (FDA) has accepted the company's Biologics License Application (BLA) for its anti-tissue factor pathway inhibitor (anti-TFPI) candidate marstacimab for individuals living with hemophilia A or hemophilia B without inhibitors to Factor VIII (FVIII) or Factor IX (FIX). The European marketing authorization application (MAA) for marstacimab also passed validation and is currently under review by the European Medicines Agency (EMA).

The FDA has set a Prescription Drug User Fee Act (PDUFA) action date in the fourth quarter of 2024, and a decision from the European Commission is anticipated by the first quarter of 2025. If approved in the U.S. and EU, marstacimab is expected to become the first once-weekly subcutaneous treatment for people living with hemophilia B and the first treatment administered as a flat dose for people living with hemophilia A or B.

"Marstacimab has demonstrated that it may be an efficacious treatment option with once-weekly, subcutaneous flat-dose administration via an auto-injector pen, for appropriate patients, if approved. This is critical as intravenous infusions are typically required for people living with these diseases today," said James Rusnak, M.D., Ph.D., Senior Vice President, Chief Development Officer, Internal Medicine and Infectious Diseases, Research and Development, Pfizer. "We look forward to progressing the review of this novel therapy with the FDA, EMA, and global regulatory authorities to bring this important medicine to patients globally."

For more than five decades, the most common treatment approach for hemophilia A and B has been factor replacement therapy, which replaces missing clotting factors to facilitate proper blood coagulation.[i] Marstacimab is a novel, investigational treatment for hemophilia that is designed to restore hemostasis by inhibiting TFPI. For appropriate patients living with hemophilia A and B, the goal of this treatment is to prevent potentially life-threatening bleeds with a once-weekly, subcutaneous flat-dose administration.

The submissions for marstacimab are based on efficacy and safety data from the Phase 3 BASIS trial (NCT03938792). Key findings were recently presented at the American Society of Hematology (ASH) Annual Meeting and Exposition on December 9, 2023. The inhibitor cohort of the BASIS trial has completed enrollment and is expected to read out as early as late 2024.

About Marstacimab

Marstacimab is a human monoclonal immunoglobulin G isotype, subclass 1 (IgG1) that targets the Kunitz 2 domain of tissue factor pathway inhibitor (TFPI), a natural anticoagulation protein that functions to prevent the formation of blood clots. Marstacimab is in development as a prophylactic treatment to prevent or reduce the frequency of bleeding episodes in individuals with hemophilia A or hemophilia B with or without inhibitors.

In September 2019, the U.S. FDA granted Fast Track designation to marstacimab for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in hemophilia A with inhibitors or hemophilia B with inhibitors.

Pfizer has three Phase 3 programs investigating treatments for people living with hemophilia: marstacimab, fidanacogene elaparvovec (hemophilia B), and giroctocogene fitelparvovec (hemophilia A).

About the BASIS study

BASIS is a global Phase 3, open-label, multicenter study evaluating annual bleed rate (ABR) through 12 months of treatment with marstacimab, an investigational, novel subcutaneous therapy option, in approximately 145 adolescent and adult participants ages 12 to <75 years with severe hemophilia A (defined as FVIII <1%) or moderately severe to severe hemophilia B (defined as FIX activity ?2%) with or without inhibitors. Approximately 15% of participants are adolescents (ages 12 to <18 years old). This study is comparing treatment with a run-in period for patients prescribed factor replacement therapy or bypass therapy during a six-month Observational Phase with a 12-month Active Treatment Phase, during which participants receive prophylaxis (a 300 mg subcutaneous loading dose of marstacimab, followed by 150 mg subcutaneously once weekly) with potential for dose escalation to 300 mg once weekly.

Pfizer is also conducting BASIS KIDS, an open-label study investigating the safety and efficacy of marstacimab in children ages 1 to <18 years with severe hemophilia A or moderately severe to severe hemophilia B with or without inhibitors. The study will compare 12 months of historical standard treatment to marstacimab prophylaxis.

About Hemophilia

Hemophilia is a family of rare genetic blood diseases caused by a clotting factor deficiency (FVIII in hemophilia A, FIX in hemophilia B), which prevents normal blood clotting. Hemophilia is diagnosed in early childhood and impacts more than 400,000 people worldwide.[ii] The inability of the blood to clot properly can increase the risk of painful bleeding inside the joints, which can cause joint scarring and damage. People living with hemophilia can suffer permanent joint damage following repeated bleeding episodes.[i,ii]

For decades, the most common treatment approach for hemophilia A and B has been factor replacement therapy, which replaces the missing clotting factors. Factor replacement therapies increase the amount of clotting factor in the body to levels that improve clotting, resulting in less bleeding.[iii,iv] Approximately 25-30% of people with hemophilia A and 3-5% of people with hemophilia B are unable to continue taking factor replacement therapies because they develop inhibitors to FVIII and FIX.[v,vi]

In a survey of people in the U.S. receiving prophylaxis for hemophilia A or B, nearly one-third of those that receive treatment and have high compliance – defined as taking 75% or more of their prescribed infusions – stated that the time-consuming nature of prophylaxis was the most significant challenge of the regimen.[vii,viii] Nearly 60% of those that took the less than the prescribed number of infusions reported that the time commitment was the primary reason for missing infusions.

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 more than 170 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 Twitter 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 December 11, 2023. 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 marstacimab, an investigational anti-tissue factor pathway inhibitor, and Pfizer's hemophilia programs for fidanacogene elaparvovec and giroctocogene fitelparvovec, including their potential benefits and applications for marstacimab pending with the FDA and the EMA, 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, 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; whether or when the inhibitor cohort of the BASIS trial will be successful; 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; whether and when any applications may be filed with regulatory authorities in particular jurisdictions for marstacimab, fidanacogene elaparvovec or giroctocogene fitelparvovec; whether and when the applications for marstacimab pending with the FDA and the EMA may be approved and whether and when regulatory authorities may approve any such other applications that may be pending or filed for marstacimab, fidanacogene elaparvovec or giroctocogene fitelparvovec may be approved by regulatory authorities, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether marstacimab, fidanacogene elaparvovec and giroctocogene fitelparvovec will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of marstacimab, fidanacogene elaparvovec and giroctocogene fitelparvovec; 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, 2022 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 .

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