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# Pfizer Doses First Participant in Phase 3 Study Evaluating anti-TFPI Investigational Therapy, Marstacimab, for People With Severe Hemophilia A and B With or Without Inhibitors

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*– If approved, marstacimab has potential to be a best-in-class treatment option among novel non-factor agents –*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the first participant has been dosed in the Phase 3 BASIS study of marstacimab (PF-06741086), an anti-tissue factor pathway inhibitor (anti-TFPI) being evaluated for the treatment of people with severe hemophilia A or B, with or without inhibitors.

BASIS is a global Phase 3, open-label, multicenter study that will evaluate annualized bleed rate (ABR) through 12 months on prophylaxis treatment with marstacimab, an investigational, novel subcutaneous therapy, in adolescents and adults with hemophilia A or B compared to a run-in period on replacement therapy with FVIII or FIX clotting factor, respectively, or bypass therapy (i.e., treatments that “bypass” the need for clotting factor treatment to help the body form a normal clot). The primary endpoint is impact on ABR through 12 months following prophylaxis treatment with marstacimab. The incidence and severity of thrombotic events will also be assessed.

“Our approach to hemophilia research includes the investigation of multiple mechanisms to help address the needs of all people with hemophilia, including those with hemophilia A or B, and with or without inhibitors, and targeting TFPI provides a novel approach to improve blood coagulation,” said Brenda Cooperstone, Chief Development Officer, Rare Disease, Pfizer Global Product Development. “Based on the Phase 2 study findings to date, marstacimab may have the potential to offer improved bleed control via subcutaneous injection and potentially eliminate the need for prophylactic factor replacement, providing an enhanced treatment option compared to factor replacement therapy.”

The completed Phase 2 study results demonstrated that treatment with marstacimab showed significant (>75%) reductions in ABR for all participants in the study population. The participants were monitored in a long-term extension study, which showed sustained efficacy up to 12 months and no thrombotic events or treatment-related serious adverse events in 20 participants receiving weekly subcutaneous marstacimab doses at or above the dose to be studied in the BASIS Phase 3 pivotal trial (300 mg subcutaneous loading followed by 150 mg subcutaneous weekly).

**About the BASIS study**

BASIS is a global Phase 3, open-label, multicenter study evaluating annualized bleed rate through 12 months on treatment with marstacimab, an investigational, novel subcutaneous therapy option, in approximately 145 adolescent and adult participants between ages 12 to <75 years with severe hemophilia A or B (defined as factor VIII or factor IX activity <1%, respectively), with or without inhibitors. Approximately 20% of participants will be adolescents (ages between 12 to <18 years old). This study is comparing treatment with a run-in period on patients' prescribed factor replacement therapy or bypass therapy during a 6-month Observational Phase with a 12-month Active Treatment Phase, during which participants will 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.

**About Marstacimab (PF-06741086)**

Marstacimab (PF-06741086) is a human monoclonal immunoglobulin G isotype, subclass 1 (IgG1) that targets the Kunitz 2 domain of tissue factor pathway inhibitor (TFPI). Marstacimab is in development as a prophylactic treatment to prevent or reduce the frequency of bleeding episodes in individuals with severe hemophilia A or B (defined as factor VIII or factor IX activity <1%, respectively) with or without inhibitors. In September 2019, the U.S. Food and Drug Administration (FDA) granted Fast Track designation to marstacimab for use in combination with inhibitors as a potential treatment for hemophilia A and B.

**About Hemophilia**

Hemophilia is a genetic hematological rare disease that results in a deficiency of a protein that is required for normal blood clotting—clotting factor VIII in hemophilia A and clotting factor IX in hemophilia B. The severity of hemophilia that a person has is determined by the amount of factor in the blood. The lower the amount of the factor, the more likely it is that bleeding will occur which can lead to serious health problems.

Hemophilia A occurs in approximately one in every 5,000-10,000 male births worldwide, and the incidence of hemophilia B is one in 25,000 male births. For people who live with hemophilia, there is an increased risk of spontaneous bleeding as well as bleeding following injuries or surgery. It is a lifelong disease that requires constant monitoring and therapy.

**About Pfizer Rare Disease**

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

**Pfizer Inc.: 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 150 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.

***PFIZER DISCLOSURE NOTICE:***

*The information contained in this release is as of November 23, 2020. 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 an investigational hemophilia A / B therapy, marstacimab, including its potential benefits, 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; 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 drug applications for any potential indications for marstacimab may be filed in any jurisdictions; whether and when regulatory authorities in any jurisdictions may approve any such applications, 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 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; uncertainties regarding the impact of COVID-19 on Pfizer's 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, 2019 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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