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FDA Grants Priority Review for HYMPAVZI® (marstacimab) sBLA for the Treatment of Two Hemophilia A or B Patient Populations with Significant Medical Need

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- *Submission is to expand HYMPAVZI indication to the treatment of hemophilia A or B patients 6 years and older with inhibitors, and pediatric patients (ages 6 to 11) without inhibitors*
- *If approved, HYMPAVZI would become the first non-factor prophylactic treatment available for children aged 6 to 11 years with hemophilia B*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the U.S. Food and Drug Administration (FDA) has accepted and granted Priority Review for the company's supplemental Biologics License Application (sBLA) for HYMPAVZI® (marstacimab) to expand the approved indication to include the treatment of hemophilia A or B patients 6 years and older with inhibitors, and pediatric patients (ages 6 to 11) with hemophilia A or B without inhibitors. In the U.S., HYMPAVZI is currently approved for the treatment of patients 12 years of age and older with hemophilia A without factor VIII (FVIII) inhibitors, or hemophilia B without factor IX (FIX) inhibitors.

The FDA has set a Prescription Drug User Fee Act (PDUFA) action date in the second quarter of 2026. If approved, HYMPAVZI would offer a combination of bleed protection with a straightforward, once-weekly subcutaneous injection administration, requiring minimal preparation and no routine treatment-related lab monitoring for these difficult-to-treat patient populations.

"There is a significant medical need for younger patients with hemophilia and for those who have developed inhibitors, which neutralize factor replacement therapies and render them ineffective," said Michael Vincent, M.D., Ph.D., Chief Inflammation & Immunology Officer, Pfizer. "Based on the findings in the BASIS clinical trial program and if approved, we believe HYMPAVZI has the potential to become a transformative option for these patients that have limited or burdensome treatment options today. We look forward to progressing discussions with regulators to make this medicine available for patients."

Hemophilia is diagnosed in early childhood and impacts more than 800,000 people worldwide.[1] The inability of the blood to clot properly can increase the risk of painful bleeding, including inside the joints, which can cause joint scarring and damage.[2,3] Children's joints have growing cartilage and bone, which makes them particularly susceptible to damage caused by repeated bleeding episodes.[4] Inhibitors, or antibodies, develop in approximately 20% of people with hemophilia A and 3% of people with hemophilia B. People living with inhibitors to FVIII and FIX are unable to continue taking factor replacement therapies as they no longer prevent or stop bleeding episodes, particularly in individuals who are refractory to immune tolerance induction therapy.[1,5,6]

“For children living with hemophilia A or B between ages 6 and 11, treatment approaches that prevent bleeds are particularly important to protect growing joints,” said Guy Young, M.D., Director, Hemostasis and Thrombosis Center at Children's Hospital, Los Angeles. “HYMPAVZI would address a critical unmet medical need for these patients and those with inhibitors if approved, particularly patients ages 6 to 11 with hemophilia B who do not have non-factor treatment options available today.”

The FDA grants Priority Review to medicines that may offer significant advances in treatment or may provide a treatment where no adequate therapy exists. Priority Review designation by the FDA shortens the standard sBLA review period by four months.

The FDA also granted HYMPAVZI Breakthrough Therapy Designation for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in younger pediatric (?6 to <12 years of age) patients with hemophilia B with and without inhibitors. The FDA’s Breakthrough Therapy Designation is intended to expedite the development and review of medicines with the potential to treat a serious or life-threatening disease, when preliminary clinical evidence indicates the medicine may demonstrate substantial improvement over existing therapies.

The submission for HYMPAVZI in adults and adolescents is based on efficacy and safety data from the inhibitor cohort of the Phase 3 BASIS trial (NCT03938792). The submission for HYMPAVZI in children aged 6 to 11 years with or without inhibitors is supported by efficacy and safety data from the Phase 3 BASIS KIDS trial (NCT05611801).

The use of HYMPAVZI for the treatment of patients 12 years and older living with hemophilia A or B with inhibitors is also under review by the European Medicines Agency.

About HYMPAVZI
Discovered by Pfizer scientists, HYMPAVZI has a unique mechanism of action that is differentiated from FVIII and FIX replacement treatments. Instead of replacing missing or insufficient clotting factors, HYMPAVZI is intentionally designed to target tissue factor pathway inhibitor (TFPI), one of the body’s natural mechanisms that inhibits the initiation of blood clotting. By targeting the Kunitz 2 domain of TFPI, HYMPAVZI may help re-establish balance between bleeding and blood clot formation with the goal of offering a combination of bleed protection and straightforward administration.

HYMPAVZI is a hemophilia treatment that has received regulatory approvals in more than 40 countries for eligible patients living with hemophilia A without factor VIII inhibitors, or hemophilia B without factor IX inhibitors. HYMPAVZI was the first anti-TFPI approved in the U.S. and EU for the treatment of hemophilia A or B and the first hemophilia medicine approved in the U.S. and EU to be administered via a pre-filled, auto-injector pen. For eligible people living with hemophilia B, it is the first once-weekly subcutaneous prophylactic treatment. HYMPAVZI is a subcutaneous treatment option with a once-weekly dosing schedule and minimal preparation required for each individual administration.

About the BASIS Clinical Trial
The pivotal BASIS study is a global, Phase 3, open-label, multicenter study to evaluate the efficacy data and safety profile of HYMPAVZI in 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. The with inhibitor cohort included 48 people living with hemophilia with inhibitors who were treated with HYMPAVZI during a 12-month active treatment period (ATP) versus an on-demand intravenous regimen with bypassing agents, administered as part of usual care in a six-month observational period. During the ATP, participants received prophylaxis (a 300 mg subcutaneous loading dose of HYMPAVZI, followed by 150 mg subcutaneously once weekly) with potential for dose escalation to 300 mg once weekly. An additional

three patients in the inhibitor cohort were on routine prophylactic treatment prior to the study and not included in the primary efficacy analysis. The primary endpoint measures the treated ABR (annualized bleeding rate) during the 12-month ATP with HYMPAVZI compared to treated ABR on prior on-demand bypass therapy. For further information, visit clinicaltrials.gov.

About the BASIS KIDS Clinical Trial
The BASIS KIDS study is a global, Phase 3, open-label study investigating the safety and efficacy of HYMPAVZI in children 1 to <18 years of age with severe hemophilia A or moderately severe to severe hemophilia B with or without inhibitors. There were 68 patients aged 6 to 11 years treated with HYMPAVZI during a 12-month ATP versus routine prophylaxis with factor replacement therapy (without inhibitor), or routine prophylaxis or on-demand treatment with bypassing agents (with inhibitor), administered as part of usual care in a 12-month period prior to enrollment. During the ATP in this age group, participants received prophylaxis (a 150 mg subcutaneous loading dose of HYMPAVZI, followed by 75 mg subcutaneous once weekly) with the potential for dose escalation to 150 mg once weekly. The primary endpoint measures treated ABR during the 12-month ATP with HYMPAVZI compared to ABR on prior routine prophylaxis with factor replacement therapy, or routine prophylaxis or on-demand treatment with bypassing agents. For further information, visit clinicaltrials.gov.

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 800,000 people worldwide.[1] The inability of the blood to clot properly can increase the risk of painful bleeding, including inside the joints, which can cause joint scarring and damage. People living with hemophilia can suffer permanent joint damage following repeated bleeding episodes.[2,3] Children's joints have growing cartilage and bone, which makes them particularly susceptible to damage caused by repeated bleeding episodes.[4]

For decades, the most common treatment approach for hemophilia A and B has been factor replacement therapy, which replaces the missing clotting factors.[2,7] Factor replacement therapies increase the amount of clotting factor in the body to levels that improve clotting, resulting in less bleeding.[2,3] The burden of intravenous infusions is believed to be a barrier to treatment adherence for some people living with hemophilia due in part to inconvenience, time constraints, and poor venous access.[8,9]

Approximately 20% of people with hemophilia A and 3% of people with hemophilia B are unable to continue taking factor replacement therapies because they develop inhibitors to FVIII and FIX, respectively.[1,6,7] These patients often have higher treatment burden, including potential complications from bleeding such as hospitalization and death, as well as higher treatment-related costs.[10,11,12]

HYMPAVZI (marstacimab-hncq) U.S. Important Safety Information

Important: Before you start using HYMPAVZI, it is very important to talk to your healthcare provider about using factor VIII and factor IX products (products that help blood clot but work in a different way than HYMPAVZI). You may need to use factor VIII or factor IX medicines to treat episodes of breakthrough bleeding during treatment with HYMPAVZI. Carefully follow your healthcare provider's instructions regarding when to use factor VIII or factor IX medicines and the prescribed dose during your treatment with HYMPAVZI.

Before using HYMPAVZI, tell your healthcare provider about all of your medical conditions, including if you:

- have a planned surgery. Your healthcare provider may stop treatment with HYMPAVZI before your surgery. Talk to your healthcare provider about when to stop using HYMPAVZI and when to start it again if you have a planned surgery.
- have a severe short-term (acute) illness such as an infection or injury.
- have been told that you have a risk for blood clots.
- are pregnant or plan to become pregnant. HYMPAVZI may harm your unborn baby.

Females who are able to become pregnant:

  - Your healthcare provider will do a pregnancy test before you start your treatment with HYMPAVZI.
  - You should use effective birth control (contraception) during treatment with HYMPAVZI and for at least 2 months after the last dose of HYMPAVZI.
  - Tell your healthcare provider right away if you become pregnant or think that you may be pregnant during treatment with HYMPAVZI.
  - are breastfeeding or plan to breastfeed. It is not known if HYMPAVZI passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, and herbal supplements.

What are the possible side effects of HYMPAVZI?

HYMPAVZI may cause serious side effects, including:

- **blood clots (thromboembolic events).** HYMPAVZI may increase the risk for your blood to clot in blood vessels in your arm, leg, lung, or head which can be life-threatening. Blood clots have happened in people using HYMPAVZI. You may have an increased risk of blood clots if you have certain risk factors. Stop using HYMPAVZI and get medical help right away if you develop any of these signs or symptoms of blood clots:
  - swelling or pain in arms or legs
  - redness or discoloration in your arms or legs
  - shortness of breath
  - pain in chest or upper back
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing
- **allergic reactions.** HYMPAVZI may cause allergic reactions, including rash and itching. Stop using HYMPAVZI and get medical help right away if you develop any of the following symptoms of a severe allergic reaction:
  - swelling of your face, lips, mouth, or tongue
  - trouble breathing
  - wheezing
  - dizziness or fainting
  - fast heartbeat or pounding in your chest
  - sweating

The most common side effects of HYMPAVZI include:

- swelling, hardening, redness, bruising, and pain at injection site
- headache
- itching

These are not all the possible side effects of HYMPAVZI. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.

The full Prescribing Information can be found here.

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 www.facebook.com/Pfizer/.

Disclosure notice
The information contained in this release is as of February 6, 2026. 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 HYMPAVZI® (marstacimab), an anti-tissue factor pathway inhibitor, including its potential benefits and submission to regulatory authorities of the Phase 3 BASIS data for HYMPAVZI for the treatment of adults and adolescents living with hemophilia A or B with inhibitors and the Phase 3 BASIS KIDS data for HYMPAVZI for the treatment of children aged 6 to 11 years with or without inhibitors, 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 commercial success of HYMPAVZI; 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 applications may be filed with regulatory authorities in particular jurisdictions for HYMPAVZI for any potential indication; whether and when any such applications that may be pending or filed for HYMPAVZI (including applications submitted to the FDA and EMA for adults and adolescents living with hemophilia A or B with inhibitors and to the FDA for children aged 6 to 11 years with or without inhibitors) 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 HYMPAVZI 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 HYMPAVZI, including for the potential new indications; risks and uncertainties related to issued or future

executive orders or other new, or changes in, laws or regulations; 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, 2024 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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