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U.S. FDA Approves Pfizer’s HYMPAVZI for the Treatment of Two Additional Hemophilia A or B Patient Populations with Significant Medical Need

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- *Approval expands HYMPAVZI use to now include people with hemophilia A or B ages 12 years and older with inhibitors and pediatric patients (ages 6 to 11 years) with or without inhibitors*
- *HYMPAVZI is the first subcutaneous non-factor therapy available for pediatric patients with hemophilia B ages 6 to 11 years old*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the U.S. Food and Drug Administration (FDA) has approved an expanded indication for HYMPAVZI® (marstacimab-hncq) to include the treatment of patients with hemophilia A or B 12 years and older with inhibitors and pediatric patients (ages 6 to 11 years) with or without inhibitors. HYMPAVZI is now indicated in the U.S. for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and pediatric patients 6 years of age and older with hemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors, or hemophilia B (congenital factor IX deficiency) with or without factor IX inhibitors.

HYMPAVZI offers a combination of prophylactic bleed protection with a straightforward, once-weekly subcutaneous administration that does not require routine treatment-related lab monitoring.

“For children who have to deal with bleeding episodes from an early age and for people living with hemophilia who develop inhibitors, treatment options have been limited and are often burdensome,” said Guy Young, M.D., Director, Hemostasis and Thrombosis Center at Children's Hospital, Los Angeles. “A treatment that can reduce bleeding with straightforward, once-weekly administration has the potential to fundamentally change how patients and caregivers approach this disease, offering control with a level of simplicity this community has long needed.”

Hemophilia is typically 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 to factor replacement therapy limit treatment options for people living with hemophilia and are associated with an increased risk of uncontrolled bleeding.[5] These inhibitory antibodies develop in approximately 20% of those with hemophilia A and 3% of those with hemophilia B.[5] Many 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]

“With this expanded approval, we believe HYMPAVZI can become a transformative option and meet a significant medical need for people living with hemophilia A or B with or without inhibitors ages 6 years and older. Particularly for children ages 6 to 11 with hemophilia B who will now, for the first time, have a subcutaneous non-factor treatment available,” said Aamir Malik, Chief U.S. Commercial Officer and Executive Vice President, Pfizer. “This milestone represents the latest step in Pfizer’s more than 40-year commitment to advancing care and quality of life for people living with hemophilia, which began with the introduction of recombinant therapies and has evolved with the introduction of this once-weekly subcutaneous treatment.”

Results from the Phase 3 BASIS trial (NCT03938792) supported the approval of HYMPAVZI in adults and adolescents 12 years and older with hemophilia A or B with inhibitors. The results demonstrated the superiority of HYMPAVZI in improving key bleeding outcomes including significantly reducing mean treated annualized bleeding rate (ABR) by 93% compared to on-demand (OD) intravenous treatment with bypassing agents (1.4 [95% CI: 0.9-2.3] vs.19.8 [95% CI: 16.1-24.3]; p<0.0001).

Interim results from the Phase 3 BASIS KIDS trial (NCT05611801) supported the approval of HYMPAVZI in children ages 6 to 17 years with hemophilia A or B with or without inhibitors. Descriptive analyses, which summarize trends in the data, in patients who received HYMPAVZI demonstrated:

- In children ages 6 to 17 years old without inhibitors, a mean treated ABR of 1.8 (99% CI: 1.1-2.6) was observed in patients who received HYMPAVZI compared to a historical model-based mean ABR of treated bleeds of 3.6 (99% CI: 1.3-5.8) in patients who received routine prophylaxis
- In children 6 to 17 years old with inhibitors, a mean treated ABR of 1.4 (99% CI: 0.5-4.5) was observed in patients who received HYMPAVZI compared to a historical model-based mean ABR of treated bleeds of 18.9 (99% CI: 14.2, 25.2) in patients who received OD therapy
- In children 6 to 11 years old with inhibitors who were previously on OD therapy or without inhibitors who were previously receiving routine prophylaxis, respectively, a model-based mean treated ABR of 1.3 and 1.4 and a median ABR of 1.0 and 1.0 were observed.

The most commonly reported adverse reactions (?2%) in adult and pediatric patients 6 years of age and older with or without inhibitors were injection site reaction, headache, pyrexia, arthralgia, diarrhea, pruritus, and rash. Thromboembolic events (venous and arterial) in two patients were observed among a total of 259 patients who received HYMPAVZI in the open-label extension study. Thromboembolic events, hypersensitivity, embryofetal toxicity, and increased laboratory values of fibrin D-dimer and prothrombin fragment 1.2 are noted within the Warnings and Precautions section of the U.S. label.

This HYMPAVZI application was reviewed under FDA Priority Review, which is granted to medicines that treat a serious condition and provide a significant improvement in safety or effectiveness over available therapy. 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 on a clinically significant endpoint over available therapies.

About HYMPAVZI

Discovered and developed 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 anticoagulants, which acts to inhibit the initiation of blood clotting. By targeting the Kunitz 2 domain of TFPI, HYMPAVZI may help re-establish balance between bleeding and coagulation (or hemostasis) with the

goal of offering a combination of bleed protection and straightforward administration.

In 2024, HYMPAVZI became the first anti-TFPI approved in the U.S. and EU for the treatment of hemophilia A or B and it is the first hemophilia medicine approved in the U.S. and EU to be administered via a pre-filled auto-injector pen. HYMPAVZI is a subcutaneous treatment option with a once-weekly dosing schedule and minimal preparation required for each individual administration.

Recently, the European Commission (EC) granted marketing authorization for HYMPAVZI for the routine prophylaxis of bleeding episodes in patients 12 years of age and older weighing at least 35 kg with hemophilia A (congenital factor VIII [FVIII] deficiency) with FVIII inhibitors, or hemophilia B (congenital factor IX [FIX] deficiency) with FIX inhibitors. Pfizer is continuing to pursue expanded regulatory approvals for HYMPAVZI in other countries around the world in this indication. To date, HYMPAVZI 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.

Pfizer Hemophilia Connect offers personalized support for eligible patients in the U.S. prescribed through a Patient Case Manager, patient support resources, benefits verification, and information about the insurance coverage process. Eligible patients prescribed HYMPAVZI can call 1-888-733-2030. Full terms and conditions are available on www.pfizerhemophiliasupport.com/hympavzi.

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 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) 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 17 years of age with severe hemophilia A or moderately severe to severe hemophilia B with or without inhibitors. In the analysis, 57 patients ages 6 to 17 years old - including 34 patients between ages 6 to 11 - received 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 participants in the 6 to 11 age group, received prophylaxis (a 150 mg subcutaneous loading dose of HYMPAVZI, followed by 75 mg subcutaneous once weekly) while participants in the 12 to 17 age group received prophylaxis (a 300mg subcutaneous loading dose of HYMPAVZI followed by 150 mg subcutaneously 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 rare disorder in which blood does not clot properly. Hemophilia increases the risk of painful bleeding, including inside the joints, which can cause joint scarring and damage.[2,3] It is diagnosed in early

childhood and impacts more than 800,000 people worldwide.[1] Children living with hemophilia are especially vulnerable, as their growing cartilage and bone are susceptible to scarring and permanent 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/or 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.[1,5,7] 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. Indication and Usage
HYMPAVZI® (marstacimab-hncq) is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and pediatric patients 6 years of age and older with hemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors, or hemophilia B (congenital factor IX deficiency) with or without factor IX inhibitors.

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 or "bypassing agents" (products that help blood clot but work in a different way than HYMPAVZI).
Your healthcare provider may prescribe factor VIII or factor IX medicines or bypassing agents to treat episodes of breakthrough bleeding during your treatment with HYMPAVZI. Carefully follow your healthcare provider's instructions regarding when to use these medicines and the prescribed dose during your treatment with HYMPAVZI. **Do not use additional doses of HYMPAVZI to treat breakthrough bleeds.**

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.
- 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.

If you inject too much HYMPAVZI, call your healthcare provider or the Poison Help Line at 1-800-222-1222 or go to the nearest hospital emergency room right away.

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 your arms or legs
    - redness or discoloration in your arms or legs
    - shortness of breath
    - pain in chest or upper back
    - fast heart rate
    - coughing up blood
    - feeling 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, bleeding, and pain at injection site
- headache
- fever
- joint pain
- diarrhea
- itching
- rash

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 June 8, 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-hncq), an anti-tissue factor pathway inhibitor, including its potential benefits, an approval by the U.S. Food and Drug Administration to expand the approved indication for HYMPAVZI to include the treatment of hemophilia A or B patients 12 years and older with inhibitors, and pediatric patients (ages 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 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, 2025 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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