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European Commission Approves Pfizer’s HYMPAVZI for the Treatment of Adults and Adolescents with Hemophilia A or B With Inhibitors

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- *Approval in patients ages 12 and older based on Phase 3 data demonstrating superior bleed reduction to on-demand therapy, with continued benefit observed in open-label extension study*
- *HYMPAVZI is the only once-weekly subcutaneous treatment approved in the EU for both people living with hemophilia A or B, with or without inhibitors*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the European Commission (EC) has granted marketing authorization to expand the approved indication for HYMPAVZI® (marstacimab) to include 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.

HYMPAVZI offers a combination of superior bleed protection compared to on-demand (OD) treatment that is well-tolerated with a straightforward, once-weekly subcutaneous injection administration that does not require routine treatment-related lab monitoring for this difficult-to-treat inhibitor patient population 12 years of age and older.

Inhibitors limit treatment options for people living with hemophilia and are associated with an increased risk of uncontrolled bleeding.[1] These inhibitory antibodies neutralize factor replacement therapies and render them ineffective.[1,2] Of the more than 800,000 people in the world living with hemophilia A or hemophilia B, approximately 20% of those with hemophilia A and 3% of those with hemophilia B are unable to continue taking factor replacement therapies because they developed inhibitors to FVIII and FIX, respectively, and these therapies no longer prevent or stop bleeding episodes, particularly in individuals who are refractory to immune tolerance induction therapy.[1,2,3]

“Inhibitors present a substantial challenge for people living with hemophilia as they neutralize traditional factor replacement therapies, in turn limiting treatment options and leaving patients vulnerable to uncontrolled bleeding episodes,” said Dr. Laurent Frenzel, Head of the Hemophilia Treatment and Research Center at the Necker-Enfants malades Hospital (Paris Cité). “The approval of HYMPAVZI offers adults and adolescents in the EU a once-weekly subcutaneous option that has demonstrated the ability to reduce bleeding episodes and maintain bleed reduction based on observation to date in a long-term extension study.”

“For people living with hemophilia with inhibitors, recurring bleeding episodes can lead to damaged joints and introduce real limitations and disruptions to everyday life,” said Alexandre de Germay, Chief International Commercial Officer and Executive Vice President, Pfizer. “This approval brings a once-weekly medicine to the

EU that meets a critical need for patients who face a treatment journey that can be complex and challenging with limited options available today, representing the latest step in Pfizer's more than 40-year commitment to advancing care for people living with hemophilia. We look forward to working with regulators globally to continue bringing HYMPAVZI to those who can benefit from it."

This indication extension is based on results from the Phase 3 BASIS trial (NCT03938792) that evaluated the efficacy and safety of HYMPAVZI in adults and adolescents 12 years and older with severe hemophilia A or moderately severe to severe hemophilia B with inhibitors:

- In the active treatment period of the study, HYMPAVZI treatment resulted in a statistically significant and clinically meaningful 93% reduction in the mean treated annualized bleeding rate (ABR) (1.39 [95% CI: 0.85-2.29] vs.19.78 [95% CI: 16.12-24.27]; p<0.0001), demonstrating superiority over OD therapy.
    - Superiority (p?0.0001) of HYMPAVZI was also demonstrated across all measured bleeding-related secondary endpoints – spontaneous bleeds, joint bleeds, target joint bleeds, and total treated and untreated bleeds.
- In an interim analysis of the open-label extension trial, where patients were treated with HYMPAVZI for up to an additional 41 months (a total of 53 months of treatment with HYMPAVZI), the mean (1.19 [95% CI: 0.72-1.95]) and median (0.00 [95% CI: 0.00-1.18]) treated ABRs remained low.
- The safety profile for HYMPAVZI was consistent with Phase 1/2 results, and the most frequently reported adverse events in the study were injection site reactions, headache, pruritus, hypertension, and rash. The most serious adverse event reported from the clinical studies with HYMPAVZI was thrombosis.

This marketing authorization is valid in all 27 EU member states, as well as in Iceland, Liechtenstein, and Norway. Separately, the U.S. Food and Drug Administration (FDA) accepted and granted Priority Review for the supplemental Biologics License Application (sBLA) for HYMPAVZI to expand its 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 earlier this year. In the U.S., HYMPAVZI is currently approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients 12 years of age and older with hemophilia A (congenital FVIII deficiency) without factor VIII inhibitors or hemophilia B (congenital FIX deficiency) without factor IX inhibitors. The FDA has set a Prescription Drug User Fee Act (PDUFA) action date in the second quarter of 2026.

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.

Pfizer is also conducting BASIS KIDS, an open-label study investigating the safety and efficacy of HYMPAVZI in children <18 years of age with severe hemophilia A or moderately severe to severe hemophilia B with or without inhibitors. Pfizer is continuing to evaluate the long-term safety and efficacy of HYMPAVZI in hemophilia patients with or without inhibitors who have successfully completed treatment in BASIS and BASIS KIDS in an ongoing open-label long-term extension study.

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) 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 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.[3] 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.[4,5]

For decades, the most common treatment approach for hemophilia A and B has been factor replacement therapy, which replaces the missing clotting factors.[4,6] Factor replacement therapies increase the amount of clotting factor in the body to levels that improve clotting, resulting in less bleeding.[4,5] 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.[7,8]

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.[1,3,6] These patients often have higher treatment burden, including potential complications from bleeding such as hospitalization and death, as well as higher treatment-related costs.[9,10,11]

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 May 13, 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, an approval by the European Commission to expand the approved indication for HYMPAVZI to include 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 and an sBLA pending with the FDA, 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 the sBLA submitted to the FDA to expand its approved indication 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 earlier this year.) 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.

Prescription Medicines

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