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# Pfizer Announces Positive Topline Phase 3 Results for HYMPAVZI™ in Hemophilia A or B with Inhibitors

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- *Study demonstrates superiority, showing both statistically significant and clinically meaningful reduction in annualized bleeding rate with a generally well-tolerated safety profile compared to on-demand treatment in patients 12 years and older*
- *HYMPAVZI was administered with a straightforward, once-weekly subcutaneous injection that required minimal preparation*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced positive topline results from the Phase 3 BASIS study (NCT03938792) evaluating HYMPAVZI™ (marstacimab) for adults and adolescents living with hemophilia A or B with inhibitors. The study met the primary endpoint and key secondary bleeding endpoints demonstrating the superiority of once-weekly subcutaneous HYMPAVZI in improving key bleeding outcomes compared to on-demand treatment in a patient population where less burdensome treatment approaches are needed.[1,2,3,4]

Inhibitors, or antibodies, which neutralize factor replacement therapies and render them ineffective, may develop in people living with hemophilia.[5] Inhibitors can be diagnosed with a blood test.[6] Of the more than 800,000 people in the world living with hemophilia A or hemophilia B, 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 (Factor VIII) and FIX (Factor IX) and these therapies no longer prevent or stop bleeding episodes.[6,7]

“Patients with inhibitors tend to face frequent complications, and navigating the treatment landscape can introduce complexities and increase disease burden,”[1,2,3,4,8] said Davide Matino, M.D., M.Sc., BASIS Principal Investigator, Associate Professor of Medicine, McMaster University. “The strong bleed reduction with HYMPAVZI compared to on-demand treatment in the Phase 3 BASIS study, coupled with its weekly administration method, offers exciting potential for these patients who are in critical need of treatment options.”

The BASIS trial demonstrated that prophylactic treatment with HYMPAVZI resulted in a statistically significant and clinically relevant reduction in annualized bleeding rate (ABR) of treated bleeds in people living with severe hemophilia A or hemophilia B with inhibitors. Forty-eight people living with hemophilia were treated with HYMPAVZI during a 12-month period versus an on-demand intravenous regimen with bypassing agents, administered as part of usual care in the six-month lead-in period. HYMPAVZI was superior to on-demand treatment with a 93% reduction in ABR over 12 months (ABR 1.39 vs ABR on-demand 19.78; $p < 0.0001$). Superiority of HYMPAVZI was also demonstrated across all bleeding-related secondary endpoints—spontaneous bleeds, joint bleeds, target joint bleeds, and total bleeds.

HYMPAVZI was generally well-tolerated, consistent with the non-inhibitor cohort of the BASIS study and Phase 1/2 results. No deaths or thromboembolic events were reported.

“These encouraging results demonstrate HYMPAVZI’s potential to help people living with hemophilia A or B with inhibitors, meeting an important need for patients with antibodies that neutralize most factor-based prophylactic options used to manage bleeding episodes,” said Michael Vincent, M.D., Ph.D., Chief Inflammation & Immunology Officer, Pfizer. “HYMPAVZI represents Pfizer’s latest contribution in more than 40 years of working to advance hemophilia care, as a generally well-tolerated treatment option that could offer bleed protection with a straightforward, once-weekly subcutaneous administration in a pre-filled pen for patients with inhibitors, if approved in this patient population.”

Analyses of the full Phase 3 dataset from the inhibitor cohort of the BASIS study are ongoing, and additional data will be presented at upcoming medical meetings. Pfizer plans to discuss these data with regulatory authorities, with the goal of initiating regulatory filings for HYMPAVZI for the treatment of patients living with hemophilia with inhibitors.

Discovered by Pfizer scientists, HYMPAVZI has a 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, good tolerability, and straightforward administration.

**About the BASIS study**

The pivotal BASIS study is a global Phase 3, open-label, multicenter study to evaluate the efficacy and safety 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.

This 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 during the 12-month ATP with HYMPAVZI compared to treated ABR on prior on-demand replacement therapy. For further information, visit clinicaltrials.gov.

**About HYMPAVZI**

HYMPAVZI has received regulatory approvals in the U.S. and in Europe 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 can offer 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 1 to <18 years of age with severe hemophilia A or moderately severe to severe hemophilia B with or

without inhibitors.

**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.[7] 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.[9,10]

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. 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.[11]

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.[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.[1,2,3,4,12]

**HYMPAVZI (marstacimab) 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.
- 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. Blood clots may form in blood vessels in your arm, leg, lung, or head and can be life-threatening. 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.** Allergic reactions, including rash and itching have happened in people treated with HYMPAVZI. 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 are injection site reactions, headache, and 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 June 26, 2025. 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 plans to discuss the Phase 3 BASIS data with regulatory authorities with the goal of initiating regulatory filings for HYMPAVZI for the treatment of patients living with hemophilia with 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 any 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; 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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