

# HYMPAVZI® (marstacimab) Reduced Bleeds by 93% Compared to On-Demand Treatment in Adults and Adolescents with Hemophilia A or B with Inhibitors

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- Findings from the Phase 3 study were presented at the 67<sup>th</sup> American Society of Hematology Annual Meeting and Exposition and published in *Blood*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today presented results from the Phase 3 BASIS study ([NCT03938792](#)) evaluating HYMPAVZI® (marstacimab) for adults and adolescents living with hemophilia A or B with inhibitors. The results demonstrated the superiority of HYMPAVZI in improving key bleeding outcomes compared to on-demand (OD) treatment with bypassing agents. HYMPAVZI was administered with a straightforward, once-weekly subcutaneous injection requiring minimal preparation and no treatment-related lab monitoring.

The findings were shared today in an oral presentation at the 67<sup>th</sup> American Society of Hematology (ASH) Annual Meeting and Exposition in Orlando and published in *Blood*.

Inhibitors, or antibodies, which neutralize factor replacement therapies and render them ineffective, may develop in people living with hemophilia.<sup>1</sup> Inhibitors can be diagnosed with a blood test.<sup>2</sup> 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), respectively, and these therapies no longer prevent or stop bleeding episodes.<sup>2,3</sup>

“The emergence of inhibitors poses significant treatment challenges and can increase disease burden for people living with hemophilia A or B,”<sup>4</sup> said Davide Martino, M.D., M.Sc., BASIS Principal Investigator, Associate Professor of Medicine, McMaster University. “In patients with inhibitors, this study demonstrates HYMPAVZI’s potential as a safe and efficacious treatment option that not only significantly reduced bleeding episodes via a once-weekly subcutaneous administration, but also demonstrated improvement in certain aspects of health-related quality of life.”

In the BASIS trial, 48 adults and adolescents living with severe hemophilia A or hemophilia B with inhibitors were treated with HYMPAVZI during the 12-month active treatment phase (ATP) following a preceding OD intravenous regimen with bypassing agents, administered as part of usual care in a six-month observational phase. During the ATP, participants received HYMPAVZI as a 300 mg subcutaneous loading dose, followed by once-weekly 150 mg dosing. The study found:

- A statistically significant and clinically meaningful 93% reduction in 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 of HYMPAVZI over OD therapy.
  - These results were consistent across hemophilia type, age, and geographies.
  - Additionally, a median ABR of 0 (95% CI: 0.00-14.91) was observed with HYMPAVZI compared to 16.42 (95% CI: 0.00-69.10) with OD treatment.
- Superiority ( $p < 0.0001$ ) of HYMPAVZI was also demonstrated across all bleeding-related secondary endpoints – spontaneous bleeds (ABR 0.87 vs. ABR OD 15.27), joint bleeds (ABR 1.10 vs. ABR OD 15.15), target joint bleeds (ABR 0.79 vs. ABR OD 6.42), and total treated and untreated bleeds (ABR 4.36 vs. ABR OD 27.29).
- After six months in the ATP, HYMPAVZI demonstrated superiority and/or numerical improvement on health-related quality-of-life outcomes compared to OD therapy, via:
  - A median difference of -25.9 (95% CI: -37.5 to -14.2;  $p < 0.0001$ ) in the Haem-A-QoL physical health domain (assessed patient-reported physical burden of disease such as joint and swelling pain, difficulty moving, and time to get ready),
  - A median difference of -13.5 (95% CI: -19.8 to -7.2;  $p < 0.0001$ ) in the Haem-A-QoL total score (assessed patient-reported outcomes across 10 domains related to health-related quality of life and daily activities),
  - A median difference of 0.1043 (95% CI: 0.0060 to 0.2027;  $p < 0.0377$ ) in the EQ-5D-5L (assessed patient-reported outcomes related to mobility, self-care, usual activities, pain/discomfort, and anxiety/depression) index score.
  - A median difference of 7.5 (95% CI: -1.9 to 17.0) in the EQ-VAS score (a component of the EQ-5D-5L and overall rating of a patient's self-reported health).

HYMPAVZI was generally well tolerated. No deaths or thromboembolic events were reported in the 51 participants included in the safety population. Thirty-eight patients reported adverse events (AEs) during the ATP and the most common were COVID-19 (21.6%), upper respiratory tract infection (15.7%), fibrin D-dimer increase (9.8%), and headache (9.8%). Most AEs were mild or moderate in severity. During the ATP, one patient reported a serious AE (skin rash). The serious AE was treatment related and led to study discontinuation, and was resolved.

“It is encouraging that these data demonstrate the potential of HYMPAVZI to combine efficacy, safety, and straightforward administration for adults and adolescents living with hemophilia A or B with inhibitors and address a significant patient need,” said Michael Vincent, M.D., Ph.D., Chief Inflammation & Immunology Officer, Pfizer. “We look forward to potentially making this treatment available for these patients as Pfizer continues its ongoing effort spanning more than 40 years to improve hemophilia care.”

Pfizer has submitted these data to the U.S. Food and Drug Administration and European Medicines Agency for review.

HYMPAVZI is currently approved in more than 40 countries for the treatment of eligible patients 12 years of age and older living with hemophilia A without factor VIII inhibitors, or hemophilia B without factor IX inhibitors.

### **About the BASIS study**

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  $\geq 2\%$ ) 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 bypass therapy. For further information, visit [clinicaltrials.gov](https://clinicaltrials.gov).

### **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, good tolerability, 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 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 up to <18 years of age with severe hemophilia A or moderately severe to severe hemophilia B with or without inhibitors. Pfizer is continuing to evaluate 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 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.<sup>3</sup> 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.<sup>5,6</sup>

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.<sup>7</sup>

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.<sup>2</sup> These patients often have higher treatment burden, including potential complications from bleeding such as hospitalization and death, as well as higher treatment-related costs.<sup>8,9,10,11,12</sup>

## **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](http://www.Pfizer.com). In addition, to learn more, please visit us on [www.Pfizer.com](http://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/](http://www.facebook.com/Pfizer/).

**Disclosure notice**

*The information contained in this release is as of December 6, 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<sup>®</sup> (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 patients living with hemophilia A or B 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 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 patients living with hemophilia A or B with 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; 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](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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<sup>3</sup> World Federation of Hemophilia. World Federation of Hemophilia Global Report on the Annual Global Survey 2024. <https://www1.wfh.org/publications/files/pdf-2588.pdf>.

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