Pfizer Logo

Pfizer Highlights Scientific Advances from Growing Hematology Portfolio at American Society of Hematology Annual Meeting

Thursday, November 02, 2023 - 10:52am

- *39 presentations span ten medicines, including six in Pfizer's pipeline, being studied for the treatment of hemophilia, sickle cell disease, and blood cancer*
- *Presentations include primary analysis from pivotal Phase 3 BASIS trial of marstacimab in hemophilia A&B, initial Phase 2/3 results from GBT601 study in sickle cell disease, and data from the broad MagnetisMM multiple myeloma program supporting the favorable profile of ELREXFIO*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) will present its latest data showcasing advances in the treatment of hemophilia, sickle cell disease, and blood cancers at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition in San Diego from December 9-12. These data from 39 presentations represent continued innovation and advancement in hemophilia including pivotal findings for Pfizer's novel anti-tissue factor pathway inhibitor (anti-TFPI) candidate marstacimab and the latest findings on a next-generation investigational treatment for sickle cell disease (SCD) in GBT021601 (GBT601). Pfizer will also present the latest research in blood cancer, including for ELREXFIO (elranatamab-bcmm), a BCMA-directed bispecific antibody recently approved by the U.S. Food and Drug Administration (FDA) for the treatment of adult patients with relapsed or refractory multiple myeloma (RRMM) who have received at least four prior lines of therapy, including a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody.

"Pfizer has been advancing science in hematology for more than 30 years, starting with the introduction of recombinant factor replacement therapy, which became the standard of care for people living with hemophilia. This year at ASH we will deliver five oral presentations including the latest clinical findings from our hemophilia programs and exciting data from the GBT601 program in sickle cell disease, representing progress in our unrelenting efforts to address the broad spectrum of patient needs," said Sonal Bhatia, M.D., Chief Medical Officer, Rare Disease, Pfizer. "The findings reflect the company's scientific capabilities and use of translational science to potentially offer improved treatment options to help people living with these rare diseases."

"During ASH, we are pleased to present new clinical and real-world data in multiple myeloma from our broad development program for ELREXFIO, following recent FDA accelerated approval. This includes extended efficacy and safety results from MagnetisMM-3, highlighting sustained clinical efficacy and no new safety signals after 20 months of follow-up," said Chris Boshoff, Chief Oncology Research and Development Officer and Executive Vice President, Pfizer. "These data continue to support the potential of ELREXFIO as the next standard of care for patients with advanced multiple myeloma."

Highlights from company-sponsored abstracts include:

- Six presentations from our hemophilia pipeline, including the first presentation of primary results from the non-inhibitor cohort of the pivotal Phase 3 BASIS clinical trial evaluating marstacimab in people living with hemophilia A or B. Additionally, four-year data from Pfizer's Phase 1/2 study of investigational gene therapy giroctocogene fitelparvovec in adults living with severe hemophilia A will be presented.
- Fourteen abstracts focused on the treatment of SCD, including Phase 2a safety, efficacy, and pharmacodynamic data from the ongoing Phase 2/3 clinical trial study of GBT601. In addition, long-term safety and efficacy findings on voxelotor (over four years follow-up) will be presented as well as data illustrating insights into the experience of SCD patients and their caregivers through a social media listening study.
- Nineteen abstracts from our blood cancer portfolio, including six which continue to support the use of ELREXFIO, a B-cell maturation antigen (BCMA)-CD3-directed bispecific antibody immunotherapy, in patients with RRMM. This includes extended findings from MagnetisMM-3 cohort A (~20 months median follow-up) and a pooled analysis of efficacy and safety in Black patients with RRMM from MagnetisMM-1, MagnetisMM-3, and MagnetisMM-9, reinforcing Pfizer's commitment to health equity.

A complete list of Pfizer-sponsored accepted abstracts is available here.

Key Pfizer-sponsored oral presentations at ASH 2023 include:

Predictive Biomarker Analysis from the GBT021601 Survival Study in Townes Sickle Mice (Abstract #14) Pochron M Saturday, December 9, 9:30 – 11:00 AM PST Presentation Time: 9:45 AM PST
Efficacy and Safety of the Anti-Tissue Factor Pathway Inhibitor Marstacimab in Participants with Severe Hemophilia without Inhibitors: Results from the Phase 3 Basis Trial (Abstract #285) Matino D Saturday, December 9, 4:00-5:30 PM PST Presentation Time: 4:30 PM PST
Preliminary Results from a Multicenter Phase 2/3 Study of Next-Generation HbS Polymerization Inhibitor GBT021601 for the Treatment of Patients with Sickle Cell Disease (Abstract #274) Saraf S Saturday, December 9, 4:00 – 5:30 PM PST Presentation Time: 4:45 PM PST
Understanding the Experiences of Patients with Sickle Cell Disease and their Caregivers by Social Media Listening in the UK (Abstract #1057) Shastri O Monday, December 11, 4:30 – 6:00 PM EST Presentation Time: 4:30 PM PST
Four-Year Follow-up of the Alta Study, a Phase 1/2 Study of Giroctocogene Fitelparvovec (PF-07055480/SB-525) Gene Therapy in Adults with Severe Hemophilia ? (Abstract #1054) Leavitt A Monday, December 11, 2023, 4:30 PM - 6:00 PM PST Presentation Time: 5:15 PM

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 often diagnosed in early childhood and impacts more than 400,000 people worldwide.[1] The inability of the blood to clot properly can increase the risk of painful bleeding inside the joints and other serious or even life-threatening bleeding. People living with hemophilia can suffer permanent joint damage following repeated bleeding episodes.[1,2]

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; however, they must be administered by IV infusion on a regular basis.[2,3] Approximately 25-30% of people with hemophilia A and 3-5% of people with hemophilia B are unable to continue taking factor replacement therapies because they develop inhibitors to FVIII and FIX.[4,5]

About Sickle Cell Disease

Sickle cell disease (SCD) is a lifelong, debilitating inherited blood disorder characterized by hemolytic anemia, which drives vascular inflammation, acute pain crises and progressive end organ damage. Acute pain crisis, or vaso-occlusive crisis (VOC), occurs when sickled red blood cells, white blood cells and platelets stick to the inflamed lining of blood vessels leading to vascular occlusion, tissue ischemia and pain. Complications of SCD begin in early childhood and are associated with shortened life expectancy. Early intervention and treatment of SCD have shown potential to reduce symptoms, events, long-term organ damage, and extend life expectancy. Historically, there has been a high unmet need for therapies that address SCD and its acute and chronic complications. SCD occurs particularly among those whose ancestors are from sub-Saharan Africa, though it also occurs in people of Hispanic, South Asian, Southern European, and Middle Eastern ancestry.

About Multiple Myeloma

Multiple myeloma (MM) is an aggressive and currently incurable blood cancer that affects plasma cells made in the bone marrow. Healthy plasma cells make antibodies that help the body fight infection.[6] MM is the second most common type of blood cancer, with over 35,000 new cases of MM diagnosed annually in the U.S. and 176,000 globally.[7,8] About half of those diagnosed with MM won't survive beyond five years, and most will receive four or more lines of therapy due to relapse.[9] While disease trajectory varies for each person, relapses are nearly inevitable.[10] Real-world evidence shows that people with RRMM often become resistant to the three main classes of treatment – proteasome inhibitors, immunomodulatory agents and anti-CD38 monoclonal antibodies – after just a few rounds of therapy, and re-treating with these classes was common.[11] The goal of therapy for people with RRMM is to achieve disease control with acceptable toxicity and improved quality of life.[12]

Prescribing Information for Pfizer Medicines

Please read full Prescribing Information, including BOXED WARNING, for ELREXFIO.

Please read full Prescribing Information for OXBRYTA.

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 more than 170 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 Twitter at @Pfizer and @Pfizer News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of November 2, 2023. 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 Pfizer's hematology portfolio, marstacimab, GBT021601 (GBT601), ELREXFIO (elranatamab-bcmm), voxelotor, and giroctocogene fitelparvovec, including their potential benefits, 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, 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 any regulatory authorities for any potential indications for marstacimab, GBT021601, elranatamab-bcmm, voxelotor, giroctocogene fitelparvovec or any other product candidates; whether and when any applications that may be pending or filed for marstacimab, GBT021601, elranatamab-bcmm, voxelotor, giroctocogene fitelparvovec or any other product candidates 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 marstacimab, GBT021601, elranatamab-bcmm, voxelotor, giroctocogene fitelparvovec or any other product candidates 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 marstacimab, GBT021601, elranatamab-bcmm, voxelotor, giroctocogene fitelparvovec or any other product candidates; 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, 2022 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 .

[1] Srivastava A, Santagostino E, Dougall A, et al. WFH guidelines for the management of hemophilia, 3rd Edition; 2020. Haemophilia, 26(S6), 1–158. https://doi.org/10.1111/hae.14046.
[2] Centers for Disease Control and Prevention. Hemophilia. Last Reviewed: April 2023. https://www.cdc.gov/dotw/hemophilia/index.html.
[3] Weyand AC, Pipe SW. New therapies for hemophilia. Blood 2019;133(5):389–398. doi: https://doi.org/10.1182/blood-2018-08-872291.
[4] Centers for Disease Control and Prevention. Inhibitors and hemophilia. Last reviewed: April 2023.

https://www.cdc.gov/ncbddd/hemophilia/inhibitors.html.
[5] Peyvandi F, Garagiola I, Seregni S. Future of coagulation factor replacement therapy. J Throm Haemost. 2013;11 (Suppl. 1):84–98.
[6] Multiple Myeloma Research Foundation (MMRF): What is Multiple Myeloma?; Accessed May 30, 2023; https://themmrf.org/multiple-myeloma/.
[7] American Cancer Society: Key Statistics About Multiple Myeloma; Accessed May 30, 2023; https://www.cancer.org/cancer/multiple-myeloma/about/key-statistics.html.
[8] World Health Organization: Globocan 2020: Multiple Myeloma; Accessed May 30, 2023; https://gco.iarc.fr/today/data/factsheets/cancers/35-Multiple-myeloma-fact-sheet.pdf.
[9] Mikhael, J, Ismaila N, Cheung M, et al. Treatment of multiple myeloma: ASCO and CCO joint clinical practice guideline. J Clin Oncol. 37:1228-1263.
[10] Dimopoulos MA, Richardson P, Lonial S. Treatment options for patients with heavily pretreated relapsed and refractory multiple myeloma. *Clin Lymphoma Myeloma Leuk*. 2022;22(7):460-473. doi:10.1016/j.clml.2022.01.011
[11] Guillaume X, Horchi D, Gomez J, et al. Real-world treatment patterns of triple-class refractory (TCR) multiple myeloma (MM) across the United States (Us), Canada, and Western Europe: a retrospective chart study. American Society of Clinical Oncology (ASCO) 2023, June 2-6, in Chicago, IL.
[12] Bazarbachi AH, Al Hamed R, Malard F, Harousseau JL, Mohty M. Relapsed refractory multiple myeloma: a comprehensive overview. *Leukemia*. 2019 Oct;33(10):2343-57.

Category: Research and Pipeline, Prescription Medicines

View source version on businesswire.com: https://www.businesswire.com/news/home/20231102445115/en/

Pfizer Contacts:

Media Contact:
+1 (212) 733-1226
PfizerMediaRelations@Pfizer.com

Investor Contact:
+1 (212) 733-4848
IR@Pfizer.com

Source: Pfizer Inc.