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FDA Accepts Pfizer's Application for Hemophilia B Gene Therapy Fidanacogene Elaparvovec

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- *Regulatory application also under review with EMA*
- *Submissions based on positive Phase 3 data from BENEGENE-2 trial*

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) announced today that the U.S. Food and Drug Administration (FDA) has accepted the company's Biologics License Application (BLA) for fidanacogene elaparvovec for the treatment of adults with hemophilia B. In parallel, the European marketing authorization application (MAA) for fidanacogene elaparvovec has also been accepted and is under review by the European Medicines Agency (EMA).

Fidanacogene elaparvovec is a novel, investigational gene therapy that contains a bio-engineered adeno-associated virus (AAV) capsid (protein shell) and a high-activity variant of human coagulation Factor IX (FIX) gene. For people living with hemophilia B, the goal of this gene therapy is to enable them to produce FIX themselves via this one-time treatment rather than needing regular intravenous infusions of FIX, as is the current standard of care.

"Gene therapy marks a new era of scientific advancement, and if approved, we believe fidanacogene elaparvovec has the potential to transform the lives of people living with hemophilia B who are eligible for treatment. We look forward to continuing to work with global regulatory authorities to bring this innovative potential treatment to patients as quickly as possible," said Chris Boshoff, M.D., Ph.D., Chief Development Officer, Oncology and Rare Disease, Pfizer Global Product Development. "Patients are at the center of our legacy of innovation in hemophilia. Despite significant progress in their treatment, those living with hemophilia continue to experience disruption to daily life and need new options."

The submissions for fidanacogene elaparvovec are based on efficacy and safety data from the Phase 3 BENEGENE-2 study (NCT03861273). As previously reported, the BENEGENE-2 study met its primary endpoint of non-inferiority and superiority in the annualized bleeding rate (ABR) of total bleeds post-fidanacogene elaparvovec infusion versus prophylaxis regimen with FIX, administered as part of usual care. Fidanacogene elaparvovec was generally well-tolerated, with a safety profile consistent with Phase 1/2 results.

The FDA has set a Prescription Drug User Fee Act (PDUFA) goal date in the second quarter of 2024. Fidanacogene elaparvovec has been granted Breakthrough, Regenerative Medicines Advanced Therapy (RMAT) and orphan drug designations from the FDA.

Pfizer currently has three Phase 3 programs investigating gene therapy in populations where there is a high unmet need: hemophilia B, hemophilia A, and Duchenne muscular dystrophy. A Phase 3 trial is also ongoing investigating marstacimab, a novel, investigational anti-tissue factor pathway inhibitor (anti-TFPI) being studied for the treatment of people with hemophilia A and B with and without inhibitors.

About fidanacogene elaparvovec

Fidanacogene elaparvovec is a novel, investigational gene therapy that contains a bio-engineered AAV capsid and a high-activity variant of human coagulation FIX gene. For people living with hemophilia B, the goal of this gene therapy is to enable them to produce FIX themselves via this one-time treatment rather than needing regular intravenous infusions of FIX, as is the current standard of care.

In December 2014, Pfizer licensed fidanacogene elaparvovec from Spark Therapeutics. Under the agreement, Pfizer assumed responsibility for pivotal studies, any regulatory activities, and potential global commercialization of this investigational gene therapy.

About BENEGENE-2

The BENEGENE-2 study is a Phase 3, open label, single arm study to evaluate the efficacy and safety of fidanacogene elaparvovec in adult male participants (age 18–65) with moderately severe to severe hemophilia B (defined as FIX circulating activity of 2% or less). The main objective of the study is to evaluate the ABR for participants treated with gene therapy versus FIX prophylaxis replacement regimen, administered as part of usual care.

The study enrolled 45 participants. Eligible study participants have completed a minimum six months of routine FIX prophylaxis therapy during the lead in study (NCT03587116) and received one intravenous dose of fidanacogene elaparvovec at a dose of 5e11 vg/kg. Participants in the BENEGENE-2 study were screened with a validated assay designed to identify individuals who test negative for neutralizing antibodies to the gene therapy vector.

The BLA and MAA submissions are based on a 15-month data cut from the BENEGENE-2 study. Clinical trial participants will be followed for up to a total of 15 years, including six years in the BENEGENE-2 study and an additional nine years as part of a separate Phase 3 study (NCT05568719) to learn about the long-term safety and efficacy of fidanacogene elaparvovec.

About Hemophilia B

Hemophilia is a rare genetic bleeding disorder that causes the blood to take a long time to clot because of a deficiency in one of several blood clotting factors and is predominately found in males[1]. People with hemophilia are at risk for excessive and recurrent spontaneous and/or post-traumatic bleeding, which can be life-threatening, particularly in those with severe hemophilia. People with severe hemophilia often bleed spontaneously into their muscles or joints, or rarely into other critical closed spaces such as the intracranial space, where bleeding can be fatal[2].

According to the World Federation of Hemophilia, more than 38,000 people worldwide were living with hemophilia B in 2021[3]. People with hemophilia B have a deficiency in clotting FIX, a specific protein in the blood. Hemophilia B also is called congenital FIX deficiency or Christmas disease. The current standard of care requires recurrent intravenous infusions of either plasma-derived or recombinant FIX to control and prevent bleeding episodes[4].

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 June 27, 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 fidanacogene elaparvovec and the fidanacogene elaparvovec program, 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 drug applications for any potential indications for fidanacogene elaparvovec may be filed in any other jurisdictions; whether and when the FDA and EMA may approve the pending applications for fidanacogene elaparvovec for the treatment of adults with hemophilia B and whether and when regulatory authorities in any jurisdictions may approve any such other applications that may be pending or filed for fidanacogene elaparvovec, 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 fidanacogene elaparvovec 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 fidanacogene elaparvovec; uncertainties regarding the impact of COVID-19 on Pfizer's 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 .

Category: Medicines

[1] Hemophilia. Mayo Clinic. October 7, 2021. Accessed May 31, 2023. https://www.mayoclinic.org/diseases-conditions/hemophilia/symptoms-causes/syc-20373327.

[2] What is hemophilia. Centers for Disease Control and Prevention. August 1, 2022. Accessed May 31, 2023. https://www.cdc.gov/ncbddd/hemophilia/facts.html#:~:text=Hemophilia%20is%20usually%20an%20inherited,can%20h
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[3] Report on the annual global survey 2021 - World Federation of Hemophilia (no date) Annual Global Survey 2021. Accessed May 31, 2023. Available at: https://www1.wfh.org/publications/files/pdf-2324.pdf.

[4] What is hemophilia. Centers for Disease Control and Prevention. August 1, 2022. Accessed May 31, 2023. https://www.cdc.gov/ncbddd/hemophilia/facts.html#:~:text=Hemophilia%20is%20usually%20an%20inherited,can%20h
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