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New Gene Therapy Approval for Treatment of Hemophilia B

By: RGA

ETS Insights and Commentary: What You Should Know

Pfizer's Beqvez™ Receives FDA Approval

On April 26, 2024, the U.S. Food and Drug Administration (FDA) approved Pfizer Inc. for Beqvez (fidanacogene elaparvovec), its gene therapy for the treatment of adults with moderate to severe hemophilia B who currently use factor IX (FIX) prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes, and do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test.

According to Pfizer representatives, Beqvez has a list price of $3.5 million, pricing it the same as Hemgenix® (etranacogene dezaparvovec) from CSL Behring, which was approved in November 2022 for adults with hemophilia B.

Edition 2
Volume 4

A Division of Companion Life Insurance Company

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By the Numbers

The US Centers for Disease Control and Prevention (CDC) estimates that the incidence of hemophilia B is one in 19,283 male births. The number of people living with hemophilia B is estimated to be 3.7 cases per 100,000 males. Hemophilia B occurs in all races and ethnicities, but is more common in people of white race.

Key Considerations

In 2022, we saw approval of the first gene therapy for hemophilia B, Hemgenix, from CSL Behring and UniQure. What was thought to be an epic gene therapy has shown slow uptake in the U.S. Beqvez now provides another option for those with hemophilia B looking for a gene therapy treatment. However, some of the eligible population may currently be treated successfully with factor protein replacements and might not seek out a gene therapy option.

Beqvez is for adults with moderate to severe hemophilia B who currently use drugs to prevent bleeds or have repeated, spontaneous bleeding. Eligible individuals also must be tested to determine whether they have antibodies that neutralize Beqvez's effects.

Pfizer has set the list price of Beqvez equal to its competitor, Hemgenix, at $3.5 million. This is the first approved gene therapy for Pfizer. The company shared that this therapy has been 40-plus years in the making, and is a gene therapy that it acquired from Spark Therapeutics.

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Pfizer is proactively working with treatment centers, payers, and the hemophilia community in order to administer this therapy to patients with hemophilia B who may benefit from the gene therapy.

Per the Pfizer press release, the FDA approval of Beqvez is based on the results from the pivotal BENEGENE-2 study. This is a phase III, open-label, single-arm study that evaluates the efficacy and safety of Beqvez 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 annualized bleeding rate (ABR) for participants treated with gene therapy versus FIX prophylaxis replacement regimen, administered as part of usual care.

The Pfizer press release states that BENEGENE-2 met its primary endpoint associated with non-inferiority in the ABR of total bleeds post-Beqvez infusion versus prophylaxis regimen with FIX, administered as part of usual care. Bleeds were eliminated in 60% of patients compared to 29% in the prophylaxis arm. Beqvez was generally well-tolerated in patients who received it. The most common adverse reaction (incidence ≥5%) reported in the phase III and phase I/II clinical trials was an increase in transaminases, which was observed in 36 out of 60 patients treated at the recommended dose, and 31 out of 60 patients received corticosteroids. No deaths or serious adverse events were reported related to treatment or associated with infusion reactions, thrombotic events, or FIX inhibitors.

Hemgenix works by using an adeno-associated virus five (AAV5) vector to deliver a working version of the F9 gene to the liver cells, which then use the gene to make FIX on their own.

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Beqvez, on the other hand, uses the adeno-associated virus serotype Rh74var (AAVRh74var) capsid to deliver a functional copy of the F9 gene, allowing the liver cells to produce their own FIX. Both Hemgenix and Beqvez are a one-time therapy administered in an outpatient setting. Neither treatments are approved for individuals that have developed inhibitors. Both therapies recommend monitoring for at least three hours after infusion and require regular follow-up post administration. The most common adverse reaction associated with Beqvez is an increase in transaminase, and for Hemgenix is elevated ALT (alanine transaminase).

The therapies compete with each other and may provide options for patients who are interested in gene therapy but have developed antibodies against one of the vectors, since the two therapies utilize different AAV vectors to deliver the F9. However, it is still anticipated that utilization may be conservative at this time due to other treatment options that are available.

Briefing on Hemophilia B

Condition Overview

Hemophilia B is an inherited (genetic) blood disorder in which the body's normal clotting process is impaired due to a gene mutation. The mutation causes a deficiency or abnormality in the development of the coagulation Factor IX, which is necessary for appropriate blood clotting. Depending upon the baseline amount of Factor IX made by the individual's body, hemophilia B can be mild, moderate, or severe.

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In mild hemophilia B, bleeding may occur due to an injury, or after surgery or a dental procedure. Severe hemophilia may present shortly after birth or during toddler years when trauma-related injuries occur. This causes prolonged bleeding from minor injuries or can occur spontaneously with bleeding into vital organs, joints, and muscles. About 30 to 50% of individuals with hemophilia B have the severe form.4

Hemophilia B is usually inherited in an X-linked recessive pattern, meaning it almost always occurs in males and is passed from mother to son by a gene carried on the X chromosome. Females are typically carriers of hemophilia B meaning they have one affected (variant) copy and one normal copy of the gene but do not have any signs or symptoms of the condition. A small proportion of carrier females are at risk of abnormal bleeding.

Drug Patent Bill Could Cut the Federal Deficit by $3B, analysts predict

By: Allison Bell

A popular, bipartisan drug patent bill could narrow federal budget deficits by a total of about $3 billion over 10 years, according to the Congressional Budget Office.

CBO analysts reported last week that the new version of the Affordable Prescriptions for Patients Act bill could cut spending by $2.4 billion over the period from 2024 through 2034 and increase federal revenue by $585 million over that same period.

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The bill would prohibit drug manufacturers from using aggressive legal maneuvers to keep drugs covered by patents and avoid letting new generic competitors lower the cost.

Sen. John Cornyn, R-Texas, introduced the bill together with four Republican co-sponsors and four Republican co-sponsors.

This Congress is the 118th, and a Congress lasts for two years. Cornyn began introducing earlier versions of the current bill in the 116th Congress.

The CBO has been giving the bill better deficit-fighting scores over time. It predicted the version released in 2019 would save just $507 million, and that the version saved in 2021 would save $1.1 billion. The CBO expects the federal debt to be $16 trillion in 2034, meaning that even $3 billion in savings could amount to far less than 0.1% of the 2023 federal debt.

Patents on drugs typically last 20 years from the time a patent application is filed.

The Cornyn bill would give the Federal Trade Commission authority to rein in prescription drug "ever greening" or "patent hopping" — moves to replace old drugs that are coming off patent with freshly patented drugs.

Because the FTC is involved, the Senate Judiciary Committee has jurisdiction over the bill.

The committee approved the bill by a voice vote in February 2023. Members talked about the patent hopping at a hearing in May.

Edition 2
Volume 4

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