PHARMA

Pfizer scores FDA nod for hemophilia B gene therapy, will charge $3.5M per dose

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Pfizer will offer a warranty program for its first FDA-approved gene therapy. The hemophilia B treatment will go for $3.5M, matching the price tag on CSL and uniQure's hemophilia B gene therapy Hemgenix. (Pfizer)

Ten years after dipping its toes into the gene therapy pool in a licensing deal with Spark Therapeutics, Pfizer has gained FDA approval for the acquired treatment.

The U.S. regulator has endorsed (https://investors.pfizer.com/Investors/News/news-details/2024/U.S.-FDA-Approves-Pfizers-BEQVEZ-fidanacogene-elaparvovec-dzkt-a-One-Time-Gene-Therapy-for-Adults-with-Hemophilia-B/default.aspx) Beqvez (fidanacogene elaparvovec-dzkt) for adults with the bleeding disorder hemophilia B. It becomes the first FDA-approved gene therapy for Pfizer and the second in the indication following CSL and uniQure's hemophilia B treatment Hemgenix, which became (https://www.fiercepharma.com/pharma/csl-and-uniqures-hemophilia-b-gene-therapy-scores-approval-35-million-price-tag) the world's most expensive drug at $3.5 million when it was approved in 2022.

Pfizer had the chance to undercut its rival on price but decided to charge the same $3.5 million for Beqvez. The therapy will be available to patients this quarter, a spokesperson confirmed on Friday to Fierce Pharma.

According to the CDC, the current number of males with hemophilia living in the U.S. is around 33,000. Hemophilia A is more prevalent, with 12 cases per 100,000 U.S. males, while there are just 3.7 cases per 100,000 U.S. males for hemophilia B.

Last month, Kyowa Kirin and Orchard Therapeutics ascended (https://www.fiercepharma.com/pharma/kyowa-kirins-orchard-sets-new-gene-therapy-price-tag-425m-steepest-any-drug-us) to the highest pricing throne, placing a $4.25-million tag on Lenmeldy, their gene therapy for the rare genetic disease metachromatic leukodystrophy (MLD).

As for the price it has set for Beqvez, Pfizer said it presents a "compelling value proposition that can deliver savings for the healthcare system when you consider potential long-term benefits that would offset costs that are associated with chronic disease management today."

Beqvez can free patients from standard-of-care intravenous transfusions that are often administered several times per week or month and can cost as much as $600,000 and all the way up to $1.1 million per year, Pfizer said.

The company added that it is offering a warranty program that will provide "financial protections by insuring against the risk of efficacy failure."

In 2014, Pfizer kicked off (https://www.fiercebiotech.com/partnering/pfizer-buys-into-gene-therapy-s-renaissance-and-bets-on-spark-therapeutics) its gene therapy program, paying just $20 million upfront and $260 million in potential milestones to Spark for the product, with an agreement that the Philadelphia gene therapy specialist would handle phase 1 and 2 development and Pfizer would take it from there. Five years later, Roche bought out (https://www.fiercepharma.com/pharma/roche-bulks-up-gene-therapy-and-hemophilia-4-3b-spark-buyout) Spark for $4.3 billion, triggering its move into the gene therapy arena.

The approval is not the first for Beqvez. In January, Canada signed off (https://www.fiercepharma.com/pharma/pfizer-welcomes-new-year-hemophilia-b-gene-therapy-nod-canada) on the adeno-associated viral (AAV) vector-based treatment. Both nods are for moderate-to-severe hemophilia B patients who are negative for neutralizing antibodies to variant AAV serotype Rh74.

Hemophilia B is a rare genetic disorder that prevents normal blood clotting for people who can't generate the factor IX (FIX) protein. According to the World Federation of Hemophilia, 38,000 people have the disorder, which causes them to bleed more often and more profusely.

"Many people with hemophilia B struggle with the commitment and lifestyle disruption of regular (factor IX protein) infusions, as well as spontaneous bleeding episodes, which can lead to painful joint damage and mobility issues," Adam Cuker, M.D., the director of the Penn Comprehensive and Hemophilia Thrombosis Program, said in a release.

Pfizer also has gene therapies under investigation in phase 3 for hemophilia A (giroctocogene fitelparovec) and Duchenne muscular dystrophy (fordadistrogene movaparovec). The company also is testing an anti-tissue factor pathway inhibitor to treat people with hemophilia A and B. The FDA and European Medicines Agency are reviewing applications for that treatment, marstacimab.

Last year, Pfizer sold off (https://www.fiercebiotech.com/biotech/astrazeneca-axes-assets-inks-pfizer-deal-and-loses-key-exec-friday-news-flurry) a portfolio of preclinical rare disease gene therapies to AstraZeneca for up to $1 billion plus tiered royalties.

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