PHARMA

Vertex, CRISPR's gene-editing therapy Casgevy wins early FDA nod to treat beta thalassemia

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CRISPR | Vertex Pharmaceuticals | FDA | Casgevy (exa-cel)

The FDA has approved Vertex and CRISPR's gene-editing therapy Casgevy to treat beta thalassemia. Casgevy becomes the first therapy for the rare blood disorder to use the innovative CRISPR gene-editing technology. (Stock photo/Getty Images)

Vertex Pharmaceuticals and CRISPR Therapeutics have scored (https://investors.vrtx.com/news-releases/news-release-details/vertex-announces-us-fda-approval-casgevytm-exagamglogene) an FDA approval for their gene-editing therapy Casgevy (exa-cel) to treat transfusion-dependent beta thalassemia (TDT). The approval comes less than six weeks after the U.S. regulator signed off on Casgevy to treat patients with sickle cell disease (SCD).

With the TDT nod, Casgevy becomes the first treatment for the rare blood disorder using CRISPR gene-editing technology. Bluebird Bio also gained approval in 2022 for its gene therapy, Zynteglo, to treat TDT.

The endorsement arrived more than two months early as an FDA decision was due on March 30.

“On the heels of the historic FDA approval of Casgevy for sickle cell disease, it is exciting to now secure approval for TDT well ahead of the PDUFA date,” Reshma Kewalramani, M.D., Vertex’s CEO said in a release.

The companies have opened nine authorized treatment centers (ATCs) to administer Casgevy to patients, Vertex said. Each of the facilities can provide Casgevy in either indication.

Additional ATCs will be activated “in the coming weeks,” Vertex added.

The approval covers TDT patients ages 12 and older who need regular transfusions.

Vertex and CRISPR charge $2.2 million for the one-time treatment. A lifetime of healthcare costs in the U.S to manage TDT are estimated to exceed $5 million, the companies said.

Patients living with TDT can experience fatigue and shortness of breath. Infants may develop jaundice and feeding problems. Complications of TDT can also include an enlarged spleen, liver and/or heart, misshapen bones and delayed puberty. In the U.S., the median age of death for patients living with TDT is 37 years.

On Dec. 8 of last year, on the same day that the FDA gave a thumbs up to Casgevy for SCD, it also approved bluebird's gene therapy for the same condition. Bluebird has priced its treatment, Lyfgenia, at $3.1 million. The Cambridge, Mass. company charges $2.8 million for its TDT drug Zynteglo.

CRISPR | Vertex Pharmaceuticals | FDA | Casgevy (exa-cel) | beta thalassemia | Bluebird Bio | sickle cell disease | Lyfgenia