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Saudi Arabia approves first CRISPR/Cas9 gene-edited therapy for treatment of sickle cell disease

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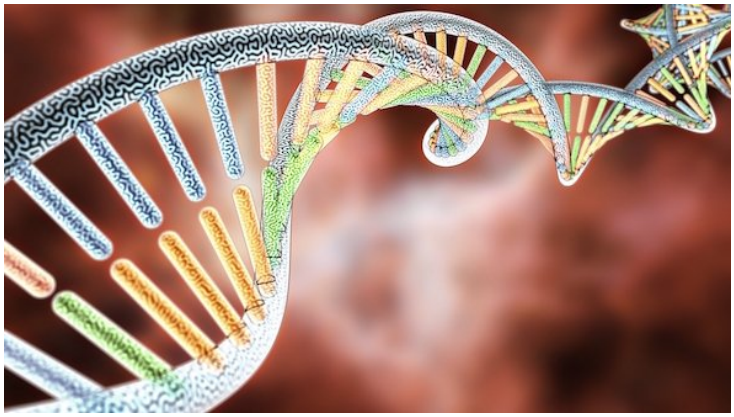
CASGEVY is first medicine ever to be evaluated through the SFDA's Breakthrough Medicines Programme



US-based Vertex Pharmaceuticals Incorporated has announced that the Saudi Food and Drug Authority (SFDA) has granted Marketing Authorisation for CASGEVY (exagamglogene autotemcel [exa-cel]), a CRISPR/Cas9 gene-edited therapy, for the treatment of sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

CASGEVY is approved for the treatment of people 12 years of age and older with SCD or TDT. The Kingdom of Saudi Arabia has among the highest prevalence rates of SCD and TDT in the world, with thousands of patients living with these genetic blood disorders.

"This approval adds to the list of firsts for CASGEVY. It represents the first medicine ever to receive SFDA Breakthrough Designation and be approved through this pathway, and Vertex's first ever regulatory approval in the Kingdom of Saudi Arabia," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex.

The Ministry of National Guard Health Affairs (MNGHA) is the first Authorized Treatment Center (ATC) in Saudi Arabia. Vertex is working to qualify additional hospitals as ATCs to bring CASGEVY to patients, including the King Faisal Specialist Hospital (KFSH). In order to enable rapid access to CASGEVY, Vertex is working to secure listing on hospital formularies to support reimbursement as soon as possible.