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ASIA EDITION

FDA lifts clinical hold on gene therapy for sickle cell disease

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CRISPR and Vertex entered into a strategic research collaboration in 2015 aimed at the discovery and development of gene editing treatments using the CRISPR/Cas9 technology to correct defects in specific gene targets known to cause or contribute to particular diseases.

CRISPR Therapeutics and Vertex Pharmaceuticals have announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold and accepted the Investigational New Drug application (IND) for CTX001 for the treatment of sickle cell disease (SCD). CTX001 is an investigational, autologous, gene-edited hematopoietic stem cell therapy for patients suffering from severe hemoglobinopathies.

In addition to the acceptance of the IND announced today, CRISPR and Vertex previously announced that they had obtained approvals of Clinical Trial Applications for CTX001 in multiple countries outside the U.S. for both ?-thalassemia and SCD. The companies remain on track to initiate a Phase 1/2 clinical study in SCD by the end of 2018 and are currently enrolling patients with transfusion dependent ?-thalassemia in a Phase 1/2 trial in ?-thalassemia in Europe.

CTX001 is an investigational **ex vivo** CRISPR gene-edited therapy for patients suffering from ?-thalassemia or sickle cell disease in which a patient's hematopoietic stem cells are engineered to produce high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is a form of the oxygen carrying hemoglobin that is naturally present at birth and is then replaced by the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion-requirements for ?-thalassemia patients and painful and debilitating sickle crises for sickle cell patients.

CTX001 is being developed under a co-development and co-commercialization agreement between CRISPR Therapeutics and Vertex. The companies have obtained approvals of Clinical Trial Applications (CTA) in multiple countries for both ?-thalassemia and sickle cell disease (SCD).

CRISPR and Vertex entered into a strategic research collaboration in 2015 aimed at the discovery and development of gene editing treatments using the CRISPR/Cas9 technology to correct defects in specific gene targets known to cause or contribute to particular diseases. Vertex has exclusive rights to license up to six new CRISPR/Cas9-based treatments that emerge from the collaboration, and CTX001 represents the first treatment to emerge from the joint research program. For CTX001, CRISPR and Vertex will equally share all research and development costs and profits worldwide.