CTX001

CTX001 is an investigational ex vivo CRISPR gene-edited therapy for patients suffering from β-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 β-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 β-thalassemia and sickle cell disease (SCD).