# Globin Gene Therapy

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## Technology description

## Summary of Invention

A team of Memorial Sloan Kettering investigators has developed a path-breaking stem-cell-based gene therapy for patients with the inherited blood disorder beta (β)-thalassemia. This condition is caused by deficient production of the β chain of hemoglobin, and for most patients it is incurable, since they lack matched donors for bone marrow or stem cell transplants.
MSK’s innovative globin gene transfer therapy offers the potential to free patients from a lifetime of red blood cell transfusions and iron chelation therapy. This potentially curative treatment involves extracting a patient’s hematopoietic stem cells (HSCs), utilizing a lentiviral vector to stably insert a functional β-globin gene, and then infusing the patient with engineered HSCs that should now be capable of producing red blood cells and hemoglobin. Preclinical proof-of-concept, IND filing, and a pilot trial testing the safety and efficacy of mobilizing CD34+ hematopoietic progenitor cells in adults with β-thalassemia major have all been achieved.
An ongoing Phase 1 trial at MSK was the first to win FDA approval to treat β-thalassemia with genetically engineered cells. Investigators are currently working on optimizing manufacturing of their vector, which is believed to achieve better expression of β-globin on a per-copy basis, and therefore the potential for superior clinical results, than other vectors utilized in treatments under development elsewhere.

## Key Publications

Boulad F et. al, Blood. 2014 Mar 6;123(10):1483-6 (PMID 24429337 )

## Market Need

β-thalassemia and sickle cell disease are the most common, severe hereditary blood disorders worldwide, with several million people affected and more than 50,000 born with these conditions each year. There is an urgent unmet need for curative therapy, which holds the potential to transform patient’s quality of life and life expectancy.

## Advantages

An ultra-orphan indication with larger, related markets and strong commercial potential
An exceptional scientific team with more than two decades of groundbreaking research in this field
Use of a vector that is well-protected on the IP front and may offer competitive advantages

## Institution

Memorial Sloan-Kettering Cancer Center

## Inventors

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