Isabelle Rivière (left), Director of the Cell Therapy and Cell Engineering Facility, and Michel Sadelain, Director of the Center for Cell Engineering

Summary

Memorial Sloan Kettering's trial to evaluate a new therapy for patients with beta-thalassemia is the first to receive FDA approval to treat this disease with genetically engineered cells.

Memorial Sloan Kettering Cancer Center will begin evaluating a new stem-cell-based gene therapy for patients with the inherited blood disorder beta (β)-

thalassemia. The clinical trial is the first to receive US Food and Drug Administration approval to treat this disease with genetically engineered cells.

This potential new treatment is a culmination of more than two decades of research led by Memorial Sloan Kettering investigators. If the trial proves to be successful, the treatment could offer hope of a cure for patients with this severe blood disease, as well as related conditions such as sickle cell disease.

“Launching this trial is a major milestone for all the people at Memorial Sloan Kettering and international collaborators who have contributed to this work,” says Michel Sadelain, who leads the Center for Cell Engineering. “Our team was the first to show this approach was possible in disease models, and I’m thrilled to be able to finally start offering this potentially curative therapy to patients.”

A Hereditary Blood Disorder

β-thalassemia is found in people of Mediterranean, Asian, and African descent. It is characterized by the inability of red blood cells to make a protein called β-globin. In sickle cell disease, a related disorder affecting the same gene, the red blood cells make an abnormal form of β-globin.

Together, 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 every year.

The current treatment for β-thalassemia is a lifetime of regular red blood cell transfusions, which are lifesaving but fraught with serious secondary complications. Bone marrow or stem cell transplants can offer a cure, but more than three-quarters of patients are unable to find a matched donor.

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Hope for Patients

The trial, which is now enrolling patients, is led by Farid Boulad, a pediatric hematologist-oncologist and transplant specialist, together with Isabelle Rivière, Director of Memorial Sloan Kettering’s Cell Therapy and Cell Engineering Facility, and Dr. Sadelain.

In the trial, patients will have their blood stem cells extracted from circulating blood — a process in which the stem cells are filtered out of the patients’ blood while their other blood cells are returned to them. Investigators will then use a vector to introduce a functional version of the β-globin gene into patients’ stem cells. Vectors are disabled viruses that cannot replicate but efficiently shuttle their genetic cargo into host cells.

“Memorial Sloan Kettering has one of the finest cell therapy facilities in the world to expand and engineer patient cells for clinical studies in patients with cancer and genetic disorders,” says Dr. Rivière.

After receiving a low dose of chemotherapy to suppress the body’s natural production of blood cells, patients will have their own genetically engineered stem cells infused back into them.

“Treating a genetic defect with a reconstructed gene is something we dreamed about in medical school,” says Dr. Boulad. “The fact that it is now a reality is amazing. It is the holy grail of the treatment of genetic disorders.“

The trial will eventually be extended to patients at other institutions, including the National Institutes of Health and the University of Washington. Dr. Sadelain’s group is coordinating with investigators in Italy and Greece and throughout Asia — areas where β-thalassemia is much more prevalent — to offer the treatment to patients there as well.

Only a small handful of diseases are currently treated with this type of gene-transfer therapy, and all but one of them are rare immune disorders. If the treatment proves effective, β-thalassemia would be by far the most common condition to be successfully treated in this way. Memorial Sloan Kettering investigators are also preparing a follow-up study to eventually treat patients with sickle cell disease in a similar fashion.

“Many of the people who have β-thalassemia live in parts of the world that are medically underserved,” Dr. Sadelain explains. “Although the disease is little known in the United States, its incidence and treatment take a large toll worldwide.”

The research that led to this FDA-approved trial was supported by the National Institutes of Health under award numbers HL53750, HL57612, and HL66952; Errant Gene Therapeutics LLC; the Cooley’s Anemia Foundation; the Leonardo Giambrone Foundation; Cooley’s Anemia International; and the Stavros Niarchos Foundation.

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