

HEALTH

Medical leap gives hope to blood disorder sufferers

APIRADEE TREERUTKUARKUL

Gene therapy for the blood disorder beta-thalassemia will be carried out in Thailand for the first time by the end of this year.

A team of doctors at Ramathibodi Hospital is studying the gene therapy technology alongside experts in Paris under a collaboration programme between the Mahidol University led by Prof Suthat Fucharoen and French-American researcher Philippe Leboulch of Harvard Medical School and the University of Paris.

The Thai doctors expect to return to Thailand to conduct a trial around December, said Dr Suradej Hongeng, of Ramathibodi Hospital's department of pediatrics.

The collaboration came about after the world's first successful treatment of beta-thalassemia with gene therapy.

A 21-year-old Frenchman treated with the therapy in 2007 now no longer has the need for blood transfusions. He previously had required transfusions every month since birth.

The successful treatment was published in the journal *Nature* last September.

Beta-thalassemia is caused when a

patient cannot produce enough of the beta-globin component of haemoglobin, the protein used by red blood cells to carry oxygen around the body. This can cause life-threatening anaemia, leading to severe damage of the body's major organs.

Gene therapy is generally the insertion, alteration or removal of genes within a patient's cells and biological tissues to treat disease.

"This success justifies the hopes placed in the use of gene therapy to treat blood diseases," said Dr Suradej, a haematology specialist.

"It is also the first time an effective technology has been developed to improve the quality of life for people with thalassemia."

An estimated 20 million Thais are carriers of thalassemia. It is one of the world's most common genetic disorders, putting an enormous financial strain on Thailand and countries located in the "Thalassemia Belt", which stretches from the Mediterranean through the Middle East and Central Asia to Southeast Asia.

About three in 800 children born in Thailand are affected by the severest form of the disorder, beta-thalassemia, requiring regular blood transfusions.

However, blood transfusions carry the risk of contracting HIV and hepatitis B and C from donors, or iron overloading.

The only known cure for the condition is through a bone marrow transplant.

However, this process is dangerous and it can be very difficult to find a matching bone marrow donor, Dr Suradej said.

He hoped the gene therapy for thalassemia treatment would eliminate the problems posed by bone marrow transplants, as well as lead doctors to adapt the technology to treat the symptoms of beta-thalassemia, such as neurological problems and muscle disabilities.