Gene therapy offers new hope for healing sickle cell disease

In a world first, this blood disease which afflicts babies has been successfully treated by gene therapy. Two years after undergoing treatment, the patient is in complete remission.

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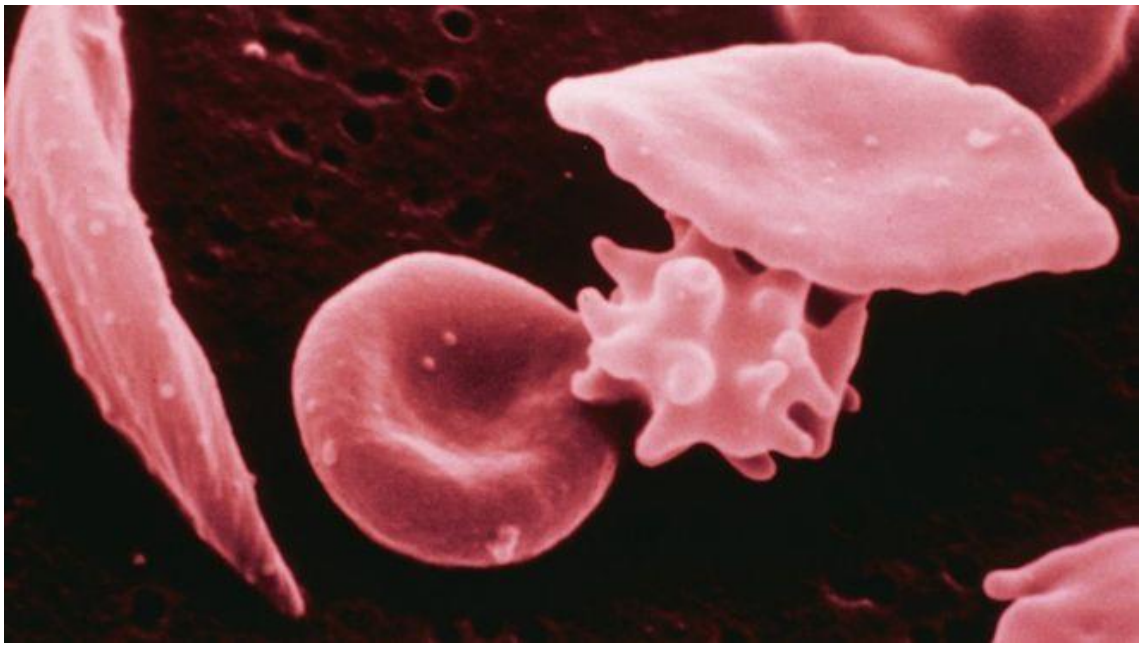

Healthy red blood cells are round, but the genetic defect makes them sickle shaped

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Great hope has been raised concerning the treatment of a blood disease affecting some 250,000 babies at birth each year around the world.

For the first time ever, a patient suffering from sickle cell disease has been successfully treated by means of gene therapy.

This breakthrough, which was announced on Thursday in an article in the *New England Journal of Medicine*, was achieved by a team from the Necker Hospital in Paris and the Imagine Institute (1).

“The patient, who is now aged fifteen, is doing very well,” explains Professor Marina Cavazzana, leader of the biotherapy department at Necker Hospital. “There is no longer any sign of the disease.

“Even though we now have two years of retreat, we prefer to remain prudent and speak of remission rather than healing,” she continued. “However, there is every indication that his condition will remain stable.”

Sickle cell disease is particularly frequent among people from the Antilles, Africa, and the Mediterranean. It is a genetic disease affecting hemoglobin, the substance in red blood cells that transports oxygen around the body.

In 2013, there were 441 children born in France with this disease, the symptoms of which vary greatly from person to person.

Most often, sickle cell causes lower resistance to infections as well as to anemia. Patients may also be victims of “vaso-occlusive” crises owing to poor blood circulation. These crises, which are often painful, may also attack certain organs, according to the Orphanet site on rare diseases.

Various treatments help reduce symptoms and diminish the frequency of crises. However, the only sustainable treatment is a bone marrow transplant.

“Mostly, this is not possible unless there is a compatible donor in the family,” says Professor Cavazzana. “And gene therapy can then offer a solution.”

In October 2014, Professor Cavazzana’s team applied the treatment to a 13-year-old adolescent suffering from severe sickle cell disease. First, doctors took samples of stem cells before introducing a “therapeutic” gene thanks to a vector developed by Professor Leboulch (CEA/University of Paris South).

Once “corrected,” the cells were later re-injected into the patient, who no longer requires treatment and has recommenced his studies.

At the moment, it is difficult to say when this therapy could be offered on a large scale.

“That is certainly the objective,” says Professor Cavazzana. “However, in the short term, we will continue clinical tests to confirm the validity of this approach.”

(1) This institute is affiliated with the Assistance Publique Hôpitaux de Paris (Paris Public Hospitals Assistance - APHP), INSERM and the University of Paris Descartes

https://international.la-croix.com/news/gene-therapy-offers-new-hope-for-healing-sickle-cell-disease/4781?utm_source=Newsletter&utm_medium=e-mail&utm_content=03-03-2017&utm_campaign=newsletter__crx_lci&PMID=ca4ce0563e46285947a35389589f090c

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