



A new success in gene therapy for Genethon, one of the partner laboratories of Evry University

A clinical trial of gene therapy has been conducted by Genethon* in France and in Great Britain and treated 7 children suffering from the Wiskott Aldrich Syndrome. Preliminary results published this week in JAMA are extremely encouraging.

The genetic disease of the immune system called « Wiskott Aldrich Syndrome » is induced by the mutation of the WAS gene which, when functional, produces the WAS protein essential to the function of white blood cells and the production of blood platelets. In this disease, the WAS protein is not produced and the cells are no longer capable of fighting off microbes and of performing their duty as sentinels of the body. The children affected by this disease, mostly boys, contract multiple infections; suffer from eczema, severe dermatitis and severe hemorrhages. They cannot live a “normal life”.

To treat this disease, children can receive a bone marrow transplant. However transplantation can be complicated and finding a compatible donor, can be sometimes impossible.

A genetic approach has been developed in Anne Galy's laboratory at Genethon to treat Wiskott Aldrich syndrome. Since the cause of disease is known and the missing gene has been identified, the teams take the patients deficient blood cells and correct them in the laboratory by inserting the functional WAS gene. The sick cells are eradicated and the patient receives the cells that have been corrected in the laboratory. These corrected cells will give birth to new cells capable of expressing the missing protein and function properly, restoring the immune system.

The clinical trial implemented in Paris and London since 2010 has treated 7 patients and, for 6 of them, a long-term follow up showed that gene therapy resolves the dermatitis problems, reduces and in some cases gets rid of infections, and stops the bleeding. A child who was particularly affected with vasculitis, has resumed walking and is now doing normal physical activities. These are very encouraging results.

This study was initiated in a mixed research unit from Evry University in Genethon, the UMR_S951 directed by Anne Galy.

*Genethon has been created in 1990 by AFM-Téléthon. Its Inserm Unit UMR_S951 is affiliated to Evry University, Inserm and The_Ecole Pratique des Hautes Etudes.

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