

Press release

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3rd complication case in DICS X gene therapy clinical trial

In May 2004, the French Health Product Safety Agency (Afssaps) authorised the restart of the gene therapy clinical trial conducted by Prof. Alain Fischer and Marina Cavazzana-Calvo, in Necker-Enfants-Malades hospital in Paris. The clinical trial is aimed at assessing efficacy of a gene therapy approach in the treatment of X-linked severe combined Immuno-deficiency (X-SCID), an inherited genetic disease.

This clinical trial, which included 11 patients, was put on hold in October 2002 (see press release October 3rd 2002), after a first notification of a complication in one of the patients had been observed, consisting in an uncontrolled T-lymphocyte proliferation. The same complication has been reported for a second patient at the end of 2002 (see Afssaps Press release dated 15 January 2003). The hold was maintained until analysis and identification of the mechanism(s) responsible. One of the patients died last October, the other is progressively recovering.

The clinical trial has been authorised to resume after the investigators proposed several protocol modifications (number of administrated cells, inclusion criteria, age of the patients to be enrolled, etc.) aimed at reducing the risk of insertional oncogenesis. Since the restart of the clinical trial, one new patient has been treated.

On January 18th, 2005, a new complication was notified to Afssaps. It concerns a third child who was 9 months old when receiving the treatment in April 2002. This complication is also a T-lymphocytes proliferation, which characteristics are still under investigation.

Following this information, and in agreement with Afssaps, the investigators and promoter decided to put on hold the clinical trial again, and wait for further investigations in an attempt to better explain the mechanism of the adverse event.

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