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Small but important: researchers hope that changes to a gene vector will reduce risks to patients.

Gene therapy put on hold as third child develops cancer

Erika Check, Washington

Scientists have halted clinical trials of gene therapy to treat a rare immune disorder — less than a year after the trials were relaunched following an earlier stoppage.

The trials use gene therapy to treat different forms of severe combined immunodeficiency disease (SCID). The first trial to be stopped was halted in October 2002, and other trials were halted three months later, after two children in the trials developed cancer. But authorities allowed them to resume during the past year because the treatment had cured many children who lack reliable alternative treatments.

Researchers have now halted the trials again, after a third patient was found to have developed cancer. The suspension is a significant setback for the nascent field of gene therapy, because SCID treatment has been its most promising application to date.

The child with cancer was a patient of Alain Fischer of the Necker Hospital in Paris. He has been using gene therapy to treat the X-linked form of SCID, which is otherwise only treatable with bone-marrow transplant and is still often fatal. Fischer's trial restarted last May, and his team has treated one child since then.

But on 24 January, the French medical regulatory authority AFSSPS announced that a child who was treated by Fischer in April 2002 now has cancer.

As a result, Fischer's trial and similar ones in the United States have been halted again. The agency also said that one of the original two patients who had been diagnosed with cancer — both of whom were in Fischer's trial — died last October.

Fischer is now investigating why the third child, who was treated at a later age than the previous two children, developed cancer. The child's cells did not seem to have the same genetic glitch that caused the first two cancers, he says, but he cautions that the analysis is still under way.

Fischer adds that he still believes in gene therapy as a treatment for X-linked SCID, because 15 children treated in this way are still alive, and 14 are doing well four years later. But his group will not treat any more children using its current gene-therapy system, he says. He adds that he plans to change a key step in the treatment by changing the vector — the modified virus that delivers the therapeutic gene to the patients.

"The efficacy is there, but we have to improve on the safety," Fischer says, adding that this is "not an uncommon situation" in medical research. ■

NIH open-access plans draw fire from both sides

Erika Check, Washington

The US National Institutes of Health (NIH) has unveiled its long-awaited plan for open access to research findings. Elias Zerhouni, the NIH's director, claimed at a public briefing on 3 February that the plan could "change the landscape" of biomedical publishing.

The policy requests that authors whose research was funded by the NIH submit copies of their papers to the agency's National Library of Medicine after they are accepted for publication. The papers will then be placed in an online archive. Authors can decide when their papers are made available to the public, but the NIH would like this to happen as soon as possible, and in any case within 12 months of publication.

Scientists pushing for open access have praised the policy, which comes into effect on 2 May. "This is a significant and positive step and I'm glad we have the policy written down," says Harold Varmus, president of the Memorial Sloan-Kettering Cancer Center in New York.

But both sides of the debate have voiced criticism. Advocates of full open access are unhappy that the policy is voluntary and does not require access within six months of publication — a deadline that Zerhouni had proposed in a draft version (*Science* 306, 1895; 2004).

"This is a retreat from the earlier version of the policy, and the retreat is unjustified and regrettable," says Peter Suber, director of the Open Access Project at Public Knowledge, a non-profit advocacy group in Washington DC.

Suber and other critics say it would put researchers in the difficult position of having to negotiate between the NIH, which wants work available as soon as possible, and journals, which may want researchers to wait.

Publishers and societies that draw income from publishing criticize the NIH's plan to archive papers on its own site instead of directing the public to journal websites. NIH officials estimate that it will cost between $2 million and $4 million a year to run.

"The NIH is proposing to create a new publishing enterprise, and it's going to have to spend a lot of money to do that," says Marc Brodsky, chief executive of the American Institute of Physics.

Zerhouni said the announcement, expected on 11 January, had been delayed at the request of the health department and the White House. ■