Promising new gene therapy for “bubble boy” disease

More than a decade ago, doctors showed dramatic progress in helping infants born with a severe deficiency in their immune systems, a condition known as “bubble boy” disease. But when one quarter of treated children developed leukemia within two to five years, researchers went back to the drawing board.

Now a new variation of that therapy, which also uses a genetically-engineered virus to help the body create disease-fighting T-cells, is showing similar early promise, and none of the children who have received the therapy have show any evidence of cancer, according to preliminary results reported in the New England Journal of Medicine.

“It’s clear that this virus is effective. It does correct the T-cell defect in these patients as well as the last one did,” coauthor Dr. David A. Williams, chairman of hematology/oncology at Boston Children’s Hospital, told Reuters Health.

“We can’t say yet there will be no leukemias. In fact, we will follow these children for 15 years altogether,” he said. But he characterized the interim results as “encouraging.”

The rare condition is officially called X-linked severe combined immunodeficiency syndrome. It gets its “bubble boy” nickname because its victims are only safe in a sterile environment. The genetic defect leaves the boys – whose single X chromosome makes them targets of the disease – unable to fight infection. In the outside world, they usually die within a year.

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