Gene therapy may offer cure for debilitating ‘bubble boy disease’

Omarion was born with a rare genetic disorder called X-linked severe combined immunodeficiency (SCID), better known as the “bubble boy disease.” Caused by a mutated gene on the X chromosome, and almost always limited to males, a baby born with X-linked SCID, or SCID-X1, lacks a working immune system.

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[M]edical scientists at St. Jude Children’s Research Hospital in Memphis, Tennessee, were then developing a bold new procedure. The strategy: introduce a normal copy of the faulty gene, designated IL2RG, into a patient’s own stem cells, which then go on to produce the immune system components needed to fight infection. [His mother Kristin] Simpson enrolled Omarion in the clinical study.
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Within four months, some of the babies were able to fight infections on their own. All eight of the initial research subjects left the hospital with a healthy immune system.

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[W]ith additional trials and continued monitoring of patients, St. Jude hopes that the therapy will earn Food and Drug Administration approval as a treatment within five years.
Simpson, for her part, is already convinced that the therapy can work wonders: Her son doesn’t live in a bubble or, for that matter, in a hospital.

Read full, original post: These scientist may have found a cure for ‘bubble boy’ disease