Genome Surgery: The perfect cure for child in a bubble diseases
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Description
Severe Combined Immunodeficiency (scid), is a primary immune deficiency.

Bone Marrow transplantation
Higher likelihood for a good outcome when:
• Transplant done early, within the first few months of life.
• Child without severe infections or failure to thrive.
• Type of SCID with normal B cell function.
• Donor = close match.

Gene therapy by gene addition
Limit: risk of cancer development

Gene therapy by gene repair for scid mouse model: the perfect cure !!!

Children with immunodeficiencies have to live in a sterile environment to be protected against infections. It is known as "child in a bubble".