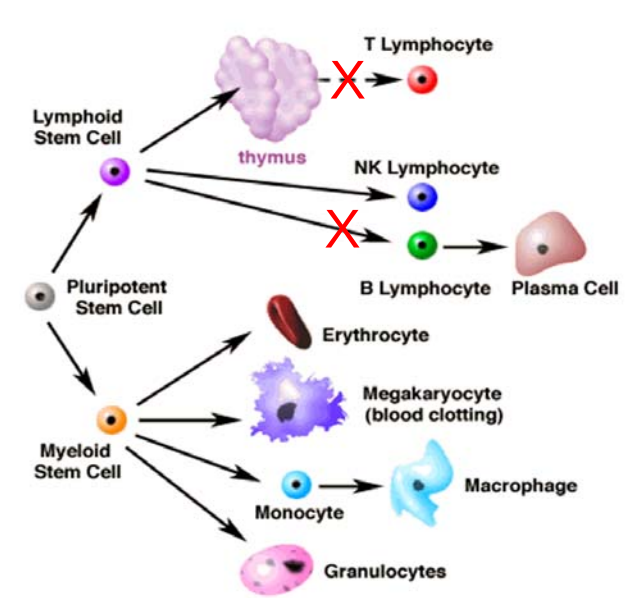
Genome Surgery: The perfect cure for child in a bubble diseases

Hayder Abdul-Razak, Céline Rocca and Rafael J. Yáñez-Muñoz

Genetic Therapy Laboratory, School of Biological Sciences, Royal Holloway-University of London, Egham, TW20 0EX

Description

Severe Combined Immunodeficiency (*scid*), is a primary immune deficiency.



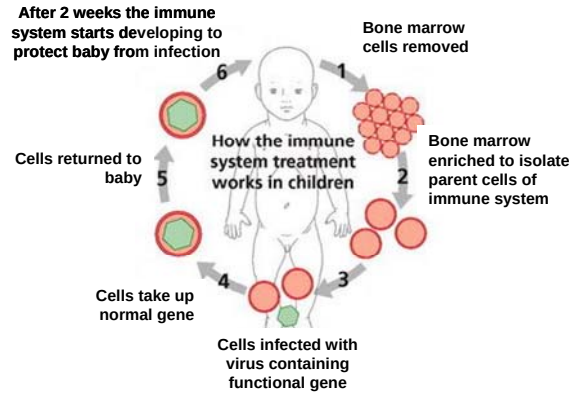
Children with immunodeficiencies have to live in a sterile environment to be protected against infections. It is known as "child in a bubble".

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 !!!