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From stem cells to functional lymphocytes: cell differentiation and gene therapy implementation for RAG-SCID

García Pérez, L.

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**From Stem Cells to Functional Lymphocytes:
Cell differentiation and gene therapy implementation for RAG-SCID**

Laura García Pérez

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Cover: Severe Combined Immunodeficiency is also commonly called the “bubble baby disease”. Babies affected with the disease need to live in a sterile environment symbolize by the bubble. The development of gene therapy to treat the disease is progressing successfully as described in this thesis allowing to reconstitute a normal immune system and therefore get out of (burst) the bubble. The development of these new complex therapies allows to progressively burst the bubbles into life.

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A mis padres y hermano por su apoyo y confianza

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