

COMPOSITIONS AND METHODS FOR STEM CELL TRANSPLANT

INNOVATION DESCRIPTION:

The present invention provides systems and methods for manufacturing and using gene corrected stem cells for gene therapy. In particular, herein provided are methods for treating Fanconi anemia in which a subject's stem cells are selected by a combination of high stringency CD34+ selection and low stringency CD34+ selection, genetically modified using a gene therapy vector encoding a FANC protein or a gene editing system, and administered to the subject.

APPLICANTS:

- FUNDACIÓN PARA LA INVESTIGACIÓN BIOMÉDICA DEL HOSPITAL INFANTIL UNIVERSITARIO NIÑO JESÚS
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FURTHER DETAILS:

Fundación para la Investigación Biomédica del Hospital Infantil Universitario Niño Jesús

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