Fanconi Anemia: Hematopoietic Stem Cell Transplant or Gene Therapy?

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Abstract

FA is a rare, multi-organ cancer-prone IBMFS associated with hematological malignancies and STs. The androgen therapy, hematopoietic growth factors, HSCT, and GT, still in the clinical trial, are various treatments for this disease. Here, we aimed to compare the advantages and disadvantages of HSCT and GT in FA cures. We perform an advanced electronic search of "FA" AND (genetics OR treatment OR HSCT OR GT OR Mosaicism), and "Allo-HSCT" AND (conditioning regimen OR complications OR GvHD OR infection OR cost) MeSH terms in non-citation and citation databases. Besides, the gray literature was searched too. This article will provide a summary of the advantages and disadvantages of HSCT and GT of FA disease. Our results show that GT has a good potential in FA treatments in the future. Furthermore, it has higher advantages and fewer disadvantages in comparison with HSCT. Systematic Review Registration: CRD42021247364 ID on PROSPERO database.

Title Page

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Abbreviations

FA	Fanconi Anemia
IBMF	Inherited Bone Marrow Failure
ICL	Inter-strand DNA Cross-Links
rhIL-3	Interleukin 3
rhGM-CSF	Granulocyte-Macrophage Colony-Stimulating Factor
rhG-CSF	Granulocyte Colony-Stimulating Factor
HSCT	Hematopoietic Stem Cell Transplantation
GT	Gene Therapy
Allo-HSCT	Allogeneic Hematopoietic Stem Cell Transplantation
GvHD	Graft versus Host Disease
MSD	Matched Sibling Donor
OS	Overall Survival
MUD	Mismatched Unrelated Donor
CMV	Cytomegalovirus
AD	Alternative Donor
Haplo-HSCT	Haploidentical Hematopoietic Stem Cell Transplantation
TCD	T-Cell Depleted
ATG	Anti-Thymocyte Globulin
CY	Cyclophosphamide
SAA	Severe Aplastic Anemia
TAI	Thoraco-Abdominal Irradiation
FLU	Fludarabine
QoF	Quality of Life
OM	Oral Mucositis
HNSCC	Head and Neck Squamous Cell Carcinomas
HC	Hemorrhagic Cystitis
PRES	Posterior Reversible Encephalopathy Syndrome
CsA	Cyclosporine A
TTP	Thrombotic Thrombocytopenic Purpura
AML	Acute Myeloid Leukemia
ST	Solid Tumor
TRM	Transplantation Related Mortality
PID	Primary Immunodeficiency Disorder
SCID	Severe Combined Immunodeficiency
HSPC	Hematopoietic Stem Cell/Progenitor Cells
iPSC	Induced Pluripotent Stem Cells

Abstract

Fanconi anemia (FA) is a rare, multi-organ cancer-prone inherited bone marrow failure syndrome (IBMFS) associated with hematological malignancies and solid tumors. The androgen therapy, hematopoietic growth factors, hematopoietic stem cell transplantation (HSCT), and gene therapy (GT), still in the clinical trial, are various treatments for this disease. Here, we aimed to compare the advantages and disadvantages of HSCT and GT in FA cures.

We perform an advanced electronic search of "Fanconi anemia" AND (genetics OR treatment OR HSCT OR GT OR Mosaicism), and "Allo-HSCT" AND (conditioning regimen OR complications OR GvHD OR infection OR cost) MeSH terms in non-citation and citation databases. Besides, the gray literature was searched too. This article will provide a summary of the advantages and disadvantages of HSCT and GT of

FA disease.

Our results show that GT has a good potential in FA treatments in the future. Furthermore, it has higher advantages and fewer disadvantages in comparison with HSCT.

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