
Failure of umbilical-related cord blood stem transplantation to correct Hemophilia A

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In the last few years a series of articles have dealt with the plasticity of bone marrow derived stem cells.¹ The possibility of the maturation of these stem cells into different lineages, including endothelial cells, seems confirmed by some laboratory results and by some preclinical research in the treatment of myocardial infarct.²

Therefore the hope that haematopoietic stem cells could be used in the treatment of some diseases by supplying cells that later mature into cardiac myocytes, endothelial cells and other cells is growing.³⁻⁴

Hemophilia A is an inherited bleeding disorder characterized by a deficiency of Factor VIII:c. Factor VIII:c is synthesized by liver sinusoidal endothelial cells and circulates in complex with von Willebrand factor. If the haematopoietic stem cells or the mesenchymal of bone marrow were able to mature into endothelial cells, a bone marrow transplantation could theoretically cure haemophilia A. We report the case of a child whose outcome seems to rule out this hypothesis.

A 6 year old child, suffering from both haemophilia A (Factor VIII:c <5%, normal value 50-134) and acute lymphatic leukaemia was submitted to a cord blood transplantation in second remission. The donor was the HLA identical sister. She was heterozygous for haemophilia (aPTT-ratio 1.21, normal value 0,8-1,18; Factor VIII:c

48%). 980×10^6 nucleated cells (3.3×10^6 CD34) were infused after a conditioning regimen consisting of TBI, Thiotepe and cyclophosphamide.

The follow up was almost uneventful and the engraftment, confirmed by DNA polymorphism and blood group, was complete.

Nevertheless after 2 years no changes were observed in the levels of Factor VIII: c.

At least in our setting a cord blood transplantation resulted unable to cure haemophilia A.

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