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Bone marrow-stem cell therapy for hemophilia A disease

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Hemophilia A (HA) is caused by mutations within the Factor VIII (FVIII) gene, which leads to depleted protein production and inefficient blood clotting. The incidence of HA is 1 in 5,000 males, constituting about 80% of all hemophilia cases, and manifests in mild to severe disease, depending on the relative expression of functional FVIII. Current therapies include fixes-dose FVIII prophylaxis, factor VIII replacement therapies, and most recently, gene therapy. Several attempts at gene therapy have failed for various reasons-including immune rejections. Liver is the primary site of FVIII synthesis; however, the specific cell types responsible for its synthesis remain controversial. Several reports have demonstrated the capacity of bone marrow stem cells (BMSCs) to transdifferentiate into hepatocytes and liver sinusoidal endothelial cells (LSECs). These finding created enormous interest because they uncovered a new property of BMCs and opened the possibility that these cells could be used in the treatment of liver injury and acute or chronic liver failure. We propose that the severity of the bleeding disorder could be ameliorated by partial replacement of mutated liver cells by healthy cells in HA mice. The study showed that BM-derived hepatocytes and endothelial cells can synthesize FVIII in liver and correct bleeding phenotype in HA mice. Thus, BM- stem cell therapy is potential alternative approach to managing HA.

Biography

Neelam Yadav has completed her PhD from Industrial Toxicology Research Centre, Lucknow and Postdoctoral studies from Stem Cell Biology Laboratory of National Institute of Immunology (NII) New Delhi, India. She is Assistant Professor in Department of Biochemistry, at Dr. R.M.L. Avadh University, Faizabad, India. She has published many papers in reputed international journals and filed one patent. She is Visiting Scientist in Department of Medicine, Albert Einstein College of Medicine, New York, USA, under Raman Fellowship Programme of University Grant Commission (UGC) New Delhi, Government of India.

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