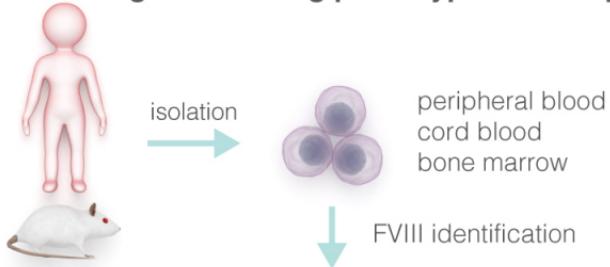


Extra-hepatic sources of FVIII potentially contribute to the coagulation cascade correcting the bleeding phenotype of Hemophilia A mice



FVIII expression

liver cells

sinusoidal endothelial cells
Kupffer cells

bone marrow and peripheral blood cells

monocytes
macrophages/dendritic cells
megakaryocytes

human tissues

spleen, lymph nodes
and lung:
endothelial cells/macrophages



transplantation



analysis

CD34+ human
cord blood cells

NOD/SCID γ Null-
hemophilia A mice

>40% engraftment of donor cells

5% of plasma FVIII activity after 12 weeks

9/12 mice survived tail-clip assay