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Cell therapy for Crilger Najjar Type I syndrome

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Crigler-Najjar Syndrome type I (CNSI) is a recessive disorder caused by a rare mutation in Ugt1a1 gene that encodes for the enzyme Ugt1 involved in bilirubin metabolism. This mutation causes high levels of unconjugated bilirubin in blood, leading to brain damage and early lethality. CNSI patients are treated with extended daily phototherapy but the only effective therapy is liver transplantation. However liver transplantation is not exempt from complications, therefore hepatocyte-like cells derived from stem cells represent an interesting alternative. The goal of this project is to evaluate the use of human adult liver stem cells (HLSC) in treating Crigler-Najjar Syndrome type I (CNSI) in a model represented by Ugt1 deficient mice closely mimicking the pathological manifestations in CNSI patients. The Ugt1+/- mice have been backcrossed with NSG for 9 generations in order to derive these mice in a pure NSG background. By recellularising decellularised rat liver scaffolds with HLSCs ex vivo, we found that HLSC can differentiate in functional hepatocytes expressing Ugt1 protein as from 7 days differentiation. A significant improvement in survival of phototherapy-treated NSG/Ugt1a1-/- pups following injection of HLSC in the liver was observed. Immunohistochemically and immunofluorescence analyses show that HLSC can engraft in the liver of NSG/Ugt1a1-/- mice and express missing enzyme. HLSCs thus show great potential not only for the treatment of CNSI but also for most metabolic liver disorders.

Biography

Elvira Famulari is pursuing Ph.D in Molecular Biotechnology Center from University of Turin Italy.

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