Gene corrected HSC transplantation: an opportunity for delivery of therapeutics to the CNS

Prof. Alessandra Biffi

Padua University, Chair, Pediatric Hematology, Oncology and Stem Cell Transplant Division, Padua University Hospital, Padua, Italy

Hematopoietic stem and progenitor cell (HSPCs) have the ability to engraft in the central nervous system (CNS) upon systemic or local transplantation and give rise to a microglia-like progeny. Prior to transplantation, HSPCs can be genetically modified so that their CNS progeny could i) deliver therapeutic molecules across the blood brain barrier and ii) modulate neuroinflammation and other local neurodegenerative processes. We will discuss recent results obtained by optimization of genetically modified HSPC transplant approaches for the treatment of neurometabolic and neurodegenerative conditions.