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Neural stem cells-based gene therapy of Rett syndrome

Mojgan Rastegar
University of Manitoba, Canada

Rett Syndrome (RTT) is the best-known example of autism spectrum disorders and the primary cause of intellectual disabilities in females. RTT results from mutations in the X-linked Methyl CpG Binding Protein 2 ($MECP^2$) gene and currently has no effective treatment. Importantly, in RTT mouse models, reactivation of the $Mecp^2$ gene after the onset of disease reverses the process and rescues the phenotype. This brings new possibility for RTT gene therapy, where reintroducing $MeCP^2$ into affected neurons may improve the symptoms. Gene Therapy is a potent and cutting edge technology for human disease, where a single gene mutation has been identified. Our ultimate goal is to find the best therapeutic approach for such neurological disorders, focusing first on RTT.

We reported the first generation of $MECP^2$ retroviral and lentiviral human gene therapy vectors. In transduced neural stem cells and cortical neurons, we showed stable and long-term expression of $MECP^2$ after transduction. We are now studying the underlying molecular mechanism of Rett Syndrome and aim to find the best possible therapeutic strategy. With advanced neuroscience and cutting edge gene therapy techniques, we study the cellular expression and molecular function of $MeCP^2$ during central nervous system development and in the adult brain. We aim to investigate the best method of gene therapy delivery in the sites of $MeCP^2$ expression in brain, comparing multiple delivery techniques. Our results will lead towards innovative therapeutic strategies for Rett Syndrome and autism that currently have no effective treatment.

Biography

Mojgan Rastegar has completed her PhD at the Université Catholique de Louvain (UCL) in Belgium and did her postdoctoral studies at the McGill University in Montreal and Hospital for Sick Children in Toronto. She is currently an Assistant Professor of Biochemistry and Medical Genetics at the University of Manitoba. She has published 19 papers in reputed journals. Her research is focused on understanding the role of epigenetics in human disease and neurological disorders, with a clear focus on finding the best therapeutic approach for Rett Syndrome and autism.

rastegar@cc.umanitoba.ca