Torrente Y, J Cell Sci Ther 2012, 3:7
http://dx.doi.org/10.4172/2157-7013.S1.005

OMICS Group Conferences
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International Conference on
Emerging Cell Therapies

October 1-3, 2012 DoubleTree by Hilton Chicago-North Shore, USA

Preclinical experience and perspectives of a clinical trial using cd133 stem cells

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Cell therapy is one promising approach to correct genetic diseases by contributing to tissue regeneration; stem cells can be isolated from a healthy donor or, when possible from the same patient. In the first case cells will be transplanted under a regime of immune suppression while in the second case, cells will have to be genetically corrected before transplantation in the same patient from which they were derived. The recent identification of different types of multi-potent stem cells, some of which are suitable for protocols of cell therapy, has disclosed new perspectives in the treatment of genetic diseases. Our previous work indicated that CD133+ stem cells, a recently identified population of progenitor cells, produce functional improvement upon intra-arterial injection in a mouse model of muscular dystrophy. Recently transplantation of engineered dystrophic canine muscle-derived CD133+ cells gave promising results in Golden Retriever dystrophic dogs, the most reliable animal model that shows a form of dystrophy very similar to and even more severe than DMD. Because of these results, we plan a pilot clinical trial, based on intra-muscular and intra-arterial transplantation of autologous engineered muscle derived CD133+ cells. Efficacy and possible adverse effects will be evaluated to test whether this approach may represent a first step towards an efficacious therapy for muscular dystrophy.

Biography

Yvan Torrente received his degree in Medicine and Surgery at the University of Milan (1995) and his Ph.D. in Biochemistry at the University of Paris (2004). In 2002 he achieved specialization in Neurology and became staff Neurologist for Dino Ferrari Center. From 2004 he has been encharged of Neurology service for Fondazione IRCCS Ca' Granda-OMP of Milan, he is assistant Professor in Neurology and Director of Stem Cell Laboratory at Department of Neurological Sciences-University of Milan. As researcher in Neurology, he has been standing out in the field of stem cells therapies for neuromuscular diseases, in particular related to DMD.

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