

## **Analysis of the efficiency of gene-cell therapy in transgenic mice with amyotrophic lateral sclerosis phenotype**

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### **Abstract**

Amyotrophic lateral sclerosis is a neurodegenerative disease characterized by progressive death of cerebral and spinal motorneurons. Using behavioral tests we studied the efficiency of gene-cell therapy in SOD1 G93A transgenic mice receiving xenotransplantation of human umbilical cord blood mononuclear cells genetically modified with adenoviral vectors encoding vascular endothelial growth factor (VEGF) and reporter green fluorescent protein (EGFP) genes. The cells were transplanted to mice on week 27 of life (preclinical stage of the disease). Behavioral tests (open field, grip strength test) showed that transplantation of umbilical cord blood mononuclear cells expressing VEGF significantly improved the parameters of motor and explorative activity, grip strength, and animal survival. Thus, gene-cell therapy based on genetically modified mononuclear cells expressing VEGF can be efficient for the treatment of amyotrophic lateral sclerosis. © 2013 Springer Science+Business Media New York.

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### **Keywords**

amyotrophic lateral sclerosis, gene-cell therapy, mononuclear umbilical blood cells, SOD1 G93A, transgenic mice, vascular endothelial growth factor