

Objective 2005 – 2006

A PHASE 1 CLINICAL TRIAL “MESENCHYMAL STEM CELLS TRANSPLANTATION IN AMYOTROPHIC LATERAL SCLEROSIS”

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The Phase 1 clinical study was approved by the Italian National Institute of Health and partially funded by the Ministry of Health. The objectives of this clinical trial were to assess the feasibility and toxicity of mesenchymal stem cell transplantation in 10 ALS patients.

The patients have already undergone the transplantation and their clinical follow-up is currently going on at the Neurological Hospital of the University of the Eastern Piedmont in Novara. Patients have been monitored by neuroradiological, neurophysiological and neuropathological assessments with the purpose on the one hand of identifying the impact of stem cell transplantation in the patients and on the other hand of developing qualitative and quantitative methods to monitor the evolution of the disease. This task was carried out by the neurologist that was awarded the two-year grant assigned by open competition thanks to the funds donated by the Vialli and Mauro Foundation to the “Maggiore della Carità” Hospital of Novara (50,000 euros).

So far the clinical trial has confirmed the results of a previous pilot study. The transplantation of autologous mesenchymal stem cells in the spinal cord of ALS patients can be carried out without serious adverse effects and there was no sign of immediate or delayed transplant-related toxicity. In a sub-group of patients, the cell-based therapy might have exerted a therapeutic effect. These data are currently being published on international magazines, all of which acknowledge the important role played by the Vialli and Mauro Foundation, and will shortly be made available.

The cells, which have previously been collected from the patient’s bone marrow, have then been isolated and expanded at the Stem Cell Transplantation and Cellular Therapy unit of the Regina Margherita Hospital in Turin. To this purpose, the hospital has been provided with new equipment, thanks to a donation of 38,300 euros by the Vialli and Mauro Foundation. At the same time a study has been accomplished by the Transplant Centre of the Regina Margherita Hospital to compare the characteristics of the autologous mesenchymal stem cells of ALS patients and those of healthy donors, thus proving for the first time that the cells of both groups display similar features and might therefore have a clinical application in stem cell treatment for ALS.

The results, which are currently being published, will be available on the website of the magazine:

www.ingentaconnect.com/content/cog/ct/2008/00000017/00000003%3Bjsessionid%3D3vsb5t7mm
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Moreover the results obtained by the animal experimental model of the disease (SOD mice) are currently being printed. Following the transplantation of human autologous mesenchymal stem cells, an improvement has been recorded in the survival rate and in the development of the disease, which can be traced back to the effects of the transplanted cells on the main pathogenetic mechanisms of the disease, in particular as regards the microglial activation. The publication will be available online, on the website of the magazine:

<http://www.sciencedirect.com/science/journal/09699961>

To conclude: so far our studies have proven for the first time with international scientific publications, that stem cell clinical trials can be accomplished in ALS patients and have cleared the way to a promising, though controversial, field of investigation for researchers. On the basis of our experience, new clinical trials will be carried out also in the United States with neuronal stem cells in Amyotrophic lateral sclerosis and a European multicentre study has been launched for Multiple Sclerosis, another major disabling disease.

Moreover we believe that beside the merely scientific interest, our trials provide a concrete answer to our patients, who demand stem cells trials for ALS, in order to avoid desperate trips to countries where there is a real and frightful “stem cells business”.

Therefore, as doctors and researchers, we firmly believe in the importance of carrying on this complex, though very promising, research project.

We call upon the Vialli and Mauro Foundation to support us once again in our work, renewing the two-year grant to the Maggiore Della Carità Hospital of Novara, to be awarded by open competition to a neurologist with specific experience and skills in clinical trials with ALS patients.

Yours faithfully,

Dr.Letizia Mazzini

