Arne Jensen, CEO of BrainRepair UG and head of the Campus Clinic Gynaecology Bochum, Germany, discusses recent advances in brain repair using cord blood-derived stem cells that have been granted 'Orphan Medicinal Product Designation' by the European Commission

Cord blood stem cells for brain repair

Each year, thousands of children incur brain damage that results in cerebral palsy, the most common disability in childhood, for which there is no cure at present beyond supportive and occupational care. Cerebral palsy leads to complex disabilities, including movement and posture disorder, chronic pain, mental retardation, inability to walk, and a life expectancy in severe cases below 18 years, to name only a few. About 17 million people worldwide live with cerebral palsy, generating global healthcare costs of more than €13 trillion. This reflects both the magnitude of the personal, medical, and socioeconomic burden of this brain disorder and the overt unmet therapeutic needs of the paediatric population. About 70,000 newborns are affected by brain damage each year in the European member states.

But there is hope

More than a decade ago, preclinical studies demonstrated that human cord blood cells given systemically to neonatal rats after brain injury have the ability to actively migrate (so-called 'homing') from the periphery into the damaged brain areas to induce a healing process that prevented the development of spastic (stiff) paresis by release of proteins such as anti-inflammatory cytokines, growth factors, and chemokines. Also, recovery of gross motor function, fine motor co-ordination, muscle strength, and somatosensory brain processing was observed. These findings spurred cord blood stem cell brain research worldwide and led to successful clinical applications.

Use of own umbilical cord blood

Cord blood is an abundant source for stem cells that is easily accessible, lacks ethical concerns, and is particularly safe when used in a so-called 'autologous setting' where the donor is also the recipient. However, cord blood is regrettably discarded after birth as medical waste in most of the cases. But this might change in the near future because first documented clinical applications and clinical trials in children using own cord blood stem cells demonstrated both safety and efficacy in the treatment of brain damage of various causes. Notably, the treatment with stem cells containing cord blood mononuclear cells caused a significant functional neuroregeneration, including reduced spastic paresis, the key symptom of cerebral palsy, and in individual cases recovery of cognition, vision, active and receptive speech competence.

Favourable regulatory aspects

Based on convincing preclinical studies and first clinical applications the European Commission has spearheaded regulation by granting the first ever 'Orphan Medicinal Product Designation' for autologous cord blood for the treatment of brain damage in preterm (EC/3/16/1744) and term-born infants (EC/3/16/1743), thus paving the way for clinical trials and market availability in this utterly important field of medicine.

It is to be hoped that the precious resource of cord blood will be preserved in the future to allow for treatment of children in need.

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