Neurodegeneration: moving from technology to application notes

Laura Calzà

Università di Bologna

Regenerative and reparative medicine represent an extraordinary opportunity for the development of innovative therapies also for multifactorial, high impact diseases. However, “the best innovative medicine”, as defined by the design and in vitro testing, frequently fails when tested in vivo. A successful approach for in vivo application of innovative medicines such as conventional drugs must consider, among others, the multiple underling pathogenic mechanisms, their temporal evolution, and the tissue/organ barriers. Central nervous system diseases are probably the most challenging target for new medicines. At the same time, this medical area is looking for innovative therapies to meet an urgent and dramatic medical need and regenerative and reparative medicine are regarded as promising approaches.

Staring from the long experience of the lab in the field, the major obstacles facing the development of new therapies for lesions and chronic degenerative diseases of the CNS will be reviewed, considering neonatal encephalopathy, traumatic spinal cord injury, and Alzheimer’s disease as pivot conditions. The focus will be on repair capability of the white matter by endogenous stem and precursor cells, as prerequisite for neuroprotection and neurorepair. Innovative medicine will consider cell therapies, nanomedicines, electroceutical, biological drugs. Strategies to reduce failures in translating promising in vitro result into in vivo proof-of-concept study will be discussed, addressing key questions to be considered already in the design of the R&D path of new medicines, as the expected biological effect, and administration timing/route according to the natural history of the disease.