3-JPS-4 JPS Satellite Symposium

Regenerative Medicine and Drug Development using Stem Cells

Hideyuki Okano

Dept. Physiol., Keio Univ. Sch. Med.

There is an increasing interest in the induced pluripotent stem cells (iPSCs)-based regenerative medicine for various diseases. We have been developing regenerative medicine of spinal cord injury (SCI) by the transplantation of neural stem/progenitor cells (NS/PCs)-transplantation for many years. In a series of our previous efforts, we have addressed the issues of safety and tumorigenesis using iPSCs-derived NS/PCs (iPSCs-NS/PCs). The first-in-human clinical study of iPSC-based cell therapy for subacute SCI was approved by the government on February 18, 2019 as class I regenerative medicine protocol, which will be provided for under Japan's Act on the Safety of Regenerative Medicine.
I will also talk about our new clinical trial for ALS using a drug named ropinirole (ROPI: known as D2 receptor agonist), which was identified in the iPSCs-based phenotypic screening of ALS patients-derived motor neurons with FDA-approved drug library (Fujimori et al., Nat Med, 2018). Based on our findings on the potential anti-ALS action of ROPI, we started A Phase I/IIa, to verify the safety and tolerability of "ROPI" in subjects with ALS (ROPALS trial) from December, 2018. Notably, we will also generate iPSCs from patients and compare in vitro and in vivo effects of ROPI on ALS phenotypes.