S39-6 Approach for the development of low-toxic DDS based on the control of tissue distribution and intracellular trafficking

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Development of molecular biology enabled us to clarify the mechanism of pathogenesis with genetic analysis. Meanwhile, the category of biotechnology-based medicine was expended from the typical low-molecular weight drug to macromolecules including protein, siRNA and DNA. Especially, gene therapy is expected to be an ultimate one for intractable and genetic disease with a progression of bioinformatics. To realize gene therapy, novel nanotechnology is essential which can deliver the DNA with low toxicity. To establish an efficient delivery system with minimum toxicity, tissue targeting is important issue. Furthermore, improvement of gene expression efficiency per 1 particle is also required to reduce an injection dose.

In intracellular space, a dynamic organelle networks is formed in viscous cytoplasm containing proteins and RNA. To develop an efficient gene delivery system applicable in the high-serum condition, sophisticated nanotechnology is essential to overcome intracellular barriers, which is equipped with various functional devices into a single particle so that each function operate at the appropriate time and place. In this symposium, we will show our recent approached to deliver the nucleic-acid based drugs to tissue, and to control and/or analysis of an intracellular trafficking.