GS01-1 Gene delivery using dry powder inhalation

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Gene transfection is an innovative therapeutic strategy for intractable and lethal disease. The successful key for gene therapy is how to deliver therapeutic genes into targeted organs and cells. Recently, the application of inhalation therapy, which enables the direct pulmonary delivery, is progressing for local (pulmonary) and systemic action. Among them, dry powder inhalation has some advantages including compact device and easy handling, but has hardly been applied to pulmonary gene therapy. So far, we have successfully prepared dry gene powders containing plasmid DNA by supercritical carbon dioxide technique, and demonstrated that the dry gene powder formulation exhibits higher gene transfection efficiency and higher storage stability compared with its solution formulation. To apply it to clinical use, on the other hand, several conditions including preparation method and formulation compositions must be optimized for higher pulmonary gene transfection.

In this symposium, we will report the physicochemical and gene expressing characteristics of the novel dry gene powders prepared by spray-freeze drying technique as well as supercritical carbon dioxide one. Furthermore, as a method for simultaneous evaluation of pulmonary powder delivery and gene expressing effect, we will describe the merit of dual imaging dry powders.