

Non-Viral Gene Therapy

~ Design of Novel Gene Vector and Application for Healthcare ~

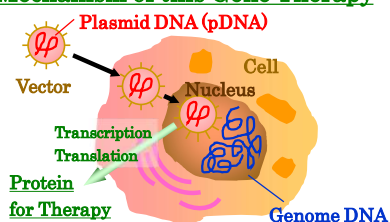
KEY INVENTION

A gene therapy technology using non-viral vector (polymer/pDNA complex) has been developed.

Advantages of Non-Viral Vector

- High Safety (Low Risk of Immunogenicity and Genome Integration)
- Intravenous Injection Available (Difficult in case of viral vector)
- No size limitation of gene to be carried (AAV: 4,800 bases < Human: 8,446 bases)

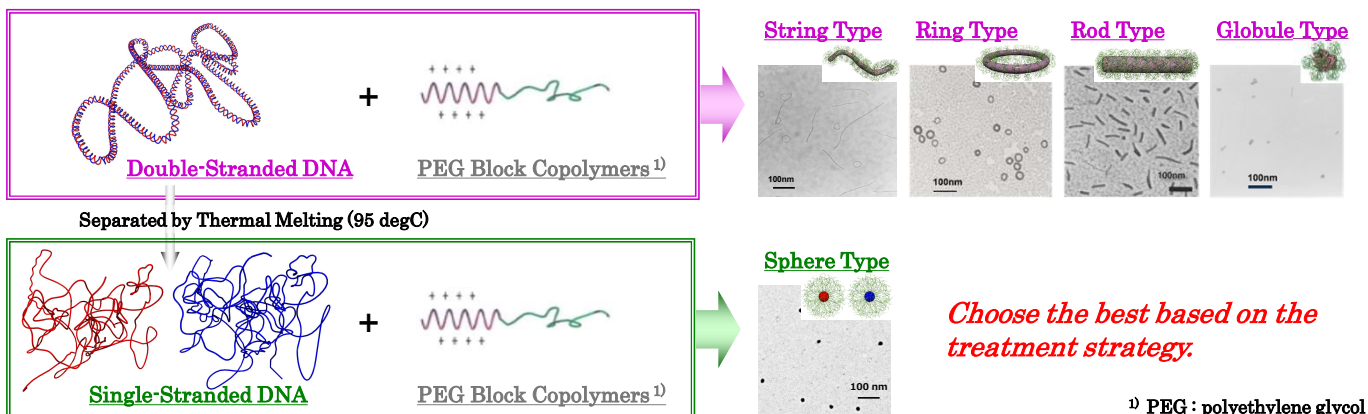
Mechanism of this Gene Therapy



SUMMARY of INVENTION

[High-Order Structure of Polymer/pDNA Complex]

Several high-order structures has been designed using different types of polymers (PEG Block Copolymers¹⁾).



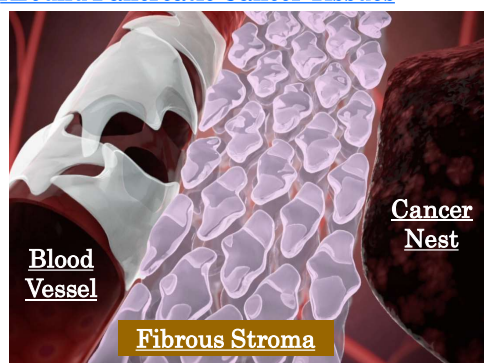
ADVANTAGE of INVENTION

[Application for Pancreatic Cancer Therapy]

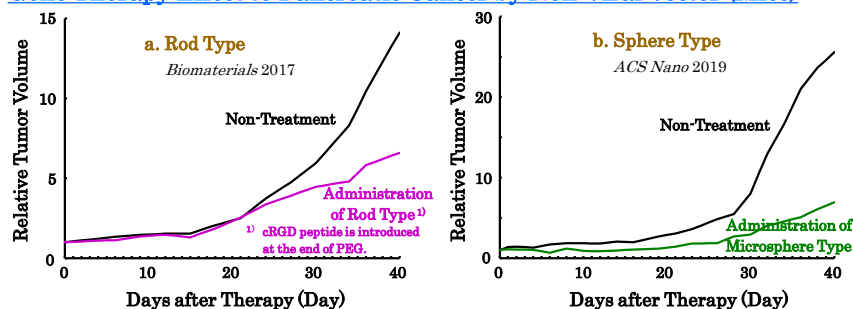
In case of general solid cancers, ca.100 nm of particles pass through the blood vessel spaces and accumulate in the cancerous tissues (EPR Effect). However, 50 nm or less of particles are required to access the cancer cells in case of pancreatic cancers due to the **fibrous stroma** between the blood vessels and the cancerous nests.

- ➔ a. Rod Type (76 nm) to target cancer vasculature cells and express antiangiogenetic protein
- ➔ b. Sphere Type (26 nm) to target cancer cells and express suicide protein

Around Pancreatic Cancer Tissues



Gene Therapy Effect to Pancreatic Cancer by Non-Viral Vector (Mice)



Inhibitory Effect of Non-Viral Vector to Pancreatic Cancers

APPLICATION expected

- ◎ Use as a therapeutic agent for refractory cancers such as pancreatic one
- ◎ Application for a basic therapy of genetic disorders such as an immunodeficiency disorder agent
- ◎ Application for a gene editing or vaccine

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Licensable Patent

Title of Invention :

International Publication No. :

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Nucleic Acid-Encapsulating Polymer Micelle Complex
and Method for Producing Same

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