The 16th Korea-U.S. Forum on Nanotechnology

Targeted delivery of CRISPR/Cas9 protein complex by functional nanoparticle for fusion gene editing in cancer cells

Seongchan Kim

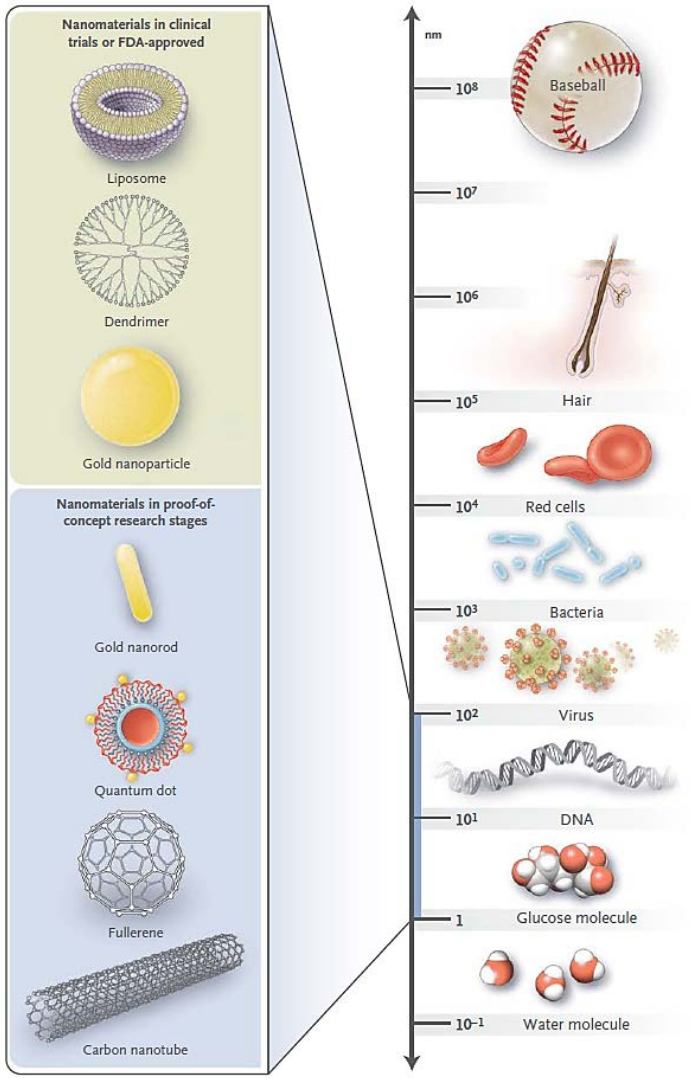
Post-doctoral fellow

Center for Biomaterials, Biomedical Research Institute

Korea Institute of Science and Technology (KIST)

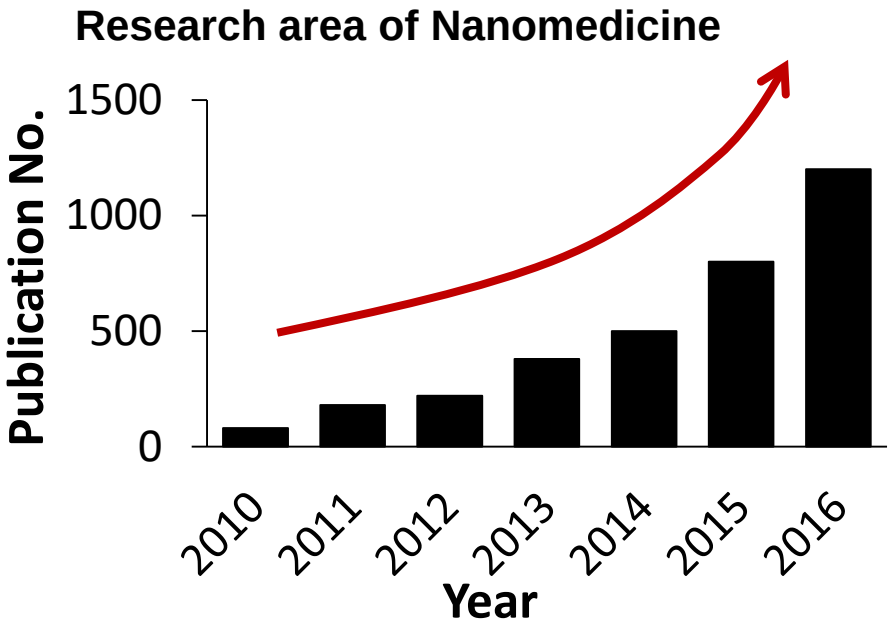
Introduction

Development of nanomedicine



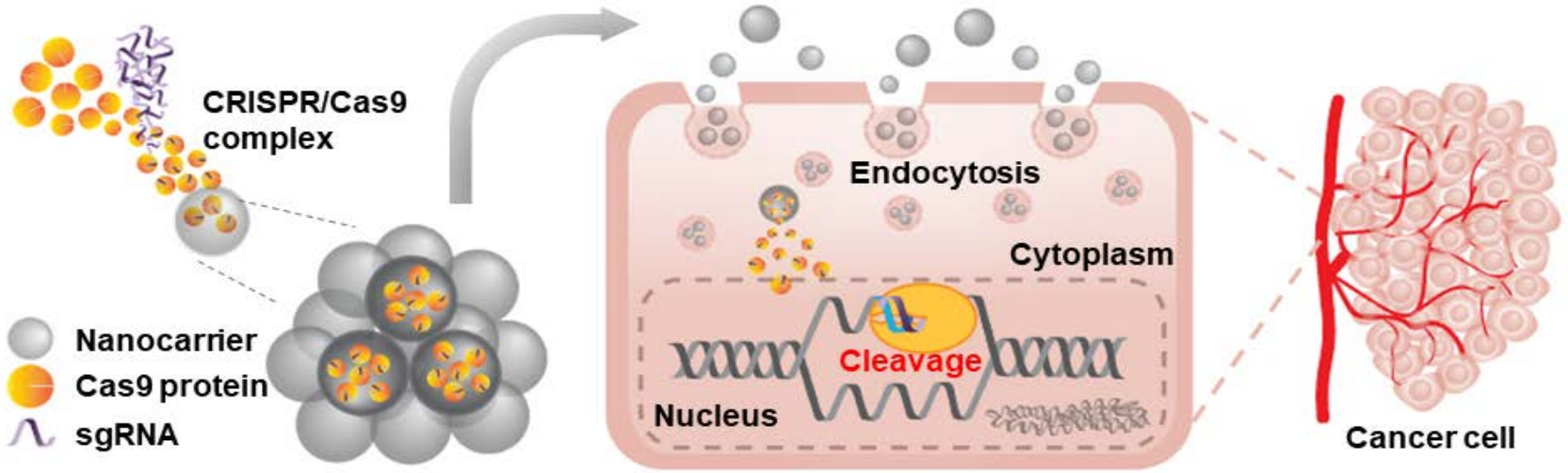
Key is “Nanoplatform”

1) Discovery of a **new nano-structured materials**
2) Development of systems via a **novel approach**
3) Application in the field of **unmet need**

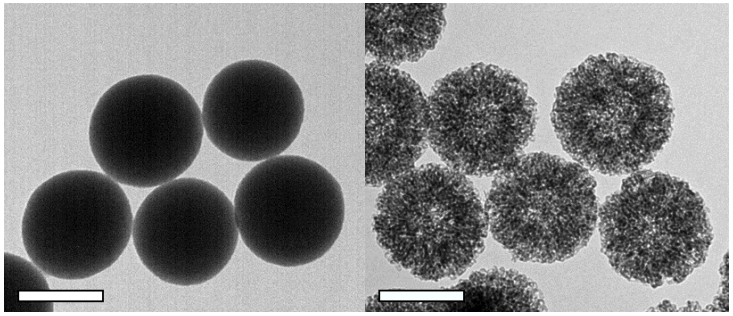


Development of CRISPR/Cas9 protein complex delivery system

Overall strategy



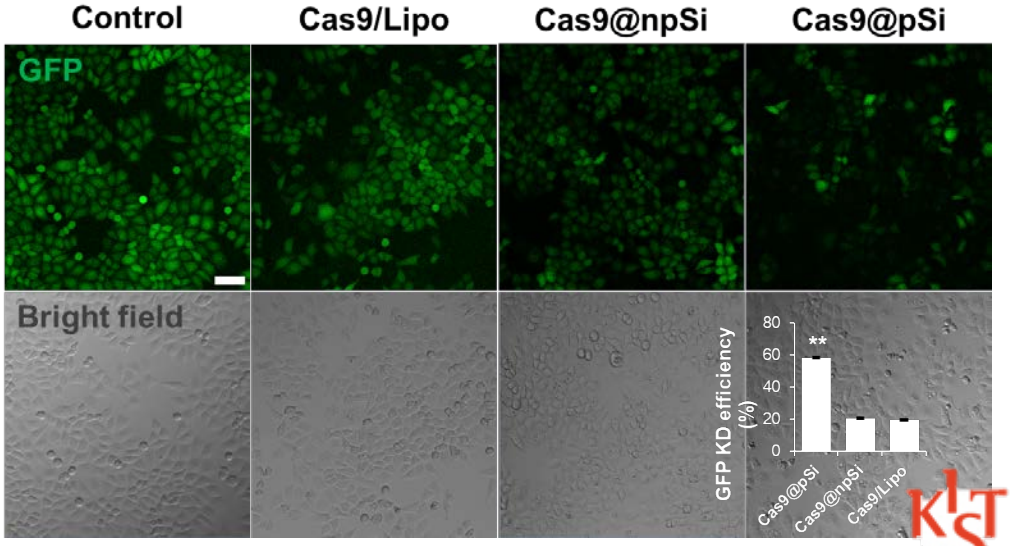
TEM image of Nanocarriers



particle	BET surface area (m^2/g)	Pore volume (mL/g)	Mean pore size (nm)	Zeta potential (mV)
npSi	1130	0.67	3.07	25.2
pSi	359.75	1.18	20.25	24.5

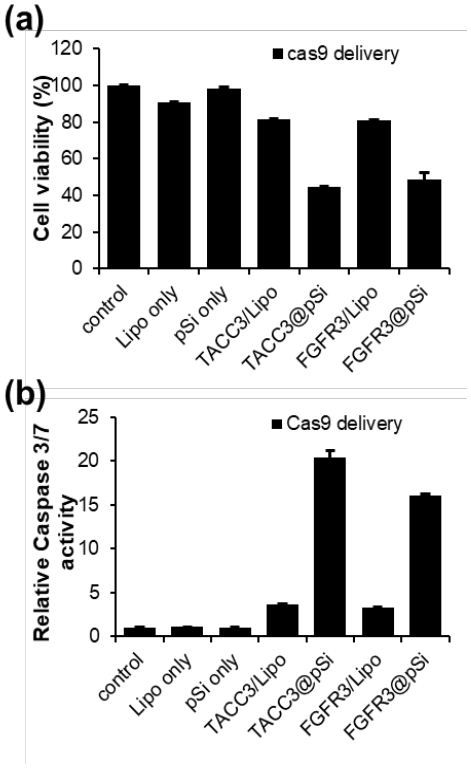
(unpublished data)

Regulation of GFP expression

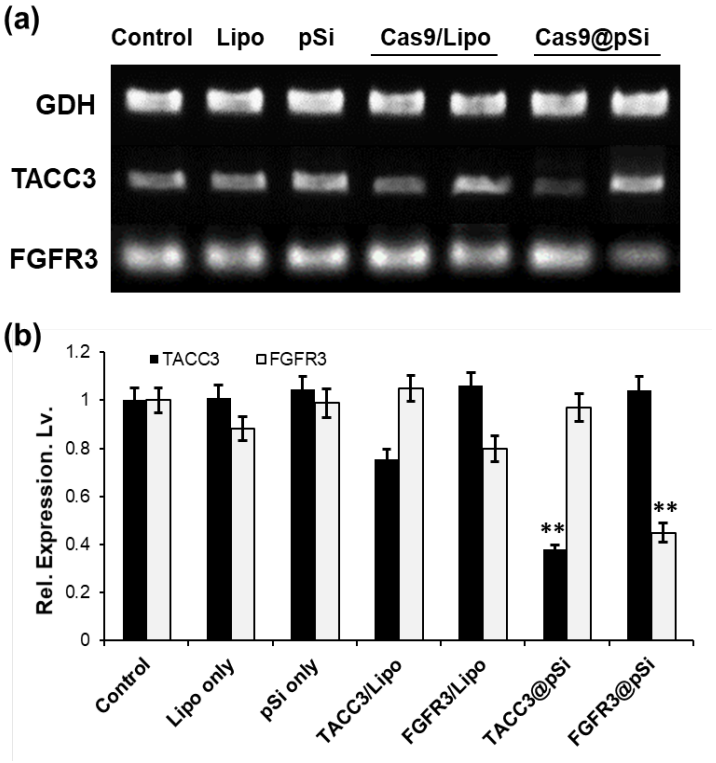


Regulation of target gene *in vitro*

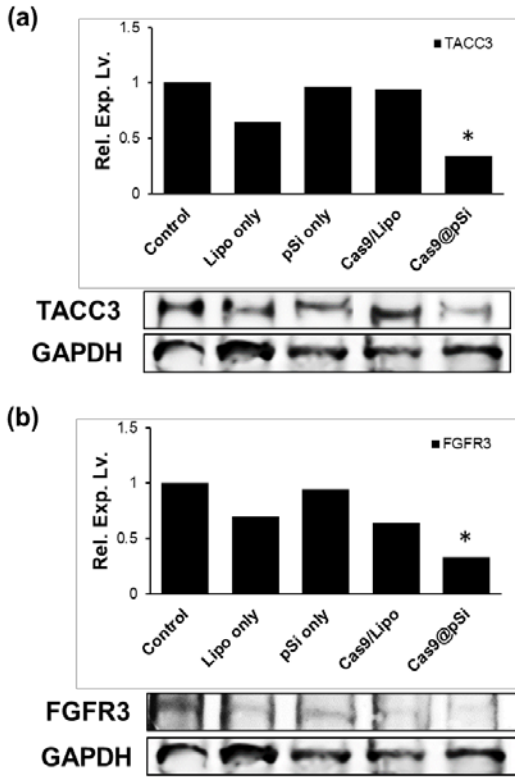
Cell viability



RT-PCR for mRNA Lv.



Western blot for Protein Lv.

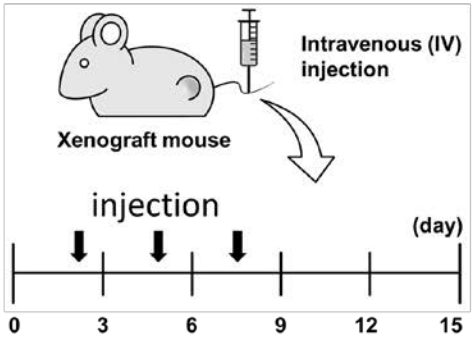


(unpublished data)

Regulation of target gene *in vivo*

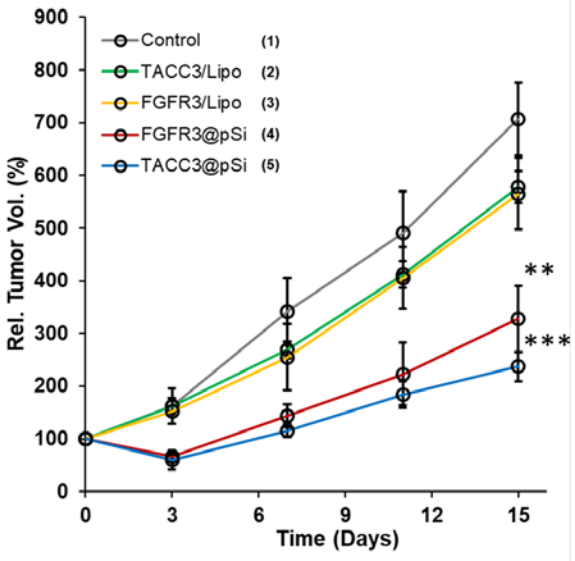
Anticancer effect in HeLa-tumor bearing mice

(a)



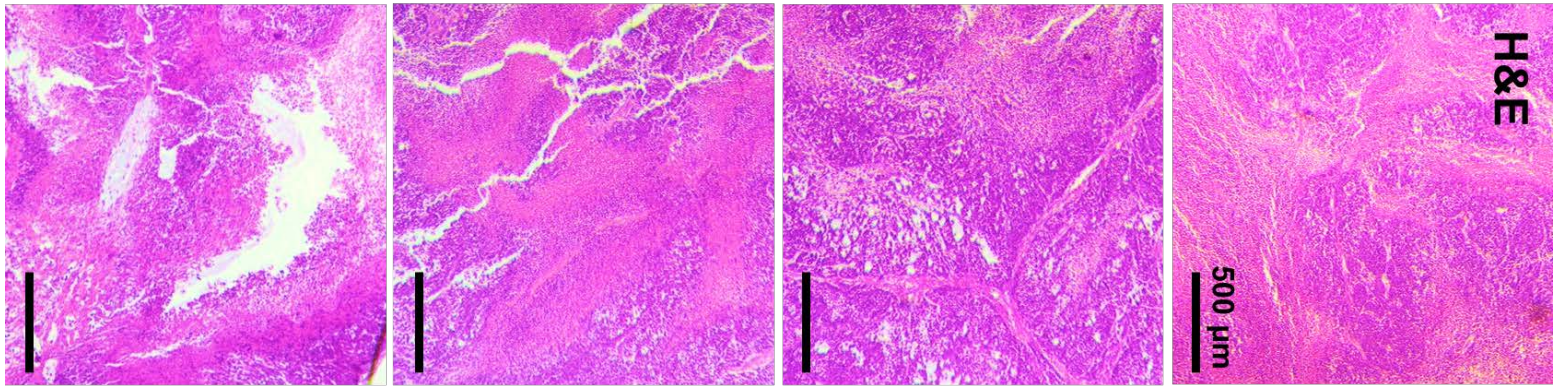
(b)

(c)



Histological study

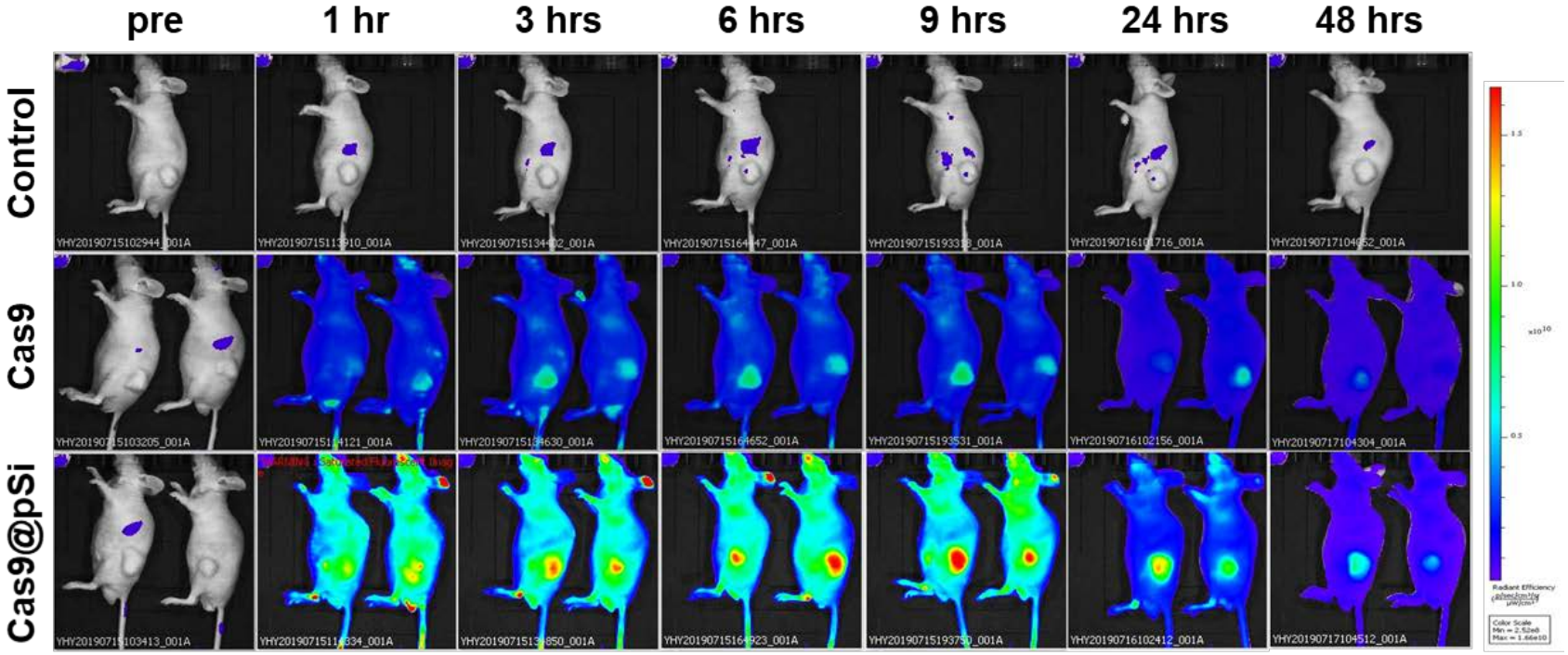
Cas9@pSi | Cas9/Lipo | pSi only | Control



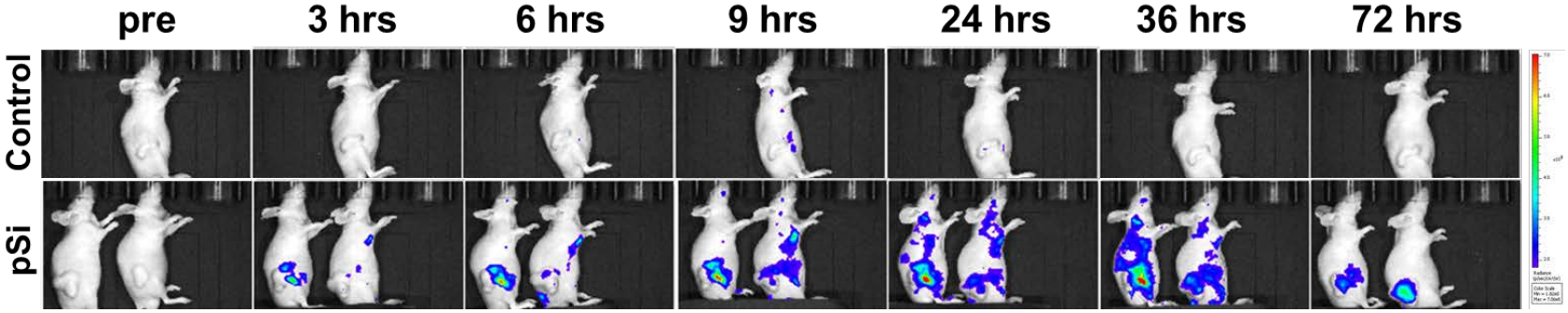
(unpublished data)

Biodistribution *in vivo*

Real-time imaging of Cas9 protein



Real-time imaging of Nanocarrier



(unpublished data)

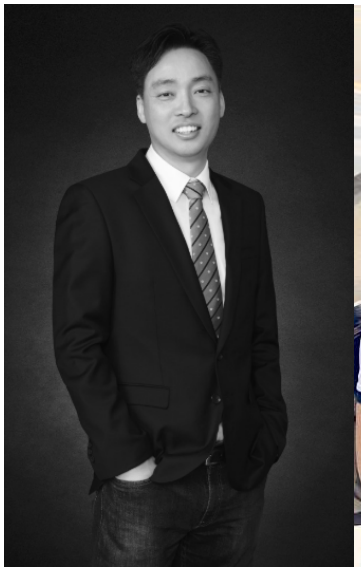
Summary

In this study, we reported a novel therapeutic platform based on CRISPR/Cas9 protein complex for targeted cancer treatment.
We developed an efficient **protein delivery system** based on silica nanocarrier with **expanded pores**.

The present work is the **first demonstration** of a Cas9 protein-based gene editing approach both *in vitro* and *in vivo*, with high gene regulation efficacy, low cytotoxicity and immune response achieved by the **systemic administration**.

We believe that the studies can provide a strong foundation for basic research in the field of nanomedicine and the **long-term technical progress of gene therapy into an effective clinical application**.

nano BNMLab
BIOMEDICAL NANO MONITORING LABORATORY



Dr. Hyojin Lee
Dr. Seongchan Kim
Seunghoe Kim
Eunhye Park

Dr. Youngdo Jeong
Minsu Kim
Dongkap Kim

Dr. Kwan Hyi Lee
Dr. Hojun Kim
Sungwook Park
Rashid Tonmoy

Project was supported by **Basic Science Research Program** through the **NRF** funded by **Ministry of Education**

NRF 한국연구재단 National Research Foundation of Korea
KIST Korea Institute of Science and Technology

Thank you for your attention