National Cancer Center (NCC) Japan, NCC Seed Acceleration Program（NCC SAP）

Development of DDS best sutable for cancer therapy

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Vision

- Many nucleic acid (NA) medicines such as antisense oligo and siRNA have been developed, but problem is lack of useful drug delivery system (DDS) which carries NA to the target lesions.
- We have shown successful therapeutic efficacy of refractory cancers by various NAs loaded on our DDS in mouse models.
- Through this project, we established extended production system of DDS and we are ready to distribute our DDS world-widely.

Marketability

- In 2028, the market of nucleic acid medicine is estimated to reach 24 billion dollars. It is pointed out that DDS is a key factor as a crucial contributor for extension of the market.
- With our DDS, 24 billion dollars could be turned up to 5-10 folds.

Innovation

Compared with conventional DDSs, the following points are advantages.

I. Cancer specific delivery, devoid of normal organs.
II. 100% endosomal escape achieved while others <1%.
III. Materials are cheap.

Partnering

【 Expected partners 】

Pharmaceuticals, CDMO

【 Expectation 】

drug manufacture

Research Outline

Key Words: # **DDS, #siRNA, #microRNA, #cancer**

- Our DDS delivers massive NA to tumors in mice compared with liposome（Fig. I）.
- Injection of apoptosis-inducing NA on conventional DDS killed mice due to liver failure. With our DDS mice were healthy and apoptotic cells were rarely noted in liver (Fig. 2).
- In contrast to conventional DDS, our DDS is devoid of accumulation of NA in normal organs (see red line, Fig. 3).

Fig. 1 Comparison with Liposome

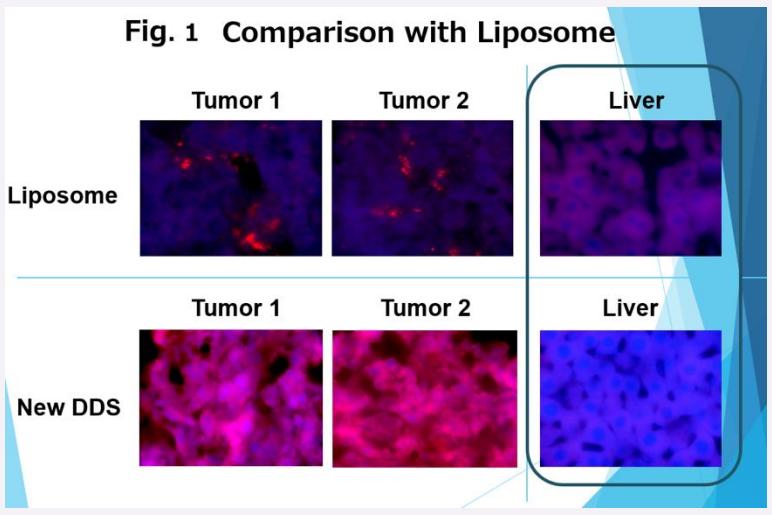


Fig. 2 Damage of LncRNA to liver

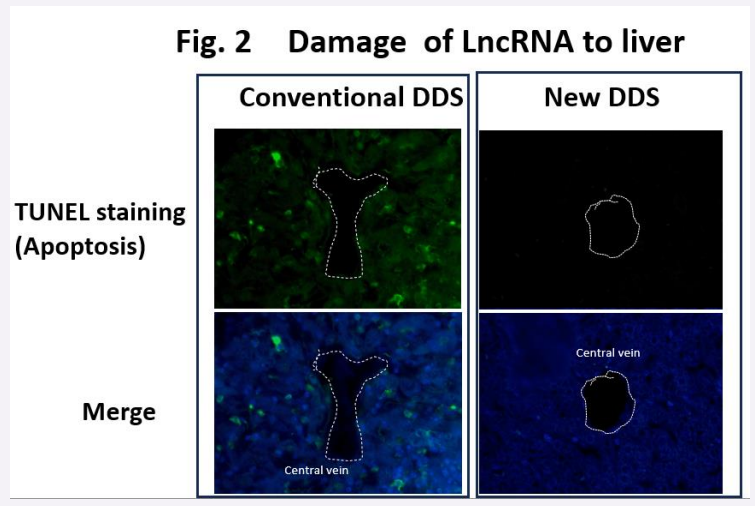
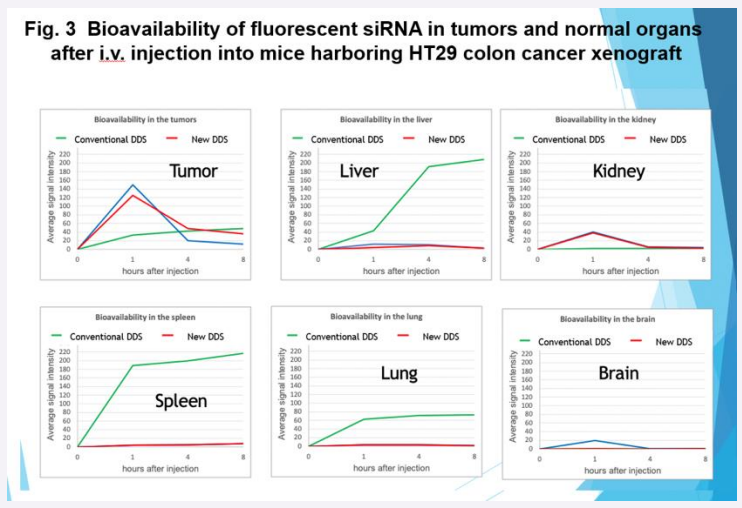


Fig. 3 Bioavailability of fluorescent siRNA in tumors and normal organs after i.v. injection into mice harboring HT29 colon cancer xenograft



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