Development of nanotechnology for nucleic acid medicine for tumor treatment



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Vision

- Nucleic acid medicines are expected to be developed as drugs that can specifically regulate nucleotide sequences upstream of genes. However, the lack of effective delivery technology remains a challenge.
- > The aim is to develop anti-cancer nucleic acid medicines by establishing practical synthetic methods for proprietary technologies and building development strategies.
- Original Technology: Developed a lipid-free technology as an organ/cell delivery method for novel nucleic acid medicines.

Marketability

- The market for nucleic acid medicines is expected to reach approximately 3 trillion yen (30 billion USD) by 2030.
- The LNP market, which has gained significant attention due to applications like mRNA vaccines, is expected to reach approximately 1.5 trillion yen (15 billion USD) by 2030.
- The market for refractory cancers, which are difficult to treat with standard therapies or tend to recur after treatment, is projected to grow to approximately 3 trillion yen (30 billion USD) by 2030.

Innovation

Research Outline

The existing technology of lipid nanoparticles (LNP) has a high accumulation in the liver, and the low delivery efficiency to tumors remains a challenge.

RION, being lipid-free, has the characteristic of avoiding the adsorption of lipid-carrying proteins. It also exhibits low liver accumulation and preferential accumulation in tumors. Additionally, RION has a higher drug encapsulation capacity than LNP.

Partnering

[Expected partners]

Pharmaceuticals · Chemical/Fibers · Medical institute · IT, Electronics/Digital · Biotech/Drug Discovery Service · Machinery/Device · CMO/CDMO/CRO/SMO · Food/Beverages · Medical/Diagnosis/Research Devices · Venture capitals

[Expectation]

Investigational drug manufacturing, support for planning and conducting nonclinical safety and clinical trials, and start-up support

Key Words: DNA, RNA, ASO, DDS, Nanotechnology

We developed a lipid-free technology for organ/cell delivery as a new nucleic acid medicine delivery technology. Comparison of the disadvantages of existing nanocarriers with RION:

※existing nanocarriers (LNP etc···)

- High liver accumulation (>90%)
- Low blood stability (<15 minutes)

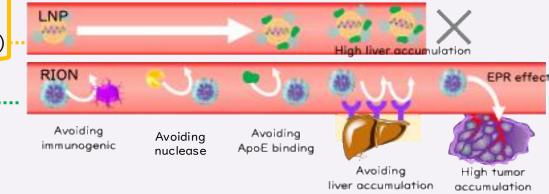
RION contributes to 'nucleic acid medicines'



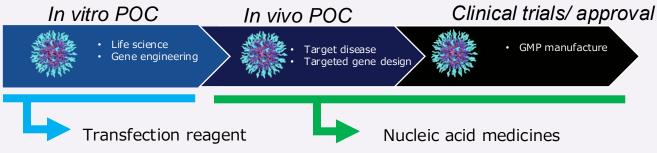
- Low encapsulation rate of the active ingredient (<5%)
- Side effects caused by carriers (excluding the active ingredient)

©RION : DDS technology with high tumor accumulation ability

- High tumor accumulation
- High blood stability (> 2h)
- High nucleic acid medicine encapsulation ability
- Self-assembling nucleic acid without Lipids



The technology is expected to expand from in vitro assays, where nucleic acid delivery is difficult, to in vivo applications where nucleic acid delivery is challenging.



Miyamoto, Noriko, et al. (2023) Journal of Drug Delivery Science and Technology, 88: 104902. Miyamoto, Noriko, et al. (2023) Advanced Therapeutics 6: 2200265.

Patent lists

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Title : ARTIFICIAL NUCLEIC ACID AND NUCLEIC ACID DELIVERING METHOD
USING SAME
No. : PCT/JP2022/019343(JP, US, CHN, EU, AU)
Applicant
            : JST
          : Miyamoto Noriko
Inventor
Date : 2021/4/28
Status
           : IP disclosure
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Title : USE OF STRUCTURE FOR NUCLEIC ACID DELIVERY IN TREATMENT OF HEMATOPOIETIC TUMOR : PCT/JP2024/018410 No. Applicant : JST : Miyamoto Noriko, Others Inventor : IP disclosure Status

Applicant : JST Inventor : Miyamoto Noriko Status : IP Application