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March, 2022

NOF EXHIBITS AND PRESENTS AT ADVANCING GENE THERAPY 2022 (MARCH 28-30)

NOF CORPORATION is exhibiting and presenting at AGT: Advancing Gene Therapy 2022, held at Hilton Boston/Woburn, MA between March 28th and March 30th.

Conference website: https://biogatesc.com/events/Gene-Therapy/

Presentation Titles:

① COATSOME® SS-Lipids as novel, biodegradable, ionizable Lipids for mRNA Therapeutic

② COATSOME® SS-Lipids as novel, biodegradable, ionizable Lipids for nucleic acid delivery and Next Generation Vaccines

Date and Time:

① March 29, 2022 at 9:30 – 10:10 EST (DAY 2, Session: Vector Products & Innovative Delivery Platforms, Keynote)

② March 30, 2022 at 16:05 – 16:30 EST (DAY 3, General Session)

Abstract:

① mRNA has broad potential as a therapeutic and multiple current clinical efforts are focused on vaccination, protein replacement therapies, and for gene editing. While much of the current clinical progress is due to advances in mRNA manufacturing and chemical modification, intracellular delivery remains a significant hurdle. Recently, lipid nanoparticles composed of ionizable lipids have emerged as a new class of delivery system but pose new challenges due to inflammatory responses and organ toxicity. At NOF, we developed an improved series of ionizable lipids, COATSOME® SS-Lipid, that contains multiple structural motifs to increase biodegradation. Studies of mRNA transfection efficiency and rodent toxicity studies have shown that biodegradation, among other properties, contributes significantly to improved tolerability and RNA expression levels. This presentation will provide a detailed examination of the barriers at cellular level to RNA delivery and the role of the innate immune system in mediating lipid toxicity. Finally, the future outlook for development of next generation LNP platforms will also be discussed.

② Nucleic acid vaccines, DNA and messenger RNA (mRNA), have emerged as promising modalities for infectious disease and for cancer immunotherapy due, in part, to shortened manufacturing cycles and high potency. Currently, there is limited understanding of the mechanisms of antigen presentation and induction of specific T-Cell responses critical to long-term immunity. Lipid nanoparticles (LNP) composed of ionizable lipids are important components of such vaccines as they can convey and present the nucleic acid effectively to the immune system. Further improvements in LNP carriers require a reduction in the 1) systemic toxicity, 2) improved endosomal escape, 3) potent and T-cell specific adjuvanting function and 4) targeting to specific antigen presenting cells.

Previously we have reported that lipid nanoparticles composed of SS-lipids can deliver pDNA or mRNA in mice to liver, solid tumors, and other organs via the IV route and achieve high levels of expression. We also evaluated the safety of the lipids in mice where doses of up to 175 mg/kg were well tolerated.

After subcutaneous administration, the LNPs containing an SS-E, an SS-lipid with vitamin E scaffolds, elicited a higher gene expression activity in comparison with the other LNPs composed of the SS-lipids with different hydrophobic scaffolds. Immunization with the SS-E-LNPs encapsulating plasmid DNA that encodes ovalbumin (OVA, a model tumor antigen) or profilin (TgPF, a potent antigen of Toxoplasma gondii) induced substantial anti-tumor or antiprotozoan effects, respectively. These findings suggest LNP composed of SS-E lipid can be effective delivery systems for DNA vaccines.

Visit our booth during the conference, or contact our regional offices below:

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