SiSaf Announces Positive Preclinical Data in its SiS-ADO2 siRNA Program to Treat Rare Genetic Bone Disorder Osteopetrosis

2022-10-02

SiSaf Ltd, an RNA delivery and therapeutics company, is pleased to announce positive data confirming safety and efficacy of its Bio-Courier™ next generation silicon stabilized hybrid lipid nanoparticles (sshLNP) as an effective non-viral delivery system for Autosomal Dominant Osteopetrosis 2 (ADO2) siRNA therapy.

The data was presented at the American Society of Bone and Mineral Research (ASBMR) 2022 Annual Meeting, 9-12 September, Austin, Texas, USA by SiSaf's collaborators from the University of L'Aquila, Italy.

SiSaf's lead in house program, SIS-101-ADO, is an ADO2-specific siRNA combined with SiSaf's Bio-Courier platform, that aims to suppress the expression of the mutant CLCN7 gene and thereby rescue bone mass and quality to nearly normal levels.

The new data confirmed that sshLNPs can be designed for specific cell lines using different formulations. It also confirmed sshLNP is an effective non-viral delivery system for AD02, with the data showing down regulation of the CLCN7 gene. Most importantly, the data confirms the safety profile of siRNA delivered by the sshLNP technology in an animal model, with no side effects at the end of the full-length study in which rodent model received three injections per week for four weeks.

Dr Suzanne Saffie-Siebert, CEO of SiSaf Ltd said, "Skeletal rare diseases still pose unmet therapeutic needs as the major challenge related to intrinsic difficulty in targeting bone. SiSaf's silicon stabilised hybrid lipid nanoparticles constitutes a next-generation non-viral vectors able to retain both integrity and stability of constructs over time and offering considerable payloads of biologicals without requiring any cold-chain distribution."

In presenting the data, Dr Antonio Maurizi from Professor Anna Maria Teti's team in the Department of Biotechnological and Applied Clinical Sciences concluded, "Given obtained results, SiSaf sshLNP™ constitutes the most promising bone targeting non-viral vector available for addressing rare skeletal disorders by safely and effectively delivering Biologicals of interest."

SiSaf is developing the trial design and preparing the IND data package for a planned Phase I clinical study of SIS-101-ADO, with the aim of submitting an application to the U.S. FDA within the next six months.

SiSaf has an ongoing collaboration with Professor Teti's group and is working to extend its current pipeline of Bio-Courier targeted siRNA therapies to cure other genetic skeletal disorders. Its strategy is to develop its pipeline of novel therapies through clinical studies and then seek collaborative partnerships with marketing expertise in genetic skeletal disorders and rare diseases.

Read the original article on PR Newswire.