

NEWS RELEASE



January 25, 2022

Nippon Shinyaku and Capricor Therapeutics enter into an Exclusive Partnership for Commercialization and Distribution of CAP-1002 for the Treatment of Duchenne Muscular Dystrophy in US

KYOTO, Japan, January 25, 2022 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan, President: Toru Nakai) announced today that Nippon Shinyaku and Capricor Therapeutics (Headquarters: California, USA, CEO: Linda Marbán, NASDAQ: CAPR) have entered into an exclusive distribution agreement for CAP-1002 for the treatment of Duchenne muscular dystrophy (DMD) in the territory of US.

DMD is a progressive muscular dystrophy caused by a deficiency of the dystrophin protein leading to weakness of skeletal, cardiac, and pulmonary muscles. DMD has a variety of genotypic variants, and therapeutic agents have been developed for patients with DMD caused by specific genetic mutations, thus contributing to the fulfillment of unmet medical needs. However, treatment for DMD patients due to the other type of genetic mutations and treatment for relatively older, non-ambulant DMD patients is still unsatisfactory, and effective therapies are required to be developed.

CAP-1002 is comprised of human allogeneic cardiosphere-derived cells. Exosomes (extracellular vesicles) secreted by CAP-1002 are thought to be the mechanism of action of CAP-1002 and have been shown to reduce oxidative stress, inflammation, fibrosis, and increase myocyte generation, as well as improve motor and cardiac function. CAP-1002 is expected to be effective in a wide range of DMD patients, regardless of the type of genetic mutation, and a Phase II clinical trial (HOPE-2 study) suggested improved upper limb function and improved cardiac function in non-ambulatory DMD patients. CAP-1002 has received orphan drug designation and RMAT designation from the U.S. Food and Drug Administration (FDA) and Capricor is currently ready to initiate a Phase III clinical trial (HOPE-3 study) at several US health institutions. After the BLA approval in US, CAP-1002 will be marketed by our wholly owned subsidiary NS Pharma, Inc. (New Jersey, USA, President: Tsugio Tanaka).

Nippon Shinyaku is focusing on the field of intractable, rare disorders, and have commercialized our in-house developed DMD treatment, Viltepso® (an antisense exon skipping agent) in the US. Executing this agreement with Capricor, Nippon Shinyaku aims to contribute further to the treatment for patients suffering from DMD.

About exosomes

Exosomes (extracellular vesicles) are vesicles of about 30 to 150 nanometers in diameter surrounded by a lipid-doublet membrane that are secreted by cells. Exosomes were previously considered relating to the excretion of intracellular waste products, but in recent years, it has been discovered and well known that exosomes contain proteins and nucleic acids and act as important mediators of intercellular communication. Research and development of clinical applications, such as therapeutic, diagnostic, and DDS, using this function has been actively pursued.

About Orphan Drug Designation

The Orphan Drug Designation is provided for orphan drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the US, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. With orphan drug designation, the FDA grants a seven-year market exclusivity and provides tax incentives to the orphan drugs and biologics.

About RMAT designation

RMAT (Regenerative Medicine Advanced Therapy) is an FDA-designated system for advanced regenerative medicine therapies targeted at developing serious diseases that have shown certain effect in clinical trials based on the 21st Century Treatment Act (21st Century Cures Act) in US. Companies with RMAT designations are given the opportunity for priority review and accelerated approval for the product.

About Nippon Shinyaku

Our mission is to help people lead healthier and happier lives. Through creating unique medicines that will bring hope to patients and families struggling with illness, we aim to be an organization trusted by the community. Please visit our website (<https://www.nippon-shinyaku.co.jp/english/>) for products or detailed information.

About Capricor Therapeutics

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a biotechnology company focused on developing transformative cell and exosome-based therapeutics and vaccines for treating and preventing a broad spectrum of diseases. Capricor is also developing its exosome technology as a next-generation therapeutic platform for the delivery of nucleic acids, including mRNA to treat or prevent a variety of diseases. For more information, visit <https://www.capricor.com>.

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