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Chugai In-Licenses Gene Therapy Delandistrogene Moxeparvovec (SRP-9001) for Duchenne Muscular Dystrophy

- Chugai obtained exclusive marketing rights in Japan for delandistrogene moxeparvovec (SRP-9001), an investigational gene therapy for Duchenne muscular dystrophy
- EMBARK, global pivotal phase III study of delandistrogene moxeparvovec is currently conducted by Sarepta Therapeutics in partnership with Roche

TOKYO, December 16, 2021 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it concluded a license agreement with Roche (SIX: RO, ROG; OTCQX: RHHBY) for delandistrogene moxeparvovec (SRP-9001), an investigational gene therapy for Duchenne muscular dystrophy (DMD) currently under development by Roche and Sarepta Therapeutics Inc. (NASDAQ:SRPT).

Under the license agreement between Roche and Chugai, Chugai obtained exclusive marketing right in Japan for delandistrogene moxeparvovec, and Roche will receive an upfront fee and milestone payments. Sarepta is currently conducting a global phase III EMBARK study for delandistrogene moxeparvovec in patients with DMD in partnership with Roche. Sarepta will lead the future global clinical studies including in Japan based on an agreement between Roche and Sarepta. Chugai will be responsible for the regulatory filing and marketing of the therapy in Japan.

“I am very pleased to conclude this license agreement for delandistrogene moxeparvovec, which is the first gene therapy project in Chugai’s development pipeline. Our goal is to provide innovations that can fulfill unmet medical needs. External partnership is a very important approach for us to deliver innovative new modalities to patients in Japan,” said Chugai’s President and CEO, Dr. Osamu Okuda. “We will work closely with Roche and Sarepta to help improve the lives of people with DMD, a serious and progressive neuromuscular disease, through this project.”

Delandistrogene moxeparvovec is an investigational gene transfer therapy that uses AAVrh74 vector to deliver the micro-dystrophin-encoding gene directly to the muscle tissue for the targeted production of the microdystrophin protein. Delandistrogene moxeparvovec was designated as an orphan drug in the US, EU and Japan.

Chugai will continue to effectively utilize the research and development resources of the Roche Group to find innovative new drugs so as to satisfy unmet medical needs.

About Duchenne muscular dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is an X-linked rare degenerative neuromuscular disorder leading to progressive muscle weakness, diminished quality of life and premature death [1-3]. DMD affects approximately one in every 3,500-5,000 male births worldwide [1-3]. The prevalence in Japan is assumed to

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be 4,000-5,000 [4].

[Reference]

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