SEAL Therapeutics enters into collaboration agreement with Rutgers, The State University of New Jersey, to support its innovative gene therapy for muscular dystrophy

Basel, Switzerland, March 18, 2022 – SEAL Therapeutics announces the signing of a collaboration agreement with Rutgers, The State University of New Jersey, in support of its SEAL technology program for the treatment of LAMA2-related muscular dystrophy (LAMA2 MD or MDC1A).

SEAL Therapeutics has entered into a collaboration agreement under which the company will support research by Prof. Peter Yurchenco, a pioneer in a novel gene therapy approach for the treatment of LAMA2 MD. The work to be conducted under this agreement will support the methods and gene sequences and constructs for the treatment of LAMA2 MD already filed for patent protection by Rutgers and exclusively licensed to SEAL Therapeutics.

"We are very pleased being able to continue our long-standing collaboration with Prof. Yurchenco, following our common goal to advance innovative gene therapy treatment options for patients with LAMA2 MD", said Prof. Markus Rüegg, Co-Founder and CEO of SEAL Therapeutics. "Our combined effort has led to the development of our proprietary SEAL Technology as innovative and unique gene therapy approach to overcome the detrimental consequences of laminin α2-deficiency as seen in the muscle tissue of patients with LAMA2 MD."

“Gene replacement is a promising therapeutic option for the treatment of LAMA2 MD. The continued collaboration with Prof. Rüegg and his team and the funding now provided by SEAL Therapeutics to my research group will advance this important work,” said Peter D. Yurchenco, MD, PhD, Professor at Rutgers Robert Wood Johnson Medical School, USA. “Collaborating with the Basel team, we have been working on continuously optimizing linker proteins engineered from extracellular matrix proteins, which will aid in advancing such gene therapy approach towards clinical use.”

About LAMA2 MD (Merosin-deficient congenital muscular dystrophy or MDC1A)
Congenital muscular dystrophies (CMDs) are a group of genetic muscle diseases with onset at birth or very early infancy, which cannot be treated. The more than 30 known forms of these neuromuscular diseases differ in the type of genetic defect and in the severity of disease progression. The muscles of
the affected children progressively lose strength and degenerate over time. Progressive muscle weakness, joint contractures and respiratory insufficiency characterize most CMDs and patients often die before they reach adulthood.

Laminins are proteins of the extracellular matrix that are important in many tissues for the development, stability and survival of interacting cells. LAMA2-related muscular dystrophy (LAMA2 MD, also called MDC1A), is one of the most common forms of CMD. It is caused by mutations in the LAMA2 gene encoding the α2 subunit of laminin-211, a protein that stabilizes muscle fibers. Children affected by LAMA2 MD usually suffer from poor muscle tone and strength already at birth, and are therefore called “floppy infants”. Most of the affected children never learn to walk independently. The respiratory muscles are also weak and continue to degenerate, resulting in organ failure.

About the Simultaneous Expression of Artificial Linker (SEAL) technology

The innovative gene therapy approach (called SEAL technology), developed by Prof. Markus Rüegg and Prof. Peter Yurchenco and their teams over the past 20 years, overcomes the lack of laminin-α2 in muscle tissue by providing molecular connections with other laminins and with the plasma membrane of the muscle fibers. Available data demonstrate that the simultaneous expression of two specifically designed linker proteins functionally corrects the primary pathology of laminin-α2 deficiency, leads to sustained improvement in muscle histology, increased muscle mass and strength, improved body weight, and results in a remarkable increase in life span compared to untreated animals [1-8].

About Rutgers, The State University of New Jersey

Rutgers, The State University of New Jersey, is a leading national research university and the state of New Jersey’s preeminent, comprehensive public institution of higher education. Established in 1766, the university is the eighth-oldest higher education institution in the United States. More than 70,000 students and 23,400 full- and part-time faculty and staff learn, work and serve the public at Rutgers locations across New Jersey and around the world.

About SEAL Therapeutics AG

SEAL Therapeutics AG, a spin-off of the Biozentrum of University of Basel, develops proprietary SEAL technology as potential gene therapy treatment of LAMA2-related muscular dystrophy (LAMA2 MD; also called MDC1A). The Company combines technology from the Biozentrum, University of Basel and Rutgers, The State University of New Jersey. SEAL Therapeutics intends to team-up with and support a qualified pharma partner with experience in advanced gene therapy technologies for clinical development and registration with the ultimate goal to make this innovative treatment approach available to LAMA2 MD patients and their families.
References


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