University of Tokyo and Amplo Biotechnology sign Exclusive License Agreement for patent applications to advance novel DOK7 gene therapy for neuromuscular diseases

Summary

University of Tokyo today announced the signing of an exclusive license agreement for patent applications with Amplo Biotechnology, a new adeno-associated virus (AAV) gene Therapy Company, to develop an AAV expressing DOK7 gene for the treatment of several neuromuscular diseases.

Contents:

Professor Yuji Yamanashi of the Institute of Medical Science at the University of Tokyo is an inventor of the DOK7 gene therapy, notes that neuromuscular junction function loss leads to the loss of motor function including breathing and swallowing. His research group previously identified DOK7 as an essential protein for neuromuscular junction formation. This group, in collaboration with David Beeson's group of University of Oxford, also identified a neuromuscular junction disorder caused by abnormalities in DOK7 gene: DOK7 myasthenia (references 1, 2). In mice, systemic administration of an AAV expressing DOK7 gene (AAV-D7) has shown therapeutic benefit in models of DOK7 myasthenia, Emery-Dreifuss Muscular Dystrophy and Amyotrophic Lateral Sclerosis (references 3, 4).

Amplo Biotechnology identified the potential of DOK7 to significantly improve the life of many patients suffering from neuromuscular disorders. Amplo Biotechnology believes "The data set in DOK7 myasthenia and in ALS is impressive, significantly increasing survival in mouse models of these devastating diseases. We, at Amplo, are excited to move the therapy forward for the benefit of patients."

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About Amplo Biotechnology
Amplo is a gene therapy company focused on developing novel AAV therapies for rare neurodegenerative and neuromuscular disorders.

References:

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