Universität Basel

Research Project

Gene therapy for LAMA2 MD / MDC1A

Third-party funded project

Project title Gene therapy for LAMA2 MD / MDC1A
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Organisation / **Research unit**
Departement Biozentrum / Pharmacology/Neurobiology (Rüegg)
Department
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Muscular dystrophies are progressive, muscle wasting diseases of skeletal muscle fibers. So far, none of these monogenetic diseases, which often manifest in children, can be treated. The research group of Markus Rüegg has developed a treatment option in mouse models for a severe congenital muscular dystrophy, called LAMA2 MD or MDC1A. This treatment allows the mice to reach an almost normal lifespan while untreated mice die at the age of a few months. The recent advances in adeno-associated virus (AAV)-based gene therapy now make it possible to translate this treatment to human patients. The submitted proposal aims at critically testing the efficacy and potency of this gene therapeutic approach. To this end, AAV vectors will be applied systemically at different concentrations and at different time-points and their curative capacity will be measured. This innovative research will be conducted in collaboration and with the support of Santhera Pharmaceuticals Ltd., a Switzerland-based SME that has successfully conducted the first-ever phase 1 clinical trial in children suffering from LAMA2 MD. Thus, this program will pave the way to a possible treatment of patients. The clinical expertise of Santhera Pharmaceuticals Ltd. in combination with the preclinical expertise of the group of Markus Rüegg in developing this treatment will assure it will reach the patients as fast as possible.

Keywords LAMA2 MD, muscular dystrophy, gene therapy, laminin
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