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Establishment of a human-based in vitro functional NMJ system for ALS drug screening

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The neuromuscular system represents the major functional outputs of the central nervous system (CNS). Its dysfunction is closely related to many neurodegenerative diseases, including ALS (Amyotrophic Lateral Sclerosis). This study aims to develop a defined human-based functional neuromuscular system for the study and drug development of ALS disease. We have developed a defined *in vitro* system in which functional neuromuscular junctions (NMJs) can be formed with human stem cells as the sources. With the implementation of PDMS chamber system, this NMJ system is then modified by separating motoneurons and muscle cells, in which the functional analysis of NMJ formation can be better controlled. Motoneurons and muscle cells will be differentiated from the iPSCs derived from diseased patients. These patient-derived cells will then be incorporated into this functional NMJ system for the pathological and therapeutic study of disease. The outcome of this study would generate efficient functional *in vitro* model which would be clinically applicable to the study and treatment of various NMJ pathologies.

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