microRNA-induced Direct Conversion of Human Fibroblasts into Motor Neurons

**Background:**
Neurodegenerative diseases such as Amyotrophic Lateral Sclerosis (ALS) and Spinal Muscular Atrophy (SMA) are neurological disorders that selectively affect motor neurons. ALS is a fatal late-onset neurodegenerative disease, affecting approximately 30,000 patients in the United States alone. There are currently only two FDA-approved medications available for treatment, part of which is due to the difficulty of studying motor neurons both *in vitro* and *in vivo*. Few animal models are available for ALS studies and deriving patient models with pluripotent stem cells can take a significant amount of time, with low yields. Additionally, the genetic mutations involved in ALS can widely vary across patients.

**Technology Description:**
This technology is a novel method to directly convert human fibroblasts into human motor neurons via microRNA. The method is highly efficient and notable in that donor age marks and positional information are retained to maintain cellular “age”. This direct conversion of patient-derived cells is particularly beneficial to the study of such late-onset diseases, as cellular age is maintained as opposed to cellular rejuvenation in induced pluripotent stem cells (iSPCs). The specificity, speed, and efficiency make this method distinctly advantageous for neurodegenerative disease studies as the technology simultaneously provides patient-derived motor neurons for both disease modeling and regenerative medicine.

**Advantages:**
- Direct reprogramming of fibroblasts with approximately 80% efficiency
- Reprogrammed cells display gene expression analogous to endogenous motor neurons
- Cellular age is maintained in converted cells
- Universal conversion without reliance on available pluripotent cells


**Patent/Patent Application:** Patent Pending

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**Application Space**
Developmental biology, Neurodegenerative disease, Amyotrophic lateral sclerosis (ALS), Regenerative medicine