Exploring the metabolic signature of Amyotrophic Lateral Sclerosis patients-derived cells

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Abstract:
Amyotrophic lateral sclerosis (ALS) is a relentlessly fatal neurodegenerative disease characterized by a selective degeneration of upper motor neurons in motor cortex and lower motor neurons in brainstem and spinal cord, leading to rapid paralysis and death[1]. To date, the pathogenesis is largely unknown and effective therapies still lacking.
A massive amount of evidence shows the pivotal role of abnormalities in energy metabolism and mitochondrial dysfunction in ALS, suggesting a strong association between mitochondria metabolism disorder and neurodegeneration[2,3].
Here, we are elucidating the metabolic profile of patients-derived in vitro models of ALS aiming to obtain a more in-depth understanding of metabolism in health and disease and to provide a platform to unravel the pathological mechanisms underpinning ALS. Defining the role of the impaired metabolism might identify novel therapeutic targets and design innovative treatments for ALS patients.

[1] Brown et al., (2017), Amyotrophic Lateral Sclerosis, doi: 10.1056/NEJMra1603471.
[2] Tefera et al., (2021), doi: 10.1186/s13578-020-00511-2.
[3] Vandoorne et al., (2018), doi: 10.1007/s00401-018-1835-x