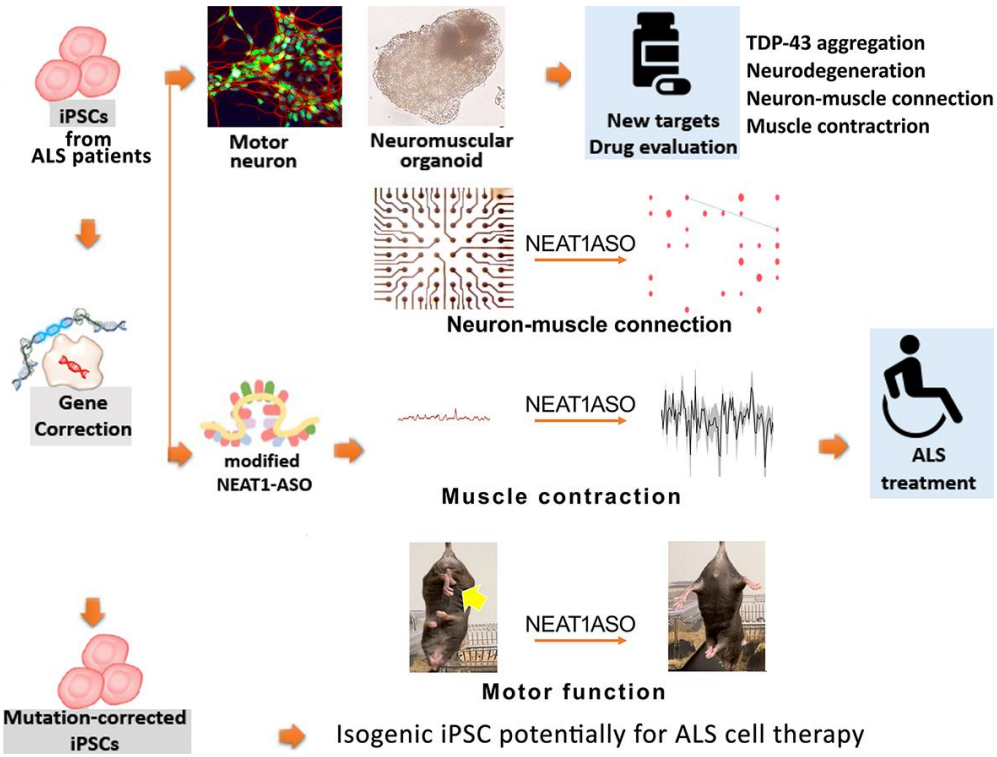
反義寡核苷酸降低lncRNA 作為肌萎縮性側索硬化症的治療劑

主要領域 神經退化性疾病

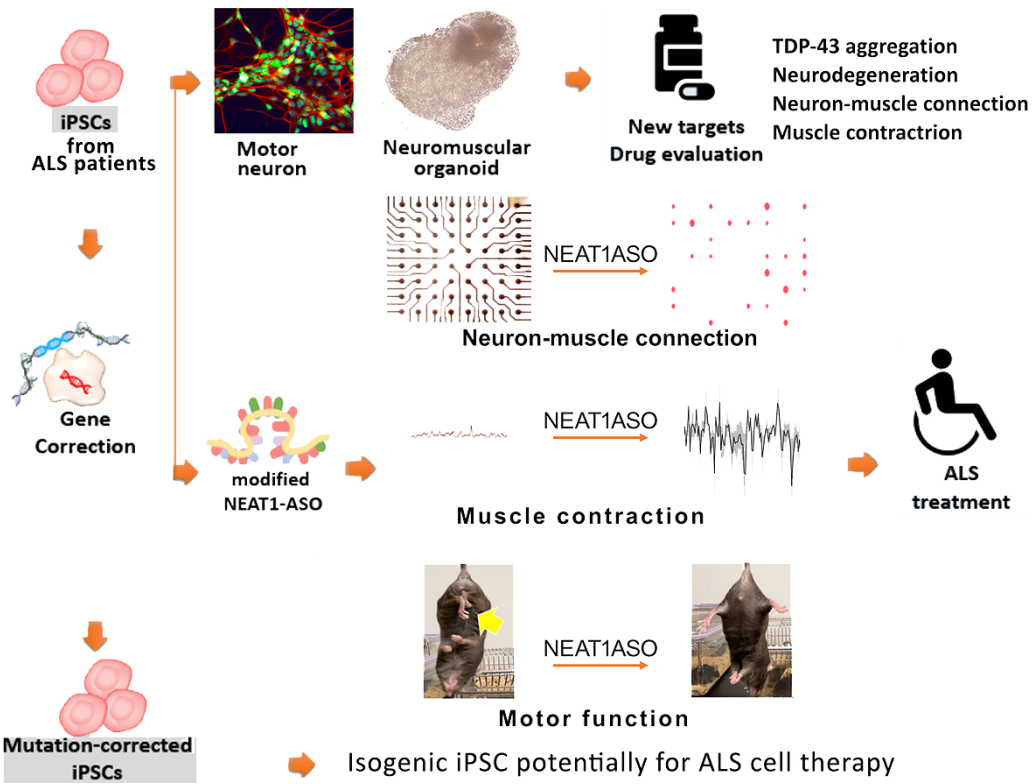
- 產品/技術簡介
  - 以iPSC技術發展肌萎縮性側索硬化症(ALS)的治療劑 ，建立疾病篩藥細胞平台並進行新藥研發與確效。
  - 針對LncRNA NEAT1, 設計NEAT1ASO 為ALS藥物，可降低TDP43不正常堆疊與神經細胞死亡，維持肌肉收縮能力。
- 應用:**神經退化性疾病新藥**
  - **治療TDP43 aggregation 導致之神經退化性疾!**
  - **減緩ALS肌肉功能缺損!**
- 優勢
  - 尚無有效治療ALS 之藥物。
  - NEAT1ASO 為有療效且具專利保護之小分子藥物。
- 專利現況
  - 中華民國發明專利 : I729363(2021)
  - 美國專利公開號：US20190282605A1(2022獲准)



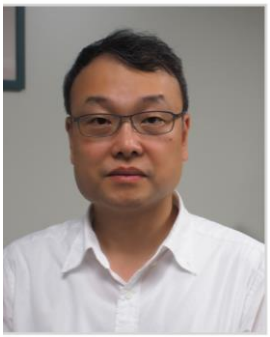
Antisense oligonucleotides targeting lncRNAs as therapeutics for amyotrophic lateral sclerosis

Research Area Neurodegenerative Disease

- Technical statement
  - ALS-iPSC as in vitro models for drug screening/validation platform and identify novel drug targets.
  - Developed ASOs drugs targeting LncRNA NEAT1 (NEAT1ASO) prevents TDP43 aggregations and maintain motor function.
- Applications
  - **Treatment of TDP43 related neurodegenerative diseases !**
  - **Treatment of ALS patients with impaired motor function !**
- Advantages
  - No effective drug is available for ALS.
  - NEAT1ASO is effective and patent-protected therapeutic drug.
- Patent status
  - Taiwan patent No. I729363(2021);
  - USA patent Pub. No. US20190282605A1 (allowed 2022)



計畫主持人 Project PI 郭紘志



計畫成員 Member

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