

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

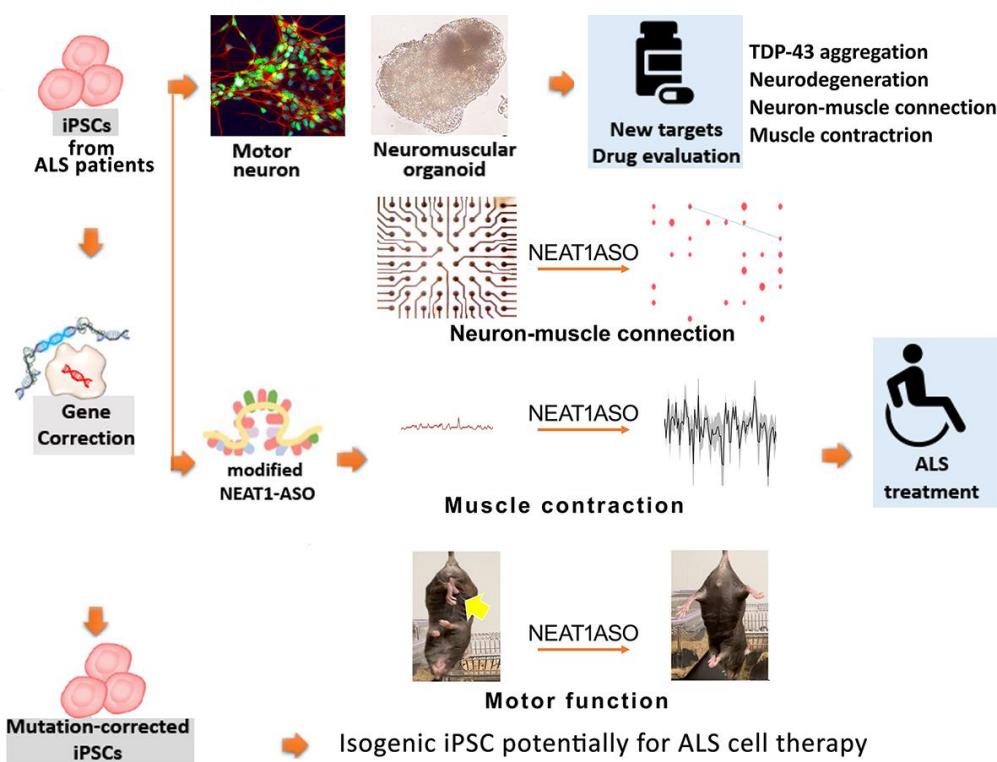
- 治療TDP43 aggregation 導致之神經退化性疾!
- 減緩ALS肌肉功能缺損!

■ 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

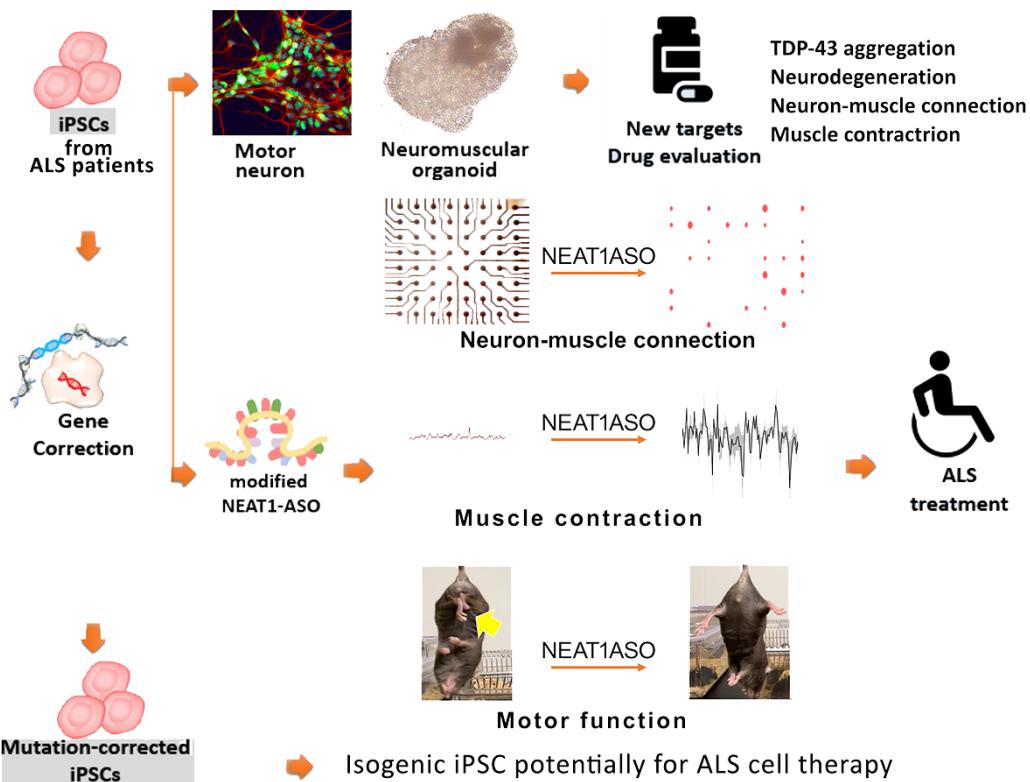
- Treatment of TDP43 related neurodegenerative diseases !
- Treatment of ALS patients with impaired motor function !

▪ 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)



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