

Talarozole Improves Key Features Of Leigh Syndrome

Talarozole is a promising drug candidate for Leigh Syndrome

Invention

Leigh Syndrome is progressing rare disease leading to neurodegenerative processes starting in early childhood. The disease is incurable and usually leads to death in early childhood. The invention discloses the azole compound talarozole as a promising drug candidate for the treatment of Leigh disease. The inventors could demonstrate that talarozole rescued neuronal morphogenesis in Leigh neurons, lowered lactate release and improved growth rate in Leigh midbrain organoids. Mechanistically, talarozole modulated the retinoic acid pathway and membrane-associated lipid metabolism. Hence talarozole seems to be a promising first-in-class candidate for the treatment of Leigh Syndrome by rescuing defect energy metabolism.

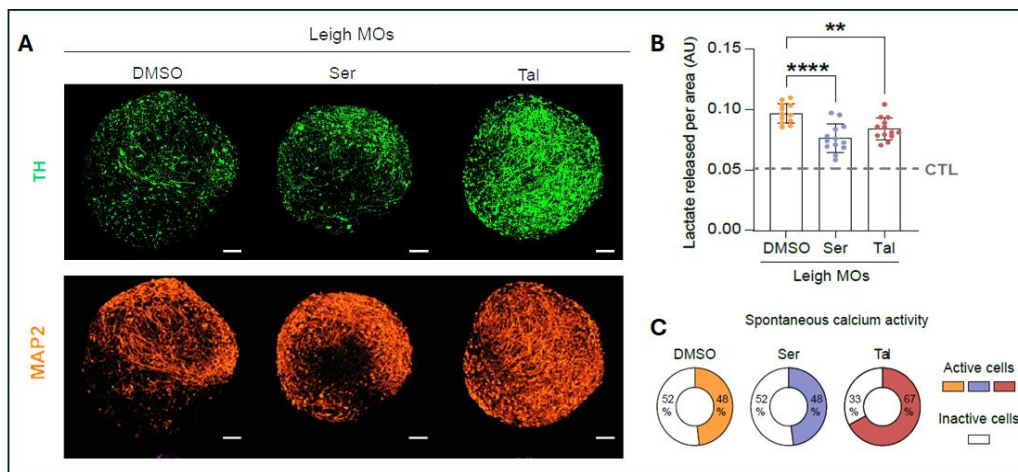


Figure 1: Talarozole improves neuromorphogenesis in Leigh organoids without impacting mitochondrial anchoring

As shown in Figure 1:

(A) Midbrain organoids (MO) from Leigh syndrome patients express low level dopaminergic marker tyrosine hydroxylase (TH) and dysregulated pattern of expression of dendritic marker MAP2. Treatment with Talarozole (Tal) corrects these defects.

(B) The pathological increase of lactate produced by Leigh midbrain organoids is reduced following treatment with Talarozole (Tal).

(C) The functionality of Leigh midbrain organoids is significantly improved following treatment with Talarozole (Tal).

Commercial Opportunities

The invention offers talarozole for the treatment of Leigh Syndrome.

Current Status

A PCT-application was filed for the invention (PCT/EP2025/056901) on 13.03.2025.

Relevant Publications

Deep learning-driven neuromorphogenesis screenings identify repurposable drugs for mitochondrial disease. Menacho et al. 2024. bioRxiv 2024.07.08.602501;

doi: <https://doi.org/10.1101/2024.07.08.602501>

An invention from the Heinrich-Heine-University Düsseldorf and University of Luxembourg.

Advantages

- Compound for treatment of Leigh Syndrome
- First-in-class candidate
- Experimental validation available

Technology Readiness Level

1 2 3 4 5 6 7 8 9
Experimental proof of concept

Sector(s)

- Pharmaceutical industry

Ref.-No.

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