Drug discovery for mitochondrially inherited Leigh syndrome (MILS)


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SUMMARY

The team has developed a novel assay system based on patient-derived induced pluripotent stem cells (iPSCs) to identify compounds for treating Leigh syndrome. Using this assay, a class of drugs applicable for repurposing that restore the cellular disease phenotype has been identified. The team has initiated a compassionate use treatment for a terminal ill patient. The patient has recovered significantly. Based on these results a clinical study is planned. Leigh syndrome is a rare severe mitochondrial disease affecting children where treatment options are lacking.

PROJECT ACHIEVEMENTS DURING & AFTER SPARK

- Identified and validated compound class for treatment of Leigh syndrome
- Performed compassionate treatment
- Plan to prepare phase I/II orphan drug repurposing trial

LONG-TERM GOALS

- Run a clinical study