

Therapeutic Strategy by Drug Repositioning against Lowe Syndrome

Existing FDA-approved drugs (rapamycin and statins) are repurposed as a therapeutic strategy to ameliorate symptoms of Lowe Syndrome and potentially other genetic conditions by targeting shared molecular pathways.

Researchers at Purdue University have developed a therapeutic strategy for Lowe Syndrome (LS), a currently untreatable genetic disorder characterized by cognitive deficiencies, bilateral congenital cataracts and renal dysfunction that leads to the early death of those affected, often from kidney failure. The Purdue treatment method has the potential to reverse symptoms and offer patients a higher quality of life. The researchers designed this treatment regimen using rapamycin and statins, drugs approved by the FDA for other indications because of on those drugs' ability to target two molecular pathways implicated in LS. The drugs produced a phenotype similar to normal cells in a variety of tests on LS patient cells. This technology addresses the molecular basis of LS and promises to ameliorate symptoms. This strategy also has the potential to help those suffering from other genetic conditions in which the same cellular pathways are affected.

Related Publication:

Lowe syndrome patient cells display mTOR- and RhoGTPase-dependent phenotypes alleviated by rapamycin and statins

Hum Mol Genet. 2020 Jun 27;29(10):1700-1715

doi: 10.1093/hmg/ddaa086

Advantages:

- Uses FDA Approved drugs with low risk of adverse effects
- Addresses the molecular basis of symptoms in Lowe Syndrome

Applications:

- Therapeutic for Lowe Syndrome

Technology ID

2017-AGUI-67722

Category

Biotechnology & Life
Sciences/Biomarker Discovery &
Diagnostics
Biotechnology & Life
Sciences/Bioinformatics &
Computational Biology
Artificial Intelligence & Machine
Learning/AI Model Optimization
& Acceleration Tools
Pharmaceuticals/Drug Discovery
& Development
Pharmaceuticals/Small Molecule
Therapeutics

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- Strategy could be applied to other genetic conditions

Technology Validation:

The Purdue researchers analyzed the effect of rapamycin and statins on the cellular and molecular phenotypes of Lowe Syndrome patient cells.

TRL: 3

Intellectual Property:

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Keywords: Lowe Syndrome therapeutic strategy, rapamycin, statins, genetic disorder treatment, mTOR pathway, RhoGTPase pathway, FDA approved drugs, renal dysfunction, congenital cataracts, cognitive deficiencies