

## Treatment of Lowe Syndrome with Repurposed Drugs

**Track Code:** 2017-AGUI-67722

**Categories:**

- Pharmaceuticals

**Keywords:**

- Children
- drug therapeutics
- genetic disease
- Genetic Diseases
- kidney
- Lowe Syndrome
- Neurological
- ocular
- Pediatric
- rare disease
- Rare Pediatric
- renal
- Therapeutics

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
- 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.

**People:**

- Aguilar, Ruben Claudio (Project leader)

**Intellectual Property:**

**Application Date:** July 8, 2021

**Type:** Utility-Gov. Funding

**Country of Filing:** United States

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