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GAL KINASE INHIBITOR TO TREAT GALACTOSEMIA

THERAPEUTICS

Small molecule inhibitor of Galactokinase to treat a rare metabolic disorder, Galactosemia.

TECHNOLOGY TYPE

Rare Diseases
Small Molecule
Oncology

STAGE OF DEVELOPMENT

Prototype in development

IP PROTECTION

Nationalized PCT Issued in the United States

Galactokinase Inhibitors for the Treatment and Prevention of Associated Diseases and Disorders
US9447087B2

Continuations Pending in the United States

Galactokinase Inhibitors for the Treatment and Prevention of Associated Diseases and Disorders
US20170035760A1
US20190030031A1

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Reference Number: U-5171

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TECHNOLOGY SUMMARY

Galactosemia is a rare, inherited condition where people cannot metabolize galactose due to mutations in GALT, GALE, and GALK1. The disorder appears in 1 in 40,000 live births and can be fatal if left untreated, but no long-term treatment exists. Although removal of galactose from the diet can prevent death, galactosemia patients still experience intellectual and speech deficits, motor function loss, ataxia, and infertility due to galactose accumulation.

Accumulation of Gal1-p results in a unique form of endoplasmic reticulum stress in fibroblasts of patients with Galactosemia. A small molecule GALK inhibitor can alleviate symptoms of Galactosemia by reducing accumulation of human galactose. GALK inhibitor leads have been identified for testing in animal models. Patient cell lines, crystal structure data, and animal models for Galactosemia are available for rapid development of an IND enabling candidate.

FEATURES AND BENEFITS

- First-in-class small molecule therapeutic for Galactosemia.
- Reduces toxicity.
- Utilizes a novel mechanism of action.
- Demonstrates potential extended applications in cancer with elevated AKT signaling.

RECENT PUBLICATIONS

Odejinmi, S.I., Rascon, R.G., Tang, M., Vankayalapati, H., Lai, K. (2011). Structure-activity analysis and cell-based optimization of human galactokinase inhibitors. *ACS Medicinal Chemistry Letters*. 2(9): 667-672. doi: [10.1021/ml200131j](https://doi.org/10.1021/ml200131j)

INVENTOR PROFILE

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