Cincinnati Children's | Innovation Ventures

Genetic Diseases

Novel Treatment to Ensure Efficacious Dosage for Multiple, Rare Genetic Diseases

Brief Description of Technology

Optimal dosing regimen for treating seizures and self-aggressive behavior

TECHNOLOGY ID

2019-0505

BUSINESS OPPORTUNITY

Exclusive License

TECHNOLOGY TYPE

Small Molecule

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Technology Overview

Clinicians use a common, FDA approved drug to treat symptoms such as seizures and self-aggressive behavior. In some genetically identified patients, too low of a dose is not effective and too high of a dose is not optimal. Sub-optimal dosing may also cause sleep problems, dyslipidemia, diabetes, and growth problems. We are working on refining the dosing regimen for patient sub-populations.

Applications

Smith Kingsmore Syndrome, Tuberous sclerosis, PMSE, and other similar genetic diseases.

Advantages

- Repurposing an FDA approved drug.
- A biomarker for optimal dosing.
- Strategy likely applies to other mTORopathies.

Investigator Overview

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