

# **Repurposing Drug Libraries and Artificial Intelligence for the Discovery of New Treatments for Cystinosis**

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The processes that take place in the degradative and recycling compartments of the cell (named lysosomes) are affected in cystinosis. We have discovered that a small molecule (potential medication) that increases the availability of the recycling receptor, improves cellular and kidney function in cystinosis. However, it could take many years to get new drugs into the clinic. To accelerate the discovery of medications with similar drug activity to the small molecule we have identified, we are utilizing a computer-based modeling technique named molecular docking. We are using this artificial intelligence approach to screen drug libraries with FDA approved compounds with the idea that newly identified compounds will be rapidly repurposed as their clinical utilization (doses, toxicology) has already been established. We have screened a best-in-class drug repurposing library containing nearly all small molecules that have reached clinical development with around 12,000 compounds. We will present data supporting the use of this new strategy to accelerate the discovery of new therapeutics for cystinosis.