Small Molecule Binders of a Human Peptide for Therapeutic Use

Molecules that may be used to treat the symptoms of an inherited protein deficiency that causes developmental issues

Inventors from Georgia Tech's School of Biology have conducted a study that casts aside the mechanical underpinnings of the disorder in favor of discovering small-molecule binders that have the potential to alleviate symptoms. Through their threading/structure-based virtual ligand screening algorithm, they identified nine distinct ligands that may bind to the NGly1 with therapeutic effects. Inventors have identified ligands binding to the NGly1 protein as a possible therapeutic solution to disorders caused by NGly1 mutants.

Summary Bullets

- First discovery of small-molecules binding to the central catalytic domain of NGly1
- Could be used to treat the inherited genetic disorder resulting from NGly1 deficiency

Solution Advantages

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- Could be used to treat the inherited genetic disorder resulting from NGly1 deficiency

Potential Commercial Applications

- Therapeutic properties useful for treating the symptoms brought about by NGly1 mutant

Background and More Information

Peptide N-glycanases (NGly1) is an enzyme responsible for removing proteins from the body that are not functioning normally. Recently, it has been demonstrated that mutations in this gene are responsible for an inherited disorder of the endoplasmic reticulum-associated degradation pathway (the cell component responsible for detecting and eliminating misfolded, aggregated, and unassembled proteins). However, the literature is unclear whether the disorder is a result of mutations leading to loss-of-function, loss of substrate specificity, or loss of protein stability. When these gene errors exist, those afflicted may exhibit movement disorder (choreoathetosis), liver disease, developmental delay, weak muscle tone, and small head size.

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