(SD2017-252) Strategy for in vivo Depalmitoylation of Proteins and Therapeutic Applications Thereof

Tech ID: 30462 / UC Case 2017-252-0

BACKGROUND

The neuronal ceroid lipofuscinoses (NCLs), commonly grouped together as Batten disease, are the most common neurodegenerative lysosomal storage diseases of the pediatric population. No cure for NCL has yet been realized. Current treatment regimens offer only symptomatic relief and do not target the underlying cause of the disease. Although the underlying pathophysiology that drives disease progression is unknown, several small molecules have been identified with diverse mechanisms of action that provide promise for the treatment of this devastating disease. On this point, several researchers have reported the use of potential drugs for NCL patient lymphoblasts and fibroblasts, along with neurons derived from animal models of NCL disease. Unfortunately, most of these studies were inconclusive or clinical trials or follow-up results were not available. High concentrations employed and toxicity of the small molecules are clear disadvantages to the use of some of the corresponding derivatives as potential drugs. To circumvent these effects, development of nontoxic alkyl cysteines would be useful for the non-enzymatic and chemo-selective depalmitoylation of S-palmitoyl proteins, which hold good promise as an effective treatment for neuronal ceroid lipofuscinoses.

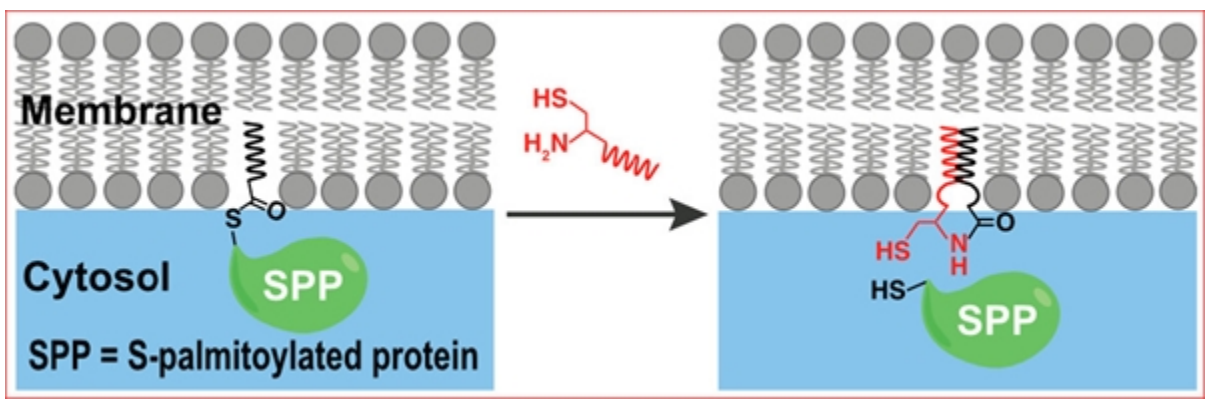
TECHNOLOGY DESCRIPTION

Researchers from UC San Diego have developed an NCL-based depalmitoylation strategy for the chemo-selective cleavage of S-palmitoyl groups in vivo. This technology efficiently depalmitoylates proteins at the plasma membrane and internal membranes and is poised to allow the development of novel potential nontoxic drugs for efficient targeting of thioesterase deficiency diseases, such as infantile neuronal ceroid lipofuscinosis.

APPLICATIONS

This compound has potential therapeutic applications for a number of diseases in which palmitoylation is dysregulated such as cancer, Alzheimer's, Infantile Neuronal Ceroid Lipofuscinosis. This technology is poised to become a powerful tool that will aid studies of protein depalmitoylation in vivo.

STATE OF DEVELOPMENT



INTELLECTUAL PROPERTY INFO

This technology is patent pending and available for licensing.

RELATED MATERIALS

- Rudd AK, Brea RJ, Devaraj NK. Amphiphile-Mediated Depalmitoylation of Proteins in Living Cells. J Am Chem Soc. 2018 Dec 19;140(50):17374-17378. doi:10.1021/jacs.8b10806. Epub 2018 Dec 10. - 12/19/2018

PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	2019/178166	09/19/2019	2017-252

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OTHER INFORMATION

KEYWORDS

palmitoylation; Batten disease; neuronal ceroid lipofuscinoses (NCLs); research reagent; therapeutics

CATEGORIZED AS

- **Medical**
  - Disease: Cancer
  - Disease: Central Nervous System
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Research Tools

RELATED CASES

2017-252-0

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