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XNA enzymes to Validate and Treat Genetic Diseases

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OTHER INFORMATION

CATEGORIZED AS

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 - » Therapeutics
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 - » Nucleic Acids/DNA/RNA

RELATED CASES

BRIEF DESCRIPTION

Allelic proteins are often considered undruggable targets, because therapeutics that interfere with these proteins while leaving the wild-type protein unharmed are difficult to come by. Researchers at UCI have developed a xeno-nucleic enzyme (XNAzyme) that offers a solution to this problem by selectively cleaving the mRNA of mutant alleles while leaving the wild-type mRNA unharmed. This novel gene silencing technology offers an efficient, safe, and effective approach to treating genetic diseases.

SUGGESTED USES

- Validation of disease-causing mutations
- Treatment for genetic diseases
- Research tool for silencing genes
- Treatment for infectious diseases and cancer

FEATURES/BENEFITS

- Efficiency: maintains multiple turnover activityunder physiological conditions
- Activity: functions in cells and under physiological conditions
- Stability: highly resistant to nuclease digestion in biological environments
- Specificity: exhibits sequence-specific mRNA cleavage
- Engineerable: easily modified to cleave desired sequences
- Synthesis: constructed by solid-phase synthesis that avoid biological contaminants

TECHNOLOGY DESCRIPTION

Many diseases, such as cancer, produce healthy and disease-causing versions of the same protein in our cells.Establishing therapeutics that can effectively treat these diseases has become a grand challenge in precision medicine, as it is extremely difficult to distinguish RNA messages that differ by a single mutation. Conventional gene silencing technologies, such as antisense, siRNA, and CRISPR, all suffer from this problem.

Inventors at UCI have addressed this issue by developing an XNAzyme that can site-specifically cleave mRNA strands at precise nucleotide positions. This reagent silences the expression of disease-associate proteins by cutting the RNA templates that encode the disease-associated proteins while leaving the healthy RNA templates unharmed.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20240392298	11/28/2024	2020-350
Patent Cooperation Treaty	Published Application	WO 2022/146868	07/07/2022	2020-350

STATE OF DEVELOPMENT

In vitro biochemical studies are complete, in cellulo studies are in progress. Inventors have demonstrated that XNAzyme molecules can find, bind, and cleave target RNA, resulting in the reduction of a fluorescent protein.

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