DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Government-Owned Inventions; Availability for Licensing

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: The National Institute of Allergy and Infectious Diseases (NIAID), an institute of the National Institutes of Health (NIH), Department of Health and Human Services (HHS), is giving notice of the invention listed below, which is owned by an agency of the U.S. Government and is available for licensing to achieve expeditious commercialization of results of federally funded research and development. Foreign patent applications are filed on selected inventions to extend market coverage for companies and may also be available for licensing.

FOR FURTHER INFORMATION CONTACT: Inquiries related to this licensing opportunity should be directed to: David Yang at 240-695-6406, or David.Yang@niaid.nih.gov. Licensing information may be obtained by communicating with the Technology Transfer and Intellectual Property Office, National Institute of Allergy and Infectious Diseases, 5601 Fishers Lane, Rockville, MD 20852: tel. 301-496-2644. A signed Confidential Disclosure Agreement will be required to receive copies of unpublished information related to the invention.

SUPPLEMENTARY INFORMATION: Technology description follows:

Gene Editing for ALPK1 p.Thr237Met.

Description of Technology:

ROSAH syndrome is a rare genetic disease caused by a mutation in the human alpha kinase 1 (ALPK1) gene (p.Thr237Met), leading to vision loss, swollen optic nerves, dry mouth, enlarged spleen, and frequent headaches. Researchers in the Laboratory of Clinical Immunology and Microbiology (LCIM) at the National Institute of Allergy and Infectious Diseases (NIAID) have developed a new approach that can precisely fix the ALPK1 mutation without causing

unwanted changes in the patient's DNA. This method uses a base editor combined with a guide RNA to safely and efficiently convert the pathogenic thymine of the mutation back to cytosine. In laboratory tests, this gene editing technology successfully repaired the mutation in patient-derived affected cells with high accuracy and no side effects.

This therapy could be delivered directly to the eye or salivary glands, or patient cells could be corrected outside the body and then returned to the patient, offering hope for personalized treatment to restore vision and improve quality of life for people with ROSAH syndrome.

This technology is available for licensing for commercial development in accordance with 35 U.S.C. § 209 and 37 CFR part 404, as well as for further development and evaluation under a research collaboration.

Potential Commercial Applications:

- Personalized therapy for individuals with disease secondary to the ALPK1 p.Thr237Met genetic variant.

Competitive Advantages:

- Highly accurate tool that directly repairs the faulty gene that causes ROSAH syndrome, while avoiding unwanted changes elsewhere in DNA.
- Corrects the mutation in most patient cells with few or no mistakes.
- Can be delivered directly to affected areas (e.g., eye or salivary glands) or can treat patient cells outside the body.
- Custom therapy for people with the ALPK1 mutation.
- Effective in cells that don't divide, unlike older gene editing methods.

Development Stage:

- Pre-Clinical

Inventors: Dr. Christina Torres Kozycki, Dr. Colin L. Sweeney, Dr. Uimook Choi, all of NIAID.

Intellectual Property: HHS Reference No. E-044-2024-0. Provisional Patent Application No.

63/733,836, filed on December 13, 2024, and PCT Patent Application No. PCT/US2025/059432, filed on December 12, 2025.

Licensing Contact: To license this technology, please contact David Yang at 240-695-6406, or David.Yang@niaid.nih.gov, and reference E-044-2024-0.

Research Opportunity: The National Institute of Allergy and Infectious Diseases is seeking statements of capability or interest from parties interested in collaborative research to further develop, evaluate, or commercialize this technology. Area of specific interest includes human clinical trials. For collaboration opportunities, please contact David Yang at 240-695-6406, or David.Yang@niaid.nih.gov.

Dated: March 18, 2026.

Surekha Vathyam,

Director,

Technology Transfer and Intellectual Property Office,

National Institute of Allergy and Infectious Diseases.

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