

## Press release

# Breakthrough Technologies for Precise Genetic Medicine Advance in \$6M NIH Challenge

**The National Institutes of Health (NIH) announces winners for second phase of the TARGETED Challenge, accelerating development of next-generation delivery systems for genetic therapies**

**SAN FRANCISCO, California - 12 MAY 2025** – The United States National Institutes of Health (NIH) today announced the winners of Phase 2 of the [Targeted Genome Editor Delivery \(TARGETED\) Challenge](#), a competition to improve technologies for delivering genome editing tools to cells in the body.

Launched in May 2023, the \$6 million TARGETED Challenge aims to advance the current state of in vivo delivery technologies for genome editors in two target areas: 1) Programmable Delivery System for Gene Editing, and 2) Crossing the Blood-Brain Barrier. The challenge is funded through the NIH Common Fund's Somatic Cell Genome Editing (SCGE) program, which seeks to improve the efficacy and specificity of gene editing approaches to help reduce the burden of common and rare diseases caused by genetic changes.

The Phase 2 winners were selected based on their experimental data demonstrating successful delivery and editing performance, along with their innovative methodologies and technologies that effectively address the challenge of targeted gene editing delivery. In addition to the monetary prize, each winner will advance to Phase 3 of the challenge, where they will have the opportunity to prepare their technologies for testing in large animal models through NIH-supported independent evaluation.

The following teams and organizations have been selected as winners for Phase 2 of the TARGETED Challenge:

### **Target Area 1: Programmable Delivery System for Gene Editing**

Winning Solutions \$250,000 USD Prize

- **Exosome Engineers (University of Nebraska-Lincoln)** from Lincoln, NE and Durham, NC: *Editing the Genome in Any Tissue of Choice Through Programmable Milk Exosomes*
- **Perelman School of Medicine at the University of Pennsylvania** from Philadelphia, PA: *Targeted Delivery of Genome Editing Machinery to Lungs, Systemic Endothelium, and Muscles*
- **David R. Liu Group, Broad Institute of MIT and Harvard** from Cambridge, MA: *Tissue*

*Specific Targeted eVLPs Through Barcoded Lentiviral Screening and Rational Engineering*

- **Beth Israel Deaconess Medical Center and University of Washington** from Boston, MA and Seattle, WA: *ENTER: Elastin-based Nanoparticles for Therapeutic delivERY, Self-Assembled Protein Nanoparticles for Targeted Gene Editor Delivery*
- **Ben Deverman Vector Engineering Laboratory, Broad Institute of MIT & Harvard** from Cambridge, MA: *Engineering Receptor-Targeted AAVs with Predictable Cellular and Species Tropism*

Winning Solutions \$50,000 USD Prize

- **Columbia-Leong Oral Editing** from New York, NY: *Oral Nonviral Gene Editing System*
- **Helex, Inc.** from Boston, MA: *EPIC-Cure™ platform*
- **Medical College of Wisconsin and Università degli Studi di Milano (University of Milan)** from Milwaukee, WI and Milan, Italy: *One System, Any Target*
- **ProEdit (University of Washington)** from Seattle, WA: *ProEdit: Programmable Genome Editing to Target Diverse Organ and Cell Lineages*
- **Smart Gene Darts (SGD) (Case Western Reserve University)** from Cleveland, OH: *Smart Programmable Lipid Nanoparticle Platform for Gene Editing*

**Target Area 2: Crossing the Blood-Brain Barrier**

Winning Solutions \$250,000 USD Prize

- **Crisaptics Trans-BBB Genome Editing Team (University of Maryland School of Medicine)** from Baltimore, MD: *Crisaptics Trans-BBB*
- **PERCEPT (Innovative Genomics Institute at UC Berkeley)** from California and Ohio, USA : *Chemically engineered CRISPR enzymes for accessible whole-brain genome editing*
- **Icahn School of Medicine at Mount Sinai** from New York, NY: *Blood-brain barrier-crossing lipid nanoparticles for genome editing*
- **STEP Team (Yale University)** from New Haven, CT: *BRAIN TARGETED-STEP RNPs for Delivery of Genome Editing to the Brain*

Phase 3 of the challenge will be divided into two parts. In Phase 3a, winners will demonstrate that their technology is ready for large animal testing. Those who successfully complete Phase 3a will receive additional prize funding and advance to Phase 3b, where their technologies will undergo independent testing and validation in large animal models. The top-performing solutions in Phase 3b will be awarded up to \$625,000.

The TARGETED Challenge is expected to conclude in 2027, with the announcement of the final winners who successfully demonstrate their ability to deliver genome editing tools effectively to target tissues.



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### **About the NIH SCGE Program**

The NIH Common Fund's [Somatic Cell Genome Editing \(SCGE\)](#) program is working to improve the efficacy and specificity of gene editing approaches to help reduce the burden of common and rare diseases caused by genetic changes. SCGE is developing quality tools to perform and assess effective and safe genome editing in non-reproductive ("somatic") cells of the body. These research tools will be made widely available to the research community to reduce the time and cost required to develop new therapies.

**For more information, contact:**

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