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UNITED FOR MEDICAL RESEARCH

# NIH RESEARCH IN ACTION

**How NIH-funded research is making a difference for patients, families and communities**

## Clinical Trials

Clinical trials are an essential part of the medical innovation process, helping move promising treatments from the lab to the people who need them. But for those affected by disease, clinical trials often mean more than progress. They offer hope — that new or better treatments are on the horizon or that they might participate in a trial and have access to the latest treatments and specialized medical care. Learn more about clinical trials here.

**Here are three examples of clinical trials supported by the National Institutes of Health (NIH) that are offering new hope for better treatments for both rare and common conditions.**

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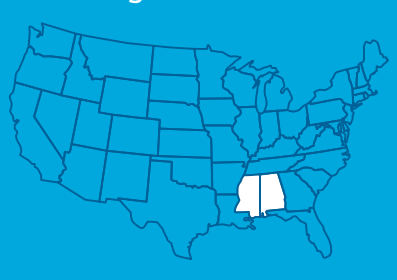
### Identifying Sustainable Methods to Support Better Diabetes Management in the Deep South

States located in the Deep South, such as Alabama and Mississippi, have the highest rates of type 2 diabetes and some of the worst outcomes due to diabetes-related cardiovascular disease and chronic kidney disease. Social determinants of health such as reduced health care access, quality of care, poverty, transportation barriers and food insecurity negatively impact diabetes management and health outcomes.

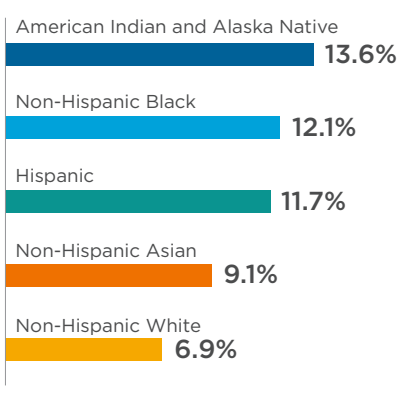
The four-year FREEDOM study, led by researchers at the University of Alabama (UAB) in conjunction with the **University of Mississippi Medical Center (UMMC)**, aims to identify effective and sustainable interventions that can improve diabetes related health outcomes among Black adults with sub-optimal social determi nants of health. Interventions being evaluated are digital health coaching, food box delivery, and remote patient monitoring. The study is supported by the **National Institute on Minority Health and Health Disparities**, part of the NIH, and the Forge AHEAD Center.

**15+%** adults in Alabama and Mississippi with diagnosed diabetes



**11.6%** average U.S. diabetes rate

**U.S. prevalence of diabetes by race and ethnicity (CDC data):**




“Our study will leverage multi-health care system and health care system-industry partnerships to promote health equity and to improve diabetes outcomes in socially vulnerable Black adults living in the Deep South.”

**TAPAN MEHTA, PhD**
*Vice Chair for Research, University of Alabama at Birmingham Department of Family and Community Medicine*

#### CLINICAL TRIAL

**304 Black adult patients with type 2 diabetes and related cardiovascular disease and chronic kidney disease will be enrolled in the FREEDOM study through researchers at UAB, UMMC, Cooper Green Mercy Health Systems and Pack Health. Study participants will get referred to a diabetes educator and may receive access to health coaching, food box delivery, and remote patient monitoring. By identifying the most effective intervention package, researchers aim to build a sustainable, health system-driven program capable of aiding those with type 2 diabetes.**

View trial at ClinicalTrials.gov LEARN MORE

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### Groundbreaking Clinical Trial Demonstrates Effectiveness of Gene Editing to Treat Inherited Form of Blindness in Adults and Children

A proof of concept study led by scientists from Harvard Medical School and Mass Eye and Ear, **Oregon Health Sciences University**, and University of Pennsylvania, evaluated the safety and effectiveness of EDIT-101, an experimental gene editing treatment developed by Editas Medicine, a spinoff founded by researchers at Harvard, Massachusetts General Hospital and Massachusetts Institute of Technology. Initial results of the BRILLIANCE study were published in May 2024.

EDIT-101 was designed to edit a mutation in the CEP290 gene, which provides instructions to create a protein that is critical for sight. People with this gene mutation have a rare condition that is commonly called Leber Congenital Amaurosis Type 10, or LCA10, for which there is currently no Food and Drug Administration-approved treatment.

The study’s primary focus was to determine whether the approach was safe and how well it worked before proceeding further. In addition to funding from Editas, the research was supported by a **National Eye Institute Center Core Grant for Vision Research** from the NIH and other organizations.

#### CLINICAL TRIAL

**For this trial, the goal was to inject a CRISPR medicine that could reach the retina and restore the function of a key gene and protein that enable light-sensing cells to work properly in people with LCA. All 14 trial participants, including 12 adults (ages 17 to 63) and two children (ages 10 and 14), received a single injection of the EDIT-101 gene-editing medicine designed to repair CEP290 in one eye. Eleven out of 14 participants experienced improvement on at least one vision-related outcome, while six had improvements on two or more.**

View trial at ClinicalTrials.gov LEARN MORE

**CRISPR**

**Is a technology that enables the editing of genes by precisely cutting DNA and then using natural DNA repair processes to modify the gene in the desired manner. CRISPR is short for “clustered regularly interspaced short palindromic repeats.”**

**Image credit**
Mass Eye and Ear, a member of Mass General Brigham

“This research demonstrates that CRISPR gene therapy for inherited vision loss is worth continued pursuit in research and clinical trials.”

**ERIC A. PIERCE, MD, PhD**
*William F. Chatlos professor of Ophthalmology and director of the Ocular Genomics Institute in the Department of Ophthalmology at Mass Eye and Ear and Harvard Medical School*

“Our patients are the first congenitally blind children to be treated with gene-editing, which significantly improved their ability to see things like daylight. Our hope is that the study will pave the road for treatments of younger children with similar conditions and further improvements in vision.”

**TOMAS S. ALEMAN, MD**
*The Irene Heinz-Given and John LaPorte Research Professor in Ophthalmology, Penn Medicine*

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### Using Wearable Technology to Prevent Stroke and Reduce Reliance on Blood Thinners

This seven-year study looks at an alternative strategy to prevent stroke in people with atrial fibrillation (AFib) that combines wearable heart monitoring technology with as-needed use of blood thinning medication. The study is a collaboration between Northwestern University Feinberg School of Medicine and the **American Heart Association**. It is supported by a grant from the **National Heart, Lung and Blood Institute**, part of the NIH.

AFib is the most common heart rhythm disorder in adults, affecting 2.5 to 5 million Americans. The current standard of care for patients with AFib is continuous use of blood thinners to reduce their risk of blood clots and stroke. However, blood thinning medication can increase the risk of bleeding and carries a financial cost for the patient as well.

Many people with AFib experience infrequent heart rhythm episodes. Current evidence suggests that the risk of stroke increases in the weeks following an AFib episode, then returns to a baseline level. Given these factors, the study is investigating whether limiting blood thinner use to only these high-risk periods is as effective as current practice, while potentially offering the added benefit of reducing bleeding risk.


#### CLINICAL TRIAL

**Approximately 5,400 people with AFib will be enrolled in the seven-year trial through health centers in 27 states. Trial participants will receive either the current standard of care or will use an app and Apple Watch to monitor for irregular heart rhythm. They will be directed through the app to take blood-thinning medicine for a few weeks following the episode to protect against stroke. These patients can then discontinue taking the medication if they do not have another episode.**

View trial at ClinicalTrials.gov LEARN MORE

**1 in 7 strokes**
are caused by AFib

**12.1 million**
people in the U.S. will have AFib by 2030

“Many of these patients are on blood thinners for the rest of their lives even if they have infrequent episodes of atrial fibrillation. If we can show this strategy is equally protective against stroke and reduces bleeding, that could save lives, reduce cost and improve quality of life.”

**ROD PASSMAN, MD**
*Director of the Center for Arrhythmia Research and the Jules J. Reingold Professor of Electrophysiology, Northwestern Feinberg School of Medicine*