

## NIH RESEARCH MATTERS

## 2016 Research Highlights

With NIH support, scientists across the United States and the world conduct wide-ranging research to improve the health of our nation. Groundbreaking NIH-funded research often receives top scientific honors. In 2016, these honors included 1 NIH-supported Nobel Prize winner and 5 NIH-funded recipients of top awards from the Lasker Foundation. Here's just a small sampling of the research accomplishments made by NIH-supported scientists in 2016. For more health and medical research findings from NIH, visit [NIH Research Matters](#).

## Clinical Advances

### Prevention, Diagnosis, and Treatment of Human Disease



#### [Blood pressure management for seniors](#)

High blood pressure, or hypertension, affects 1 in 3 American adults. In a large clinical study, researchers found that seniors who aimed for a target systolic blood pressure level lower than commonly recommended (less than 120 mm Hg compared to 140 mm Hg) had a reduced risk of cardiovascular disease and death. The findings will help older adults with hypertension and their doctors make more informed decisions about blood pressure goals.



#### [Islet transplantation restores blood sugar control in type 1 diabetes](#)

Diabetes is a disorder in the regulation and use of glucose. In type 1 diabetes, the body's own immune system attacks and destroys pancreatic beta cells that make insulin. Researchers used pancreatic islet cell transplantation to successfully treated people with difficult cases of type 1 diabetes. The procedure and the use of antirejection drugs were associated with some side effects. Researchers continue to monitor participants to assess the experimental procedure.



#### **Food allergy prevention**

The standard approach to food allergy prevention is to avoid allergenic foods, such as peanuts. Researchers are developing several approaches to protect infants and children with peanut allergy. These include [regular consumption of peanut products early in life](#); including [eating small, gradually increasing amounts of peanut protein](#); and a [wearable patch that delivers tiny amounts of peanut protein through the skin](#). More work will be needed to assess these regimens.



**Experimental malaria vaccine protects adults for more than a year**

Roughly half of the world’s population is at risk for malaria, despite improved prevention and control efforts. Scientists have been working to develop a vaccine that provides long-term, reliable protection from malaria. An experimental vaccine protected healthy adults from infection for more than a year after immunization. The vaccine is now being tested in larger trials.



**Genetic misdiagnoses of heart condition in black Americans**

Genetic testing can help identify people at risk for certain conditions. Scientists found that several genetic variations previously linked with hypertrophic cardiomyopathy (abnormally thick heart muscle) were harmless. The variants, which were much more common in black Americans than in white Americans, resulted in a higher misdiagnosis of black Americans. The findings highlight the importance of including diverse populations in genomic studies.



**Long-term benefits of age-related macular degeneration treatments**

Age-related macular degeneration (AMD) is the leading cause of vision loss among older Americans. AMD often has few symptoms in its early stages, but causes loss of central vision in later stages. Researchers examined the 5-year outcomes of using the drugs Avastin and Lucentis to treat AMD. The results showed that almost half of the participants had 20/40 vision or better, confirming the long-term benefits of the therapy.



**Meditation and cognitive-behavioral therapy ease low back pain**

Most people experience low back pain at some point in their lives. Treatment choices include over-the-counter and prescription drugs, cold and hot compresses, exercise, and in some cases, surgery. Researchers found that mindfulness-based stress reduction and cognitive-behavioral therapy both alleviated chronic low back pain in adults. The results validate more treatment options for people with back pain to consider with their health care providers.

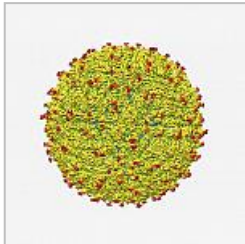


**Helmet-based ventilation eases respiratory distress**

Acute respiratory distress syndrome (ARDS) is a life-threatening condition that occurs when fluid builds up in the lungs and blocks oxygen from entering the bloodstream. If a person doesn’t receive enough oxygen, the organs can’t function properly. Researchers compared noninvasive oxygen delivery methods—a helmet versus a face mask—for patients with ARDS. The trial was stopped early because the helmets proved more effective than the face masks for treating this condition.

## Promising Medical Advances

### Findings with Potential for Enhancing Human Health



#### **Zika research advances quickly**

The Zika virus has spread worldwide since 2015, but there are no vaccines or effective treatments. This year, researchers decoded the [structure of the virus](#), providing clues to how it enters human cells. They identified novel ways to inhibit Zika, testing a [human-derived antibody in mice](#) and [screening for promising compounds](#). Five [experimental vaccines](#) were evaluated in monkeys, [with one now being tested in people](#).



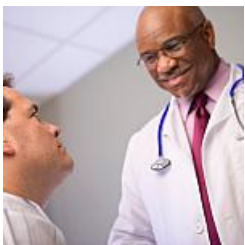
#### **[Spinal cord stimulation helps paralyzed people move hands](#)**

More than a quarter of a million Americans are living with spinal cord injuries. Spinal cord damage can lead to serious disabilities, including paralysis. In a proof-of-concept study, electrical stimulation of the spinal cord helped 2 people with quadriplegia improve voluntary movement and use of their hands. The study represents the first step in using the approach to improve hand function for people with cervical spinal cord injury.



#### **[Developing novel ear infection treatments](#)**

Ear infections are the most common reason parents bring their children to the doctor's office. Infections are often treated with a 7–10 day course of oral antibiotics. But getting young children to take the medication can be difficult. Researchers designed an easier way to administer ear infection medication by engineering a gel to deliver antibiotics directly into the ear. This method was used to successfully treated ear infections in chinchillas.



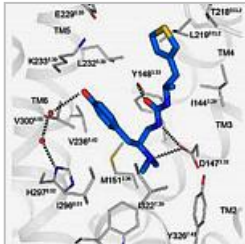
#### **[Biomarker signatures of prostate cancer](#)**

Prostate cancer is the second most common cancer in men in the United States. More than half of prostate cancers don't become life-threatening, but doctors don't have a way to reliably predict which will likely to cause problems. Researchers discovered biomarkers in urine samples that were unique to 2 different prostate cancer stages. The findings suggest a noninvasive way to diagnose prostate cancer and assess tumor progression.



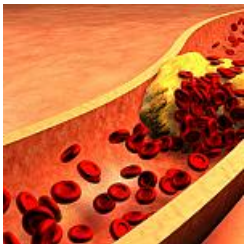
#### **Gene editing shows promise in different disease models**

Gene-editing techniques like CRISPR/Cas9 can successfully replace faulty genes, and scientists have been exploring their therapeutic potential. This year, NIH-funded scientists showed the approach holds promise as a gene therapy for 3 diseases in animal or cell models: [Duchenne muscular dystrophy](#) (in mice); [sickle cell disease](#) (in blood stem cells from affected people); and [the inherited eye disorder retinitis pigmentosa](#) (in rats).



### [Designing more effective opioids](#)

Opioids are a class of powerful pain-relieving drugs that are generally safe when taken for a short time and as prescribed by a doctor. However, they're frequently misused because they also produce euphoria. Researchers used computer simulations to screen millions of molecules for opioid-like pain-relieving properties. The analyses allowed them to create a molecule that effectively alleviates pain in mice, but with fewer side effects than the opioid morphine.



### [When HDL cholesterol doesn't protect against heart disease](#)

High-density lipoproteins (HDL) are thought to help remove cholesterol from the body. Higher levels of HDL have been associated with a lower risk of cardiovascular disease. However, scientists discovered a genetic mutation that raises HDL cholesterol levels but also increases the risk for heart disease. The findings suggest that levels of HDL cholesterol may not be as important as how well it functions to remove cholesterol from the body.

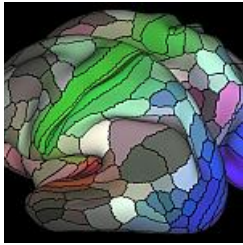


### [Redefining health and well-being in older adults](#)

Many traditional models for assessing health in older adults focus on disease. But health is more than just the absence of disease. It also includes your physical, psychological, and social well-being. Researchers developed a "comprehensive model" to assess health that includes measures of health behaviors; psychological health; sensory function; and frailty. The findings may help doctors better assess and manage the quality of life and health of older adults.

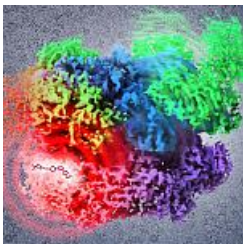
## Insights from the Lab

### Noteworthy Advances in Basic Research



#### [An expanded map of the human brain](#)

A detailed map of the human brain can reveal its organization, connections and function. Previously generated brain maps have been limited by technology or size. Scientists created a high-resolution map of the human brain and identified 180 distinct areas in each half of the outmost layer, the cerebral cortex. The study provides new insights and tools for understanding the roles of specialized brain regions in health and disease.



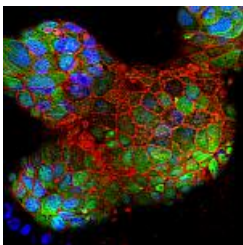
#### [Visualizing a cancer drug target at atomic resolution](#)

Determining the 3-D structure of a protein at a fine level of detail is important for drug development. Understanding how the drug and protein interact at an atomic level can allow scientists to design new drugs. Using cryo-electron microscopy, researchers were able to view, in atomic detail, the binding of a potential small molecule drug to a key protein in cancer cells. The results illustrate how the imaging technique can help advance drug development.



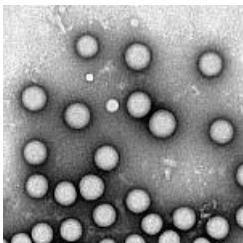
#### [Cone snail venom reveals insulin insights](#)

Many people with diabetes rely on injections of synthetic insulin to keep blood sugar levels in check. Human insulin molecules take time to act on blood glucose levels. Researchers found that a fast-acting insulin from the cone snail can bind and activate the human insulin receptor. The 3-D structure and other findings from the study provide insights for designing rapid-acting insulins to better manage diabetes.



#### [3-D cell placenta model mimics development, microbial resistance](#)

The placenta serves as a barrier to protect the developing fetus from toxins and infectious microbes, but some viruses can cross the placenta and potentially harm the unborn baby. Researchers developed a 3-D cell culture system that mimics aspects of placental development and microbial resistance. The system may help researchers understand how pathogens that cause diseases such as herpes, HIV, and Zika pass from a mother to her unborn child.



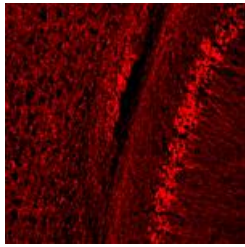
#### [Nanoparticles target, transform fat tissue](#)

Adults have 2 types of fat: white fat to store excess calories and brown fat, which can burn energy to create heat and help maintain body temperature. Scientists designed nanoparticles to target white fat and convert it to calorie-burning brown fat. The nanoparticles slowed weight gain in obese mice without affecting food intake. This proof-of-concept work could lead to new therapies to treat obesity.



# National Institutes of Health

*Turning Discovery Into Health*



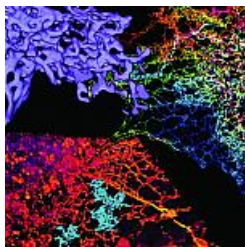
## [Boosting brain's waste disposal system may slow neurodegenerative diseases](#)

Several neurological disorders are characterized by proteins that accumulate in the brain. Activating the cell's waste disposal system restored brain function in a mouse model of tauopathies—a set of neurodegenerative disorders that include Alzheimer's disease. The findings suggest that enhancing disposal activity with drugs during early stages of neurodegenerative diseases may help lessen brain damage and dementia.



## [How sperm are activated](#)

Many different factors can affect a couple's ability to conceive. One is sperm motility; once inside the female reproductive tract, sperm use their tail-like appendages to swim for the egg. Researchers identified a molecular pathway that activates sperm. The findings may lead to new approaches for male contraception and treatments for infertility that result from problems with sperm mobility.



## [Re-envisioning the endoplasmic reticulum](#)

The endoplasmic reticulum (ER) is a complex organelle inside the cell that makes and distributes many substances the cell needs, such as proteins, lipids, and sugars. Advanced imaging techniques revealed a new, more accurate picture of how the peripheral ER is structured. The findings may yield new insights for some genetic diseases that arise from mutations in proteins that help control the shape of the ER.