

NIH...

Turning Discovery Into Health

U.S. Department of Health & Human Services
National Institutes of Health



THE 21ST CENTURY NATIONAL INSTITUTES OF HEALTH



APPLYING CUTTING-EDGE TECHNOLOGIES...

...surveying huge sets of biological components

TRANSLATING DISCOVERIES...

...turning molecules into medicines and cells into cures

HELPING HEALTH CARE...

...personalizing therapies and behaviors to people and communities

ACTING GLOBALLY...

...finding vaccines and fighting worldwide chronic diseases

NURTURING CREATIVE MINDS...

...exploring the unknown and improving our health

From the NIH Director

As the nation's medical research agency and the largest source of funding for medical research in the world, the National Institutes of Health (NIH) has been a driving force behind many decades of advances that have improved the health of people in every corner of America.

The 21st century is a time of rapid growth and change, and we continue to generate new knowledge that can be applied toward improving health. The NIH investment has a multiplicity of effects, creating hundreds of thousands of high-quality jobs as we support thousands of scientists in universities and research institutions in every state across America and across the globe.

Throughout this booklet, you can see first-hand how NIH research results have spared millions from suffering. Because of NIH research, we have vaccines to protect us from cervical cancer, flu, shingles, and meningitis. Because of NIH research, survival from the most common childhood leukemia is now 90 percent. Because of NIH research, effective medicines and lifestyle changes have slashed rates of heart disease and stroke.

Yet we continually face new challenges. America is getting older, and chronic diseases consume the vast majority of our health care dollars. Rising rates of obesity, diabetes, and Alzheimer's disease threaten to reverse hard-fought longevity gains earned from our past medical research investment. The vexing problem of health disparities limits the reach of science discoveries to all people. Although we know this problem is a mix of genes, the environment, individual health practices, and socioeconomics, we need to learn much more.

Expansive and routine global travel and communication have flattened our world, making it necessary to think without borders when it comes to our nation's health. Emerging and re-emerging infectious diseases, the threat of bioterrorism, and the looming crisis of chronic conditions in the developing world require constant vigilance.

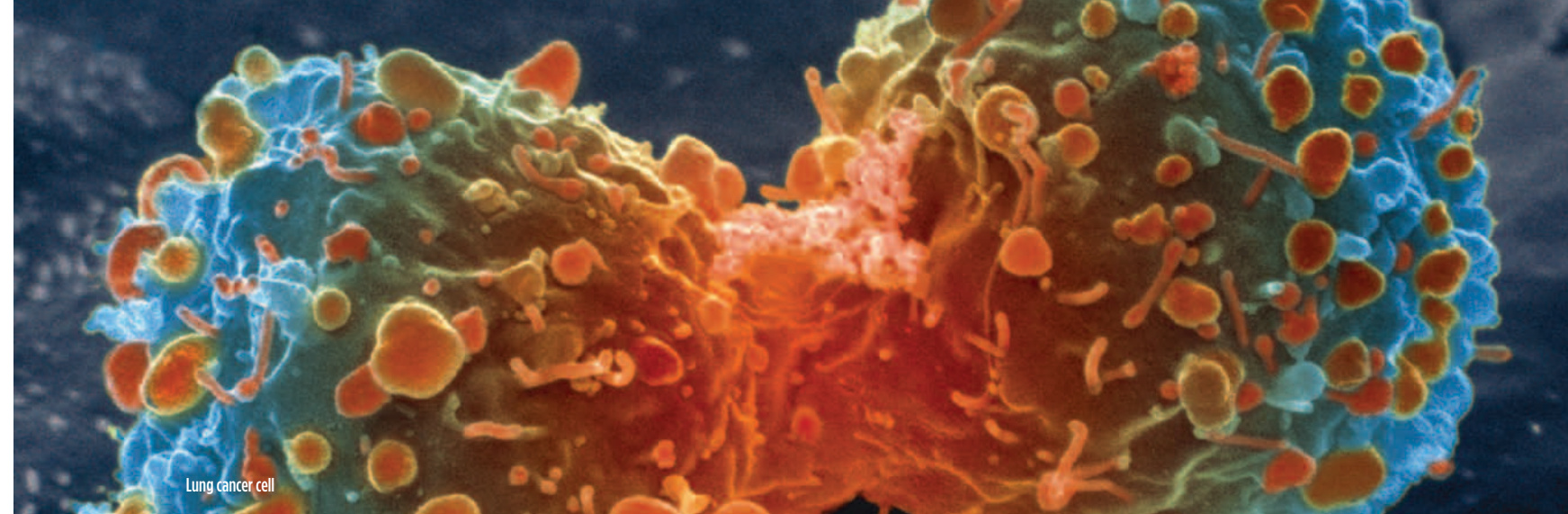
With the help of the best scientific minds, NIH is meeting these challenges head-on. Imagine the future, when today's investment in medical research will have given us the ability to regenerate lost or injured body parts, practical ways to tailor health outcomes with individualized prescriptions, and millions of lives saved from preventable deaths caused by smoking and other unhealthy behaviors.

This is a remarkable time of discovery, and the opportunities in science and medicine are at once exciting and urgent. The NIH investment will continue to bring us new ways to cure disease, alleviate suffering, and prevent illness. By bringing the best science to the people who need it most, NIH is empowering Americans to embrace healthy living through informed decision-making. Job number one for me is to be sure NIH can make significant, lasting contributions to public health. This effort will change forever the health of ourselves, our families, our country, and the world.

Francis S. Collins, M.D., Ph.D.
Director, NIH

CHRONIC DISEASES

Nearly half of all Americans have a chronic medical condition. These diseases now cause more than half of all deaths worldwide. If current trends continue unabated, annual deaths due to chronic diseases will reach 36 million by 2015.



2 CARDIOVASCULAR DISEASE

Fifty years ago, it was not uncommon for Americans to die of heart attacks in their 50s or 60s. We didn't know what caused cardiovascular disease, which by the late 1940s caused half of all U.S. deaths. Few people knew that in 1945, when President Franklin Delano Roosevelt met in Yalta with Winston Churchill and Josef Stalin, his blood pressure was an alarmingly high 260/150. Roosevelt's death two months later was a wake-up call to the nation about the growing epidemic of cardiovascular disease.

We have come a long way. NIH-funded research, beginning with the Framingham Heart Study in the late 1940s, helped define the concept of risk factors and changed the course of public health. Today, the death rate for heart disease, the leading cause of death in the United States, has dropped by more than 60 percent since 1940. The death rate for stroke—the third most common cause of death—declined by 70 percent over the same time period.

Now we have new and more effective treatments such as clot-buster drug therapy to open up blocked arteries, and minimally invasive techniques that prevent heart attacks. Quitting smoking, controlling high blood pressure, and regular exercise, as well as therapies like statins, aspirin, and beta-blockers, are preventing heart attack, sudden death, and stroke. Increasingly, we are able to pinpoint those at highest risk for future illness—even before any symptoms appear—and offer them effective prevention strategies.

Imagine the Future...

Personal gene chips predict risk for high blood pressure, kidney disease, high cholesterol, diabetes, obesity, and heart disease.

Doctors routinely use minimally invasive, image-guided procedures to preempt heart disease.

Recovery to Discovery:

The Cancer Genome Atlas

Cancer is an enemy that requires attack from all sides. The results of studies on the basic biology of cancer have led to fundamental biological insights that have, through translational research and development, led to dozens of new drugs, with many more to come. Health campaigns that promote the value of screening continue to save lives. NIH-funded researchers are using Recovery Act funds to dig deeper: to build comprehensive maps of the key genomic changes in 20 major types and subtypes of cancer. They will store knowledge in The Cancer Genome Atlas, or TCGA, a powerful tool for scientists and physician-investigators throughout the world to discover precise and effective methods to diagnose, treat, and ultimately prevent cancer. To date, TCGA has produced a comprehensive molecular classification system for the most common and aggressive brain tumor, glioblastoma multiforme, as well as for



another deadly disease, ovarian cancer. Researchers have now read the complete DNA sequence of cancer cells from a patient with acute myeloid leukemia, providing the first complete, comprehensive cancer genome in history.

CANCER

NIH-funded research has led a revolution in how we think about cancer. Ten or 20 years ago, cancer treatment was mostly reactive, specific to a particular organ, and associated with difficult therapy and a poor quality of life. Today, cancer therapy is more proactive, targeted, less toxic, and personalized. Effective screening programs have saved thousands of lives.

Basic research in cancer biology has led to targeted treatments with very few side effects. NIH-sponsored clinical trials showed in 2005 that adding the drug trastuzumab to standard chemotherapy cut the risk of breast cancer recurrence by 40 percent for women whose tumors are genetically matched to this drug. This is the most dramatic improvement in the post-surgical treatment of breast cancer ever described.

Our understanding of genetic susceptibility to cancer has grown dramatically in recent years through the application of technologies that grew from the Human Genome Project. Over 70 common genetic variants have been discovered that are associated with risk for 15 cancers to date, including cancers of the breast, prostate, colon, pancreas, brain, bladder, testis, and lung, as well as chronic lymphocytic leukemia and non-Hodgkin lymphoma. In 2007—for the first time in history—the absolute number of cancer deaths in the United States went down.

Imagine the Future...

Individualized risk prediction allows more rational application of screening procedures, enabling early detection and cure.

Nanomedicine targets cancer cells precisely, with limited toxicity to healthy cells.

Oncologists select cancer drugs based on the precise DNA changes in a person's tumor.

RESEARCH PAYS More than 50 percent of U.S. economic growth since World War II has come from science and technology.¹

¹ Rising Above the Gathering Storm: http://www.nap.edu/catalog.php?record_id=11463

CHRONIC DISEASES



4 DIABETES

In the 1950s, about one in five people died within 20 years after being diagnosed with type 1 diabetes, formerly known as juvenile diabetes. Almost all of them developed diabetic retinopathy, which accounted for about 12 percent of new cases of blindness between the ages of 45 and 74. People with diabetes relied on inaccurate urine tests to track their blood sugar and crude, animal-derived insulins to control it.

In 1983, NIH began the Diabetes Control and Complications Trial, which enrolled 1,441 people with type 1 diabetes. This landmark study was stopped early because the results showed so clearly that careful control of blood sugar reduced eye, kidney, and nerve complications by 50 percent to 75 percent. In a follow-up study 10 years later, researchers learned that rates of heart disease and stroke had gone down by 50 percent. Today, people with type 1 diabetes are living longer and healthier lives, and new technologies help them keep tight control of their blood sugar using continuous glucose monitors and insulin pumps that deliver rapid-acting, bioengineered human insulin.

Today, we know a lot more about type 2 diabetes as well; we know that family history, obesity, and physical inactivity are risk factors for this condition formerly known as adult-onset diabetes. NIH-funded research has shown us that type 2 diabetes can be delayed or prevented. Basic lifestyle interventions—modest weight loss and regular exercise—slash type 2 diabetes risk by 58 percent over three years in people with pre-diabetes. But type 2 diabetes accounts for 90 percent of cases and has been increasing at an alarming rate due to the rise in obesity in the United States.

Imagine the Future...

An artificial pancreas—perhaps an implantable device—automatically senses a person's blood sugar and adjusts insulin dosage precisely.

Preventing obesity preempts type 2 diabetes and its complications.

Stem cell research leads to the ability to replace failing insulin-producing cells.

5 ASTHMA

A few decades ago, we knew very little about asthma and still less about how to manage it. Most people couldn't pursue life's normal activities. This was especially difficult for children, who often had to forego routine playtime, and playing sports was unthinkable.

In the 1950s, scientists first learned that asthma symptoms were not just due to muscle tightening in the lungs triggered by environmental exposures, but were caused by underlying, chronic airway irritation and swelling. This insight led to successful anti-inflammation treatments that allowed doctors to prevent symptoms, and to tailor treatments to an individual's disease severity.

Another major advance was the development of inhalers that could deliver vaporized medicine—airway-opening drugs and steroids—directly to the lungs, thereby avoiding the need for a pill that can cause multiple side effects in other organs. In addition, educational research created effective programs that helped people manage their asthma by working in partnership with their

doctor. Because of the NIH research investment, people with asthma now lead active lives at school, work, and play.

In recent years, scientists supported by NIH have made important progress in asthma treatment in vulnerable populations, such as school-age children living in poor, urban areas across the United States. They have learned that when added to medical treatment, individualized, home-based intervention programs—for instance, those that emphasize the use of mattress covers, air filters, and professional pest control—are cost-effective. Such tailored interventions reduce emergency-room visits, decrease asthma medication use, and improve the health of at-risk children.

Imagine the future...

Interventions early in life—perhaps even before a child is born—prevent asthma from ever taking hold.

A protective vaccine, tailored to an individual's asthma triggers, eliminates the disease in those at risk.

Molecules to Medicines

Clinical Trials

A vital part of medical research is testing treatments—drugs, behaviors, and surgeries—in people, to see what works, as well as to determine whether a new treatment is better than what's currently available. Today, clinical trials are a mainstay of medical research. They have led to numerous, effective therapies for just about every disease and condition described throughout this booklet. They are the lifeblood of turning discovery into health. Clinical trials are carefully monitored to protect people who participate. In addition, NIH clinical research is federally regulated with built-in safeguards that protect the health and privacy of participants while addressing an important research question. Why volunteer for a clinical trial? People who participate in clinical trials can play a more active role in their own health care, gain access to new research treatments before they are widely available, and help others



by contributing to medical research. However, there can be risks, since like all research, the outcomes are not known. Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate. In this process, researchers provide details about the risks and potential benefits, and a participant may withdraw from a trial at any time. For more information about clinical trials, visit ClinicalTrials.gov, an NIH resource that catalogs and explains thousands of ongoing clinical studies for a wide range of diseases and conditions.

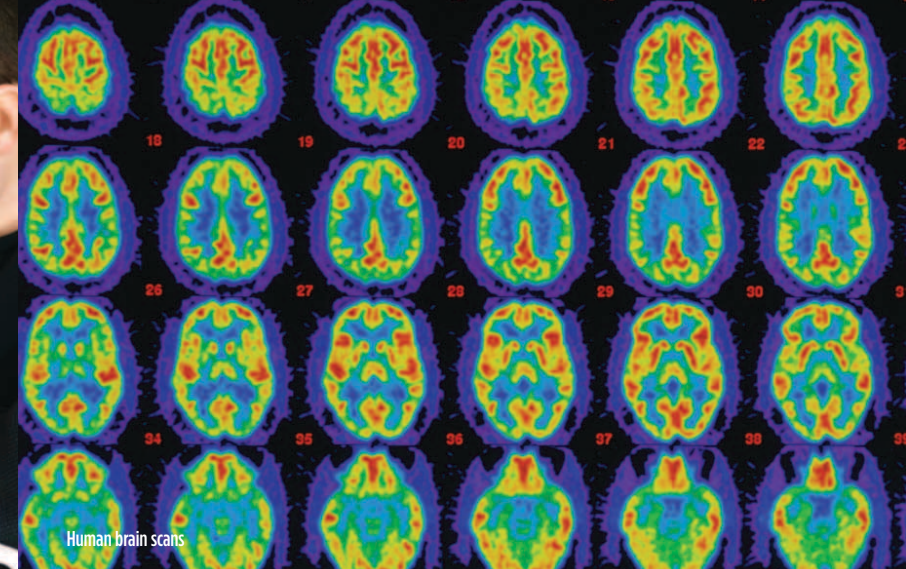
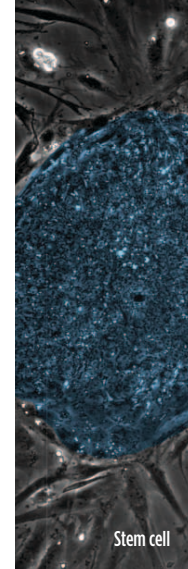
RESEARCH PAYS

Every dollar of NIH support returns \$2.25 in goods and services in just one year.²

² In Your Own Backyard: How NIH Funding Helps Your State's Economy: <http://www.familiesusa.org/issues/global-health/publications/in-your-own-backyard.html>

A HEALTHY MIND

Only a few centuries ago, philosophers found it inconceivable that our ability to think and to feel was due to the actions of a physical organ, the brain. We now know that this amazing biological machine is also central to many chronic diseases including addiction, mental illness, and movement disorders.



6 DEPRESSION

Everyone occasionally feels depressed, but for some these feelings do not go away within a couple of weeks. Until just recently, people with serious, long-lasting depression had few options. In many cases, people suffering from depression viewed their condition as a sign of personal weakness and never sought treatment. Untreated depression increases risk for substance abuse, heart disease, and suicide. Since suicide kills nearly twice as many Americans as homicide, detecting and treating depression saves lives.

NIH-funded research has indicated that depressive illnesses are disorders of the brain, and they are highly treatable. Brain-imaging technologies, such as functional magnetic resonance imaging (fMRI), have shown that the brains of people who have depression look different from those of people without depression. The parts of the brain responsible for regulating mood, thought, sleep, appetite, and behavior appear to function abnormally. Researchers have recently successfully treated some forms of depression with

localized brain stimulation, a method that has also been effective for Parkinson's disease or some forms of epilepsy. While current antidepressant medicines are usually effective by relieving symptoms over many weeks, new drugs appear to take effect much sooner.

Our ability to effectively treat mental illnesses like depression has changed the lives of millions of Americans and their loved ones. Today, the vast majority of people, even those with the most severe depression, can get better with medications or cognitive behavioral therapy, or a combination of both.

Imagine the Future...

Increased education and awareness steers more people with depression to effective therapies, saving millions in health care costs and lost productivity in the workplace.

Genetic markers for depression risk match patients to the best treatments for them.

Antidepressants relieve depression within hours, reducing the rate of suicide, substance abuse, and disability.

PARKINSON'S DISEASE

Fifty years ago, the discovery that people "frozen" and shaking due to Parkinson's disease could regain control of movement from the drug levodopa was nothing short of a miracle. While this treatment helps to replenish levels of the brain chemical dopamine that is depleted in Parkinson's, in the later stages of the disease, levodopa causes debilitating side effects and doesn't work very well. NIH-funded basic brain research contributed to the development of a variety of new therapies to address this problem.

An unexpected observation in 1982 that people who used certain illicit drugs contaminated with the chemical MPTP developed Parkinson's symptoms provided the chance for an unusual breakthrough. Scientists learned that MPTP-treated animals also developed Parkinson's, a finding that uncovered a key disruption in brain circuitry. Going further, researchers then discovered that electrical stimulation of those same circuits in monkeys, then in people, improved Parkinson's symptoms. Today,

deep brain stimulation is an approved therapy that benefits thousands of people with Parkinson's disease every year.

Recently, NIH researchers figured out how to reprogram ordinary skin cells from people with Parkinson's into the nerve cells that die in this disease. This research provides a powerful new approach to study Parkinson's disease, and it is an important step toward the development of effective cell replacement therapies.

Imagine the Future...

"Neuroprotective" drugs and gene therapy rescue nerve cells threatened by Parkinson's disease and also prevent its progression.

Skin cells from people with Parkinson's are converted into dopamine-producing cells that can be transplanted back into the brain, alleviating symptoms even in the late stages of the disease.

Environmental triggers of Parkinson's are known and avoidable.

Recovery to Discovery:

A Shot Against Addiction

One of the nation's most formidable health threats is smoking, which kills more than 400,000 Americans every year and consumes about \$193 billion in health care costs. People who start smoking in their teens have the hardest time quitting. Using NIH Recovery Act funds, Nabi Biopharmaceuticals is testing whether a nicotine vaccine, called NicVax, can help smokers quit for good. The vaccine works by prompting the immune system to make antibodies that sop up nicotine while it is still the bloodstream, keeping the addictive substance away from the brain where it causes cravings. Early results are promising—a significant number of people who develop high antibody levels in response to NicVax are more likely to be smoke-free a year later than those who did not get the vaccine. Any successful effort to cut smoking rates will affect health



dramatically, preventing chronic obstructive pulmonary disease, lung cancer, heart disease, and other conditions. Other Recovery Act-funded researchers are pursuing similar vaccines to treat addiction to drugs of abuse like methamphetamine and cocaine.

ADDICTION

For much of the 20th century, people viewed addiction as a moral failing, a simple lack of willpower. We had no idea that drugs of abuse could actually change the structure and function of the brain—eroding exactly those circuits that govern free will and control over behavior. We also didn't appreciate fully the wide-ranging harmful health effects of drug abuse on, for example, heart and lung diseases, hepatitis, and HIV/AIDS.

Today, thanks to NIH research, we know how drugs of abuse act in the brain, as well as which circuits they target to drive the compulsive behaviors of addiction. We also know that chronic drug abuse causes changes in the brain that can persist long after a person stops using drugs and contribute to the potential for relapse. This knowledge has underscored the need to view addiction as a chronic condition.

NIH research has already led to effective prevention strategies, especially those targeted at youth, who are the most vulnerable. New medicines and proven behavioral therapies help to counter the devastating effects of addiction on mood, judgment, decision-

making, and overall health—leading many toward acquiring the life skills and behaviors needed for recovery.

One dramatic success story is that of buprenorphine, a drug that treats heroin and other opiate addictions effectively. This medicine can now be prescribed in the privacy of a doctor's office, rather than obtained only from methadone clinics—a change that extends its availability and reduces stigma around its use.

Imagine the Future...

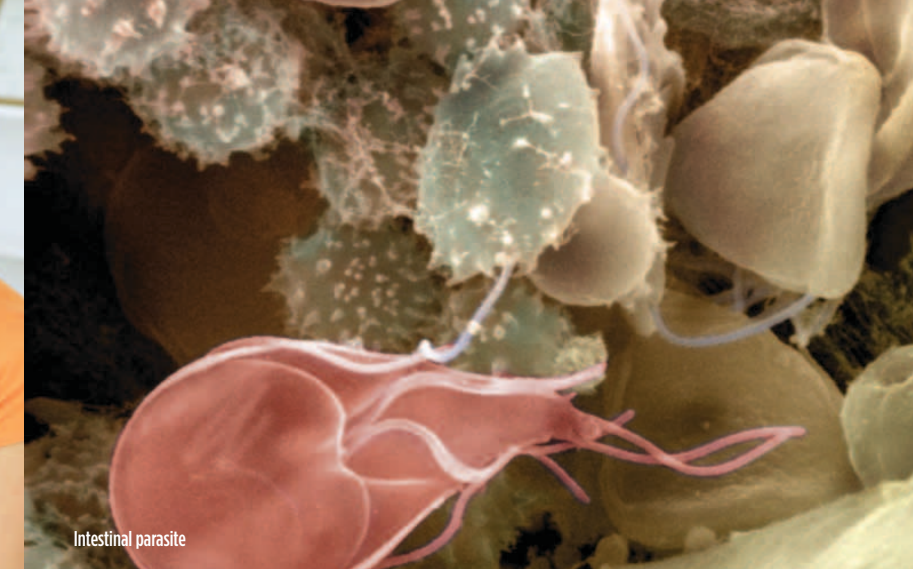
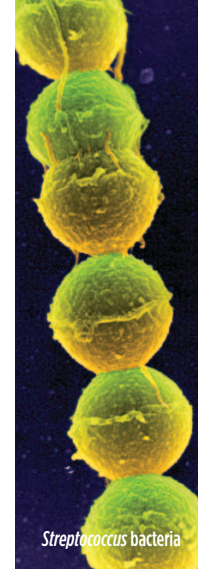
Genetic discoveries and noninvasive imaging technologies "tell the future" about addiction risk and provide an ability to personalize therapies.

New medications target all addiction-disrupted circuits, helping people appreciate natural rewards—like chocolate or music—while regaining control over drug abuse.

Long-lasting forms of medicines help people stay off drugs—saving lives, reducing crime, and improving work productivity.

INFECTIOUS DISEASES

For more than 60 years, NIH research in infectious diseases has led to new therapies, vaccines, diagnostic tests, and other technologies that have improved the lives of millions of people in the United States and around the world.



8 HIV/AIDS

On June 5, 1981, federal health officials reported the first cases of a new and fatal disease. By the late 1980s, acquired immunodeficiency syndrome, or AIDS, was spreading rapidly throughout the world with no effective therapy available to treat the several hundred thousand people who were infected in the United States and worldwide. Today, more than a million people in the United States are living with HIV/AIDS.

In 1989, NIH researchers made a number of major discoveries about how the human immunodeficiency virus, or HIV, destroys the body's immune system that ultimately leads to full-blown AIDS. In 1996, NIH-funded researchers discovered a new class of drugs, known as protease inhibitors. When used in combination with other AIDS drugs, these medicines attack HIV in several ways at once, extending the lives of HIV-infected people. The discovery and development of new drugs have turned HIV infection from a death sentence into a chronic disease for those who have access to, and can tolerate, these powerful medicines.

Today, in the United States, there is less than a 1 percent chance that a child will become infected by his or her HIV-infected mother if she is taking anti-HIV medicines. In 2009, NIH-funded scientists reported the first evidence that an HIV vaccine could provide partial protection—the first signal from any human study that a protective vaccine for HIV may be possible. NIH continues a vigorous HIV/AIDS research program to study the basic biology of HIV and related complications, as well as to develop and test new drugs and prevention approaches.

Imagine the Future...

[New medicines and prevention stem the spread of HIV/AIDS in the developing world.](#)

[The ultimate defeat of HIV/AIDS is a toolkit of HIV prevention interventions, including safe, effective, and widely available vaccines, microbicides, and behavioral interventions.](#)

INFLUENZA

Each year in the United States, seasonal influenza, or flu, kills more than 36,000 people and hospitalizes 200,000 more. The flu sweeps through communities, creating an epidemic, and seasonal flu outbreaks are an unfortunate mainstay of the late fall and winter. They affect between 5 and 20 percent of Americans every year. Most people who get the flu get better within a week, but for some the flu and its complications can be life-threatening.

From decades of NIH-funded research, scientists know that some types of flu, the “influenza A” strains, have the potential to be quite dangerous. A few of these strains set off global outbreaks in 1918, 1957, and 1968, fueling fears recently about similar potential for the H1N1 flu that emerged in 2009. Because of the unpredictability of how and when flu emerges—and what impact it will have when it spreads—there is an urgent need to search for new drugs to combat the flu.

NIH-funded researchers continue to study the basic biology of flu, and they recently discovered a type of antibody that neutralizes and protects against several subtypes of flu. By gripping onto a common attachment site on the surface of influenza A viruses, the “super”-antibody appears to prevent the virus from fusing with and entering into human cells.

Imagine the Future...

[Public health officials use precise, real-time surveillance tools to stop flu spread.](#)

[New vaccine production methods shorten the time required to prepare vaccines against emerging pathogens.](#)

[A universal vaccine protects adults and children against both seasonal and pandemic flu.](#)

GLOBAL HEALTH

Science and disease have no borders, and thanks to NIH-funded research by U.S. and international teams, we have made important progress in both infectious and chronic, non-communicable diseases that affect people across the globe.

Now we have modern research tools and an ability to read the genetic language of disease-causing bacteria, viruses, and parasites. As such, we can begin to develop totally new approaches to well-known threats like malaria, tuberculosis, and HIV/AIDS, but also to microorganisms that cause “neglected” diseases, so-named because they have gone understudied for many years, yet sicken or kill billions of people worldwide. According to the World Health Organization, diarrheal disease kills 1.8 million people every year. By far, most of this burden is caused by unsafe water supply, sanitation, and hygiene, and mostly is concentrated in children in developing countries. NIH-funded research on prevention and treatment of diarrheal diseases, like cholera and rotavirus, has made vital contributions to public health throughout the world.

Chronic non-communicable conditions like cancer, heart disease, and obesity contribute to more than half of all deaths in the developing world. NIH is committed to mitigating this developing catastrophe by providing not only needed financial and human resources, but also in fostering teamwork between scientists, health organizations, and regional governments.

Imagine the Future...

[New technologies diagnose tuberculosis in a few minutes and predict the right drug for the right patient.](#)

[A malaria vaccine prevents more than a million deaths a year in the developing world.](#)

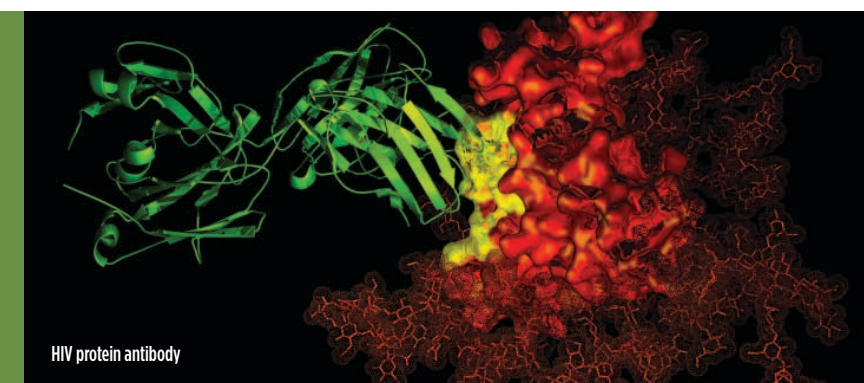
[New behavioral strategies greatly reduce the incidence of smoking-related illness around the world, preventing millions of deaths.](#)

Recovery to Discovery:

HIV Test and Treat?

Recent and intriguing findings from the world of HIV/AIDS research have led to a proposed public health strategy called “test and treat.” About 60 percent of new cases of HIV/AIDS are due to transmission from people who have high viral loads but don't know they are infected. Researchers generated a mathematical model of HIV spread among heterosexuals in South Africa, which predicted that universal voluntary testing and immediate treatment for infected individuals who meet medical guidelines can dramatically quell the spread of HIV to very low levels.

This is a provocative idea, and NIH is carefully gathering information to determine if the approach could possibly



work in the real world, including in Washington, D.C., where at least 3 percent of the population is living with HIV/AIDS. NIH is supporting research to find out whether this universal, voluntary HIV testing strategy is possible, practical, and cost-effective. Using Recovery Act funds, NIH is conducting a series of studies that will, over the course of two years, answer the question of whether we can reach high-risk populations, which is necessary to evaluate the test-and-treat paradigm.

CHILDREN'S HEALTH

NIH research addresses health and well-being of children throughout their lives, from fetal development through adolescence. This research helps children, adults, families, and communities improve health so that our children can fulfill their dreams.



10 AUTISM

Current estimates suggest that around one in 100 U.S. children appears to be affected by an autism spectrum disorder, and new diagnoses are on the rise. These children and teens have trouble socially, have a range of language-related problems, and may exhibit repetitive behaviors or narrow, obsessive interests. Although we don't know what causes autism, NIH-funded research has shown that both genetics and environment appear to contribute. Although there is no cure, medications and behavioral interventions may ease symptoms.

In the past decade, NIH-funded research has taught us much more about autism and related conditions, as well as how parents can help their children manage it. NIH works with other government agencies, professional organizations, and patient advocacy groups to accelerate research and to increase awareness. This team effort is changing the way children, teens, and young adults with autism view the world, and helps parents, teachers, and friends understand that these individuals are part of "the spectrum."

Despite intense efforts, much of the biology of autism spectrum disorder continues to be a mystery to researchers. Renewed hope comes from research in which NIH-funded scientists have identified a few genetic factors that affect autism risk, pointing to genes that form and strengthen connections between brain cells. Following up on these important clues, researchers plan to decode the complete genomes of people with autism. Other studies will look at the effect of a mother's exposures during pregnancy, including medication, infection, and other environmental triggers.

Imagine the Future...

Environmental and genetic risk factors accurately identify children for subtypes of autism spectrum disorder, enabling prevention or personalized, effective treatments as early as possible.

New molecular therapies based on detailed understanding of the brain circuits affected by autism help reduce or prevent the onset of symptoms.

Behavioral therapies allow people on the autism spectrum to reach their full potential and productivity.

HEARING

Imagine the life of a child who never hears the blare of a trumpet, a mother's soothing words, or the bark of a dog. Thirty years ago, most deaf or severely hearing-impaired children were not diagnosed until they were two to three years old and didn't get treatment when it could help the most. As a result, these children fell behind, left with lifelong limitations in career and coping skills.

Thanks to NIH-funded research, thousands of children born deaf each year are able to hear through a remarkable technology called the cochlear implant, an electronic device that mimics the function of cells in the inner ear. In 1984, the FDA approved the first cochlear implant for use in adults ages 18 and older, and in 2000, for children as young as 12 months of age. In 2009 in the United States, roughly 41,500 adults and 25,500 children have received cochlear implants.

Research has shown that children who receive a cochlear implant at a young age develop language skills at a rate comparable to children with normal hearing, and many can succeed in mainstream classrooms. NIH-supported scientists have found that the benefits of fitting a cochlear implant for a child's ear far outweigh its costs. A cochlear implant costs approximately \$60,000—for surgery, adjustments, and usage training. In comparison, the services, special education, and adaptation related to a child who is deaf before age three costs more than \$1 million.

11

Imagine the Future...

Improvements in cochlear implants allow hearing-impaired or deaf children to hear sounds just like non-deaf people.

Environmental and genetic causes of age-associated hearing loss are known, allowing identification of those at risk and enabling them to avoid exposure.

OBESITY

During the past 30 years, the incidence of obesity has more than doubled among children ages two to five and more than tripled among tweens and teens. Childhood obesity can increase risk for many serious health problems such as type 2 diabetes, high blood pressure, and heart disease.

NIH is tracking obesity trends vigilantly and supports a range of research approaches to help children eat well and stay fit. For example, in 2009, NIH's We Can!™ program partnered with Subway® Restaurants to reach families throughout the United States and beyond with practical tips and tools to help children and their parents stay active and maintain a healthy weight.

We Can!'s extensive outreach in communities across the United States helps community-based educators and leaders to implement and distribute We Can!'s hands-on curricula for youth and for parents. Currently, more than 1,000 We Can! community sites nationwide and in 11 other countries have committed to providing We Can! programs at the local level.

Imagine the Future...

Knowing how genes and the environment lead to obesity creates personalized, targeted therapies for children and their parents.

Thousands of deaths and billions of health care dollars are saved from avoiding obesity-related complications in adulthood.

Recovery to Discovery:

Tackling Tooth Decay

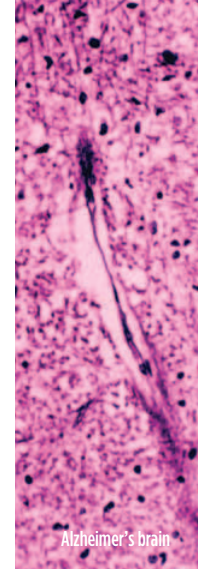
Poor oral health remains a serious problem for some of the most vulnerable members of our society. Low-income children, especially those from racial or ethnic minority groups, are especially at risk for untreated tooth decay, which can have consequences far beyond the mouth, causing discomfort and pain, poor school performance, unhealthy eating, and infections that are difficult to treat. Through NIH-funded research, practical, cost-effective solutions are possible. One is fluoride varnish, a clear liquid coating brushed directly onto a child's teeth that slowly releases fluoride over several months, strengthening teeth and preventing decay. Recovery Act-funded scientists are doing more to break down oral health disparities by developing culturally sensitive educational information for



parents of children especially prone to early childhood caries, a particularly devastating form of dental caries that is highly prevalent among low income, preschool-aged Latino children. The intervention will be delivered by promotoras, who are community-based Latina lay health workers.

AGING

The U.S. population is aging. By 2050, one in five Americans will be 65 or older. The clock is ticking for new discoveries that will help people enjoy their older years, remaining healthy and independent.



12 ALZHEIMER'S DISEASE

At the turn of the 20th century, German psychiatrist Dr. Alois Alzheimer noted unusual behavioral symptoms—including short-term memory loss—in a middle-aged patient. After his patient died, Dr. Alzheimer used staining techniques to view his brain, revealing nerve cells, plaques, and tangles seen in people who suffer from this tragic disorder. Little progress was made over the next 75 years, and as recently as 30 years ago, most people viewed dementia, or “senility,” as a normal part of aging.

Today, as many as 5.1 million Americans suffer from Alzheimer's, and health officials estimate that the aging of the U.S. population may result in three times as many cases by 2050. NIH-funded clinical studies are testing a range of new treatments for Alzheimer's, including anti-oxidants, statins, gene therapy, and anti-depressants. Totally new therapies are under consideration, too.

Genetic and other basic research have unearthed four culprit genes, as well as knowledge about how toxic molecules build up in the brain. Recently, NIH-supported researchers measured and tracked levels of biomarkers in spinal fluid that appear to signal the onset of the earliest stages of Alzheimer's.

Imagine the Future...

[Sensitive imaging scans and blood tests detect the risk of Alzheimer's disease early.](#)

[Designer medications and/or vaccines prevent Alzheimer's in people at risk.](#)

ARTHROSIS

More than 100 different forms of arthritis affect tens of millions of Americans, causing pain, swelling, physical deformities, and disabilities. The most common form, osteoarthritis, results from trauma, infection, or aging of the body's joints. Arthritis has an enormous impact on the nation's productivity and on quality of life for so many of our citizens.

Over the past decades, scientists have made important strides toward helping people manage osteoarthritis. This research has shown that successful treatment programs involve a combination of patient-guided therapies, including exercise, weight control, and rest and relief from stress on joints. In addition, novel approaches to combining pain medicines, surgery, and complementary and alternative therapies have provided millions with relief from this debilitating, common disorder.

The outlook for rheumatoid arthritis, which often appears much earlier in life, has dramatically improved. As recently as two decades ago, treatment was limited, and included aspirin and a gold-containing suspension, both of which didn't work well and had serious side effects. NIH-funded research led to three FDA-approved medicines that have had a remarkable effect on the lives of people who live with rheumatoid arthritis. Many patients view these “TNF-blockers” as miracle drugs because of their life-changing effects to reduce pain and swelling, increase energy, and even help repair joint damage.

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Imagine the Future...

[Bioengineered replacement joints restore full function to people with arthritis.](#)

[Personalized prescriptions of medicine and exercise enable people with arthritis to stay active throughout life.](#)

Recovery to Discovery:

Healthy Bones for Life

Osteoporosis is a “silent” disease, since symptoms don't appear until bones start to break. One out of every two women and one in four men over 50 will have an osteoporosis-related fracture in their lifetime, and 34 million more Americans are at risk because they have low bone mass density. Although current drugs reduce the risk of fractures in people with weak bones, new strategies are needed because America's population is getting older, and this problem will only worsen with time. Older people who break a hip, for example, often need an artificial joint or other implant that surgeons attach to the bone with a cement-like substance that doesn't always stick properly. Scientists are using Recovery Act funds to make this process more lasting and reliable. Bioengineers and clinicians are developing and testing new materials more durable than the



cements typically used to stabilize fragile bones. In another project aimed at prevention, Recovery Act-supported researchers are looking into how the bone-building effects of childhood activity affect bone health during adulthood.

VISION LOSS

Not long ago, age-related macular degeneration, or AMD, was an untreatable disease—a major cause of blindness and the leading cause of new cases of blindness in people over age 65. The disorder made it difficult, if not impossible, to read, recognize faces, drive a car, or perform even simple tasks that require hand-eye coordination.

With baby boomers nearing retirement, AMD is an urgent health problem affecting millions of Americans. Thanks to NIH research, we know a lot about the underlying causes of vision loss from AMD, such as the formation of abnormal, leaky blood vessels in the eye. Laser-based treatments, like verteporfin injection, remain effective in destroying these abnormal vessels and stabilizing advanced AMD. The most recent FDA-approved drug, ranibizumab injection, blocks vessel leakage and in many cases reverses vision loss.

Prevention strategies are also showing promise. An NIH-funded clinical study found that a daily regimen of antioxidant vitamins

and minerals delays the onset of advanced AMD by 25 percent. Follow-up studies are under way to examine other dietary factors that may slow or prevent vision loss from AMD.

Exciting new findings from the genomics world show still more promise. NIH-supported researchers recently identified subtle alterations in two immune system-related genes that account for 75 percent of AMD risk. These discoveries will allow researchers to understand what causes AMD and how to preempt it long before it has a chance to damage vision. A novel drug and cell-based delivery method has been tested by NIH-funded researchers, leading to expedited regulatory approval from the FDA for trials to treat AMD.

Imagine the Future...

[A prescription of inexpensive dietary supplements prevents vision loss from AMD.](#)

[Implantable sustained-release capsules deliver genetically engineered cell therapy to damaged eyes.](#)

PERSONALIZED MEDICINE

Personalized medicine is the science of individualized prevention and therapy. And the future is now: Millions of people have already been touched by the era of personalized medicine that has grown directly from research.



14 A poignant example is the case of a woman with lung cancer that had spread to her brain. Diagnosed in 2002, this 44-year-old—a vegetarian who had never smoked—underwent various therapies to stave off what seemed inevitable. Then came a miracle: she learned of a clinical trial testing a new drug, gefitinib, that for some tumors appeared to work as a genetic smart weapon. Her tumor was one of those, and she is alive today thanks to medical research.

The Human Genome Project and thousands of follow-on studies are helping scientists develop other, gene-targeted treatments. Some of these enable doctors to forego disfiguring surgery that is unneeded based on a tumor's individual nature.

NIH research is working hard to solve the puzzle of how genes and lifestyle connect to create our lives and our health. Today, researchers can scan and compare entire genomes very quickly. These studies have already turned up disease “signatures” for type 2 diabetes, heart disorders, prostate cancer, Crohn’s disease, Parkinson’s disease, and AMD. More are identified every few months.

PHARMACOGENOMICS

Thanks to NIH-funded basic research that gave us genetic engineering and launched the \$40 billion biotech industry, DNA is a household name. Virtually every biomedical research lab and pharmaceutical company throughout the world uses the power of the genomic revolution every day to demystify diseases and find new cures. Within five years, the complete DNA instruction book of an individual will read out for less than \$1,000, making genetic analysis a routine part of medical care.

Remarkable advances in the field of pharmacogenomics—how individuals react differently to medicines—indicate that we are moving away from “one-size-fits-all” medicine. Increasingly, we are learning how to identify glitches in our DNA scripts that reveal what drugs may be dangerous—or completely ineffective—for certain people. This information will help doctors calculate precise dosages that match a person’s DNA.

Collectively, research results in this important area of biomedicine are prompting the FDA to consider changing the labeling requirements for important medicines taken by millions of Americans. Already, pharmacogenomic information is contained in about 10 percent of labels for drugs approved by the FDA to treat a range of conditions including HIV/AIDS, cancer, seizures, and cardiovascular disorders.

Imagine the Future...

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Doctors use a patient’s genetic profile—not just weight or age—to determine the best drug and the optimal dose.

Testing a drug only in those likely to benefit from it makes clinical trials faster and less expensive, and gets a good drug to market quickly.

Recovery to Discovery:

What Works Best?

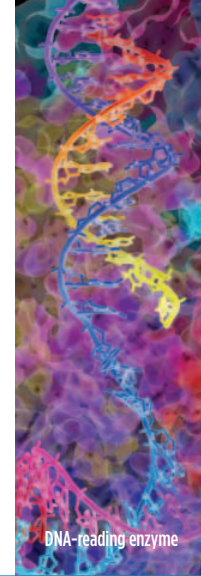
Comparative effectiveness research evaluates various therapies, gathering evidence to determine what works best. It is a cornerstone of personalized medicine, in which smart medical choices get the right treatment to the right patient at the right time. NIH’s long-time investment in comparative effectiveness research has informed experts who craft clinical guidelines used by doctors in choosing effective care for their patients. New knowledge from NIH-funded comparative effectiveness research has already changed the way we treat diabetes, high blood pressure, schizophrenia, and HIV/AIDS. Recently NIH invested \$400 million of Recovery Act funds to study the value of genomic and pharmacogenomic screening, compare strategies for diagnosing and treating diseases like cancer and addiction, analyze health behavior consequences



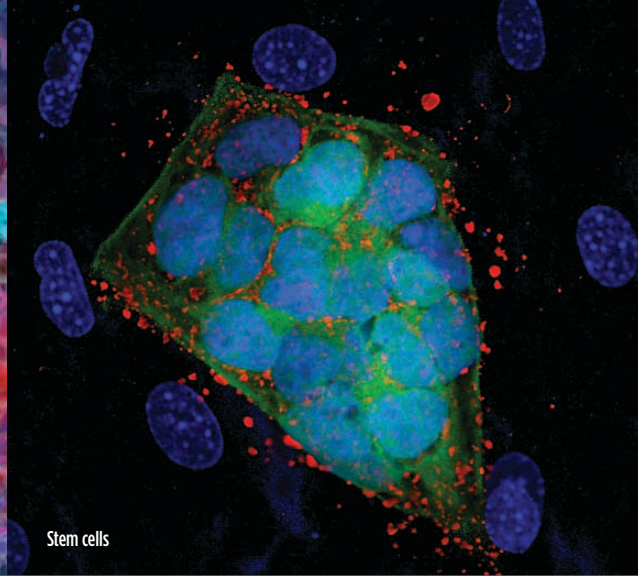
of different health insurance policies, and evaluate different ways to manage chronic pain.

RESEARCH PAYS NIH support stimulates hundreds of new businesses each year and is vital to maintaining U.S. leadership in science, which has been losing ground in recent years.

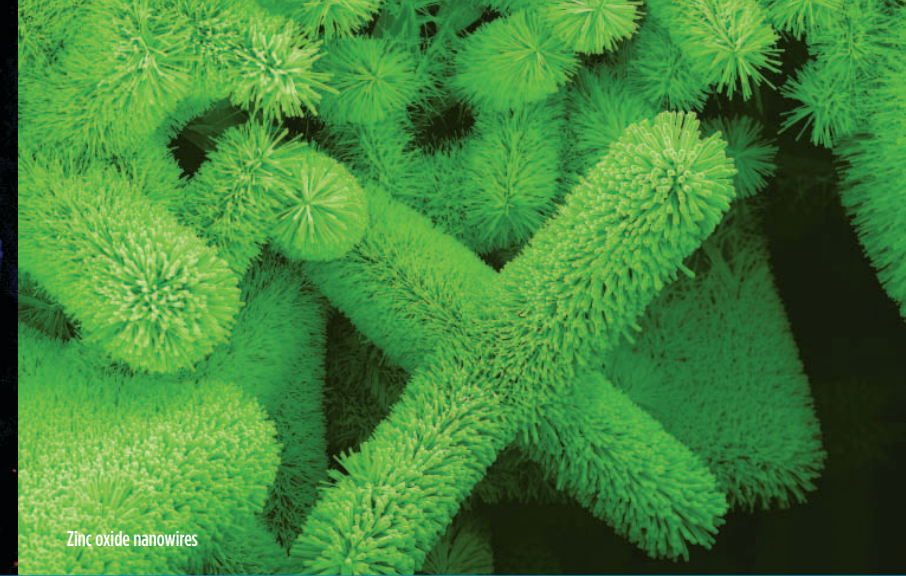
PERSONALIZED MEDICINE



DNA-reading enzyme



Stem cells



Zinc oxide nanowires

16 STEM CELLS

Stem cell research holds great promise for biomedical research—from helping us to understand better how diseases develop and spread, to serving as accurate screens for new drugs, to developing cell-based therapies for diabetes, heart failure, Parkinson's, and many other conditions that affect millions of Americans. There are two basic types of human stem cells: embryonic stem (ES) cells and non-embryonic, or “adult” stem cells. Just a few years ago, scientists learned how to make a third type, by reprogramming ordinary skin cells that have already “grown up” into those that look and act like cells from an embryo. These cells have been named induced pluripotent stem cells, or iPS cells.

NIH research is progressing on multiple fronts, to understand better the differences between the three stem cell types and to create patient-specific cells for in-depth study of many diseases. The ability to create iPS cells is a significant breakthrough, since the reprogramming technique is relatively simple to perform with standard laboratory methods, and because skin cells are easy to gather and grow. The most exciting aspect of this research is its potential to speed progress toward achieving personalized therapies. With refinements, this method could yield an unlimited supply of customized cells.

Regenerative medicine is moving towards a day when we can repair and replace damaged tissues. In time, we will be able to make insulin-secreting pancreatic cells, bone cells to heal breaks and defects, and eye and ear cells to restore vision and hearing. NIH researchers are hard at work using stem cells as a powerful tool to study neurological disorders like Parkinson's, Huntington's, amyotrophic lateral sclerosis (ALS), and spinal cord injury, to name a few.

Imagine the Future...

Stem cells restore motor function in spinal cord injury.

Screening new drugs is quick and much less costly due to rapid and accurate early testing for specific disease applications.

Bioengineered bones and cartilage ease joint disorders, improving the quality of life for millions.

IMAGING

For most of the history of medicine, doctors relied on their senses—mainly vision, hearing, and touch—to diagnose illness and monitor a patient's condition. The NIH research investment over the last few decades has transformed this scenario, providing the ability to see diseases hidden from view.

Various three-dimensional medical imaging techniques in particular, such as computed tomography (CT), magnetic resonance imaging (MRI), nuclear medicine, and ultrasound, have revolutionized medicine. Today, doctors use sophisticated and accurate imaging methods not only to find disease, but also to treat it:

- Image-guided surgical techniques have improved our ability to remove seizure-causing brain regions in patients with debilitating and untreatable epilepsy.
- MRI makes a profound difference in finding breast and prostate cancer early, when these cancers are less advanced and easier to treat.
- Minimally invasive, image-guided procedures have reduced the need for open surgery to repair blocked coronary arteries, remove gallbladders, and take tissue samples to diagnose disease.
- Nuclear medicine and ultrafast CT scanning can non-invasively identify diseased tissue that may lead to heart attacks and other heart diseases, even in people without symptoms.
- MRI- and CT-guided procedures allow heart surgeons to view the coronary arteries in 3-D, pinpointing the location of blockage.

Imagine the Future...

17

Portable and inexpensive, Star Trek-like imaging devices in the doctor's office make disease diagnosis quick and accurate.

Non-invasive, molecular-based procedures, that are both diagnostic and therapeutic, cut hospital stays, and save billions in health care costs.

RESEARCH PAYS

On average, every NIH grant creates seven high-quality jobs.

LOOKING FORWARD



18 Thanks in large part to NIH-funded medical research, Americans are living longer, healthier lives. Life expectancy in the United States has jumped from 47 years in 1900 to 77 years today, and disability in people over 65 has fallen dramatically in the past 30 years. But our nation still faces a number of serious chronic illnesses. Cardiovascular disease, cancer, diabetes, and mental health problems such as depression and schizophrenia continue to cause premature death and disability. NIH has made strides in these areas over the past 20 years, but our work is not done.

Science has always been a marathon, not a 100-yard dash.

Fortunately, the 21st century is a truly revolutionary period for science, technology, and medicine. The “new biology” is an exciting time when physicists and physicians, chemists and computer scientists, engineers and economists, and mathematicians and materials scientists combine their creative talents and natural curiosity to solve important health problems. Many concepts and tools central to understanding and improving health have come from basic, untargeted research, and we anticipate this will continue into the future.

Revolutionary new ideas often come from unexpected directions. Engaging and training the next generation of scientists is crucial to building on our strong foundation. One of the keys to solving the problem of health disparities is assuring that tomorrow’s scientific workforce reflects the rich diversity of the U.S. population. Smart investments in medical research and in the intellectual vigor of our increasingly interdisciplinary and diverse research community have the potential to keep our nation healthy, strong, and competitive for years to come. In the global 21st century, the greatest hope for a future of good health for all lies in medical research—and the promise has never been greater.

Now is the time to be bold.

NATIONAL INSTITUTES OF HEALTH

NIH is the nation’s medical research agency—making important medical discoveries that improve health and save lives. Composed of 27 Institutes and Centers, NIH provides leadership and financial support to researchers. More than 83 percent of NIH’s \$31 billion budget goes to more than 300,000 research personnel at over 3,000 universities, medical schools, and other research institutions in every state and throughout the world. NIH has supported more than 130 Nobel Prize winners. In addition, intramural research conducted on its own campus in Bethesda, Maryland—and a few other locations—is world renowned and has contributed to countless medical advances. The NIH Clinical Center is the largest hospital in the world totally dedicated to clinical research.

NIH CENTERS

- CIT** Center for Information Technology
- CSR** Center for Scientific Review
- FIC** John E. Fogarty International Center
- NCATS** National Center for Advancing Translational Sciences
- NCCAM** National Center for Complementary and Alternative Medicine
- CC** NIH Clinical Center

NIH INSTITUTES

- NCI** National Cancer Institute
- NEI** National Eye Institute
- NHLBI** National Heart, Lung, and Blood Institute
- NHGRI** National Human Genome Research Institute
- NIA** National Institute on Aging
- NIAAA** National Institute on Alcohol Abuse and Alcoholism
- NIAID** National Institute of Allergy and Infectious Diseases
- NIAMS** National Institute of Arthritis and Musculoskeletal and Skin Diseases
- NIBIB** National Institute of Biomedical Imaging and Bioengineering
- NICHD** *Eunice Kennedy Shriver* National Institute of Child Health and Human Development
- NIDCD** National Institute on Deafness and Other Communication Disorders
- NIDCR** National Institute of Dental and Craniofacial Research
- NIDDK** National Institute of Diabetes and Digestive and Kidney Diseases
- NIDA** National Institute on Drug Abuse
- NIHES** National Institute of Environmental Health Sciences
- NIGMS** National Institute of General Medical Sciences
- NIMH** National Institute of Mental Health
- NIMHD** National Institute on Minority Health and Health Disparities
- NINDS** National Institute of Neurological Disorders and Stroke
- NINR** National Institute of Nursing Research
- NLM** National Library of Medicine

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For more about all NIH Institutes, Centers, and Offices please visit <http://www.nih.gov/icd>

Because of NIH Research...



- Blood-thinning drugs prevent strokes and save lives
- Fluoride in water protects our teeth for life
- Our blood supply is clean and safe from viruses like HIV and hepatitis
- Cholesterol-lowering statins prevent heart disease
- Effective medicines treat millions with depression
- Quitting smoking prevents many diseases like cancer, heart disease, and COPD
- Medicines block mother-to-child HIV transmission
- Artificial skin allows severe burns to heal
- The sequencing of the human genome has opened a world of unprecedented opportunities for science, medicine, and health

Imagine the Future...

<http://www.nih.gov>



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