Revolutionizing Science

NIH fuels the biomedical research enterprise—cultivating world-class scientists and catalyzing new scientific fields, tools, and resources that have changed how science is done.

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Partnerships

NIH catalyzes the research enterprise by fostering partnerships to advance research fields in new ways and new directions.

ALZHEIMER'S DISEASE NEUROIMAGING



Image credit: National Institute on Aging, NIH.

Launched in 2004, the NIH-funded Alzheimer's Disease Neuroimaging Initiative (ADNI) is a public-private partnership between NIH, nonprofits, and private industry designed to understand and develop tools to track Alzheimer's disease. With 57 sites, ADNI aided the development of methods and tools that can detect the hallmark signs of this disease.

- ADNI researchers aided the development of methods and tools to detect the hallmark signs of Alzheimer's disease that can be seen with PET scans of the brain and found in spinal fluid and blood.
- An analysis in August 2020 revealed that open-access ADNI data had been requested by 24,500 users, resulting in 131 million downloads of data and 2,300 manuscript submissions.
- The successful ADNI model inspired similar initiatives for Parkinson's disease and multiple sclerosis.

WORKFORCE DIVERSITY



Image credit: John Powell.

The NIH-funded Diversity Program Consortium (DPC) created a network of institutions improving training and mentoring to enhance individuals' success in biomedical careers. DPC efforts provided mentorship to over 10,000 scientists, including those from underrepresented groups, and resulted in hundreds of publications.

- DPC fosters successful research careers among scientists from diverse backgrounds and advances biomedical research through a series of partnership programs, including Building Infrastructure Leading to Diversity (BUILD), National Research Mentoring Network (NRMN), and Coordination & Evaluation Center (CEC).
- DPC efforts resulted in 343 publications, as of November 2021, based on pilot projects and evaluations conducted on the activities of the consortium.



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COVID-19 DIAGNOSTICS



Image credit: iStock.

NIH facilitated the high-speed development of COVID-19 diagnostic technologies by applying a new model for innovation and collaboration between government, academia, and the private sector that can shape how technologies are developed in the future and be leveraged for other public health problems.

- The Rapid Acceleration of Diagnostics (RADx[®]) initiative enabled the accelerated development of COVID-19 diagnostic tests, reducing development timelines from years down to months.
- As of November 2021, the RADx[®] initiative received over 800 submissions for COVID-19 testing technologies and supported more than 100 companies.
- As of November 2021, support from the initiative led to 35 FDA emergency use authorizations (EUAs), including the first overthe-counter EUA for at-home COVID-19 tests. The cumulative capacity of manufactured tests and test products exceeds 950 million.



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Partnerships

ALZHEIMER'S DISEASE NEUROIMAGING

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Research Approaches

NIH revolutionizes science through wide-reaching influence on approaches to the way research is conducted.

CLINICAL TRIALS



Image credit: National Institute of Diabetes and Digestive and Kidney Diseases, NIH.

In 1953, NIH implemented a formal policy to protect the health of human subjects participating in NIH clinical trials, pioneering such safeguards in clinical research. This was subsequently emulated by research organizations worldwide in the form of institutional review boards and research ethics committees.

 NIH human subject protections are consistently updated, improving the trust of research participants and leading to many approved therapies, such as vaccines and cancer treatments.

HIGH-RISK, HIGH-REWARD



Image credit: Office of Intramural Training & Education, NIH.

NIH supports research that is highrisk, high-reward, meaning that it is highly innovative and has potential to lead to scientific breakthroughs, but may struggle to get funded through more traditional avenues. High-risk, high-reward research led to the development of imaging approaches, treatment technologies, and scientific techniques that have revolutionized science.

- Optogenetics is a new imaging technology, developed due to NIH's investment in high-risk, high-reward research, that has revolutionized neuroscience by enabling scientists to use light to control the activity of brain cells.
- The NIH Common Fund High-Risk, High-Reward Program has supported research on how gene editing can treat genetic disorders, such as Duchenne Muscular Dystrophy.
- NIH-supported researchers developed a new technology leading to the discovery of 25 new antibiotics, at least one able to treat difficult to treat infections.



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FRAMINGHAM HEART STUDY



Designed to understand heart health and disease, the NIH-funded Framingham Heart Study has also advanced our understanding of many other conditions and disorders, including obesity, diabetes, dementia, Parkinson's disease, osteoporosis, chronic obstructive pulmonary disease, and cancer.

- Launched in 1948 in Framingham, Massachusetts, this study aimed to unravel causes of heart disease by following a group of individuals over time.
- The tools and methodologies developed by Framingham scientists are commonly applied to understand chronic disease today.
- As of 2020, more than 4,000 articles based on Framingham research have been published. While many are focused on cardiovascular disease, Framingham also informed our understanding of many other diseases and conditions including genetic patterns and inheritance of common diseases.

FRAMINGHAM METHODS



Image credit: Shutterstock.

The NIH-supported Framingham Heart Study has had a wide ranging and enduring impact on how research is conducted, from data sharing to epidemiologic tools and methodologies.

- The success of Framingham made it a model for later prospective cohort studies.
- Epidemiologic tools and methodologies first developed by Framingham scientists, such as the statistical methods to estimate the risk of someone developing a disease, are now commonly applied to understand chronic disease.
- This study also set a new standard for widely sharing research data, allowing many researchers to investigate factors not addressed in the original study.



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COVID-19 ANIMAL MODELS



After the discovery of the virus that causes COVID-19, NIH quickly worked to identify and develop new animal models to study infection and disease progression. For example, genetic engineering techniques were used to develop animal models that mimic how COVID-19 behaves in people.

NIH provided animal models and resources to accelerate preclinical testing of vaccines and therapeutic agents for COVID-19.

As new infectious diseases are discovered, especially during pandemics, it is critical to quickly understand disease processes and the mechanisms by which infections are established for the development of vaccines and therapeutics as well as to test these approaches.

Image credit: Conny V/iStock/Getty Images Plus.



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National Institutes of Health Turning Discovery Into Health

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Research for All

NIH broadens the impact of research findings by making research more inclusive and applicable to all.

SEX AS A BIOLOGICAL VARIABLE



Image credit: National Institute of Mental Health, NIH.

NIH established the Sex as a Biological Variable (SABV) Policy requiring that all eligible funding applicants consider the influence of sex in their research to better understand health and disease for all. Since this policy, other funding agencies adopted similar policies reflecting the importance of sex as a biological variable.

- SABV applies to funding applications proposing studies with vertebrate animals and humans, of which NIH receives tens of thousands of applications annually.
- A historical focus on the biology of men, including use of male cells and animal models, contributed to the development of medications and treatments that are less effective or potentially harmful for women.
- SABV set a standard for the scientific community, paving the way for discoveries of important sex influences in many diseases and conditions.

TRIBAL NATIONS



Image credit: Mona Makela/Shutterstock.

NIH community engagement efforts with American Indian/Alaska Native (AI/AN) communities are paving new ways for revolutionizing science and making the scientific process more inclusive. Ensuring Tribal Nations have the opportunity to provide meaningful input to NIH policies, programs, and activities impacting their communities is a critical component of honoring Tribal sovereignty.

- In 2021, NIH held individual Tribal Consultations with all 574 federally recognized AI/AN Tribes in all 10 HHS regions across the U.S. to ensure Tribal Nations had the opportunity to provide meaningful input to the new NIH Tribal Consultation Policy.
- Throughout the COVID-19
 pandemic, NIH supported Tribal
 Nations in making informed
 decisions regarding participation
 in COVID-19 research and vaccine
 clinical trials, leading AI/AN
 communities to have the highest
 rates of vaccine uptake
 compared to any other racial or
 ethnic group.



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INCLUSION IN CLINICAL TRIALS



NIH set the global standard that clinical research populations should reflect the diversity of actual patient populations, requiring the inclusion of women, individuals from underrepresented racial and ethnic minority populations, and individuals of all ages in clinical trials.

Image credit: Wake Forest School of Medicine.

of people were excluded from clinical trials, meaning that individuals received medical drugs and treatment that were not developed with them in mind.
Today, NIH requires that all clinical research applications address the inclusion of women

A person's race, ethnicity, age,

Prior to the 1990s, certain groups

sex, and gender impact their

experience with health and

disease.

address the inclusion of women, individuals from underrepresented racial and ethnic minority populations, and individuals of all ages. Phase 3 trials must provide plans to analyze results broken out by sex/gender and race/ethnicity.

CANCER SURVIVORSHIP



Image credit: Matthew Zachary.

Improvements in cancer treatments have helped more people to live longer with the disease, resulting in an increased population of cancer survivors. In 2019, about 5% of the U.S. population were cancer survivors. To support this growing population, NIH has promoted the development of a new field of research on cancer survivorship.

- In 2019, about 5% of the U.S. population were cancer survivors; 18% of those survivors had lived 20 years or more past diagnosis.
- In 1996, NIH created the Office of Cancer Survivorship, dedicated to research on cancer survivorship.
 In the 25 years it has been in existence, the office's research portfolio has increased over 90%.



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HEALTH AND RETIREMENT



The Health and Retirement Study (HRS) is the most comprehensive population-representative study of adults over age 50 in the U.S., with 17 international HRS sister studies, supporting cross-national comparisons on more than half of the world's population.

Image credit: National Institute on Aging, NIH.

participants contributing
 comprehensive long-term data
 on health and cognitive status—
 including genetics, demographic,
 economic, and familial
 characteristics, along with
 Medicare data.
 HRS has yielded more than 5,800

The HRS has over 20,000

- HRS has yielded more than 5,800 journal articles, reports, book chapters, and dissertations.
- HRS researchers gather cognitive testing results and collect DNA and blood samples to ensure that the biomarkers—biological indicators of disease—being developed using these samples will be applicable to the widest range of people possible.

RESILIENCE RESEARCH



Image credit: Photo by Fortune Vieyra on Unsplash.

NIH research was critical in incorporating resilience research frameworks and tools into research plans at the federal and international level. Understanding how resilience—an individual's capacity to resist, adapt, recover, and grow from challenges—plays a role in health is important for understanding differences in health outcomes across diverse populations.

- NIH-supported resilience research frameworks are pioneering the science of resilience and advancing new studies to better understand how people respond to challenges or stressors—promoting resilience.
- Strengthening health systems through the lens of resilience has reached beyond NIH to a government-wide approach—the new Federal Plan for Equitable Long Term Recovery and Resilience—and to international organizations—including the World Health Organization and the European Observatory on Health Systems and Policies.



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Research for All

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Research for All

HEALTH AND RETIREMENT

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Research Tools

NIH leads the charge on developing new research tools that have broad applications, pushing the boundaries on multiple research fronts.

CRYO-EM



Image credit: Huilin Li, Brookhaven National Laboratory, and Bruce Stillman, Cold Spring Harbor Laboratory.

NIH funded the development and dissemination of cryo-electron microscopy (cryo-EM), a tool that enables high-resolution images of proteins and other biological structures. Cryo-EM has helped researchers identify potential new therapeutic targets for vaccines and drugs.

- An NIH-funded researcher was awarded the 2017 Nobel Prize in Chemistry for their work characterizing proteins using cryo-EM.
- Since 2018, the NIH-supported National Centers for Cryo-EM enabled researchers to determine the structure of more than 300 proteins, including the SARS-CoV-2 spike protein, and trained more than 1,000 investigators in this cutting-edge technique.

SMALL MOLECULE SCREENING



Image credit: National Center for Advancing Translational Sciences, NIH.

Thanks to NIH, publicly funded researchers now have access to resources and tools with the capacity to screen large numbers of small molecules, helping them to more efficiently study genes and discover treatments for human diseases. Researchers used these resources to develop FDA-approved treatments for ulcerative colitis and relapsing forms of multiple sclerosis.

- This advancement in small molecule research makes it easier for scientists to use and understand molecular compounds in basic research and drug development.
- The NIH Common Fund Molecular Libraries and Imaging Program also launched PubChem, an open chemistry database that contains information on chemical structures, properties, and biological activities of over 100 million compounds, including small molecules.
- NIH also developed tools and resources to help scientists conduct preclinical research, with a focus on small molecule screening.



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SINGLE CELL ANALYSIS



Image credit: NIH.

NIH fostered a technological revolution in single cell analysis research, leading to the development of cutting-edge tools, methods, platforms, and cell atlases to identify and characterize features of single cells within a variety of human tissues. These technologies are available to the entire research community to foster additional breakthroughs in research.

- The human body contains approximately 37 trillion cells, carefully organized in tissues to carry out the daily processes that keep the body alive and healthy. Analysis of single cells poses many technological challenges.
- Between 2012 and 2017, the NIH Common Fund Single Cell Analysis Program found a threefold increase in the number of single cell analysis projects funded by NIH and an approximate doubling of relevant publications.
- Understanding cells at the individual level may lead to new understandings of development, health, aging, and disease.

CELL CULTURE TECHNOLOGY



Image credit: David Sone.

NIH scientists created Matrigel, a specialized gel that promotes cell growth on a 3-D surface that mimics the environment within the body. Today, Matrigel is widely used in labs around the world to study cells that were previously impossible to grow and to investigate complex cell activities in a more relevant environment.

- Prior to this invention, scientists grew cells in a flat layer in plastic culture dishes, which was not sufficient to grow specialized cells, like stem cells.
- Using Matrigel, researchers discovered new insights into nerve growth, the formation of blood vessels, and stem and cancer cell biology. It is also being used to screen cancer drugs and to support development of artificial tissues that can mimic organ function.
- More than 13,000 scientific papers have cited the use of Matrigel in their studies.



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CANCER GENOME ATLAS



Image credit: Darryl Leja, National Human Genome Research Institute, NIH.

The Cancer Genome Atlas (TCGA) is a landmark NIH cancer genomics program that transformed our understanding of cancer by analyzing tumors from 11,000 patients with 33 different cancer types. Findings from TCGA identified new ways to prevent, diagnose, and treat cancers, such as gliomas and stomach cancer.

- TCGA showed that different cancers can share molecular traits regardless of the organ or tissue they are found in. This enabled the emergence of precision medicine in oncology cancer treatment based on molecular traits rather than the tissue in the body where the cancer started.
- TCGA generated over 2.5 petabytes (1 petabyte = 500 billion pages of standard printed text!) of data on genes, proteins, and their modifications in cancer by bringing together 20 collaborating institutions across the U.S. and Canada.

RECOMBINANT DNA



Image credit: National Human Genome Research Institute, NIH.

Because of NIH-funded research on recombinant DNA technology, researchers developed techniques that can enable the production of large quantities of important peptides—the building blocks of proteins—which can be used to produce certain medicines.

- Scientists use specialized molecules to snip out a specific gene from a long strand of DNA, creating recombinant DNA by inserting it into bacterial or yeast cells. These cells reproduce quickly and, following the gene's instructions, make large amounts of the desired peptide.
- These techniques enabled the production of synthetic insulin to treat diabetes.
- Medicines produced using these techniques have been used for more than 30 years.
- In 1980, an NIH-funded researcher received a Nobel Prize for research on recombinant DNA.



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IMAGING TECHNOLOGY



Image credit: Clinical Center, NIH.

Significant innovation in clinical imaging technology is a result of NIH-funded research. Imaging technologies now have higher resolution and greater sensitivity, with new categories of imaging, like digital 3D reconstructions, now being commonly used.

- A new type of positron emission tomography (PET) that looks for prostate cancer specific proteins has been found to be 27% more accurate than standard methods for detecting prostate cancers.
- NIH-supported improvements in PET technologies resulted in a more sensitive technology that can capture scans in under a minute and reduce the dose of dye given to patients.
- NIH-funded research led to the development of nuclear magnetic resonance imaging, which won a Nobel Prize, and is the same technique used in MRIs in clinical settings.



Revolutionizing Science

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Research Tools

CRYO-EM

- 1. Transformative High-Resolution Cryoelectron Microscopy Program: <u>https://commonfund.nih.gov/CryoEM</u>
- 2. Cryo-Electron Microscopy Program Centers: <u>https://www.cryoemcenters.org</u>
- 3. Zhang K, et al. *bioRxiv* [Preprint]. 2020:2020.08.11.245696. Update in: *QRB Discov*. 2020;1:e11. PMID: <u>32817943</u>.
- 4. NIH Nobel Laureates: <u>https://www.nih.gov/about-nih/what-we-do/nih-almanac/nobel-laureates</u>
- 5. Cressey D, et al. Nature. 2017;550(7675):167. PMID: 29022937.

SMALL MOLECULE SCREENING

- 1. Molecular Libraries and Imaging: https://commonfund.nih.gov/molecularlibraries/index
- 2. Preclinical Research Toolbox: https://ncats.nih.gov/expertise/preclinical
- 3. PubChem: https://pubchemdocs.ncbi.nlm.nih.gov/statistics
- 4. Molecular Libraries and Imaging Program Highlights: https://commonfund.nih.gov/Molecularlibraries/programhighlights
- 5. Article: Ozanimod accepted for priority review by FDA for the treatment of ulcerative colitis: <u>https://www.scripps.edu/news-and-events/press-room/2021/20210203-rosen-roberts-ozanimod-fda-ulcerative-colitis.html</u>
- Article: U.S. Food and Drug Administration Approves Bristol Myers Squibb's Zeposia[®] (ozanimod), an Oral Treatment for Adults with Moderately to Severely Active Ulcerative Colitis: <u>https://news.bms.com/news/corporate-financial/2021/U.S.-Food-and-Drug-Administration-Approves-Bristol-Myers-Squibbs-Zeposia-ozanimod-an-Oral-Treatment-for-Adults-with-Moderately-to-Severely-Active-Ulcerative-Colitis1/default.aspx</u>

SINGLE CELL ANALYSIS

- 1. NIH Single Cell Analysis Program: https://commonfund.nih.gov/singlecell
- 2. Roy AL, et al. *Sci Adv*. 2018;4(8):eaat8573. PMID: <u>30083611</u>.
- 3. The Human BioMolecular Atlas Program: <u>https://commonfund.nih.gov/HuBMAP</u>
- 4. HuBMAP Data Portal: https://portal.hubmapconsortium.org/
- 5. Cellular Senescence Network: https://commonfund.nih.gov/senescence
- 6. LungMAP: <u>https://www.lungmap.net/</u>
- 7. GenitoUrinary Development Molecular Anatomy Project: <u>https://www.gudmap.org/</u>

CELL CULTURE TECHNOLOGY

- 1. Simian M, et al. J Cell Biol. 2017;216(1):31-40. PMID: 28031422.
- 2. Article: An Interview with Hynda Kleinman: <u>https://irp.nih.gov/catalyst/v21i4/alumni-news</u>
- 3. Kleinman HK, et al. Semin Cancer Biol. 2005;15(5):378-86. PMID: 15975825.
- 4. Article: Hair today, gone tomorrow: NIDCR'S Hynda Kleinman takes off for new horizons: https://nihsearch.cit.nih.gov/catalyst/2005/05.11.01/page4.html

National Institutes of Health

Revolutionizing Science

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Research Tools

CANCER GENOME ATLAS

1. The Cancer Genome Atlas Program: <u>https://www.cancer.gov/about-nci/organization/ccg/research/structural-genomics/tcga</u>

RECOMBINANT DNA

- 1. NIGMS-Supported Nobelists: <u>https://www.nigms.nih.gov/pages/GMNobelists.aspx</u>
- 2. Article: Celebrating the discovery and development of insulin: www.niddk.nih.gov/news/archive/2021/celebrating-discovery-development-insulin
- 3. National Institute of General Medical Sciences. *The New Genetics*. 2010. <u>https://nigms.nih.gov/education/Booklets/the-new-genetics/Documents/Booklet-The-New-Genetics.pdf</u>

IMAGING TECHNOLOGY

- 1. Article: Commemorating the 50th Anniversary of the National Cancer Act (NCA50): Clinical Imaging Then and Now: <u>https://dctd.cancer.gov/NewsEvents/20210712_NCA50.htm?cid=soc_ig_en_enterprise_nca50</u>
- 2. EXPLORER Total Body PET Scanner: https://health.ucdavis.edu/radiology/myexam/PET/Equipment/explorer.html
- 3. Badawi RD, et al. J Nucl Med. 2019;60(3):299-303. PMID: <u>30733314</u>.
- 4. Article: PSMA PET-CT Accurately Detects Prostate Cancer Spread, Trial Shows: <u>https://www.cancer.gov/news-events/cancer-currents-blog/2020/prostate-cancer-psma-pet-ct-metastasis</u>
- 5. NIGMS-Supported Nobelists: <u>https://www.nigms.nih.gov/pages/GMNobelists.aspx</u>
- 6. Magnetic Resonance Imaging (MRI): <u>https://www.nibib.nih.gov/science-education/science-topics/magnetic-resonance-imaging-mri</u>



Revolutionizing Science

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Resource Sharing

NIH spearheads the sharing of resources across the research enterprise to advance science in ways that were not previously possible.

GENOMIC DATA SHARING



Image credit: Janusz Dutkowski and Trey Ideker, University of California, San Diego.

Data and resources shared by NIH significantly bolstered the research community's ability to investigate the effects of gene behavior on complex diseases. This widespread sharing of information led to new insights into type 1 diabetes, coronary artery disease, bipolar disorder, and schizophrenia, among others.

- Variations in genes can affect the way that genes behave in health and disease.
- In 2014, NIH released the Genomic Data Sharing Policy, which set forth expectations and facilitated the sharing of genomic research data broadly and responsibly.
- The NIH Common Fund Genotype-Tissue Expression Project created a catalog of genetic variants that includes over 17,000 RNA-sequencing samples from 948 donors, and a related online resource stores and shares data that has been used to support over 7,000 publications.

GENOMIC DATA



Image credit: Ernesto del Aguila III, National Human Genome Research Institute, NIH.

The NIH-funded Genomic Data Commons (GDC) enables researchers to share data in a way that allows the data to be reused and compared across different projects. This fosters a culture of collaboration and makes it much easier for researchers to access highquality datasets for their research.

- The GDC contains data from multiple cancer genomic datasets, including the Cancer Genome Atlas (TGCA) and the Therapeutically Applicable Research to Generate Effective Therapies (TARGET) database.
- The GDC currently contains 2.9 petabytes (1 petabyte = 500 billion pages of standard printed text!) of primary sequencing data from over 60 projects. Since its inception in 2016, the GDC has been cited in over 400 publications.



Downloaded from: <u>https://www.nih.gov/about-nih/what-we-do/impact-nih-research</u>

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INSTRUMENT SHARING



Image credit: Rhoda Baer, National Institute of Arthritis and Musculoskeletal and Skin Diseases, NIH.

NIH accelerates scientific discovery through supporting the purchase of state-of-the-art commercially available instruments that are too expensive to be acquired by an individual researcher. These instruments are used on a shared basis across institutions, enabling them to be used by multiple researchers and projects.

- Shared resources are costefficient and beneficial to thousands of researchers in hundreds of institutions nationwide.
- Since 2012, NIH funded the purchase of over 1,100 scientific instruments, which advanced and enabled new NIH-funded research.
- Over 11,000 research publications have cited the use of NIH-supported instruments. Through this program, biomedical researchers gained access to modern scientific instruments that they would not otherwise have been able to use due to the exceedingly high cost of purchasing advanced scientific equipment.

CANCER STATISTICS



Image credit: Ernesto del Aguila III, National Human Genome Research Institute, NIH.

NIH and CDC fund the nation's premiere resource for cancer statistics, the Surveillance, Epidemiology, and End Results (SEER) program. SEER collects data from multiple cancer registries covering ~48% of the U.S. population—which can support an enormous variety of research studies and be used to inform nonprofit, state, and federal reports and policy changes.

- SEER data are used to measure progress against cancer and identify challenges that need to be addressed.
- SEER has also been linked to the Medicare database, creating a valuable source of detailed information on Medicare recipients with cancer.
- SEER data have been used as the primary dataset in >17,000 publications and have been referenced in >86,000 publications.



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STEM CELLS



Image credit: National Eye Institute, NIH.

NIH-supported science enabled the first U.S. clinical trial of patientderived stem cell therapy to treat age-related macular degeneration. By developing scientific resources that could be shared with the broader scientific community, NIH accelerated the development of stem cell-based therapies that are potential tools for regenerative medicine.

- The clinical trial used a type of patient-derived stem cell called induced pluripotent stem cells (iPSCs), which are adult cells reprogrammed to behave like embryonic stem cells—a type of stem cell that can become any cell in the body.
- NIH developed and made available several iPSC cell lines for research and clinical use.
- Other research groups are now using this protocol to develop therapeutics and working to overcome technical hurdles that will enable these cells to be used beyond research, such as in regular clinical practice.

BEHAVIORAL SCIENCE



Image credit: Ernesto del Aguila III, National Human Genome Research Institute, NIH.

NIH-funded research improved our understanding of human behavior and transformed how human behavioral research is conducted. For example, NIH supports a collection of more than 100 behavioral science measures, targets, and experimental outcomes in a publicly accessible repository.

- The NIH Common Fund Science of Behavior Change Program demonstrated how interventions focused on behavioral change, such as adherence to medical regimens, can influence health behaviors and be measured across the whole person at the psychological, behavioral, social, and biological levels.
- Human behavioral interventions are relevant to many disease conditions, such as disrupted sleep, type 2 diabetes, and chronic pain.



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CLINICALTRIALS.GOV



Image credit: National Eye Institute, NIH.

Since 2000, the NIH-supported website, ClinicalTrials.gov, has provided easy access to information about nearly 400,000 publicly and privately supported clinical studies on a range of diseases and conditions in all 50 states and 220 countries. This resource is available to patients, their family members, health care professionals, and researchers.

- NIH worked with FDA to develop the ClinicalTrials.gov website, which launched in 2000.
- Anyone can use ClinicalTrials.gov to find a study for themselves or someone else, see results of completed studies, and learn how clinical research works. Researchers can also use the site to share up-to-date information about their clinical research.
- ClinicalTrials.gov has information on nearly 400,000 registered studies, and in the past 12 months, the site has had 51 million unique visitors and 252 million page views.

PUBMED



Image credit: Darryl Leja, National Human Genome Research Institute, NIH. Available online since 1996, PubMed is a free NIH-supported resource that facilitates the search and retrieval of biomedical and life sciences literature. The PubMed database contains more than 33 million citations and abstracts.

- PubMed is the most frequently used scientific and medical literature database in the world.
- PubMed has more than 2.5 billion daily searches by researchers, medical practitioners, and the public.



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Resource Sharing

GENOMIC DATA SHARING

- 1. NIH Genomic Data Sharing Policy: <u>https://sharing.nih.gov/genomic-data-sharing-policy</u>
- 2. Database of Genotypes and Phenotypes: <u>https://www.ncbi.nlm.nih.gov/gap/</u>
- 3. Genotype-Tissue Expression Program: https://commonfund.nih.gov/Gtex
- 4. GTEx Portal: <u>https://gtexportal.org/home/</u>
- 5. Genotype Tissue Expression (GTEx) Project: <u>https://www.genome.gov/Funded-Programs-Projects/Genotype-</u> <u>Tissue-Expression-Project</u>
- 6. Gamazon ER, et al. *Nat Genet*. 2015;47(9):1091-8. PMID: <u>26258848</u>.
- 7. Fromer M, et al. Nat Neurosci. 2016;19(11):1442-1453. PMID: 27668389.
- Article: Gene Duplication: New Analysis Shows How Extra Copies Split the Work: <u>https://directorsblog.nih.gov/2016/05/31/gene-duplication-new-analysis-shows-how-extra-copies-split-the-work/</u>

GENOMIC DATA

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- 2. Grossman RL, et al. N Engl J Med. 2016;375(12):1109-12. PMID: 27653561.

INSTRUMENT SHARING

- 1. S10 Instrumentation Search Results in RePORTER: https://reporter.nih.gov/search/xI5Pmyns9UCZNT4kOtydkw/projects?shared=true
- 2. NIH S10 Instrumentation Programs: Awards: <u>https://orip.nih.gov/construction-and-instruments/s10-instrumentation-programs/filterable-awards-table</u>

CANCER STATISTICS

1. Surveillance, Epidemiology and End Results (SEER) Program Metrics: https://seer.cancer.gov/about/factsheets/metrics.pdf

STEM CELLS

- 1. Regenerative Medicine Program: <u>https://commonfund.nih.gov/stemcells</u>
- 2. Sharma R, et al. Sci Transl Med. 2019;11(475):eaat5580. PMID: <u>30651323</u>.
- 3. Article: NIH launches first U.S. clinical trial of patient-derived stem cell therapy to replace dying cells in retina: <u>https://www.nih.gov/news-events/news-releases/nih-launches-first-us-clinical-trial-patient-derived-stem-cell-therapy-replace-dying-cells-retina</u>
- 4. Article: NIH researchers rescue photoreceptors, prevent blindness in animal models of retinal degeneration: <u>https://www.nih.gov/news-events/news-releases/nih-researchers-rescue-photoreceptors-prevent-blindness-animal-models-retinal-degeneration</u>
- 5. Stem Cell Translation Laboratory: https://ncats.nih.gov/stemcell
- 6. AMD Integrative Biology Initiative: <u>https://www.nei.nih.gov/about/goals-and-accomplishments/nei-research-initiatives/regenerative-medicine/amd-integrative-biology-initiative</u>



National Institutes of Health Turning Discovery Into Health

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Revolutionizing Science

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Resource Sharing

BEHAVIORAL SCIENCE

- 1. Science of Behavior Change: https://commonfund.nih.gov/behaviorchange
- 2. Science of Behavior Change Related Activities: https://commonfund.nih.gov/behaviorchange/related
- 3. The Measures of the Science of Behavior Change: <u>https://measures.scienceofbehaviorchange.org/</u>
- 4. NIH Science of Behavior Change: <u>https://www.nia.nih.gov/research/dbsr/science-behavior-change-sobc</u>
- 5. Science of Behavior Change Program Highlights: https://commonfund.nih.gov/behaviorchange/programhighlights
- 6. Special issue of *Health Psychology*: The Science of Behavior Change: Implementing the Experimental Medicine Approach: <u>https://psycnet.apa.org/PsycARTICLES/journal/hea/39/9</u>

CLINICALTRIALS.GOV

1. ClinicalTrials.gov: <u>https://clinicaltrials.gov/</u>

PUBMED

1. PubMed Database: <u>https://pubmed.ncbi.nlm.nih.gov/</u>



Revolutionizing Science

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Scientific Breakthroughs

NIH supports pivotal research breakthroughs that result in the emergence of new research fields, great leaps in our scientific understanding, and novel scientific techniques that can be harnessed for wide ranging applications.

GREEN FLUORESCENT PROTEIN



Image credit: Katti Prasanna, Ph.D., Muscle Energetics Laboratory, National Institute of Arthritis and Musculoskeletal and Skin Diseases and National Heart, Lung, and Blood Institute, NIH.

Green fluorescent protein (GFP), which causes jellyfish and other organisms to glow green, has been used to understand genetics, cell biology, developmental biology, neurobiology, cancer, and brain diseases. NIH-supported research led to the discovery of GFP and contributed to its adaptation in biomedical research.

- GFP revolutionized biomedical science and played a critical role in understanding genetics and cell biology. Today, it is widely used in the pharmaceutical and biotechnology industries.
- GFP played a crucial role in a 2012 research project involving the integration of stem cells into existing heart muscle in the hope of developing new treatments for damaged heart tissue.
- Researchers won the Nobel Prize in Chemistry in 2008 for their work with GFP.

MRNA VACCINE



Image credit: National Institute of Allergy and Infectious Diseases, NIH.

Decades of NIH-supported research, including investments in HIV research, revolutionized vaccine development, leading to the first two FDA-approved vaccines for COVID-19. These vaccines use mRNA to train the body to recognize SARS-CoV-2, the virus that causes COVID-19.

- Traditionally, vaccines work by introducing a weakened or inactivated virus, or a virus protein, into the body to induce an immune response against the virus.
- With this new class of vaccines, the cellular messenger, mRNA, delivers instructions to cells to induce an immune response against the virus.
- Both Moderna and Pfizer/BioNTech vaccines use an mRNA sequence of the SARS-CoV-2 spike protein discovered by NIH scientists and collaborators.



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NOBEL PRIZES



Image credit: NIH.

NIH researchers are leaders in their fields. Dozens of NIH-supported scientists have received Nobel Prizes for their groundbreaking achievements. These prizes recognize those who have conferred the greatest benefit to humankind in the past 12 months.

- As of 2022, 169 researchers either at NIH or whose research was supported by NIH have received or shared 101 Nobel Prizes.
- Often called "America's Nobels," the Lasker Award recognizes the contributions of scientists, physicians, and public servants who have made major advances in the understanding, diagnosis, treatment, cure, and prevention of human disease.
- The 214 NIH Lasker awardees, as of 2022, include extramural researchers, intramural researchers, and institutional award recipients.

HUMAN GENOME PROJECT



Image credit: National Human Genome Research Institute, NIH.

The Human Genome Project—an international effort supported in part by NIH that brought together engineers, computer scientists, mathematicians, and biologists revolutionized the field of genomics, spurred the development of novel technologies and analytical tools, established a commitment to open science and data sharing, and changed the face of the scientific workforce.

- Today, there are more than 1.25 million results for the search term "genomics" in PubMed, up from around 400,000 in 2003.
- NIH maintains a human genome reference sequence and data repositories of anonymized datasets, which host over 1,887 studies.
- Genomic data sharing promotes public benefit from federally funded research because it prevents the same research from being performed or paid for twice, and open approaches to data sharing continue to build on the foundation set by the Human Genome Project.



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THE GENETIC CODE



Image credit: NIH.

In the 1960s, NIH researchers discovered the process by which the genetic code of DNA is translated into proteins via messenger RNA, launching the genetic revolution. This discovery has touched nearly every field of science, from anthropology to zoology, revealing our underlying biology, including the cause of many diseases.

- Often described as "cracking the genetic code" this Nobel Prizewinning advance revolutionized research, touching nearly every field of science.
- This NIH research formed the foundation for personalized, gene-based medicine and new treatments for many diseases including cancer, sickle cell disease, cystic fibrosis, and other genetic disorders, both rare and common.

GENE EDITING



Image credit: Ernesto del Aguila III, National Human Genome Research Institute, NIH.

New gene-editing techniques, developed through NIH-supported research, are faster, cheaper, more efficient, and have the potential to correct the DNA code inside living cells. These technologies allow for disease-causing genetic material to be added, removed, or altered within cells and are currently being tested to treat genetic diseases.

- Gene-editing harnesses the natural process of DNA repair, in which a broken section of DNA triggers a cell's repair mechanism to stitch together the break.
- CRISPR/Cas9, the most widely used gene editor, was discovered through NIH-funded basic research on how bacteria defend themselves from viruses.
- Gene-editing techniques are being pursued as ways to diagnose viruses and to treat genetic diseases.
- In 2019, NIH spent \$391 million on gene therapy research, funding 827 grants, and spent \$46 million on gene therapy clinical trials, funding 69 grants.



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Scientific Breakthroughs

GREEN FLUORESCENT PROTEIN

1. Golden Goose Awardee 2012: Green Fluorescent Protein: https://www.goldengooseaward.org/01awardees/gfp

MRNA VACCINE

- 1. Decades in the Making: mRNA COVID-19 Vaccines: <u>https://covid19.nih.gov/nih-strategic-response-covid-19/decades-making-mrna-covid-19-vaccines</u>
- 2. Coronavirus Vaccines and Prevention: <u>https://www.niaid.nih.gov/diseases-conditions/coronavirus-vaccines-prevention</u>
- 3. COVID-19 mRNA Vaccines: https://www.niaid.nih.gov/sites/default/files/mRNA%20Vaccine%20Development.pdf
- 4. Article: A gamble pays off in 'spectacular success': How the leading coronavirus vaccines made it to the finish line: <u>https://www.washingtonpost.com/health/2020/12/06/covid-vaccine-messenger-rna/</u>
- 5. Shepherd BO, et al. *Curr HIV/AIDS Rep*. 2022;19(1):86-93. PMID: <u>35089535</u>.

NOBEL PRIZES

- 1. NIH Nobel Laureates: https://www.nih.gov/about-nih/what-we-do/nih-almanac/nobel-laureates
- 2. The Nobel Prize: <u>https://www.nobelprize.org/</u>
- 3. NIH Intramural Research Program Nobel Prizes: <u>https://irp.nih.gov/about-us/honors/nobel-prize</u>
- 4. NIH Intramural Research Program Lasker Awards: <u>https://irp.nih.gov/about-us/honors/lasker-award</u>

HUMAN GENOME PROJECT

- 1. Genomics Articles in PubMed: https://pubmed.ncbi.nlm.nih.gov/?term=genomics
- 2. Green ED, et al. Nature. 2020;586(7831):683-692. PMID: <u>33116284</u>.
- 3. RefSeq curation and annotation of the human reference genome: <u>https://www.ncbi.nlm.nih.gov/refseq/about/human/</u>
- 4. AnVIL: NHGRI's Genomic Data Science Analysis, Visualization, and Informatics Lab-Space: https://anvilproject.org/

THE GENETIC CODE

- 1. Griffiths AJF, et al. (2000). An Introduction to Genetic Analysis. (7th ed.). W. H. Freeman.
- 2. Deciphering the Genetic Code: https://www.acs.org/content/acs/en/education/whatischemistry/landmarks/geneticcode.html
- 3. Marshall Nirenberg: Deciphering the Genetic Code: <u>https://history.nih.gov/display/history/Nirenberg+Introduction</u>
- 4. Biographical Overview of Marshall Nirenberg: <u>https://profiles.nlm.nih.gov/spotlight/jj/feature/biographical</u>

GENE EDITING

- 1. Gene Editing: https://www.nih.gov/news-events/gene-editing-digital-press-kit
- 2. Estimates of Funding for Various Research, Condition, and Disease Categories (RCDC): <u>https://report.nih.gov/funding/categorical-spending#/</u>



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