

# NIH...

## Turning Discovery Into Health

### OUR KNOWLEDGE

NIH plays a key role in the expansion of biomedical knowledge. NIH-funded research leads to tens of thousands of new scientific findings every year. These fundamental advances and technological developments expand our understanding of health and living systems, and form the building blocks required for translational and clinical advances to occur. NIH also fosters the generation of new knowledge within the scientific community by supporting training within the research workforce.

The following represent some of the key ways NIH contributes to advancing our knowledge:

#### *Supporting and Training World-class Researchers*

- **Over 95% of the NIH budget** goes directly to research awards, programs, and centers; training programs; and research and development contracts.<sup>1</sup>
- NIH funds scientists across the country and even across the globe. Each year, NIH awards more than **60,000 research and training grants**. These support:
  - approximately **300,000 researchers**
  - at more than **2,500 universities** and organizations
  - in every state.<sup>2</sup>
- NIH also supports the next generation of researchers. In 2016, NIH grants directly **supported the training of more than 9,500 pre-doctoral students and almost 5,900 post-doctoral fellows through training grants**<sup>3</sup> as well as many other students through extramural research grants.<sup>4</sup>
- NIH also directly employs scientists in its intramural program. **11% of the NIH budget**<sup>5</sup> funds **1,200 Principal Investigators and more than 4,000 Postdoctoral Fellows** working at NIH laboratories in Maryland, North Carolina, and Montana.<sup>6</sup>
- NIH's researchers are leaders in their fields.
  - [153 NIH-supported researchers](#),<sup>7</sup> including [22 intramural researchers](#),<sup>8</sup> have been awarded **Nobel Prizes**.
  - 195 NIH-supported researchers, including [36 intramural researchers](#), have received **Lasker Awards**, which recognize researchers and clinicians for contributions to medicine.<sup>9</sup>

## *Building a Knowledge Base for All Scientists*

- NIH grantees publish their research findings in scientific journals, which are a major avenue for how scientists share knowledge.
  - In 2016 alone, over 115,000 articles acknowledged NIH grant support.<sup>10</sup>
  - Each [R01 grant](#), NIH's most common type of research project grant, leads to an average of **7.36 published research articles**.<sup>11</sup>
  - Those articles go on to be referred to by other scientists; the publications from a single grant accumulate an average of almost **300 citations** in the academic literature.
- NIH supports a repository of research findings via the National Library of Medicine (NLM).
  - NLM's PubMed/MEDLINE is now the **most frequently used scientific and medical database in the world**.<sup>12</sup>
  - This database had **nearly 2.8 billion searches** in 2015 by researchers, medical practitioners, and the general public, who accessed **more than 28 million available journal citations and dispatched an average of over 6,500 million bits of data per second**.<sup>13</sup>
- A recent study found that **NIH funding contributed to at least the fundamental basic research underlying every one of the 210 new FDA-approved drugs from 2010-2016**.<sup>14</sup>
- NIH grants lead to novel inventions and patents.
  - NIH research funding directly yields approximately **6 new patents for every \$100 million** of grant and contract funding.<sup>15</sup>
  - Each year's new round of funding can be expected to generate at least **100 to 120 new inventions**.<sup>16</sup>
- NIH investment also spurs private-sector patents, because the biotechnology and pharmaceutical industries build on knowledge generated by NIH funding.
  - Every **\$10 million** increase in NIH funding for a particular scientific field generates **3.26 additional private sector patents** in that field.
  - That translates to **one additional private-sector patent for every two additional NIH grants**.<sup>17</sup>
  - While only approximately **10 percent of NIH grants directly produce patents** within 25 years, over **30 percent of NIH grants produce publications that go on to be cited by patents** from the private sector over the same period.<sup>18</sup>
  - Some of the patents that cite NIH-funded publications go on to be used in FDA-approved drugs. Over a 27 year period, **5% of NIH grants** produce publications that are cited in patents awarded during the development of **an FDA approved drug**.<sup>19</sup>

## *Harnessing New Knowledge for Biomedical Advances*

- **Knowledge from NIH-funded research spreads to other scientific areas.** More than half of private sector biomedical patents are in a different disease area from the NIH grants they cite.<sup>20</sup>
- NIH intramural researchers **revolutionized understanding of how nerve cells communicate**, laying a foundation for the development of many **targeted medications for depression and anxiety** and leading to a Nobel Prize in 1970.<sup>21</sup>
- In the 1960s, NIH-supported research on the immune system led to the discovery and characterization of the human leukocyte antigen (HLA) system. Today, these **genes are used to match donors to hosts for organ transplantation** and minimize rejection in the host.<sup>22</sup>
- The NIH Clinical Center was the **first to adapt an electronic medical record to collect data from an outpatient clinical trial** in 1985.<sup>23</sup> The Clinical Center continues to push the envelope for improvements and innovations in how research is done.
- NIH-funded research is helping to **personalize treatments for back pain by identifying when surgery would be beneficial**.<sup>24</sup> NIH grantees are using this knowledge to create new tools for making treatment decisions, including a calculator that can predict whether a patient is a good candidate for surgery.<sup>25</sup>
- Gleevec®, developed in part with NIH support, was **the first cancer drug approved by the FDA that directly targeted a signaling molecule inside the cell**,<sup>26</sup> and launched a trend of developing targeted molecular medicines based on a deep understanding of the genes and molecules that cause disease.<sup>27</sup>
- NIH-funded research also identified **specific disease-related genes which can be targeted with cutting-edge genetic therapies**, including genes for spinal muscular atrophy (Spinraza™),<sup>28</sup> a rare form of inherited vision loss (Luxturna™),<sup>29</sup> and other retinal diseases such as Leber congenital amaurosis.<sup>30</sup>
- **NIH-funded research developed an easy saliva test to identify Cytomegalovirus (CMV) infection in infants**<sup>31</sup>, a major cause of hearing loss in children that is often passed down from a mother to her unborn child.
- **NIH-funded scientists have developed methods to transform stem cells into brain cells**.<sup>32</sup> These methods offer a versatile way for scientists to study brain cell function, for example, by growing cells from people with genetic changes related to disease.
- The research used to create the *Haemophilus influenzae B* vaccine has been applied to create several **vaccines against other disease-causing bacteria**, such as pneumococci, meningococci, *Salmonella typhi*, group B streptococci, and *E coli*,<sup>33</sup> and stimulated new strategies for **developing effective vaccines for infants**.

### **Spotlight: Understanding the Basics of Alzheimer's Disease**

- Alzheimer’s disease is an irreversible brain disorder that **slowly destroys memory and thinking skills**, and eventually the ability to carry out the simplest tasks. It is the most common cause of dementia among older adults.<sup>34</sup>
- As recently as 30 years ago, very little was known about Alzheimer’s disease.
- Research supported by NIH and other organizations has **greatly expanded knowledge and understanding** of brain function, risk factors, treatment, and prevention.
  - NIH-supported studies have used images of the brain to uncover dramatic insights into how the disease starts. Because of that work, new treatments can now be tested at the earliest stages of disease, ideally even before symptoms have appeared.
  - **More than 90 drugs** are in clinical trials for Alzheimer’s disease, and many more are in the pipeline.<sup>35</sup>
- The [Accelerating Medicines Partnership](#), an NIH-led public-private partnership to transform and accelerate drug development, launched a new [Alzheimer’s Big Data portal](#) for use by the research community.
- With NIH’s support, many scientists and physicians are now working together to identify and understand the genetic, biological, and environmental factors that, over many years, cause Alzheimer’s. This effort is bringing us closer to better treatment for and prevention of this devastating disease.
- The NIH’s National Institute on Aging provides much [more information](#) on current efforts to combat Alzheimer’s disease.

### *Spotlight: Taking on Chronic Obstructive Pulmonary Disease (COPD)*

- COPD is a progressive disease that makes it **hard to breathe**. “Progressive” means the disease gets worse over time. COPD is the third leading cause of death in the United States and a major cause of disability.<sup>36</sup>
- COPD includes two main conditions, emphysema and chronic bronchitis. Most people with COPD have both.
- **NIH-funded studies are investigating** questions such as:
  - What genes contribute to COPD
  - Who is susceptible to COPD
  - How to identify the disease at early stages
  - Understanding subtypes of COPD
  - How COPD progresses
- NIH funding is leading the way to new treatments: Large studies are already **testing several potential treatments** in people who have COPD, and new research is opening the door to ways to tailor treatments to stop the disease.

- The NIH's National Heart, Lung, and Blood Institute provides much [more information](#) on current efforts to address COPD.

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<sup>1</sup> <http://report.nih.gov/NIHDatabook/Charts/Default.aspx?showm=Y&chartId=5&catId=1> and NIH Office of Budget, FY 2017 Budget Justification <https://officeofbudget.od.nih.gov/pdfs/FY17/31-Overview.pdf>

<sup>2</sup> NIH Office of Budget, FY18 Budget Executive Summary page 19:

<http://officeofbudget.od.nih.gov/pdfs/FY18/ExecutiveSummary.pdf>

<sup>3</sup> <https://report.nih.gov/NIHDatabook/Charts/Default.aspx?sid=0&index=1&catId=17&chartId=52>

<sup>4</sup> Lindsay Pool et al. "Size and characteristics of the biomedical research workforce associated with U.S. National Institutes of Health extramural grants" *FASEB J.* 2016 Mar;30(3):1023-36.

<http://www.ncbi.nlm.nih.gov/pubmed/26625903>

<sup>5</sup> <https://www.hhs.gov/about/budget/fy2017/budget-in-brief/nih/index.html>

<sup>6</sup> <http://irp.nih.gov/about-us/what-is-the-irp>

<sup>7</sup> <https://www.nih.gov/about-nih/what-we-do/nih-almanac/nobel-laureates>

<sup>8</sup> <https://irp.nih.gov/about-us/honors/nobel-prize>

<sup>9</sup> <https://www.nih.gov/about-nih/what-we-do/nih-almanac/lasker-awards>

<sup>10</sup> [https://www.nlm.nih.gov/bsd/funding\\_support.html](https://www.nlm.nih.gov/bsd/funding_support.html)

<sup>11</sup> Supplementary Material, Danielle Li and Leila Agha. "Big names or big ideas: DO peer-review panels select the best science proposals?" *Science* 24 April 2015: Vol. 348 no. 6233 pp. 434-438

<http://www.sciencemag.org/content/suppl/2015/04/22/348.6233.434.DC1/Li-SM.pdf>

<sup>12</sup> <http://www.ncbi.nlm.nih.gov/pubmed>

<sup>13</sup> <https://www.nlm.nih.gov/pubs/factsheets/nlm.html>

<sup>14</sup> "Contribution of NIH funding to new drug approvals 2010-2016." Cleary, EG et al. *PNAS* 2018. <https://doi.org/10.1073/pnas.1715368115>

<sup>15</sup> "Patents as Proxies Revisited: NIH Innovation 2000 to 2013" Battelle Technology Partnership Practice, prepared for The Academy of Radiology Research, 2015.

[http://www.battelle.org/docs/tpp/battelle\\_2015\\_patents\\_as\\_proxies.pdf](http://www.battelle.org/docs/tpp/battelle_2015_patents_as_proxies.pdf)

<sup>16</sup> Kalutkiewicz, Michael J. and Ehman, Richard L., Patents as proxies: NIH hubs of innovation, *Nature Biotechnology*, June 2014.

<sup>17</sup> Pierre Azoulay et al. "Public R&D Investments and Private-sector Patenting: Evidence from NIH Funding Rules" NBER working paper, 2015. <http://www.nber.org/papers/w20889>

<sup>18</sup> Danielle Li et al. "The applied value of public investments in biomedical research" *Science*, Mar 30. pii: eaal0010. doi: 10.1126/science.aal0010 <https://www.ncbi.nlm.nih.gov/pubmed/28360137>

<sup>19</sup> Danielle Li et al. "The applied value of public investments in biomedical research" *Science*, Mar 30. pii: eaal0010. doi: 10.1126/science.aal0010 <https://www.ncbi.nlm.nih.gov/pubmed/28360137>

<sup>20</sup> Pierre Azoulay et al. "Public R&D Investments and Private-sector Patenting: Evidence from NIH Funding Rules" NBER working paper, 2015. <http://www.nber.org/papers/w20889>

<sup>21</sup> <https://irp.nih.gov/accomplishments/the-first-understanding-of-how-brain-cells-communicate>

<sup>22</sup> [https://www.nobelprize.org/nobel\\_prizes/medicine/laureates/1980/press.html](https://www.nobelprize.org/nobel_prizes/medicine/laureates/1980/press.html)

<sup>23</sup> <https://irp.nih.gov/accomplishments/first-electronic-medical-record-to-support-clinical-research>

<sup>24</sup> <http://www.dartmouth.edu/sport-trial/>

<sup>25</sup> <http://caligari.dartmouth.edu/SpinalOutcomes>

<sup>26</sup>

<https://www.fda.gov/Drugs/DrugSafety/PostmarketDrugSafetyInformationforPatientsandProviders/ucm110502.htm>

<sup>27</sup> A Decade of Innovation in Rare Diseases. PhRMA 2015. [http://www.phrma.org/sites/default/files/pdf/PhRMA-Decade-of-Innovation-Rare-Diseases.pdf?\\_hstc=46830328.81a11c0b4f136a2de8e187a6149732a0.1](http://www.phrma.org/sites/default/files/pdf/PhRMA-Decade-of-Innovation-Rare-Diseases.pdf?_hstc=46830328.81a11c0b4f136a2de8e187a6149732a0.1)

<sup>28</sup> <https://directorsblog.nih.gov/2017/11/21/clinical-trials-bring-hope-to-kids-with-spinal-muscular-atrophy/>

<sup>29</sup> <https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm589467.htm>

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<sup>30</sup> <https://www.nih.gov/news-events/news-releases/nih-funded-study-points-way-forward-retinal-disease-gene-therapy>

<sup>31</sup> <http://www.nejm.org/doi/full/10.1056/NEJMoa1006561#t=article>

<sup>32</sup> Paşca, S. P., Portmann, T., Voineagu, I., Yazawa, M., Shcheglovitov, O., Paşca, A. M., ... Dolmetsch, R. E. (2011). Using iPS cell-derived neurons to uncover cellular phenotypes associated with Timothy Syndrome. *Nature Medicine*, 17(12), 1657–1662. <http://doi.org/10.1038/nm.2576>. (PMID: [22120178](https://pubmed.ncbi.nlm.nih.gov/22120178/))

<sup>33</sup> [Robbins, JAMA, 1996](#), [Lasker Foundation page](#), and [NICHD Press Release](#)

<sup>34</sup> <https://www.nia.nih.gov/alzheimers/publication/alzheimers-disease-fact-sheet>

<sup>35</sup> <https://www.nia.nih.gov/health/alzheimers/dementia-research-and-clinical-trials>

<sup>36</sup> <http://www.cdc.gov/nchs/fastats/leading-causes-of-death.htm>