Exceptional Opportunities in Medical Science
A View From the National Institutes of Health

As the world’s largest source of biomedical research funding, the US National Institutes of Health (NIH) has been advancing understanding of health and disease for more than a century. Scientific and technological breakthroughs that have arisen from NIH-supported research account for many of the gains that the United States has seen in health and longevity.

For example, an infant born today in the United States can look forward to an average lifespan of about 79 years—nearly 3 decades longer than one born in 1900. Age-adjusted death rates from cardiovascular disease have declined by more than 70% since 1963, with more than half of that decline coming in the last 20 years. Meanwhile, cancer death rates have decreased about 1% annually for the past 15 years. National Institutes of Health–funded research has also led to development of vaccines to protect against an array of life-threatening diseases, including influenza, meningitis, and cervical cancer. As compelling as these facts are, emerging scientific advances provide the opportunity to go even further and faster: this is a time of unprecedented promise in medical research.

Personalized medicine, also referred to as precision medicine, is a promising area for improving health outcomes. For most of medicine’s history, and with notable exceptions like blood transfusion, physicians have been forced to approach prevention and treatment of disease based on the expected response of an average patient because that was the best that could be done. However, a more precise, personalized approach to medicine is becoming possible. One major reason is that the cost of sequencing a human genome has declined substantially and is approaching $1000—an astounding figure considering that it cost about $400 million to produce the first sequence of the human genome a little more than a decade ago.

The leading edge of this new era of precision medicine is cancer. The Cancer Genome Atlas and other NIH-funded research is showing that, even for the same type of cancer, each patient’s tumor harbors a unique set of genes driving malignant growth, and that set of drivers often can predict how that particular cancer will respond to therapy. With an increasingly long list of targeted therapeutics for various biological pathways involved in the development and progression of cancer, there is the opportunity to begin matching information about the genomic changes in an individual’s tumor with the drugs to counteract those changes—and to develop new drugs that target pathways not previously identified.

Genomic information also can predict illness in healthy individuals. In the near future physicians can look forward to a medical landscape in which the pairing of affordable, efficient DNA sequencing and electronic health records could be used to inform a lifetime of health care strategies. Combined with the use of mobile health technology to assist in real-time monitoring of factors such as diet, exercise, blood pressure, heart rate, and blood chemistries, this approach could lead to more precise ways of preventing and managing chronic diseases.

Many would agree that the 86 billion neurons in the human brain represent the most challenging frontier for medical research, and to tackle this in innovative ways, the NIH has just made a major investment in the new Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative. This pioneering, multiagency, research venture will enable the creation of new tools, such as wearable MRI scanners and microelectrodes that can record from thousands of neurons simultaneously, to examine the activity of billions of nerve cells, networks, and pathways in real time. Successful pursuit of the BRAIN initiative will help to provide a foundational platform for major advances in Alzheimer disease, Parkinson disease, autism, schizophrenia, epilepsy, traumatic brain injury, and many other brain disorders.

The NIH is also strongly supporting scientific discovery in the realm of stem cells. This technology is not only revolutionizing the study of disease, it holds promise for major advances in treatment. For example, researchers studying type 1 diabetes have spent decades trying to find a reliable way to replace the pancreatic islets that harbor insulin-producing beta cells. Recently a laboratory partially supported by the NIH has developed an approach that can differentiate stem cells, potentially derived from any individual, into functional beta cells. With the possibility of combining this kind of stem cell research with new methods of generating targeted mutations in mammalian cells at high efficiency—for example, CRISPR-cas9 genome editing systems—it is clear that an entirely new window of opportunity is opening.

The challenges of infectious disease are still common and potentially devastating. Responding to the outbreak of Ebola virus disease in West Africa, the NIH recently initiated emergency human testing of 2 investigational vaccines designed to protect against Ebola infection. If those trials meet with early success, they will be scaled up swiftly. Researchers are also using new insights into the structure of viruses to improve vaccine design for some common infectious diseases, including influenza. Researchers funded by the NIH are...
now working on a “universal” flu vaccine designed to protect against virtually all strains of the flu for extended periods. Such a vaccine could potentially reduce the need for annual flu vaccination and, even more importantly, could protect against a future global flu pandemic.

These new and powerful strategies for vaccine development, based on identifying antigenic regions of the virus that are invariant between different isolates, also apply to human immunodeficiency virus and AIDS. Although this disease is now compatible with a nearly normal lifespan because of antiretroviral regimens made possible in part by 3 decades of NIH-supported research, a vaccine is still sorely needed. That opportunity looks more encouraging than ever. Progress with other medical and behavioral preventive strategies is also being made. An AIDS-free generation may soon be within reach.

Recently, the increasing public health problem of antibiotic resistance has received considerable attention. In the United States, antibiotic-resistant bacteria are associated with approximately 2 million infections a year, and 23,000 deaths. In light of such daunting statistics, the White House has joined with leaders from government, academia, and public health to create a multi-pronged approach to combat antibiotic resistance. Areas in which NIH-funded biomedical science is poised to make progress include improved detection and tracking of pathogens by simple and accurate diagnostics, identification of novel drugs for treating bacterial infections, and development of a clinical trials network poised to test new antibiotics in patients infected with highly resistant organisms.

Although all of these ambitious scientific endeavors offer exceptional promise for advancing human health, the effect that unprecedented budget pressures are having on biomedical research cannot be ignored. Due to inflation, the NIH budget has lost almost 25% of its purchasing power over the last decade (figure in the Supplement). The decline has had important consequences; the NIH once funded 1 in 3 research proposals, but now only has enough resources to support 1 in 6. As a result, a great deal of excellent science is being left unfunded.

These challenging fiscal times are also impeding the ability of the NIH and NIH-funded institutions to recruit and retain the brightest minds in science. Successful biomedical research relies on a steady supply of highly trained and creative scientists who can bring new insights to the basic understanding of biology and advance translation of these insights. If the United States wants talented, young scientists—especially physician-scientists—to pursue high-risk research that will improve human health over the long term, timely and stable funding for biomedical research is needed.

Investment in NIH research not only improves public health but also has high rates of social and economic return. For example, the US government’s $3.8 billion initial investment in the Human Genome Project (HGP) has resulted in nearly $1 trillion in economic growth—a 178-fold return on investment. Likewise, the results of the Women’s Health Initiative’s estrogen plus progestin clinical trial, which cost NIH approximately $250 million, have translated into an estimated net economic return of $37 billion in the past decade—a 140-fold return on investment.

Other nations have witnessed the US success and are scaling up their own research investments. Between 1999 and 2009, Asia’s share of worldwide research and development expenditures increased from 24% to 32%, whereas US expenditures decreased from 38% to 31%. By 2022, if current growth rates hold, China’s funding will surpass that of the United States. Although investments by other countries to the global biomedical research enterprise are welcome, the long-term leadership of the United States in this area ought to be a high priority.

The 21st century is the century of biology. The nation that invests in biomedical research will reap untold rewards in its economy and the health of its people. The NIH is committed to leading the way in this time of exceptional opportunities in medical science, and in translating new discoveries to improve the health of the nation and the world.

ARTICLE INFORMATION

Conflict of Interest Disclosures: The author has completed and submitted the ICMJE Form for Disclosure of Potential Conflicts of Interest and none were reported.

REFERENCES