Reinvigorating Biomedical Research Funding

Since the five-year doubling of the NIH budget, completed in 2003, federal support for basic research has been under increasing pressure - intensified in 2013 by sequestration and congressional efforts to reduce the national deficit. In this new age of austerity, past research funding models are unsustainable. Consequently, universities, private research institutes and industry must explore new ways to work together. In turn, these collaborations must be supported by federal and state policies. California and America depend on world-class science and innovation to improve public health and fuel economic growth.

Recently, an all-star panel of corporate, university and biomedical institute leaders convened at the Salk Institute for Biological Studies in San Diego to explore these issues and discuss potential solutions. Organized by the California Healthcare Institute (CHI), the Pipeline for Life event proposed a number of innovative ways to augment research funding and boost public-private partnerships as the federal government slashes discretionary spending.

The Crisis in Research Funding

Never in human history has our basic understanding of health and disease offered so much promise. Still, there are many diseases with inadequate treatments, and scientific breakthroughs are the only hope for patients. Unfortunately, budgetary constraints across industry and academia are making it difficult for researchers to take full advantage of cutting-edge science. NIH funding has been stagnant for a decade, actually declining when adjusted for inflation. As a result, too many worthy projects go unfunded. To make matters worse, sequestration has reduced the NIH budget by 5 percent across the board.¹

Declining research budgets are not restricted to the NIH. Industrial R&D has also been hit, as patents expire and cash flows from branded drugs have been drastically reduced. Many companies, large and small, face patent cliffs, limiting their resources just when they need to invest in research to refill their development pipelines.

Elected officials, pharmaceutical and biotech CEOs, university chancellors and researchers share one common goal: find creative ways to work within tight budgets to advance the best science in the world. The potential returns on investment are huge. Better therapies for cancer and Alzheimer’s alone will improve health and extend life for millions, mitigating the looming fiscal crisis in Medicare and Medicaid.

Transforming the way basic research is organized and funded is a shared responsibility. While government and Congress must stabilize and sustain adequate funding, academia and industry must find greater efficiencies and develop new tools to maximize research’s return on public investment.

The problems are serious but not unsolvable. While the United States faces vast unmet healthcare needs, groundbreaking technologies, innovative researchers and the world’s most sophisticated scientific infrastructure can yield fresh solutions.

Genetic engineering and genomic sequencing evolved in similar ways, beginning with basic research grants from the NIH. Such government investment has the capacity to catalyze economic growth, much in the same way computers and the Internet, which also benefited from government support, spurred growth in previous decades.

"Should the public sector be the major driver?" asked Michael Marletta, Ph.D., president and CEO of The Scripps Research Institute. "I don't see any way that we can continue the way research has been done in this country without it."

As David Gollaher, Ph.D., president and CEO of CHI noted, commercial companies are reluctant to invest in basic research because no single enterprise can capture all the value from a basic discovery.

"Government has long-term stability to see through projects that may take 20 to 30 years," noted Larry Goldstein, Ph.D., who directs the UC San Diego Stem Cell Program. "Companies don't necessarily last that long."

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A similar revolution is taking place with genomic sequencing. Built on findings from the NIH-funded Human Genome Project, the ability to sequence whole genomes is having a profound impact on health care, genetic screening, disease diagnostics, agriculture, criminal justice and biomedical research. At least 350 biotechnology-based products resulting from the Human Genome Project are currently in clinical trials.2

The Consequences of Failure

One of the greatest scientific successes of the 20th Century was the Salk polio vaccine. It would be hard to overestimate the impact this dreaded virus had on society. In the early 1950s, there were more than 40,000 cases each year.\(^3\) Parents and children lived in fear; public facilities were completely shut down. With the vaccine, and subsequent innovations that built on its success, polio was put on a path toward extinction.

Unfortunately, we face new threats. In some ways, Alzheimer’s disease is the new polio. This incurable neurodegenerative condition affects 5.2 million Americans, a number that will triple by 2050. The disease slowly erodes cognitive capacity until patients no longer recognize loved ones or even remember their own names.

The economic impact is enormous. It’s estimated that the nation spends $203 billion each year on Alzheimer’s and dementia care. That number is expected to approach $1.2 trillion (in current dollars) by 2050. Much of that expense will be borne by Medicare and Medicaid. These numbers do not even account for the sacrifices made by caregivers. It’s estimated that family and friends provided 17.5 billion hours in Alzheimer’s and dementia care in 2012.\(^4\)

Underinvestment also erodes human capital. Of particular concern is the potential loss of young scientists, which could affect the quality of research for decades. Postdoctoral researchers are faced with a catch-22: they have to develop a grant base to have any hopes of attaining a faculty position but the scarcity of resources makes that virtually impossible.

“If you are a young scientist trained in the United States, you have the opportunity to go somewhere else—China, Singapore, Israel, Brazil,” said Rep. Peters. “In the United States, we face the risk of losing our lead in science.”

Creating New Models to Advance Innovative Research

“We have to accept the reality that we face a starkly different future,” said Michael Friedman, M.D., CEO at City of Hope. “We need to get over our nostalgia for the good old days; we need to think about things entirely differently, not just a little differently. This is more than just research; this is the survival of academic health centers in the United States.”

Dr. Friedman noted that old approaches are likely to fail. In order to thrive, academic institutions must break down silos, find new efficiencies and develop new funding models to augment critical NIH grants and drug royalties. One approach is to share expensive resources.

“Think of big assets that cost a quarter billion dollars — every institution doesn’t need them,” said Dr. Friedman.


Michael Friedman, M.D., CEO, City of Hope
He believes the biomedical community should take cues from physicists and astronomers, who routinely share multi-billion dollar particle accelerators and telescopes.

Other examples of capital-intensive resource sharing are emerging throughout the country. In San Diego, the Sanford Consortium for Regenerative Medicine unites stem cell research from UC San Diego, Salk, The Scripps Research Institute, Sanford-Burnham Medical Research Institute and the La Jolla Institute for Allergy and Immunology. These arrangements do more than preserve precious resources; they encourage the sharing of ideas so critical to advance biomedical research.

“We need to find ways to reward collaboration and break down institutional barriers,” said Dr. Goldstein. “It’s not just good economics; it’s good science.”

New Approaches to Partnerships

Dr. Gollaher asked R. Sanders Williams, M.D., president of the J. Gladstone Institute to describe his organization’s innovative approach to corporate partnerships. “The way we’ve done it is to approach a corporate relationship not as one size fits all,” he said.

Dr. Williams noted several ways Gladstone is collaborating with industry partners. For example, the Institute may have developed an assay that could help a drug company decide whether to move a project forward—a decision that could save the company millions of dollars.

Another new model of collaboration Dr. Gollaher cited was the April 2013 agreement between pharmaceutical giant GlaxoSmithKline and San Diego-based venture capitalists, Avalon Ventures, to build a $495 million fund to create new drug-discovery companies. This novel approach will provide critical early-stage funding to quickly move promising research into the drug pipeline. The partnership is expected to create as many as ten new companies.

“We try not to do things internally that can be done better somewhere else,” said John Martin, Ph.D., chairman and CEO of Gilead Sciences. This principle was echoed by Nils Lonberg, Ph.D., senior vice president, Biologics Discovery California, Bristol-Myers Squibb. “It makes little sense for a corporation to build an internal capability if that application is available externally. If an academic institution can do something more efficiently than we can, we latch on to that.”

New Collaborations

In another break from the past, private foundations are increasingly funding companies that conduct biomedical research. Organizations such as the ALS Association, Bill & Melinda Gates Foundation, the Juvenile Diabetes Research Foundation and many others are investing in for-profit companies conducting research in their particular areas of interest.

“We’re having very significant collaborations with foundations,” said Dr. Gregory of Sanofi-Genzyme. “And money is coming in from these foundations, which is something you never would have seen ten years ago, to fund research and develop products.”

Another important refinement is to increase multi-party agreements. While in the past, collaborations were mostly one-to-one, Dr. Williams notes that there’s a growing trend towards three or more organizations pooling intellectual resources to maximize their ability to fight disease.

Sharing Key Technologies

For these efforts to succeed, research institutes and universities are working together to find better ways to facilitate technology transfer. Academic institutions are rapidly prioritizing moving research to companies that can translate it into treatments. Inefficient tech transfer sometimes kill projects, as lengthy negotiations may waste time and money.

“The efficiency we worry about more than anything, more than even the dollar efficiency, is the efficiency of time,” said Dr. Martin.

Technology transfer is an evolving issue. Universities and institutes want to work with industry to develop the best models to get important technologies to patients as quickly as possible.

“There are continuous efforts to make technology transfer easier,” said Michael Drake, M.D., chancellor at UC Irvine. “Everyone would like to say that the ideas got to the public so patients could be treated immediately. I’m very much on the side of making it easier for information to get out to people who are going to translate it into products.”

A different model of public support is embodied in the California Institute for Regenerative Medicine (CIRM). Approved in 2004 and funded by California’s taxpayers, CIRM has invested billions of dollars in stem cell research, leading to new advances and a number of clinical trials. Dr. Goldstein notes that California has the wherewithal to fund a great variety of research, not just stem cells.

“I’m going to argue that the State of California has to be a major funder in this space,” said Dr. Goldstein. “It’s in our best interest to seed funding our own long-term research interests.”

**Rewarding Efficiency**

Biomedical research promises extraordinary return on investment. From a policy perspective, improving health could spare Medicare, Medicaid and other programs from the bruising financial impact of Alzheimer’s disease, type 2 diabetes and other conditions.

“If we delay the onset of major diseases by ten years, we could eliminate our healthcare deficit,” said Rep. Scott Peters.

Consortia are a proven way to share expensive resources and disseminate new ideas. Grants that encourage their creation could eliminate duplicative technologies in high tech hubs throughout the country. This is not without precedent. During the past decade, the NIH has used grant mechanisms to increase translational research, improve collaboration among scientists at different institutions, and tackle rare diseases. In 2012, the National Cancer Institute provided a $20 million grant to create a UC San Diego-based international consortium to study chronic lymphocytic leukemia.

Policymakers can also help determine national healthcare strategies, specifically by identifying the nation’s most immediate research needs and maximizing support in those areas. Diseases such as Alzheimer’s, cancer and type 2 diabetes; rare conditions like Fahr’s Disease and Spinal Muscular Atrophy; and enabling technologies, such as stem cells, should receive focused attention, ameliorating or eliminating a number of public health crises.

“The classic political science theory is that government should step in when no one else can do so, and I think we have that situation in these critical disease areas,” said Dr. Williams. “The diseases are too complicated to attract the scale of industry investment that these societal problems deserve.”

By itself, scientific innovation is not enough, policy must also keep pace. The costs of failure could not be higher: untreatable diseases, skyrocketing healthcare costs, the United States losing its leadership position in biomedical research.

Elected officials, academic and industry leadership and scientists must step up to embrace workable solutions. Strategic investments now will reduce human suffering, save billions of dollars in healthcare costs and support a new economic boom.
Pipeline for Life Speakers

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CHI-California Healthcare Institute

CHI-California Healthcare Institute is a non-profit public policy research organization for California's biomedical R&D industry. CHI represents more than 275 leading medical device, biotechnology, diagnostics and pharmaceutical companies and public and private academic biomedical research organizations. CHI's mission is to advance responsible public policies that foster medical innovation and promote scientific discovery. CHI's website is www.chi.org. Follow us on Twitter @calhealthcare, Facebook, LinkedIn and YouTube.

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