Building A Stronger Foundation For A New Century Of Treatments And Cures

ABSTRACT In the past two years, the United States has made a historic investment in biomedical research. But innovative medicines often stall in the pipeline from microscope to market. To deliver the next generation of cures and treatments to help Americans live longer, healthier, and more comfortable lives, strong, strategic partnerships both within government and among government, academic, industry, and nonprofits are needed at every stage of drug development. In this article I describe actual and potential efforts on the part of the US government—including the Biomarkers Consortium and National Institutes of Health Therapeutics for Rare and Neglected Diseases program—to work with other stakeholders to advance biomedical research and development.

At the turn of the twentieth century, the life expectancy for an American was just over forty-seven years. By 2006, Americans were living an average of nearly seventy-eight years. Diseases that had once been a death sentence were eradicated or cured. Conditions that had once been disabling were now manageable.

Many of these gains were made possible by the great leaps in biomedical science that have been made in those years, from the discovery of insulin to the identification of effective therapies for HIV.

At the beginning of a new century, we can see even bigger opportunities ahead. New treatments, such as cell-based therapies, which introduce new cells into body tissues to treat diseases such as diabetes, show great promise. Unlocking the human genome could facilitate personalized treatments for diseases such as cancer, based on each person’s unique DNA.

Yet these gains are not guaranteed. Progress toward new treatments and cures for cancer, Alzheimer’s disease, and HIV/AIDS will be made only if there is a robust pipeline that carries promising ideas forward from the microscope to the medicine chest. And the only way to achieve that goal is with a new level of coordination among all of the partners in the research process.

That was the consensus at a town hall meeting I attended in Kansas City, Missouri, last summer that brought together representatives from government, academic, industry, and nonprofits for an open conversation about how to get a bigger bang for our research buck.

From Research To Final Product

The road from the research lab to an approved and marketable drug is a long one, and today there are many detours and obstacles along the way. Despite billions of dollars invested every year in research and development, just twenty-one new drugs made it to market in 2008.

The good news is that President Barack Obama believes deeply in the potential of biomedical research. One of the first laws he signed was the American Recovery and Reinvestment Act of 2009, which invested an additional $10.4 billion over two years in the National Institutes of Health (NIH), with the vast majority of those funds going directly to scientific research. This was the single largest boost to biomedical re-
search in American history and reflects President Obama’s belief—which he shared in September 2009 when he announced these grants—that biomedical research “holds promise like no other area of human endeavor.”

But to enhance the drug pipeline, more is needed than writing bigger checks. Resources must be invested so they will have the largest impact possible and will deliver greater numbers of cutting-edge drugs, therapies, and medical devices more quickly to the patients who need them the most.

**Stronger Public-Private Partnerships**

In the past, government has generally limited its role to investment in basic research, and private industry has taken over the drug development process from there. Today it can take many years and hundreds of millions of dollars to bring one new drug from discovery to the marketplace, and most new projects fail in the early stages. Time is money, and large pharmaceutical companies have become increasingly reluctant to invest in research until late in development, when a drug is almost ready for a clinical trial. But too many discoveries never reach that point, especially when they have no broad commercial market.

To eliminate the leaks and chokepoints in our medicine pipeline, researchers in Rockville, Maryland, or Palo Alto, California; at Harvard or the University of Kansas, must have the resources that enable them to assume some of the risks of development.

That is why government has begun to broaden its role from funding discovery to building innovative networks of all of the stakeholders in the development process. These networks include many partners, including foundations, such as the Bill & Melinda Gates Foundation, which are deploying funds to generate treatments and cures for diseases that disproportionately affect people in the world’s poorest countries; and patient advocacy groups, which can help collect patient blood, tissue, and other samples for basic research and connect their members with clinical trials.

One such network is the Biomarkers Consortium, a public-private partnership that includes scientists from the National Cancer Institute, the Food and Drug Administration (FDA), major pharmaceutical companies, and nearly twenty major cancer research centers across the country.

In March 2010 the consortium announced the launch of the I-SPY 2 clinical trial, which is designed, funded, and managed by consortium members. This groundbreaking model uses genetic or biological markers from a patient’s breast tumor to select the treatments that appear most likely to work in that particular person. The goal is to improve the odds of survival for women with high-risk, fast-growing breast cancers.

Another example of partnership is the NIH Therapeutics for Rare and Neglected Diseases program, which links small companies or academic labs doing basic research on diseases such as sickle-cell disease and hookworm, with government researchers who can use high-throughput screening and medicinal chemistry to move these discoveries closer to clinical trials. Cures for rare and neglected conditions probably won’t generate much profit, but they will offer hope and healing to many in desperate need.

And there are promising road maps for successful future collaboration such as the Cures Acceleration Network, which was enacted in the Affordable Care Act of 2010. It gives the NIH director the flexibility to fund a variety of innovative projects from the public, private, nonprofit, and academic sectors, with the goal of advancing research in areas where success is not a given but the payoff could be very high. With projects like these, some of the roadblocks that have held back the development of new medicines can be eliminated.

The US Department of Health and Human Services (HHS) is also exploring other innovative approaches to building stronger partnerships. For example, we are exploring ways to set up a strategic investment fund that will jump-start research on medical countermeasures. Medical countermeasures are the medications, vaccines, diagnostic tests, medical equipment, and supplies that are often our best defense in a public health emergency, such as a pandemic flu outbreak. But right now, private companies don’t always have much commercial incentive to create countermeasures for rare conditions such as the Ebola virus or exposure to nonmedical radiation—even though these countermeasures would be critical in the event of an Ebola outbreak or a nuclear explosion. HHS is trying to fix that.

And HHS also recognizes that stronger collaboration is not only a matter of bringing government and the private sector together. That’s why we’re also working to forge stronger partnerships inside government.

For example, NIH-supported researchers might share insights about emerging tools and technologies with FDA experts working to develop standards for safety and effectiveness. At the same time, the FDA could help inform the NIH’s translational efforts by identifying important regulatory issues that needed to be considered in the early stages of research planning.

The more communication there is between re-
The more communication there is between researchers and regulators, the stronger our research pipeline will be. That's why the NIH and FDA have formed a Joint Leadership Council, cochaired by the NIH director, Francis Collins, and the FDA commissioner, Margaret Hamburg. The council brings together senior leadership from both agencies to identify and follow through on opportunities for collaboration on a wide range of issues.

Recently, HHS took another step forward toward the goal of strengthening our drug pipeline when the NIH Scientific Management Review Board recommended that the NIH create a new center devoted to translational science. As currently envisioned, the main role of the new center would be to generate new approaches for building bridges to link basic discovery research with the development of new therapeutics and clinical care. The NIH is currently gathering input from the public and a broad range of stakeholders, and if all goes as planned, the new center will be established by October 1, 2011.

Conclusion
Recent advances in biomedical research have provided a huge opportunity to develop a new generation of cures and treatments that could dramatically improve the lives of Americans and people around the world. But government can’t do this alone. Public-private partnerships are needed to build new paradigms for drug development, not just when it’s time to manufacture and market a product, but from the beginning of the development process. It is important to keep looking closely for the points where good ideas stop moving forward, and then ask what steps are needed to keep the momentum going.

That means having a broader conversation among stakeholders in government, academia, industry, and nonprofits about how everyone can do better. The research taking place in laboratories and medical centers across America today holds limitless potential to improve people’s lives with new treatments and cures. But that potential can be reached only if all work together.

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