FDA Commissioner Margaret Hamburg would like to see the U.S. develop a comprehensive national strategy to encourage and support biomedical research and innovation and to transform discoveries into new therapies.

“We need to ensure we are adequately investing in research and making the right investments. We have to look at economic policies. We have to look at reimbursement policies and we have to look at regulatory policies and pathways,” she said Sept. 12 during the 2013 National Health Research Forum sponsored by Research!America.

“As a nation we really need to make sure that we have everything aligned properly to support the advance of innovation and the leveraging of the kinds of science and opportunities that exist today. That isn’t happening, and I think that can potentially really undermine both our ability to deliver on science and the ability of our nation to maintain its preeminence in this important arena,” she noted.

There are many pieces to such a strategy, which requires input from all stakeholders. The right players – industry, academia, patients, consumers, government and policy makers – need to come to the table “to really delve more deeply into this important conversation and really come up with some meaningful strategies,” Hamburg told reporters after the meeting.

“It’s only by coming together to look at the full range of issues that we will be able” to examine in an integrated way the barriers to biomedical product development and reduce them, she said.

No Formal Strategy Development Efforts Yet

There is as yet no formal effort to produce a biomedical innovation strategy. The White House is one possibility to take the lead. The President’s Council of Advisors on Science and Technology (PCAST) already has looked at some of the issues and made recommendations in 2012 for improving the drug approval process at FDA. It also called for additional funding for the National Institute of Health’s basic research programs (“Recipe For Doubling Innovative Medicines Includes New Approval Pathway, FDA Management Changes” — “The Pink Sheet,” Oct. 1, 2012).

One impediment to biomedical advances is the funding available in the current era of federal budget cuts. Hamburg acknowledged the difficulty and pointed out that if one part of the biomedical ecosystem “isn’t adequately resourced from a dollar and human point of view, you’re going to see problems throughout the system. We need to really recognize how important this is to the health of individuals, the health of the economy and the health of the health care system. So we all need to be pulling in the same direction together,” Hamburg said.

William Hait, global head of Janssen Pharmaceutical Cos., suggested a model for biomedical innovation could be the national war on cancer that was launched in 1971 when President Richard Nixon signed the National Cancer Act. The country now is “seeing enormous benefits from that year-in, year-out multi-billion dollar investment in cancer research, he noted. “That has led to fundamental understanding of cancer that we never had before, which allows us to make drugs that are no longer as toxic and as miserable as chemotherapy and that in some cases are even more effective. It takes that kind of push, that sustained push.”

Hamburg stressed that the U.S. should be at the forefront of biomedical innovation. “We’re watching other nations really take on this challenge and we need to be leading the pack,” she said.

The European Commission and European pharmaceutical industry plan to renew their Innovative Medicines Initiative with $4.5 billion over the next seven years to finance development of therapies that carry challenges unlikely to be overcome by governments or companies acting on their own (“Europe Renews Innovative Medicines Initiative, With Funding Boost” — “The Pink Sheet” DAILY, Jul. 10, 2013).

NIH Director: Next 'Cure for Cancer' Lost to Sequester
Marrecca Fiore
Sep 18, 2013

Billions of dollars in research funding and thousands of health-related jobs have been lost to the sequester, putting the nation's healthcare system at risk, said panelists speaking at a health forum last week.

National Institutes of Health Director Francis Collins, MD, said his institute has lost $1.7 billion in federal funding since the start of sequestration and stands to lose another $600 million on October 1.

"People are demoralized," said Dr. Collins, speaking at the 2013 National Health Research Forum held at the Newseum last week in Washington, DC. "That is research that could have been the next cure for cancer or the next Nobel Prize. But we'll never know."

Last Thursday's forum, titled "Straight Talk About the Future of Medical and Health Research," was hosted by Research!America and featured 3 panel discussions that included representatives from government, industry, research, and academia.

Although some of the discussion focused on new and innovative ways that government, academia, and the private sector could partner on research, much of the conversation was devoted to public sector cuts in research funding, many of which stem from the sequester.

"Are we ready to speak forcefully and candidly to our officials about the problems they've created through their inaction?" asked Research!America Chair John Edward Porter, JD, a former US congressman from Illinois, during his opening remarks.

"Are we ready to talk to them about the research that's not being conducted and the number of scientists leaving the profession, the missed opportunities, the lost jobs, the threat to our global competitiveness?"

Porter said Research!America's polling shows that the majority of Americans support research as a means to lower healthcare costs, and half are willing to pay more taxes if the money is used to support medical research.

He added that Congress can no longer "kick the can down the road" when it comes to medical research. "They either have to get the job done or they have to get out of the way."

Other speakers expressed similar concerns.

"Outbreaks Won't Be Detected..."

Centers for Disease Control and Prevention Director Thomas Frieden, MD, MPH, said tens of thousands of federal, state, and local healthcare jobs have been or will be cut because of ongoing budgetary restraints. He said the results could be devastating.
"Outbreaks won't be detected, vaccines won't happen," he said, adding that there "will be costs in terms of human suffering."

Bart Peterson, JD, senior vice president of corporate affairs and communications at drug maker Lilly, the conference’s lead sponsor, said the United States has become the global leader in health innovation during the past 30 years through "sound public policy," but that status is at risk.

"Public funding for research, which is so threatened today, is absolutely critical to the future, and we care about that in the private sector as much as anybody else," said Peterson, a former mayor of Indianapolis. "The stakes are very, very high. We need new medicines, and we need new technologies in medicine."

On the regulatory side of medicine, FDA Commissioner Margaret Hamburg, MD, said the sequester has reduced and strained resources to her agency as well.

"Investment in research is key," she said. "We have to look at economic policies, we have to look at reimbursement policies, and we have to look at regulatory policies and pathways. Advancing innovation and protecting the patients that use our healthcare system go hand and hand."

Dr. Hamburg added that she and others at the agency worry further cuts may make it difficult for the FDA to retain the "very best people" to oversee the drug and device review process.


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Hamburg Says Healthcare ‘Ecosystem' Reform Triggered FDA Streamlining
By Ferdous Al-Faruque
9/13/13

http://www.fdanews.com/newsletter/article?articleId=158647&issueId=17155

Speaking to a group of industry stakeholders Sept. 12, FDA Commissioner Margaret Hamburg said a recent call to streamline the agency should be seen as part of a national “comprehensive strategy” to overhaul the healthcare system.

“There really is an ecosystem here and we have to, as a nation, address the challenges before us as an integrated whole with a real comprehensive strategy,” Hamburg said. “We have to look at economic policies, we have to look at reimbursement policies and we have to look at regulatory policies and pathways.”

Hamburg said she has been in talks with the White House and other key stakeholders to develop such a strategy. “It’s much bigger than the FDA,” she added. “My goal as FDA commissioner is to help make
sure we are delivering on the promises of science and technology, and today I see this as part of my mission even though many of the pieces of this ecosystem ... are far outside our area of activity.”

Hamburg has asked center heads to develop ideas to streamline the agency and submit their ideas to her in the next three months (WDL, Sept. 9).

Speaking on a panel at Research America’s 2013 National Health Research forum in Washington, D.C., Hamburg said she hopes they will present ideas that will include ways to improve regulatory science. Ultimately, she hopes they will find ideas to streamline the way the agency reviews product applications and modernize their business practices so they are better able to communicate with industry about FDA requirements.

“We have actually supported a lot of work externally and in partnership with industry and academia and done a lot internally to really look at how can we develop really innovative clinical trial approaches, how can we decrease the time clinical research needs to be done,” added Hamburg.

Hamburg told WDL she is also concerned with the FDA’s ability to conduct inspections on the ground. She said historically, FDA investigators have had to cover a wide range of inspections, but “in the modern era” the experience and training needed to properly do inspections are very different. “We’re trying to really modernize, professionalize, better utilize staff and provide ultimately better service to our stakeholders in terms of the job we do,” added Hamburg. “So we’re looking at how we can better organize and train staff.”

Sequester Cuts May Have Driven NIH Grant Success Rates Down to 14% in 2013
By Jocelyn Kaiser
9/13/13

http://news.sciencemag.org/funding/2013/09/sequester-cuts-may-have-driven-nih-grant-success-rates-down-14-2013

Research!America
Call to action. NIH Director Francis Collins (fourth from left) urged the audience at a Research!America forum to push for more biomedical research funding.
The automatic spending cuts and other reductions to the National Institutes of Health’s (NIH’s) budget this year have caused slightly less damage than expected, NIH Director Francis Collins said yesterday. Preliminary data show that about 50 more grants were funded than projected, he said at a forum sponsored by Research!America. But success rates may have plunged even further than the agency predicted.

NIH’s budget shrunk by 5.5% this year, to $29.15 billion. As a result, NIH expects to fund about 650 fewer grants than it did the previous year. About 150, or nearly a quarter, of those grants were from investigators hoping to renew their award, Collins said. “We’re losing what we’ve already invested in,” he lamented during a panel discussion. In May, NIH had estimated that it would make 703 fewer new and competing awards for a total of 8283, but the new figure bumps that up to 8336.

The success rate—the number of proposals receiving funding divided by the number of proposals reviewed—could drop as low as 14% or 15%, Collins told ScienceInsider. That is lower than the 17% rate that NIH had anticipated. A larger than expected rise in applications this year could be the cause of the stiffer competition.

And “it ain’t over,” Collins told his audience. Congress has not yet approved any spending bills for the 2014 fiscal year, which begins on 1 October. Instead, legislators are likely to pass a temporary stop-gap funding measure known as a continuing resolution, which would freeze agency budgets at the 2013 level until further notice. If that happens, Collins said, “we will lose another $600 million” along with several hundred more grants. (A Senate panel approved a bill that would erase this year’s sequester cut and give the agency $30.95 billion in 2014, but the House of Representatives, which has not released its version of the bill, has proposed cuts to the overall federal spending that could translate into another $5 billion cut for NIH, according to an analysis by the Federation of American Societies for Experimental Biology.)

Collins’s message to NIH’s supporters: “I still don’t think we’ve activated our case sufficiently. … We should be making a lot of noise.”

FDA, Industry, See Progress on Innovation But Worry About U.S. Losing Its Leadership
By John Reichard
9/12/2013

http://www.cq.com/doc/hbnews-4342536

Food and Drug Administration Commissioner Margaret Hamburg, in an assessment shared by industry executives, told a Washington, D.C., forum Thursday that her agency has made strides in lowering
regulatory obstacles to product innovation but said that it is critical that the nation evaluate its innovation strategy.

“I really do think this is a critical time for our nation,” Hamburg told the forum sponsored by the medical research advocacy organization Research!America.

Despite what she called the “mythology” surrounding the amount and type of clinical data the FDA requires to approve a drug, Hamburg said that “we have made very solid progress” in terms of trying to really look at what are the agency’s requirements and what is necessary.

“We have actually supported a lot of work, externally in partnership with industry and academia, and done a lot internally, to really look at how we can develop more innovative clinical trial approaches, how can we decrease the time for clinical research to be done, the number of patients,” Hamburg said.

“Some of these are very hard scientific challenges,” she cautioned. “How do you assess adequately the long term safety when somebody is using drugs not for days but for decades? And that’s where we need to work together as a scientific community.”

Hamburg appeared on a panel that also included drug industry executives.

“I think there has been a lot of progress,” agreed Bart Peterson, a senior vice president for communications at Eli Lilly. “I can think back to when I joined the company just a little over four years ago. And sort of the constant refrain we heard throughout the industry was ‘Look at the data. The number of drug approvals each year from FDA is going down.’ ”

But “the number of approvals has significantly gone up each year in the last several years,” he said. “I think the spirit is positive.”

William Hait, global head at Jannsen Research and Development, the R&D arm of pharmaceutical division of Johnson and Johnson, praised a streamlined system at FDA for reviewing potential breakthrough uses of drugs.

For drugs that show extraordinary progress in a relatively small number of patients, “the tables have changed dramatically where now the FDA and pharmaceutical companies actually work in partnership to do everything possible to get the drugs to the patients as soon as conceivably possible,” Hait said. One of his company’s drugs will probably be approved “two years earlier than we had anticipated, which means a drug with significant benefit can get to patients earlier.”

But Hamburg and the industry executives also pointed to worrisome trends.

Peterson noted that industry-academia collaboration “is the heart and soul of our industry. And we wouldn’t exist without that. And it really is a symbiotic relationship.” Promising university research can’t get turned into new drugs without drug companies completing their development. But in many instances, companies depend on academia for new technologies and early drug development work, and universities rely on grants from the National Institutes of Health to fund that work. And the NIH budget is being cut. Peterson said that “because so much of the research money that the universities use comes from the NIH, that’s why the issue of NIH funding levels is so critical to the entire ecosystem.”
Hait said the medical research infrastructure nurtured by medical school faculties is weakening. He also said that he’s worried about “a very important cultural shift that should worry a lot of people, I hope. When I was training in medical school ... it was your highest calling to become a medical researcher, to emulate the faculty that was teaching you. I can remember as a resident sneaking off to the lab.”

Today, he said, if you go to “even top medical schools in the country you don’t consistently see that. The faculty is under such pressure to raise clinical revenue,” and NIH funding is so tight, “that the people that come to training don’t see this as a career” because of the struggles their mentors now face to do research.

“If you lose the kids that are so smart and so talented, who’s going to be the next generation that continues to spur on biomedical research and the prevention and cures we seek in this country?,” Hait said. “Pharmaceutical companies will look everywhere in the world” for young researchers if they can no longer find them in the U.S.

“Trust me, we’re not limited,” he said. “But we want to see as Americans the U.S. to continue to be the leaders, and what I’m seeing because of a variety of factors, we’re losing that generation, and that’s a real problem.”

Hamburg agreed. “My concern is that as a nation we really make sure that we have everything aligned properly to support the advancement of innovation and the leveraging of this kind of scientific opportunity” that exists today, she said. “And that isn’t happening. And I think that that can potentially really undermine both our ability to deliver on science and the ability of our nation to maintain its preeminence in this important arena.

“The science can be fabulous,” she noted. But “there are many things that limit the ability of that science to come forward. We can only review what’s in the pipeline,” she said.

“I really do think this is a critical time for our nation to really look at what is our national strategy to advance biomedical product innovation. Are we doing all the things that are necessary? Can we do more? And I think we’re watching other nations really take on this challenge and we need to be leading the pack.”

Hamburg was asked whether it would be the White House that would advance the strategy. “I’ve certainly been engaged in discussions on how we can do that,” she said, along with other government leaders. “It’s much bigger than the FDA. But it’s really, really important.”

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The Future of Medical and Health Research

On Sept. 12, Research!America’s 2013 National Health Research Forum took place at the Newseum in Washington, D.C. Read more about the event below and come back to watch video highlights.

By Bara Vaida
9/12/13


Panelists at the National Health Research Forum discuss funding for health care research. Panelists from left to right include: Moderator Norm Ornstein, American Enterprise Institute, Robert Hariri, Celgene Cellular Therapeutics, Debra Lappin, FaegreBD Consulting, Francis Collins, NIH, Tony Coles, Onyx Pharmaceuticals, and Victor Dzau, Duke University Health System.

(Photo Credit: Research! America)

Top government and business leaders came together Thursday in Washington, D.C. to raise the alarm about federal cuts in biomedical and health care research and to rally science and research advocates to call members of Congress.

Over a lunch of goat cheese salad and braised chicken, panelists organized by the advocacy group Research!America, told more than one hundred scientists, researchers, health care workers, professors, federal government staff and policy advocates, that the ongoing federal budget sequestration is likely to hurt future innovation and public health. When Congress failed to pass a budget earlier this year, all government agencies had to “sequester,” or cut their budgets.

“I don’t think we have made our case sufficiently,” said panelist Francis Collins, NIH director. The NIH lost $1.7 billion in federal funding because of the sequester. “People are demoralized. On October 1, we are going to lose another $600 million that will not fund 650 grants. That is research that could have been the next cure for cancer or the next Nobel Prize. But we’ll never know.”
Leaders from health care companies, medical teaching hospitals and other federal agencies, such as the Centers for Medicare and Medicaid Services, the CDC and FDA also painted a dire picture of the state of science and medicine. Several drug company executives said they count on federal investments in the NIH and other agencies because it provides seed money for breakthrough therapies in the future. Without that money, there will be fewer new treatments, said Tony Coles, president and CEO of Onyx Pharmaceuticals.

“Make noise people!” Collins urged members of the audience, who were sitting in a Newseum conference room with a view of Capitol Hill. “Come along. Help me out!”

Other panelists talking about the impact of federal cuts said:

- **FDA Commissioner Margaret Hamburg**: The sequester has reduced the flow of user fees to the agency and strained resources. “We are worried that we won’t be able to retain the very best people for the review process” of drugs and medical devices.

- **CDC Director Tom Friedan**: His agency will have to cut “several thousand” public health workers on top of the 46,000 state and local public health jobs that were cut for state budget reasons. That means “outbreaks won’t be detected, vaccinations won’t happen” and there “will be costs in terms of human suffering.”

- **William Hait**, global head of Janssen Research & Development, an arm of Janssen, a unit of Johnson & Johnson: Federal budget cuts have hampered the ability of medical school faculty to focus on research and have dissuaded medical students toward careers in science and research. “I am worried about this cultural shift. It used to be the highest calling to go into research. Now funding is so tough students don’t see it as a career. We are losing the next generation.”

- **Patrick Conway**, chief medical officer for CMS and director of CMS’s Center for Clinical Standards and Quality: He said that government investment has paid some returns in reforming the way health care services are delivered. The Affordable Care Act penalizes hospitals if too many Medicare patients get an infection while in the hospital. “We have aligned payment incentives and now infections are down.”